BIO BLAST PHARMA LTD.
Form 424B4
July 31, 2014

Rule 424(b)(4)
Registration No.: 333-193824

3,200,000 Shares

Ordinary Shares
$11.00 per share

This is the initial public offering of ordinary shares of Bio Blast Pharma Ltd. We are offering 3,200,000 shares of our ordinary shares in this offering. Prior to this offering, there has been no public market for our ordinary shares.

The initial public offering price of our ordinary shares is $11.00 per share.

Our ordinary shares have been approved for listing on The NASDAQ Global Market under the symbol ORPN .

We are an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012 (the JOBS Act) and will be subject to reduced public company reporting requirements.

Investing in our ordinary shares involves a high degree of risk. See Risk Factors beginning on page 10.

	Per Share	Total
Initial public offering price	$ 11.00	$ 35,200,000
Underwriting discounts and commissions	$ 0.77	$ 2,464,000
Proceeds to us (before expenses)	$ 10.23	$ 32,736,000

Certain of our existing investors and their affiliated entities, as well as certain of our directors, have indicated an interest in purchasing up to an aggregate of approximately $10.0 million worth of our ordinary shares in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may sell more, less or no shares in this offering to any of these persons, or any of these persons may determine to purchase more, less or no shares in this offering. The underwriters will receive the same underwriting discounts and commissions on any shares purchased by these persons as they will on any other shares sold to the public in this offering.

We have granted a 30-day option to the underwriters to purchase up to 480,000 additional ordinary shares solely to cover over-allotments, if any.

The underwriters expect to deliver the shares to purchasers in the offering on or about August 5, 2014 through the book-entry facilities of The Depository Trust Company.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

Oppenheimer & Co. **Roth Capital Partners**

BTIG

The date of this prospectus is July 30, 2014.

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You should rely only on the information contained in this prospectus and any free writing prospectus prepared by or on behalf of us or to which we have referred you. We have not authorized anyone to provide you with information that is different. We are offering to sell our ordinary shares, and seeking offers to buy our ordinary shares, only in jurisdictions where offers and sales are permitted. The information in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our ordinary shares.

Until and including August 24, 2014, 25 days after the date of this prospectus, all dealers that buy, sell or trade our ordinary shares, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealer s obligation to deliver a prospectus when acting as an underwriter and with respect to unsold allotments or subscriptions.

For investors outside of the United States: Neither we nor any of the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus.

In this prospectus, we, us, our, the Company and BioBlast refer to Bio Blast Pharma Ltd.

Our reporting currency and functional currency is the U.S. dollar.

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PROSPECTUS SUMMARY

The items in the following summary are described in more detail later in this prospectus. This summary provides an overview of selected information and does not contain all of the information you should consider before buying our ordinary shares. Therefore, you should read the entire prospectus carefully, especially the Risk Factors section beginning on page 10 and our financial statements and the related notes appearing at the end of this prospectus, before deciding to invest in our ordinary shares.

Overview

We are a development-stage biopharmaceutical company focused on the identification, licensing, acquisition, development and commercialization of drugs for rare and ultra-rare genetic diseases. We seek to identify therapeutic platforms that offer solutions for several diseases that share a common pathophysiological mechanism, which are the functional changes that accompany a particular syndrome or disease. We focus on rare diseases with severe and debilitating manifestations, where the unmet medical need is clear, the biological mechanism of action is understood and for which there is no satisfactory treatment. Since our inception in 2012, we have developed and in-licensed potential treatments for six diseases, one of which is in a Phase 2/3 clinical trial which, if the results are positive, we believe could be a pivotal trial, another one which is in Phase 2 clinical study and an additional two of which we expect will be in mid/late stage clinical studies by late 2014 or early 2015. We believe, based on discussions with the U.S. Food and Drug Administration, or FDA, that if the Phase 2/3 trial shows positive results, the data could be considered pivotal data, considering, among other things, the applicable FDA guidance for the industry called Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products. Our current drug candidate pipeline has been either in-licensed from academic institutions or developed internally. Our strategy is based on risk diversification through multiple therapeutic platforms, diversified clinical and pre-clinical stages of our programs, and diversified diseases addressed. We use strict selection criteria of our pipeline platforms and cost-efficient drug development. This allows us to pursue multiple programs in parallel with the goal of promptly delivering safe and effective therapies to patients in dire need.

Our current pipeline is based on three platforms:

Cabaletta is a mutant protein stabilizing platform based on a small repurposed molecule, Trehalose, which is currently used as an excipient for intravenous protein drugs. We are currently conducting a Phase 2/3 clinical trial, which, if the results are positive, we believe could be a pivotal trial, to assess its efficacy and safety in treating Oculopharyngeal Muscular Dystrophy, or OPMD, and a Phase 2 clinical trial to assess its efficacy and safety in treating Machado Joseph disease, or SCA3. We believe, based on discussions with the FDA, that if the Phase 2/3 trial shows positive results, the data could be considered pivotal data, considering, among other things, the applicable FDA guidance for the industry called Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products. Subject to regulatory allowance, we plan to conduct an additional Phase 2 study in 2015 aimed at Kennedy s disease, or SBMA. Mutant unstable cellular proteins are the cause of several genetic diseases known as PolyA/PolyQ, including OPMD, SBMA and SCA3. These proteins aggregate within cells, eventually leading to cell death. Our data to date from preclinical studies from both cells and animal models indicates that our Cabaletta platform has the potential to prevent mutant protein aggregation in humans.

mPRT is a mitochondrial protein replacement platform that is based on biological components that we synthesize in bacteria. Mitochondria are cell components that supply chemical energy for normal cell functioning. This platform is

currently in preclinical development for two diseases: Friedrich s Ataxia and Ornithine Transcarbamylase Deficiency. These diseases are among over 100 genetic diseases that are caused by a missing or mutant protein that has critical role in the normal mitochondrial function. While lysosomal protein replacement platforms have been successful in replacing a missing protein in lysosomes, we believe that our mPRT platform is the first one to be successful in replacing mitochondrial proteins. Our product candidates are new fusion proteins that are a combination of the replaced protein fused with two additional sequences that facilitate its transport through biological membranes.

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BBrm is our read-through platform based on small repurposed molecules. The platform enables the read-through (or bypassing) of genetic defects called nonsense mutations or stop codons that interfere with normal protein formation and cause disease. Based on our preclinical data, we believe that this drug platform has the potential to treat six different diseases caused by nonsense mutation. Subject to regulatory approval, we plan to conduct Phase 2 clinical studies in late 2014 for our lead indication in Spinal Muscular Atrophy (SMA).

The following table summarizes our product candidate pipeline:

Product Candidates

The diseases which we are addressing have devastating consequences on the patient s health, quality of life and life expectancy. In addition, these diseases create significant burdens on the patient s family and care takers as well as on the public health resources. In all the diseases we are addressing, patients cannot be offered an alternative therapy or the current solutions are inadequate in their abilities to change the course of the disease. We believe that prompt and efficient drug development can be of substantial benefit to the patients who are suffering from these incurable diseases.

Cabaletta for the treatment of OPMD

Cabaletta is our proprietary intravenous (IV) solution of Trehalose for the treatment of OPMD. OPMD is an inherited myopathy, which is a muscle disease caused by a primary defect in muscle cells, characterized by dysphagia (difficulty in swallowing) and the loss of muscular strength and weakness in multiple parts of the body. As the dysphagia becomes more severe, patients become malnourished, lose significant weight, become dehydrated and suffer from repeated incidents of aspiration pneumonia. These last two are often the cause of death.

Trehalose is naturally-occurring and is well known for its protein-stabilizing properties. It is used in several biological systems, such as freeze drying of red blood cells and the preservation of organs for donation and as a protein stabilizer in IV pharmaceutical products. Trehalose is approved by the FDA as a GRAS (Generally Recognized As Safe) food ingredient and is listed in the U.S. National Formulary, which is a compendium of public pharmacopeial standards, which are directions for the identification of compound medicines, as well as in Europe and Japan. It is a disaccharide chemical chaperone, which is a chemical molecule comprised of two sugar components that stabilizes the folding of proteins and buffers abnormal protein aggregation, that protects against pathological processes in cells. It has been shown to prevent pathological aggregation of proteins within cells in several diseases associated with abnormal cellular-protein aggregation as well as acting as an autophagy enhancer. Autophagy is the basic catabolic mechanism that involves cell degradation of unnecessary or dysfunctional cellular components. Autophagy, if regulated, ensures the synthesis, degradation and recycling of cellular components. Trehalose has been documented as demonstrating significant efficacy in preclinical animal models of OPMD and other PolyA/PolyQ diseases.

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Trehalose is not effective when given orally since it is almost completely metabolized in the small intestine into glucose and often causes diarrhea and flatulence after ingestion of more than 50g. We have developed a proprietary IV solution of Trehalose, which we call Cabaletta™, to circumvent the breakdown of Trehalose in the gastrointestinal tract, and to enable therapeutic doses of Trehalose to reach the muscles.

We are currently conducting a Phase 2/3 multicenter clinical trial of Cabaletta to treat OPMD; if the results are positive, we believe this could be a pivotal trial. We believe, based on discussions with the FDA, that if the Phase 2/3 trial shows positive results, the data could be considered pivotal data, considering, among other things, the applicable FDA guidance for the industry called Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products. The trial is being conducted in Israel, and subject to regulatory allowances, in Canada and the USA. If our current OPMD trial is successful, we will submit a new drug application, or NDA, to the FDA.

Cabaletta for the treatment of spinocerebellar ataxia type 3 (SCA3 Machado Joseph disease)

SCA3, also known as Machado Joseph disease, is the most common disease among the cerebellar ataxias, which are a group of genetic diseases that are characterized by memory deficits, spasticity, difficulty with speech and swallowing, weakness in arms and other muscular disorders. Symptoms can begin in early adolescence and get worse over time. Eventually, SCA3 leads to paralysis, and severe cases can lead to an early death in the fourth decade of life. SCA3 is incurable, and there is currently no approved treatment for the disease.

SCA3 is caused by a mutation in the DNA that leads to the creation of a pathological protein Ataxin 3. Ataxin 3 is unstable, meaning that it aggregates within the cells and eventually leads to cell death.

Multiple reported studies in cell models have shown that Trehalose, both as an anti-mutant protein aggregator and as an autophagy enhancer, is able to reduce protein aggregates and improve cell survival in several spiocerebellar ataxias, including SCA3 cells. Additional animal studies show that activation of autophagy may be beneficial in alleviating disease symptoms. We recently started a Phase 2 clinical trial of Cabaletta to treat SCA3 in Israel.

Cabaletta for the treatment of Spino Bulbar Cerebellar Ataxia (SBMA Kennedy s disease)

SBMA, also known as Kennedy s disease, is characterized by the degeneration and loss of lower motor neurons in the brainstem and spinal cord. Patients present with weakness in muscle function, including severe difficulty in swallowing and aspiration, and other disorders. SBMA is caused by an abnormal androgen receptor protein. Like other PolyA/PolyQ diseases, the abnormal protein is unstable and aggregates within cells, eventually leading to cell death. Patients suffering from SBMA suffer from progressive neuromuscular deterioration that can end up in extreme disability and repeated aspiration pneumonia. There is currently no approved therapy for SBMA.

Studies in cell models have shown that Trehalose in its capacity both as an anti-mutant protein aggregation and as an autophagy enhancer is able to reduce protein aggregates and improve cell survival in SBMA cells. Additional studies show that activation of autophagy may be beneficial in alleviating disease symptoms in animal models.

We have entered into an agreement with NINDS (a group within the U.S. National Institutes of Health, or NIH) pursuant to which NINDS will conduct animal studies on SBMA mouse models to validate the potential efficacy of Cabaletta. NINDS will conduct the study using its own resources, and we will supply the research material. If the animal studies are successful, and subject to regulatory allowance, we plan to commence a Phase 2/3 clinical trial.

Mitochondrial protein replacement platform (mPRT)

Mitochondrial disorders are diseases, for which no cure has been found, that stem from a missing or mutated critical enzyme or protein in the cell. Our fusion proteins, comprised of TAT-MTS (het)-replacement proteins, help move proteins across biological membranes to facilitate the creation of mature proteins inside the mitochondria. Our replacement proteins are able to enter human cells and mitochondria and replace the damaged or missing proteins in the mitochondria. Our breakthrough platform technology demonstrated efficacy in several mitochondrial protein deficiencies in cells and animal models. Currently, Frataxin for Friedrich s Ataxia (BB-FA) is our most advanced mPRT preclinical program, followed by Ornithine Transcarbamylase (BB-OTC) for Ornithine Transcarbamylase Deficiency (OTCD).

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Our preclinical studies demonstrated the superiority of our proprietary approach of using a heterologous (non-native) mitochondrial targeting sequence (MTS) as part of our fusion proteins in four different aspects: expression by bacteria, cell penetration, mitochondrial processing and anchorage of target proteins, and rescue of cells in oxidative stress.

We have an exclusive worldwide and royalty-bearing license to commercialize, develop and use the technology, including certain patent applications, from the Hebrew University in Jerusalem. These patent applications cover our unique approach of using homologous and heterologous MTS in the fusion proteins.

BB-FA for the treatment of Friedrich s Ataxia

Friedreich s Ataxia is an inherited disease characterized by progressive deterioration of the muscular and nervous systems, resulting in gait disturbance (Ataxia), cognitive impairment, progressive heart disease and diabetes. Patients are usually diagnosed in the first or second decade of life, and are typically wheelchair-bound within 15 years of diagnosis. Most patients do not survive beyond the fourth decade of life. In many cases the cause of death is myopathic heart disease. The underlying causes of Friedrich s Ataxia are reduced levels of Frataxin a protein responsible for iron-sulphor clusters in the mitochondria that are critical for the mitochondria activity. Our preclinical data demonstrated successful placement of Frataxin into the mitochondria and in the treatment of oxidative stress in Friedreich s Ataxia patients cells. We are advancing the Friedreich s Ataxia program through its preclinical development throughout 2014.

BB-OTC for the treatment of ornithine transcarbamylase deficiency (OTCD)

OTCD is the most common disorder among urea cycle disorders a group of rare genetic diseases characterized by body s inability to detoxify ammonia. Ammonia is a toxic breakdown product of proteins. OTCD is caused by a mutated and ineffective form of the enzyme, ornithine transcarbamylase. As a result, ammonia accumulates in the blood causing hyperammonemia. Newborn males affected with OTCD may suffer devastating hepatic coma in the first few days after birth, and survivors typically suffer from severe cognitive, mental and metabolic disorders and growth retardation. Many do not survive the first decade of life. Ornithine transcarbamylase is part of the urea cycle complex in the mitochondria. We are using our mitochondrial protein replacement platform to replace this enzyme in the mitochondria. Our preclinical data indicates that our OTC fusion protein is well able to be transferred into the mitochondria and processed in it. We are advancing the BB-OTC program through its preclinical development through 2014.

BBrm our read-through platform

BBrm is our family of small molecule non-glycosides repurposed drugs for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation (stop codon). The platform enables the read-through (deactivation) of the genetic defects that stop synthesis of proteins called stop codons. Deactivation of disease-causing nonsense mutation can alleviate the symptoms of genetic diseases caused by these nonsense mutations. Our BBrm family of repurposed drugs is able to induce translational readthrough, restoring full-length functional proteins in diseases where the nonsense mutation results in truncated ineffective proteins. We licensed the exclusive worldwide commercial rights for our BBrm technology for stop codon inhibition of orphan diseases from the Tel-Aviv University in January 2014.

BBrm1 for the treatment of Spinal Muscular Atrophy (SMA)

Using our BBrm platform, we developed a unique therapeutic candidate for SMA. SMA is the leading genetic cause of infantile death and is caused by the loss of a functional Survival Motor Neuron 1 (SMN1). The disease is manifested by loss of muscle mass and mobility as well as severe compromise of vital functions such as respiration. Another protein called SMN2 is a nearly identical copy of SMN1, differentiated only by a silent, single-nucleotide mutation within the DNA. SMN2 partially compensates for the dysfunction of SMN1, however, the small amount of the functional protein that is produced from the SMN2 gene is not able to fully compensate for the loss of SMN1. Prior independent studies proposed that read-through agents, such as aminoglycosides, can induce the read-through of the stop codon located in the SMN2-protein, thus elongating the SMN2 and creating a full length functional protein that can compensate for the non-functioning SMN1 and alleviate the disease. This approach was also successfully tested in SMA animal models. Nonetheless, chronic administration of aminoglycosides was found to be associated with prohibitive toxicity.

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Nevertheless, we believe drug-induced read-through of premature stop codons remains a promising approach to elevate active protein expression from the SMN2 gene which is an ideal therapeutic target as it is found in all SMA patients. Our family of repurposed FDA-approved non-glycosides molecules (BBrm) induce significantly higher levels of full length functional SMN2 protein. These molecules are ineffective if administered orally or intravenously since they do not penetrate into the brain to create high enough therapeutic levels within nerve cells. Our BBrm1 product can be injected directly into the central nervous system (CNS), creating adequate drug concentration. This method of administration has the added benefit of further protection against off label use. We plan to continue our preclinical development of BBrm1 for SMA through 2014, and, if successful and subject to regulatory approval, expect to start a Phase 2 study late in 2014 or early in 2015.

Our Strategy

Our strategy is to identify, acquire, license, develop, and commercialize novel products for the treatment of rare and ultra-rare diseases in the United States, the European Union, and select international markets, with the goal of becoming a leading rare disease biotechnology company.

The critical components of our business strategy include the following:

Focus on rare and ultra-rare diseases with significant unmet medical need;
Focus on diseases and therapies with clear mechanisms of action;
Leverage our experience and relationships to in-license promising product candidates;
Develop and commercialize multiple product candidates in parallel;
Focus on excellent and rapid clinical and regulatory execution; and
Seek to retain global commercialization rights to product candidates.

Recent Developments

On February 6, 2014, we closed a private placement pursuant to which we sold to accredited investors an aggregate of 782,537 of our ordinary shares at a price of $6.07 per share for a total consideration of $4,368,000 net of expenses.

We recently initiated a Phase 2/3 clinical trial for OPMD; if the results are positive, we believe this could be a pivotal trial. We also recently initiated a Phase 2 clinical trial for SCA3.

Risks Associated with Our Business

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section entitled Risk Factors immediately following this prospectus summary. These are not the only risks we face. These risks include, among others:

We are a development-stage biopharmaceutical company and have a limited operating history on which to assess our business, have incurred significant losses since our inception, and anticipate that we will continue to incur significant losses for the foreseeable future;
Even if this offering is successful, we expect that we will need to raise additional funding before we can expect to become profitable from sales of our products;

We are heavily dependent upon the success of our product candidates, which are in the early stages of clinical development, and we cannot provide any assurance that any of our product candidates will receive regulatory approval;

Because the target patient populations of our product candidates are small, we must be able to successfully identify patients and achieve a significant market share to maintain profitability and growth;

We have not completed preclinical development of several product candidates and do not have any products approved for sale by the FDA or any other regulatory bodies;

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The insurance coverage and reimbursement status of newly-approved orphan products is uncertain and failure to obtain or maintain adequate coverage and reimbursement for our product candidates could limit our ability to market those products and decrease our ability to generate revenue;

If we are unable to obtain and maintain effective intellectual property rights for our technologies, product candidates, or any future product candidates, we may not be able to compete effectively in our markets; and

Our future success depends in part upon our ability to retain our executive team, particularly Dr. Dalia Megiddo and Mr. Udi Gilboa, and to attract, retain, and motivate other qualified personnel.

Corporate Information

We are an Israeli corporation based in Tel Aviv and were incorporated on January 22, 2012. Our principal executive offices are located at 37 Dereh Menachem Begin St., Tel Aviv 6522042, Israel, and our telephone number is: +972 722409060.

Implications of being an Emerging Growth Company

We are an emerging growth company, as defined in Section 2(a) of the Securities Act of 1933, or the Securities Act, as modified by the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. As such, we are eligible to take advantage of certain exemptions from various reporting requirements applicable to other public companies that are not emerging growth companies including, but not limited to:

only two years of audited financial statements in addition to any required unaudited interim financial statements with correspondingly reduced Management s Discussion and Analysis of Financial Condition and Results of Operations disclosure; and

not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002 and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

We intend to take advantage of these and other exemptions available to emerging growth companies. We could remain an emerging growth company for up to five years, or until the earliest of (a) the last day of the first fiscal year in which our annual gross revenue exceeds $1 billion, (b) the date that we become a large accelerated filer as defined in Rule 12b-2 under the Securities Exchange Act of 1934, or Exchange Act, which would occur if the market value of our ordinary shares that is held by non-affiliates exceeds $700 million as of the last business day of our most recently completed second fiscal quarter, or (c) the date on which we have issued more than $1 billion in nonconvertible debt during the preceding three-year period.

The JOBS Act permits an emerging growth company like us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. This means that an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We are electing to delay such adoption of new or revised accounting standards.

Implications of being a Foreign Private Issuer

Our status as a foreign private issuer also exempts us from compliance with certain laws and regulations of the Securities and Exchange Commission (the SEC) and certain regulations of The NASDAQ Stock Market, including the proxy rules, the short-swing profits recapture rules, and certain governance requirements such as independent director

oversight of the nomination of directors and executive compensation. In addition, we will not be required to file annual, quarterly and current reports and financial statements with the SEC as frequently or as promptly as United States companies registered under the Exchange Act.

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THE OFFERING

Ordinary shares offered by us

3,200,000 ordinary shares

Ordinary shares to be outstanding after this offering

14,230,480 ordinary shares

Over-allotment option

The underwriters have an option for a period of 30 days to purchase up to 480,000 additional ordinary shares to cover over-allotments, if any.

Use of proceeds

We expect to receive approximately $31.9 million in net proceeds from the sale of 3,200,000 ordinary shares offered by us in this offering (approximately $36.8 million if the underwriters exercise their over-allotment option in full), after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We currently expect to use the net proceeds from this offering for:

completing our clinical program for OPMD, estimated at $6,000,000;

completing our Phase 2 and pivotal clinical study for SCA3, estimated at $3,500,000;

Initiating and completing our Phase 2/3 clinical study for SBMA, estimated at $4,200,000;

completing our pre-clinical program for BB-FA and Phase 1 clinical study, estimated at $4,500,000;

completing our pre-clinical program for BB-OTC and initiation and completion of Phase 1 and 2A-2B clinical studies, estimated at $7,800,000;

Initiating and completing our Phase 1-2 clinical programs for SMA, estimated at $2,200,000;

Other Indications, estimated at $500,000;

Premarketing activity for OPMD, estimated at $1,500,000; and

the remainder for personnel-related costs, preclinical research, working capital, and other general corporate purposes.

Risk factors

You should read the Risk Factors section starting on page 10 of this prospectus for a discussion of factors to consider carefully before deciding to invest in ordinary shares.

NASDAQ Global Market Symbol ORPN

The number of our ordinary shares to be outstanding immediately after this offering is based on 11,030,480 ordinary shares outstanding as of the date of this prospectus. This number excludes:

456,630 shares issuable upon the exercise of share options outstanding as of July 30, 2014;

269 shares reserved as of July 30, 2014 for future grants under our equity incentive plan; and

206,702 shares issuable upon the exercise of options granted to the Executive Chairman of our Board of Directors subject to the completion of this offering.

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Unless otherwise indicated, all information in this prospectus assumes or gives effect to:

the filing of our amended and restated articles of association, which will occur immediately prior to the completion of this offering; and

no exercise of the underwriters over-allotment option.

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SUMMARY FINANCIAL DATA

The following table summarizes our financial data. We have derived the following statements of operations data for the years ended December 31, 2013 and 2012 and the balance sheet data as of December 31, 2013 from our audited financial statements included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that may be expected in the future. The following summary financial data should be read in conjunction with Management s Discussion and Analysis of Financial Condition and Results of Operations and our financial statements and related notes included elsewhere in this prospectus.

	Years Ended December 31,	
(in thousands of U.S. dollars, except share and per share amounts)	2013	2012
Statements of Operations Data:		
Research and development expenses	$732	$140
General and administrative expenses	416	86
Financial expense (income), net	(3)	3
Deemed dividend	26	
Loss attributable to holders of ordinary shares	1,171	229
Weighted average number of ordinary shares used in computing basic and diluted net loss per share	8,423,018	7,551,427

	As of December 31, 2013		
(in thousands of U.S. dollars, except share and per share amounts)	Actual	Pro forma (unaudited)[1]	As Adjusted (unaudited)[2]
Balance Sheet Data:			
Total long-term assets	$7	$ 7	$ 7
Total current liabilities	131	131	131
Shareholders equity	175	5,555	37,406

(1) After giving effect to the sale of 1,065,076 ordinary shares on January 1, 2014 at the price of $0.95 per share and to the sale of 782,537 ordinary shares on February 6, 2014 at the price of $6.07 per share, as if the sale of the shares in each case had occurred on December 31, 2013.

(2) The unaudited as adjusted column in the balance sheet data above gives effect to the sale of 3,200,000 ordinary shares in this offering at the initial public offering price of $11.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, as if the sale had occurred on December 31, 2013.

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RISK FACTORS

An investment in our ordinary shares involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this prospectus, including our financial statements and related notes thereto, before deciding to invest in our ordinary shares. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. In these circumstances, the market price of our ordinary shares could decline, and you may lose all or part of your investment.

Risks Related to Our Financial Condition and Capital Requirements

We are a development-stage company and have a limited operating history on which to assess our business, have incurred significant losses since our inception, and anticipate that we will continue to incur significant losses for the foreseeable future.

We are a development-stage biopharmaceutical company with a limited operating history. We have incurred net losses since our inception in January 2012, including net losses of $1.1 million for the year ended December 31, 2013. As of December 31, 2013, we had an accumulated deficit of $1.4 million.

We have devoted substantially all of our financial resources to identify, acquire, license, and develop our product candidates, including conducting preclinical and clinical studies and providing general and administrative support for these operations. To date, we have financed our operations primarily through the sale of equity securities. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations, or grants. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. With respect to most of our product candidates, we are in the early stages of clinical development. We have commenced a Phase 2/3 clinical trial for only one of our product candidates, and it may be several years, if ever, before we have a product candidate approved for commercialization. Even if we obtain regulatory approval to market a product candidate, our future revenue will depend upon the size of any markets in which our product candidates may receive approval, and our ability to achieve sufficient market acceptance, pricing, reimbursement from third-party payors, and adequate market share for our product candidates in those markets.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

continue our research and preclinical and clinical development of our product candidates;
expand the scope of our current clinical studies for our product candidates;
advance our programs into more expensive clinical studies;
initiate additional preclinical, clinical, or other studies for our product candidates;
change or add additional manufacturers or suppliers;
seek regulatory and marketing approvals for our product candidates that successfully complete clinical studies;
establish a sales, marketing, and distribution infrastructure to commercialize any products for which we may obtain marketing approval;

seek to identify, assess, acquire, license, and/or develop other product candidates;
make milestone or other payments under any license agreements;
seek to maintain, protect, and expand our intellectual property portfolio;
seek to attract and retain skilled personnel;

We are a development-stage company and have a limited operating history onwhich to assess our business, have i

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create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and

experience any delays or encounter issues with any of the above, including but not limited to failed studies, complex results, safety issues, or other regulatory challenges that require longer follow-up of existing studies, additional major studies, or additional supportive studies in order to pursue marketing approval.

Further, the net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

We have never generated any revenue from product sales and may never be profitable.

We have no products approved for commercialization and have never generated any revenue. Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize one or more of our product candidates. We do not anticipate generating revenue from product sales for the foreseeable future. Our ability to generate future revenue from product sales depends heavily on our success in many areas, including but not limited to:

completing research and preclinical and clinical development of our product candidates;

obtaining regulatory and marketing approvals for product candidates for which we complete clinical studies;

developing a sustainable and scalable manufacturing process for any approved product candidates and establishing and maintaining supply and manufacturing relationships with third parties that can conduct the process and provide adequate (in amount and quality) products to support clinical development and the market demand for our product candidates, if approved;

launching and commercializing product candidates for which we obtain regulatory and marketing approval, either directly or with a collaborator or distributor;

obtaining market acceptance of our product candidates as viable treatment options;

addressing any competing technological and market developments;

identifying, assessing, acquiring and/or developing new product candidates;

negotiating favorable terms in any collaboration, licensing, or other arrangements into which we may enter;

maintaining, protecting, and expanding our portfolio of intellectual property rights, including patents, trade secrets, and know-how; and

attracting, hiring, and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the European Medicines Agency, or the EMA, or other regulatory agencies, domestic or foreign, to change our manufacturing processes or assays, or to perform clinical, nonclinical, or other types of studies in addition to those that we currently anticipate. In cases where we are successful in obtaining regulatory approvals to market one or more of our product candidates, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to get reimbursement at any price, and whether we own the commercial rights for that territory. If the number of our addressable rare disease patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect, or the reasonably expected population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved. Additionally, if we are not able to generate revenue from the sale of any approved products, we may never become profitable.

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Even if this offering is successful, we expect that we will need to raise substantial additional funding before we can expect to become profitable from sales of our products. This additional financing may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit, or terminate our product development efforts or other operations.

We are currently advancing our Cabaletta platform product candidates through clinical development and our other product candidates, BB-FA, BB-OTC, and BBrm1, as well as our other early stage research projects, through preclinical development. Developing our product candidates is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates through clinical studies.

As of December 31, 2013, our cash and cash equivalents were $0.3 million. This amount does not reflect $5.3 million that we received in connection with sales of our ordinary shares in 2014. Upon the completion of this offering, based upon our currently expected level of operating expenditures, we expect that our existing cash, cash equivalents and marketable securities will be sufficient to fund our current operations for at least the next 12 months; however, we expect that we will require substantial additional capital to obtain regulatory approval for, and to commercialize, our product candidates. In addition, our operating plans may change as a result of many factors that may currently be unknown to us, and we may need to seek additional funds sooner than planned. Our future funding requirements will depend on many factors, including but not limited to:

the scope, rate of progress, results and cost of our clinical studies, preclinical testing, and other related activities;
the cost of manufacturing clinical supplies, and establishing commercial supplies, of our product candidates and any products that we may develop;
the number and characteristics of product candidates that we pursue;
the cost, timing, and outcomes of regulatory approvals;
the cost and timing of establishing sales, marketing, and distribution capabilities; and
the terms and timing of any collaborative, licensing, and other arrangements that we may establish, including any required milestone and royalty payments thereunder.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The incurrence of indebtedness could result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell, or license intellectual property rights, and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results, and prospects. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay, or discontinue one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition, and results of operations.

Even if this offering is successful, we expect that we will need to raise substantial additional funding before we can

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Raising additional capital would cause dilution to our existing shareholders, and may restrict our operations or require us to relinquish rights.

We may seek additional capital through a combination of private and public equity offerings, debt financings and collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a shareholder. Debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions such as incurring debt, making capital expenditures or declaring dividends. If we raise additional funds through collaboration, strategic alliance and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates, or grant licenses on terms that are not favorable to us.

Our recurring operating losses have raised substantial doubt regarding our ability to continue as a going concern.

As of December 31, 2013, we had an accumulated deficit of $1.4 million. Our recurring operating losses raise substantial doubt about our ability to continue as a going concern. Our financial statements include a note describing the conditions which raise this substantial doubt. As a result, our independent registered public accounting firm included an explanatory paragraph in its report on our financial statements as of and for the year ended December 31, 2013 with respect to this uncertainty. We have no current source of revenue to sustain our present activities, and we do not expect to generate revenue until, and unless, the FDA or other regulatory authorities approve our product candidates and we successfully commercialize our product candidates. Accordingly, our ability to continue as a going concern will require us to obtain additional financing to fund our operations. The perception of our ability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees.

Risks Related to the Discovery and Development of Our Product Candidates.

We are heavily dependent on the success of our product candidates, which are in the early stages of preclinical or clinical development. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.

To date, we have invested substantially all of our efforts and financial resources to identify, acquire, license, and develop our product candidates, including conducting preclinical and clinical studies and providing general and administrative support for these operations. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize one or more product candidates. We currently generate no revenue from sales of any drugs, and we may never be able to develop or commercialize a marketable drug.

Each of our product candidates is in the early stages of development and will require additional clinical development (and in some cases additional preclinical development), management of nonclinical, clinical, and manufacturing

activities, regulatory approval, obtaining adequate manufacturing supply, building of a commercial organization, and significant marketing efforts before we generate any revenue from product sales. We currently have one product candidate in Phase 2 clinical studies and another one of our product candidates has advanced into a Phase 2/3 trial which, if the results are positive, we believe could be a pivotal trial. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates.

We as a company have never submitted marketing applications to the FDA or comparable foreign regulatory authorities. We cannot be certain that any of our product candidates will be successful in clinical studies or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical studies. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

We are heavily dependent on the success of our product candidates, whichare in the early stages of preclinical or c

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We generally plan to seek regulatory approval to commercialize our product candidates in the United States, the European Union, and in additional foreign countries where we have commercial rights. To obtain regulatory approval in other countries, we must comply with numerous and varying regulatory requirements of such other countries regarding safety, efficacy, chemistry, manufacturing and controls, clinical studies, commercial sales, pricing, and distribution of our product candidates. Even if we are successful in obtaining approval in one jurisdiction, we cannot ensure that we will obtain approval in any other jurisdictions. If we are unable to obtain approval for our product candidates in multiple jurisdictions, our revenue and results of operations could be negatively affected.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, and inherently unpredictable. If we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable, typically takes many years following the commencement of clinical studies, and depends upon numerous factors. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate s clinical development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. We have not obtained regulatory approval for any product candidate, and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Applications for our product candidates could fail to receive regulatory approval for many reasons, including but not limited to the following:

the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical studies;
we may be unable to demonstrate to the FDA or comparable foreign regulatory authorities that a product candidate s safety-benefit ratio for its proposed indication is acceptable;
the population studied in the clinical program may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical studies;
the data collected from clinical studies of our product candidates may not be sufficient to support the submission of a new drug application, or NDA, or biologics license application, or BLA, or other submission or to obtain regulatory approval in the United States or elsewhere;
the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process, as well as the unpredictability of the results of clinical studies, may result in our failing to obtain regulatory approval to market any of our product candidates, which would significantly harm our business, results of operations, and prospects.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies may not be predictive of future study results.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical study process. The results of preclinical studies and early clinical studies of our product candidates may not be predictive of the results of later-stage clinical studies. Product candidates that have shown promising results in early-stage clinical studies may still suffer significant setbacks in subsequent registration clinical studies. For example, the safety or efficacy results generated to date in preclinical and clinical studies for Cabaletta and BBrm1 or BB-FA, and BB-OTC do not

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ensure that later clinical studies will demonstrate similar results. There is a high failure rate for drugs and biologics proceeding through clinical studies, and product candidates in later stages of clinical studies may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical studies. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical studies due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier studies. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. We do not know whether any Phase 2, Phase 3, or other clinical studies we may conduct will demonstrate consistent or adequate efficacy and safety sufficient to obtain regulatory approval to market our drug candidates.

We may find it difficult to enroll patients in our clinical studies given the limited number of patients who have the diseases for which our product candidates are being studied. Difficulty in enrolling patients could delay or prevent clinical studies of our product candidates.

Identifying and qualifying patients to participate in clinical studies of our product candidates is critical to our success. The timing of our clinical studies depends in part on the speed at which we can recruit patients to participate in testing our product candidates, and, although we have not yet experienced difficulty enrolling patients in clinical studies in the past, we may experience delays in our clinical studies if we encounter difficulties in enrollment in the future.

Each of the conditions for which we plan to evaluate our current product candidates is a rare genetic disease. Accordingly, there are limited patient pools from which to draw for clinical studies. For our current product candidates:

- we estimate that several thousand patients in the United States suffer from OPMD for which Cabaletta is being studied;
- we estimate that a few thousand patients in the United States suffer from SCA3 for which Cabaletta is being studied;
- we estimate a few thousand patients in the United States suffer from SBMA for which Cabaletta is being studied;
- we estimate that several thousand patients in the United States suffer from Friedrich s Ataxia for which BB-FA is being studied;
- we estimate that a few thousand patients in the United States suffer from OTC deficiency for which BB-OTC is being studied;
- we estimate that several thousand patients in the United States suffer from SMA deficiency for which BBrm1 is being studied; and
- we estimate that the prevalence of the diseases we aim at is similar and sometimes higher than the total number of patients in the United States.

In addition to the rarity of these diseases, the eligibility criteria of our clinical studies will further limit the pool of available study participants as we will require that patients have specific characteristics that we can measure or to assure their disease is either severe enough or not too advanced to include them in a study. Additionally, the process of finding and diagnosing patients may prove costly. We also may not be able to identify, recruit, and enroll a sufficient number of patients to complete our clinical studies because of the perceived risks and benefits of the product candidate under study, the availability and efficacy of competing therapies and clinical studies, the proximity and availability of clinical study sites for prospective patients, and the patient referral practices of physicians. If patients are unwilling to participate in our studies for any reason, the timeline for recruiting patients, conducting studies, and obtaining regulatory approval of potential products may be delayed.

If we experience delays in the completion of, or termination of, any clinical study of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenue from any

of these product candidates could be delayed or prevented. In addition, any delays in completing our clinical studies will increase our costs, slow down our product candidate development and

We may find it difficult to enroll patients in our clinical studies given the limited number of patients who have the dis

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approval process, and jeopardize our ability to commence product sales and generate revenue. Any of these occurrences may harm our business, financial condition, and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical studies may also ultimately lead to the denial of regulatory approval of our product candidates.

We may encounter substantial delays in our clinical studies, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical studies to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time consuming, and uncertain as to outcome. We cannot guarantee that any clinical studies will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical studies can occur at any stage of testing, and our future clinical studies may not be successful. Events that may prevent successful or timely completion of clinical development include but are not limited to:

inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of human clinical studies;

delays in reaching a consensus with regulatory agencies on study design;

delays in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical study sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical study sites;

delays in obtaining required Institutional Review Board, or IRB, approval at each clinical study site;

imposition of a clinical hold by regulatory agencies, after review of an investigational new drug, or IND, application, or equivalent application, or an inspection of our clinical study operations or study sites;

delays in recruiting suitable patients to participate in our clinical studies;

difficulty collaborating with patient groups and investigators;

failure by our CROs, other third parties, or us to adhere to clinical study requirements;

failure to perform in accordance with the FDA s good clinical practices requirements, or applicable regulatory guidelines in other countries;

delays in having patients complete participation in a study or return for post-treatment follow-up;

patients dropping out of a study;

occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;

changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;

the cost of clinical studies of our drug candidates being greater than we anticipate;

clinical studies of our drug candidates producing negative or inconclusive results, which may result in us deciding, or regulators requiring us, to conduct additional clinical studies or abandon drug development programs; and

delays in manufacturing, testing, releasing, validating, or importing/exporting sufficient stable quantities of our product candidates for use in clinical studies or the inability to do any of the foregoing.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenue. In addition, we may need to conduct additional studies to bridge our repurposed product candidates to generic products in the market. We may also be required to conduct additional safety, efficacy and comparability studies before we will be allowed to start clinical studies with our repurposed drugs. Clinical study delays could also shorten any periods during which our products

We may encounter substantial delays in our clinical studies, or we may fail to demonstrate safety and efficacy to the

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have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to obtain orphan exclusivity and to successfully commercialize our product candidates and may harm our business and results of operations.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay, or halt clinical studies and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. Patients enrolled in our study of Cabaletta for OPMD, known as our HOPEMD study, may suffer side effects associated with the use of Cabaletta. Other enzyme replacement therapies have been associated with infusion-associated reactions due to a developing allergy to the product, which can cause rashes, pain, significant clinical disease, or even death. Our BB-FA and BB-OTC product candidates may also cause these or similar side effects. Results of our studies could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, our studies could be suspended or terminated, and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny or withdraw approval of our product candidates for any or all targeted indications.

The drug-related side effects could affect patient recruitment, the ability of enrolled patients to complete the study, or result in potential product liability claims. We do not currently hold product liability insurance and do not anticipate obtaining product liability insurance until such time as we have received FDA or other comparable foreign authority approval for a product and there is a product that is being provided to third parties outside of clinical trials.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including but not limited to:

regulatory authorities may withdraw approvals of such product;
regulatory authorities may require additional warnings on the label;
we may be required to create a Risk Evaluation and Mitigation Strategy, or REMS, plan, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
we could be sued and held liable for harm caused to patients; and
our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations, and prospects.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.

If our product candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy, and other post-market information, including both federal and state requirements in the United States. In addition, manufacturers and manufacturers facilities are required to comply with extensive FDA

requirements, including ensuring that quality control and manufacturing procedures conform to current Good Manufacturing Practices, or cGMP, regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA or BLA. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, and quality control.

Any regulatory approvals that we receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or

Even if we obtain regulatory approval for a product candidate, our productswill remain subject to regulatory scrutiny

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contain requirements for potentially costly post-marketing testing, including Phase IV clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. We will also be required to report certain adverse reactions and production problems, if any, to the FDA, and to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product s approved label. As such, we may not promote our products for indications or uses for which they do not have FDA approval. The holder of an approved NDA or BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical studies to verify the safety and efficacy of our products in general or in specific patient subsets. If original marketing approval were obtained via the accelerated approval pathway, we could be required to conduct a successful post-marketing clinical study to confirm clinical benefit for our products. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval. Furthermore, any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance. Foreign regulatory authorities impose similar requirements.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, such regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

issue warning letters;
impose civil or criminal penalties;
suspend or withdraw regulatory approval;
suspend any of our ongoing clinical studies;
refuse to approve pending applications or supplements to approved applications submitted by us;
impose restrictions on our operations, including closing our contract manufacturers facilities; or
seize or detain products, or require a product recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenue from our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our operating results will be adversely affected.

Risks Related to our Reliance on Third Parties

We rely on third parties to conduct our preclinical and clinical studies and perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon third-party CROs to monitor and manage data for our ongoing preclinical and clinical programs. We rely on these parties for execution of our preclinical and clinical studies, and control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is

conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs and other vendors are required to comply with cGMP, current good clinical practices, or cGCP, and Good Laboratory Practices, or GLP, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, or EEA, and comparable foreign regulatory authorities for all of our product candidates in clinical development. Regulatory authorities

We rely on third parties to conduct our preclinical and clinical studies andperform other tasks for us. If these third pa

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enforce these regulations through periodic inspections of study sponsors, principal investigators, study sites, and other contractors. If we or any of our CROs or vendors fail to comply with applicable regulations, the clinical data generated in our clinical studies may be deemed unreliable and the FDA, EMA, or comparable foreign regulatory authorities may require us to perform additional clinical studies before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical studies comply with cGCP regulations. In addition, our clinical studies must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical studies, which would delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. In addition, our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our on-going clinical, nonclinical, and preclinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements, or for other reasons, our clinical studies may be extended, delayed, or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. CROs may also generate higher costs than anticipated. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition, and prospects.

We rely completely on third parties to manufacture our preclinical and clinical drug supplies. Our business could be harmed if those third parties fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices.

We do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture our preclinical and clinical drug supplies for use in the conduct of our clinical studies, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We rely on our manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical studies. There are a limited number of suppliers for raw materials that we use to manufacture our drugs, and there may be a need to identify alternate suppliers to prevent a possible disruption of the manufacture of the materials necessary to produce our product candidates for our clinical studies, and, if approved, ultimately for commercial sale. We do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. Although we generally do not begin a clinical study unless we believe we have a sufficient supply of a product candidate to complete such study, any significant delay or discontinuity in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical study due to the need to replace a third-party manufacturer could considerably delay completion of our clinical studies, product testing, and potential regulatory approval of our product candidates, which could harm our business and results of operations.

We are subject to a multitude of manufacturing risks, any of which could substantially increase our costs and limit supply of our product candidates.

The process of manufacturing our product candidates is complex, highly regulated, and subject to several risks, including but not limited to:

the process of manufacturing biologics, such as BB-FA and BB-OTC is extremely susceptible to product loss due to contamination, equipment failure or improper installation or operation of equipment, or vendor or operator error. Even minor deviations from normal manufacturing processes for any of our product candidates could result in reduced production yields, product defects, and

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other supply disruptions. If microbial, viral, or other contaminations are discovered in our product candidates or in the manufacturing facilities in which our product candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination; and
the manufacturing facilities in which our product candidates are made could be adversely affected by equipment failures, labor shortages, natural disasters, power failures, and numerous other factors.
Any adverse developments affecting manufacturing operations for our product candidates may result in shipment delays, inventory shortages, lot failures, withdrawals or recalls, or other interruptions in the supply of our product candidates. We may also have to take inventory write-offs and incur other charges and expenses for product candidates that fail to meet specifications, undertake costly remediation efforts, or seek more costly manufacturing alternatives.

The drug substance and drug product for our product candidates are currently acquired from single-source suppliers. The loss of these suppliers, or their failure to supply us with the drug substance or drug product, could materially and adversely affect our business.

In several cases, we rely on a single provider or manufacturer for our product candidates. For example, the raw materials used to manufacture our Cabaletta are acquired from a single third party drug products supplier. Additionally, our Cabaletta, is manufactured by a single third party manufacturer. It is possible that the Company will be required to switch providers in the unforeseen future. In such case, the process of switching suppliers and/or manufacturers may be costly and/or time consuming for us, and that may include the temporary or permanent suspension of a clinical study or commercial sales of our candidate products.

We do not currently have any other suppliers for the drug substance or drug product of our product candidates and, although we believe that there are alternate sources of supply that could satisfy our clinical and commercial requirements, we cannot assure you that identifying alternate sources and establishing relationships with such sources would not result in significant delay in the development of our product candidates. Additionally, we may not be able to enter into supply arrangements with alternative suppliers on commercially reasonable terms, or at all. A delay in the development of our product candidates or having to enter into a new agreement with a different third party on less favorable terms than we have with our current suppliers could have a material adverse impact upon on our business.

We and our collaborators and contract manufacturers are subject to significant regulation with respect to manufacturing our product candidates. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.

All entities involved in the preparation of therapeutics for clinical studies or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical studies must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We, our collaborators, or our contract manufacturers must supply all necessary documentation in support of an NDA, BLA, or Marketing Authorization Application, or MAA, on a timely basis and must adhere to GLP and cGMP regulations enforced by the FDA and other regulatory agencies through their facilities inspection program. Some of

our contract manufacturers have never produced a commercially approved pharmaceutical product and therefore have not obtained the requisite regulatory authority approvals to do so. The facilities and quality systems of some or all of our collaborators and third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements. If these facilities do not pass a

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pre-approval plant inspection, regulatory approval of the products may not be granted or may be substantially delayed until any violations are corrected to the satisfaction of the regulatory authority, if ever.

The regulatory authorities also may, at any time following approval of a product for sale, audit the manufacturing facilities of our collaborators and third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time consuming for us or a third party to implement, and that may include the temporary or permanent suspension of a clinical study or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we, our collaborators, or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA or other applicable regulatory authority can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, withdrawal of an approval, or suspension of production. As a result, our business, financial condition, and results of operations may be materially harmed.

Additionally, if supply from one approved manufacturer is interrupted, an alternative manufacturer would need to be qualified through an NDA or BLA supplement or MAA amendment, or equivalent foreign regulatory filing, which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause us to incur higher costs and could cause the delay or termination of clinical studies, regulatory submissions, required approvals, or commercialization of our product candidates. Furthermore, if our suppliers fail to meet contractual requirements and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical studies may be delayed or we could lose potential revenue.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to develop and manufacture our product candidates, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements, or other similar agreements with our collaborators, advisors, employees, and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor s discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

Risks Related to Commercialization of Our Product Candidates

If the market opportunities for our product candidates are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Because the target patient populations of our product candidates are small, we must be able to successfully identify patients and achieve a significant market share to maintain profitability and growth.

We focus our research and product development on treatments for rare and ultra-rare genetic diseases. Given the small number of patients who have the diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare and ultra-rare genetic diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including

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the scientific literature, surveys of clinics, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business.

We intend to rely on third-party manufacturers to produce our product candidates, but we have not entered into binding agreements with any such manufacturers to support commercialization. Additionally, these manufacturers do not have experience producing our product candidates at commercial levels and may not achieve the necessary regulatory approvals or produce our product candidates at the quality, quantities, locations, and timing needed to support commercialization.

We have not yet secured manufacturing capabilities for commercial quantities of our product candidates. Although we intend to rely on third-party manufacturers for commercialization, we have only entered into agreements with such manufacturers to support our clinical studies. We may be unable to negotiate binding agreements with the manufacturers to support our commercialization activities at commercially reasonable terms.

Manufacturers may not have the experience or ability to produce our product candidates at commercial levels. We may run into technical or scientific issues related to manufacturing or development that we may be unable to resolve in a timely manner or with available funds. We also have not completed all of the characterization and validation activities necessary for commercialization and regulatory approvals. If our manufacturing partners do not conduct all such necessary activities, our commercialization efforts will be harmed.

Even if we timely develop a manufacturing process and successfully transfer it to the third-party product manufacturers, if such third-party manufacturers are unable to produce the necessary quantities of our product candidates, or in compliance with cGMP or other pertinent regulatory requirements, and within our planned timeframe and cost parameters, the development and sales of our products, if approved, may be materially harmed.

We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are similar, more advanced, or more effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize our product candidates.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. We are currently aware of various existing therapies in the market and in development that may in the future compete with our product candidates.

Gene therapy, cell therapy, bone marrow transplantation and other approaches may also emerge for the treatment of any of the disease areas in which we focus.

We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies. Some of the pharmaceutical and biotechnology companies we expect to compete with include Shire, Sanofi, BioMarin, Alexion, and Roche, other smaller companies or biotechnology startups, as well as other large multinational pharmaceutical companies. Many of our competitors have substantially greater financial, technical, and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. As a result, these companies may obtain regulatory approval more rapidly than we are able to and may be more effective in selling and marketing their products as well. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring, or licensing on an

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exclusive basis, products that are more effective or less costly than any product candidate that we may develop, or achieve earlier patent protection, regulatory approval, product commercialization, and market penetration than we do. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

We currently have no marketing and sales organization. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

Although our employees may have sold other similar products in the past while employed at other companies, we as a company have no experience selling and marketing our product candidates and we currently have no marketing or sales organization. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. If our product candidates receive regulatory approval, we intend to establish a sales and marketing organization with technical expertise and supporting distribution capabilities to commercialize our product candidates in major markets, which will be expensive, difficult, and time consuming. Any failure or delay in the development of our internal sales, marketing, and distribution capabilities would adversely impact the commercialization of our products.

Further, given our lack of prior experience in marketing and selling biopharmaceutical products, our initial estimate of the size of the required sales force may be materially more or less than the size of the sales force actually required to effectively commercialize our product candidates. As such, we may be required to hire substantially more sales representatives to adequately support the commercialization of our product candidates or we may incur excess costs as a result of hiring more sales representatives than necessary. With respect to certain geographical markets, we may enter into collaborations with other entities to utilize their local marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If our future collaborators do not commit sufficient resources to commercialize our future products, if any, and we are unable to develop the necessary marketing capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We may be competing with companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

The commercial success of any current or future product candidate will depend upon the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.

Even with the requisite approvals from the FDA and comparable foreign regulatory authorities, the commercial success of our product candidates will depend in part on the medical community, patients, and third-party payors accepting our product candidates as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payors, and others in the medical community. The degree of market acceptance of any of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

the efficacy of the product as demonstrated in clinical studies and potential advantages over competing treatments;
the prevalence and severity of any side effects, including any limitations or warnings contained in a product s approved labeling;

the clinical indications for which approval is granted;
relative convenience and ease of administration;
the cost of treatment, particularly in relation to competing treatments;
the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;

The commercial success of any current or future product candidate willdepend upon the degree of market acceptan

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the strength of marketing and distribution support and timing of market introduction of competitive products;
publicity concerning our products or competing products and treatments; and
sufficient third-party insurance coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical and clinical studies, market acceptance of the product will not be fully known until after it is launched. Our efforts to educate the medical community and third-party payors on the benefits of the product candidates may require significant resources and may never be successful. If our product candidates are approved but fail to achieve an adequate level of acceptance by physicians, patients, third-party payors, and others in the medical community, we will not be able to generate sufficient revenue to become or remain profitable.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue.

Our target patient populations are small, and accordingly the pricing, coverage, and reimbursement of our product candidates, if approved, must be adequate to support our commercial infrastructure. Our per-patient prices must be sufficient to recover our development and manufacturing costs and potentially achieve profitability. Accordingly, the availability and adequacy of coverage and reimbursement by governmental and private payors are essential for most patients to be able to afford expensive treatments such as ours, assuming approval. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid for by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or reimbursed by government authorities, private health insurers, and other third-party payors. If coverage and reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about coverage and reimbursement for new drugs are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new drug will be covered and reimbursed under Medicare. Private payors tend to follow the coverage reimbursement policies established by CMS to a substantial degree. It is difficult to predict what CMS will decide with respect to reimbursement for products such as ours.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicinal products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or ma

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general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Health Care Reform Law, was passed, which substantially changes the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Health Care Reform Law, among other things, subjects biologic products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted, or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, and promotes a new Medicare Part D coverage gap discount program.

In addition, other legislative changes have been proposed and adopted in the United States since the Health Care Reform Law was enacted. On August 2, 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by the U.S. Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation s automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which, among other things, delayed for another two months the budget cuts mandated by these sequestration provisions of the Budget Control Act of 2011. On March 1, 2013, the President signed an executive order implementing sequestration, and on April 1, 2013, the 2% Medicare payment reductions went into effect. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain effective patent rights for our product candidates or any future product candidates, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection, and confidentiality agreements to protect the intellectual property related to our technologies and product candidates. Our success depends in large part on our and our licensors ability to obtain and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technology and products.

We have sought to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business. This process is expensive and time consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable

cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain and involves complex legal and factual questions for which legal principles remain unsolved. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue, and even if such patents cover our product candidates, third parties may challenge their validity, enforceability, or scope, which may result in such patents being narrowed, found unenforceable or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual

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property, provide exclusivity for our product candidates, or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

We, independently or together with our licensors, have filed several patent applications covering various aspects of our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

Although we have a number of patent applications directed to methods of use and certain compositions of matter, we do not have complete patent protection for our product candidates. For example, we do not have a patent covering the composition of matter of Trehalose, and our patent applications cover only the route of administration (IV) and use in several orphan diseases associated with trinucleotide repeat expansion (PolyA/PolyQ) diseases. With regards to our mitochondrial protein replacement therapy we do not have patent protection for the concept of attaching TAT to a protein. With our read-through technology, we do not have composition of matter protection. Instead, we have filed a use patent application of a generic family of molecules in a non-systemic route of administration for genetic neuro degenerative and neuro developmental diseases and new route of administration through direct injection to the central nervous system. In addition, our patent applications have not resulted in issued patents.

If we cannot obtain and maintain effective patent rights for our product candidates, we may not be able to compete effectively and our business and results of operations would be harmed.

We may not have sufficient patent terms to effectively protect our products and business.

Patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Although various extensions may be available, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from generic medications.

While patent term extensions under the Hatch-Waxman Act in the United States and under supplementary protection certificates in Europe may be available to extend the patent exclusivity term for Cabaletta, BB-FA and BB-OTC as well as the BBrm family of molecules we cannot provide any assurances that any such patent term extension will be obtained and, if so, for how long. In addition, upon issuance in the United States any patent term can be adjusted based on certain delays caused by the applicant(s) or the United States Patent and Trademark office, or USPTO. For example, a patent term can be reduced based on certain delays caused by the patent applicant during patent prosecution. If we do not have sufficient patent terms or regulatory exclusivity to protect our products, our business and results of operations will be adversely affected.

Patent policy and rule changes could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. We therefore cannot be certain that we or our licensors were the first to make the invention claimed in our owned and licensed patents or pending applications, or that we or our licensor were the first to file for patent protection of such inventions. Assuming the other requirements for patentability are met, in the United States prior to March 15, 2013, the first to make the claimed invention is entitled to the patent, while outside the United States, the first to file a patent application is entitled to the patent. After March 15, 2013, under the Leahy-Smith America Invents Act, or the Leahy-Smith Act, enacted on September 16, 2011, the United States has moved to a first to file system. The Leahy-Smith Act also includes a number of significant changes that affect the way patent applications will be prosecuted and may also affect patent litigation. The

Patent policy and rule changes could increase the uncertainties and costssurrounding the prosecution of our patent

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effects of these changes are currently unclear as USPTO, must still implement various regulations, the courts have yet to address any of these provisions and the applicability of the act and new regulations on specific patents discussed herein have not been determined and would need to be reviewed. In general, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

If we are unable to maintain effective proprietary rights for our product candidates or any future product candidates, we may not be able to compete effectively in our markets.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors, and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There have been many lawsuits and other proceedings involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, and reexamination proceedings before the USPTO and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. There may be

third-party patents or patent applications with claims to materials, formulations, methods of manufacture, or methods for treatment related to the use or manufacture of our product candidates. We have conducted freedom to operate analyses with respect to only certain of our product candidates, and therefore we do not know whether there are any third-party patents that would impair our ability to commercialize these product candidates. We also cannot guarantee that any of our analyses are complete and thorough, nor can we be sure that we have identified each and every patent and pending application in the United States and abroad that is relevant or necessary to the commercialization of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents

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may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire or are finally determined to be invalid or unenforceable.

Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture, or methods of use, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires or is finally determined to be invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

We may not be successful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license, or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and the rights to these formulations may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources, and greater clinical development and commercialization capabilities.

For example, we sometimes collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution s rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, we may have to abandon development of that program and our business and financial condition could suffer.

We may face competition from biosimilars, which may have a material adverse impact on the future commercial prospects of our mitochondrial protein replacment platform.

Even if we are successful in achieving regulatory approval to commercialize a product candidate faster than our competitors, we may face competition from biosimilars with respect to BB-FA and BB-OTC and future product candidates based on our protein replacement platform. In the United States, the Biologics Price Competition and Innovation Act of 2009 created an abbreviated approval pathway for biological products that are demonstrated to be highly similar, or biosimilar, to or interchangeable with an FDA-approved biological product. This new pathway could allow competitors to reference data from innovative biological products 12 years after the time of approval of the innovative biological product. This data exclusivity does not prevent another company from developing a product that is highly similar to the innovative product, generating its own data, and seeking approval. Data exclusivity only assures that another company cannot rely upon the data within the innovator s application to support the biosimilar product s approval. In his proposed

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budget for fiscal year 2014, President Obama proposed to cut this 12-year period of exclusivity down to seven years. He also proposed to prohibit additional periods of exclusivity due to minor changes in product formulations, a practice often referred to as evergreening. It is possible that the U.S. Congress may take these or other measures to reduce or eliminate periods of exclusivity. The Biologics Price Competition and Innovation Act of 2009 is complex and only beginning to be interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when any such processes may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for BB-FA and BB-OTC.

In Europe, the European Commission has granted marketing authorizations for several biosimilars pursuant to a set of general and product class-specific guidelines for biosimilar approvals issued over the past few years. In Europe, a competitor may reference data supporting approval of an innovative biological product, but will not be able to get on the market until 10 years after the time of approval of the innovative product. This 10-year marketing exclusivity period will be extended to 11 years if, during the first eight of those 10 years, the marketing authorization holder obtains an approval for one or more new therapeutic indications that bring significant clinical benefits compared with existing therapies. In addition, companies may be developing biosimilars in other countries that could compete with our products.

If competitors are able to obtain marketing approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences.

The patent protection and patent prosecution for some of our product candidates is dependent on third parties.

While we normally seek and gain the right to fully prosecute the patents relating to our product candidates, there may be times when patents relating to our product candidates are controlled by our licensors.

In addition, even where we now have the right to control patent prosecution of patents and patent applications we have licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensors and their counsel that took place prior to us assuming control over patent prosecution.

If we fail to comply with our obligations in the agreements under which we license intellectual property and other rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, we may be required to make certain payments to the licensor, we may lose the exclusivity of our license, or the licensor may have the right to terminate the license, in which event we would not be able to develop or market products covered by the license. Additionally, the milestone and other payments associated with these licenses will make it less profitable for us to develop our drug candidates. See Business License Agreements for a description of our license agreements with the Hebrew University in Jerusalem and the Tel-Aviv University.

In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business, and scientific issues. Disputes may arise regarding intellectual property subject to a licensing agreement, including but not limited to:

the scope of rights granted under the license agreement and other interpretation-related issues;
the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
the sublicensing of patent and other rights;

If we fail to comply with our obligations in the agreements under which welicense intellectual property and other righ

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our diligence obligations under the license agreement and what activities satisfy those diligence obligations;

the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our collaborators; and

the priority of invention of patented technology.

If disputes over intellectual property and other rights that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. If we or one of our licensing partners were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable.

Interference proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our ordinary shares.

We may be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants, and independent contractors do not use the proprietary information or know-how of others in their work

for us, we may be subject to claims that we or our employees, consultants, or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee s former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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We may be subject to claims challenging the inventorship of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. For example, we may have inventorship disputes arise from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Therefore, we may receive less revenue from future products if such claims are successful which in turn could impact our future profitability.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involves both technological and legal complexity. Therefore, obtaining and enforcing biotechnology patents is costly, time consuming, and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Among these countries is China where we intend to protect our intellectual property rights to the extent possible.

Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may also export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of

competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

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Risks Related to Our Business Operations

Our future success depends in part on our ability to retain our Chief Executive Officer and our Chief Financial Officer and to attract, retain, and motivate other qualified personnel.

We are highly dependent on Dalia Megiddo, M.D., our Founder and Chief Executive Officer, and Udi Gilboa, our Chief Financial Officer and Senior Vice President of Operations, the loss of their services without a proper replacement may adversely impact the achievement of our objectives. Dr. Megiddo and Mr. Gilboa may leave our employment at any time (subject, in each case, to a 60 days prior notice, under the terms of their consultancy agreements), as each one is an at will independent contractor. Recruiting and retaining other qualified employees, consultants, and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled personnel in our industry, which is likely to continue. As a result, competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical studies may make it more challenging to recruit and retain qualified personnel. The inability to recruit and retain qualified personnel, or the loss of the services of Dr. Megiddo or Mr. Gilboa without proper replacement, may impede the progress of our research, development, and commercialization objectives.

If we fail to obtain or maintain orphan drug exclusivity for our products, our competitors may sell products to treat the same conditions and our revenue will be reduced.

Our business strategy focuses on the development of drugs that are eligible for FDA and European Union orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the EMA s Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union Community. Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity. In the European Union, orphan drug designation also entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity is granted following drug or biological product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Because the extent and scope of patent protection for our products may in some cases be limited, orphan drug designation is especially important for our products for which orphan drug designation may be available. For eligible drugs, we plan to rely on the exclusivity period under the Orphan Drug Act to maintain a competitive position. If we do not obtain orphan drug exclusivity for our drug products and biologic products that do not have broad patent protection, our competitors may then sell the same drug to treat the same condition sooner than if we had obtained orphan drug exclusivity and our revenue will be reduced.

Even though we have obtained orphan drug designation for OPMD in the United States, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for a product,

If we fail to obtain or maintain orphan drug exclusivity for our products, ourcompetitors may sell products to treat the

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that exclusivity may not effectively protect the product from competition because different drugs with different active moieties can be approved for the same condition. Even with orphan drug exclusivity, if a third party were to prepare or market a Trehalose IV preparation which infringes upon our intellectual property, we may need to initiate litigation, which may be costly, to enforce our rights against such party. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.

As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, sales, marketing, financial, legal, and other resources. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees, and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

We may not be successful in our efforts to identify, license, or discover additional product candidates.

Although a substantial amount of our effort will focus on the continued clinical testing, potential approval, and commercialization of our existing product candidates, the success of our business also depends upon our ability to identify, license, or discover additional product candidates. Our research programs or licensing efforts may fail to yield additional product candidates for clinical development for a number of reasons, including but not limited to the following:

our research or business development methodology or search criteria and process may be unsuccessful in identifying potential product candidates;
we may not be able or willing to assemble sufficient resources to acquire or discover additional product candidates;
our product candidates may not succeed in preclinical or clinical testing;
our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval;
competitors may develop alternatives that render our product candidates obsolete or less attractive;
product candidates we develop may be covered by third parties patents or other exclusive rights;
the market for a product candidate may change during our program so that such a product may become unreasonable to continue to develop;
a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
a product candidate may not be accepted as safe and effective by patients, the medical community, or third-party payors.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, or we may not be able to identify, license, or discover additional product candidates, which would have a material adverse effect on our business and could potentially cause us to cease operations. Research

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programs to identify new product candidates require substantial technical, financial, and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful.

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting, and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, as well as rules subsequently implemented by the SEC and The NASDAQ Stock Market, have imposed various requirements on public companies. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as say on pay and pay parity. Recent legislation permits smaller emerging growth companies to implement many of these requirements over a longer period and up to five years from the pricing of this offering. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment, and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time consuming and costly. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain our current levels of such coverage.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. In particular, we will be required to perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report, commencing in our annual report on Form 20-F for the year ending December 31, 2014, on the effectiveness of our internal controls over financial reporting, if then required by Section 404 of the Sarbanes-Oxley Act. Our testing may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses. Our compliance with Section 404 will require that we incur substantial accounting expense and expend significant management efforts. We currently do not have an internal audit group, and we will need to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge. Moreover, if we are not able to comply with the requirements of Section 404 in a timely manner or if we identify or our independent registered public accounting firm identifies deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by NASDAQ, the SEC, or other regulatory authorities, which would require additional financial and management resources.

New laws and regulations as well as changes to existing laws and regulations affecting public companies, including the provisions of the Sarbanes-Oxley Act and rules adopted by the SEC and by NASDAQ, would likely result in increased costs to us as we respond to their requirements.

We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be directly or indirectly through our customers, subject to various federal and state fraud and abuse laws, including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act, and physician sunshine laws and regulations. These laws may impact, among other things, our proposed sales, marketing, and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

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the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;
the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
HIPAA, as amended by the Health Information Technology and Clinical Health Act, or HITECH, and its implementing regulations, which imposes certain requirements relating to the privacy, security, and transmission of individually identifiable health information;
the federal physician sunshine requirements under the Health Care Reform Laws requires manufacturers of drugs, devices, biologics, and medical supplies to report annually to the U.S. Department of Health and Human Services information related to payments and other transfers of value to physicians, other healthcare providers, and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and
state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws that may apply to items or services reimbursed by any third-party payor, including commercial insurers, state laws that require pharmaceutical companies to comply with the pharmaceutical industry s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has strengthened these laws. For example, the Health Care Reform Law, among other things, amends the intent requirement of the federal anti-kickback and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. Moreover, the Health Care Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

International expansion of our business exposes us to business, regulatory, political, operational, financial, and economic risks associated with doing business outside of the United States or Israel.

Other than our headquarters and other operations which are located in Israel (as further described below), we currently have limited international operations, but our business strategy incorporates potentially significant international expansion, particularly in anticipation of approval of our product candidates. We plan to maintain sales representatives and conduct physician and patient association outreach activities, as well as clinical trials, outside of the United States and Israel. Doing business internationally involves a number of risks, including but not limited to:

International expansion of our business exposes us to business, regulatory, political, operational, financial, and eco

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multiple, conflicting, and changing laws and regulations such as privacy regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;

failure by us to obtain regulatory approvals for the use of our products in various countries;

additional potentially relevant third-party patent rights;

complexities and difficulties in obtaining protection and enforcing our intellectual property;

difficulties in staffing and managing foreign operations;

complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;

limits in our ability to penetrate international markets;

financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products, and exposure to foreign currency exchange rate fluctuations;

natural disasters, political and economic instability, including wars, terrorism, and political unrest, outbreak of disease, boycotts, curtailment of trade, and other business restrictions;

certain expenses including, among others, expenses for travel, translation, and insurance; and

regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, or FCPA, its books and records provisions, or its anti-bribery provisions.

Any of these factors could significantly harm our future international expansion and operations and, consequently, our results of operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

Our research and development activities and our third-party manufacturers and suppliers activities involve the controlled storage, use, and disposal of hazardous materials, including the components of our product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling, and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently, and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. We do not currently carry biological or hazardous waste insurance coverage.

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Risks Related to this Offering and Ownership of Our Ordinary Shares

We may be a passive foreign investment company , or PFIC, for U.S. federal income tax purposes in the current taxable year or may become one in any subsequent taxable year. There generally would be negative tax consequences for U.S. taxpayers that are holders of our ordinary shares if we are or were to become a PFIC.

We would be treated as a PFIC for U.S. federal income tax purposes in any taxable year in which either (1) at least 75% of our gross income is passive income or (2) on average at least 50% of our assets by value produce passive income or are held for the production of passive income. Passive income for this purpose generally includes, among other things, certain dividends, interest, royalties, rents and gains from commodities and securities transactions and from the sale or exchange of property that gives rise to passive income. Passive income also includes amounts derived by reason of the temporary investment of funds, including those raised in a public offering. In determining whether a non-U.S. corporation is a PFIC, a proportionate share of the income and assets of each corporation in which it owns, directly or indirectly, at least a 25% interest (by value) is taken into account. We cannot rule out that we will not be a PFIC for our current taxable year or in the future. The tests for determining PFIC status are applied annually, and it is difficult to make accurate projections of future income and assets which are relevant to this determination. In addition, our PFIC status may depend in part on the market value of our ordinary shares. Accordingly, there can be no assurance that we currently are not or will not become a PFIC in the future. If we are a PFIC in any taxable year during which a U.S. taxpayer holds our ordinary shares, such U.S. taxpayer would be subject to certain adverse U.S. federal income tax rules. In particular, if the U.S. taxpayer did not make an election to treat us as a qualified electing fund, or QEF, or make a mark-to-market election, then excess distributions to the U.S. taxpayer, and any gain realized on the sale or other disposition of our ordinary shares by the U.S. taxpayer: (1) would be allocated ratably over the U.S. taxpayer s holding period for the ordinary shares; (2) the amount allocated to the current taxable year and any period prior to the first day of the first taxable year in which we were a PFIC would be taxed as ordinary income; and (3) the amount allocated to each of the other taxable years would be subject to tax at the highest rate of tax in effect for the applicable class of taxpayer for that year, and an interest charge for the deemed deferral benefit would be imposed with respect to the resulting tax attributable to each such other taxable year. In addition, if the Internal Revenue Service, or IRS, determines that we are a PFIC for a year with respect to which we have determined that we were not a PFIC, it may be too late for a U.S. taxpayer to make a timely QEF or mark-to-market election. U.S. taxpayers that have held our ordinary shares during a period when we were a PFIC will be subject to the foregoing rules, even if we cease to be a PFIC in subsequent years, subject to exceptions for U.S. taxpayer who made a timely QEF or mark-to-market election. A U.S. taxpayer can make a QEF election by completing the relevant portions of and filing IRS Form 8621 in accordance with the instructions thereto. We do not intend to notify U.S. taxpayers that hold our ordinary shares if we believe we will be treated as a PFIC for any taxable year in order to enable U.S. taxpayers to consider whether to make a QEF election. In addition, we do not intend to furnish such U.S. taxpayers annually with information needed in order to complete IRS Form 8621 and to make and maintain a valid QEF election for any year in which we or any of our subsidiaries are a PFIC. U.S. taxpayers that hold our ordinary shares are strongly urged to consult their tax advisors about the PFIC rules, including tax return filing requirements and the eligibility, manner, and consequences to them of making a QEF or mark-to-market election with respect to our ordinary shares in the event that we are a PFIC. See Taxation U.S. Federal Income Tax Consequences Passive foreign investment company consequences for additional information.

The market price of our ordinary shares may be highly volatile, and you may not be able to resell your shares at or above the initial public offering price.

Prior to this offering, there has not been a public market for our ordinary shares. If an active trading market for our ordinary shares does not develop following this offering, you may not be able to sell your shares quickly or at the market price. The initial public offering price for the shares will be determined by negotiations between us and representatives of the underwriters and may not be indicative of prices that will prevail in the trading market.

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The trading price of our ordinary shares is likely to be volatile. The following factors, in addition to other risk factors described in this section, may have a significant impact on the market price of our ordinary shares:

inability to obtain the approvals necessary to commence further clinical trials;
unsatisfactory results of clinical trials;
announcements of regulatory approval or the failure to obtain it, or specific label indications or patient populations for its use, or changes or delays in the regulatory review process;
announcements of therapeutic innovations or new products by us or our competitors;
adverse actions taken by regulatory agencies with respect to our clinical trials, manufacturing supply chain or sales and marketing activities;
changes or developments in laws or regulations applicable to any candidate product of our in each of our platforms;
any adverse changes to our relationship with manufacturers or suppliers;
any intellectual property infringement actions in which we may become involved;
announcements concerning our competitors or the pharmaceutical industry in general;
achievement of expected product sales and profitability or our failure to meet expectations;
our commencement of, or involvement in, litigation;
any major changes in our Board of Directors or management; and
legislation in the United States relating to the sale or pricing of pharmaceuticals.

In addition, the stock market in general, and The NASDAQ Stock Market in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of small companies. Broad market and industry factors may negatively affect the market price of our ordinary shares, regardless of our actual operating performance. Further, a systemic decline in the financial markets and related factors beyond our control may cause our share price to decline rapidly and unexpectedly.

We may be subject to securities litigation, which is expensive and could divert management attention.

In the past companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Litigation of this type could result in substantial costs and diversion of management s attention and resources, which could seriously hurt our business. Any adverse determination in litigation could also subject us to significant liabilities.

Our principal shareholders, chief executive officer and directors currently own over 80% of our outstanding ordinary shares and will own approximately 63% of our ordinary shares upon the closing of this offering. They will therefore be able to exert significant control over matters submitted to our shareholders for approval.

After this offering, our chief executive officer and directors, and shareholders who own more than 5% of our outstanding ordinary shares before this offering will, in the aggregate, beneficially own approximately 63% of our ordinary shares (assuming no exercise of the underwriters over-allotment option and no exercise of outstanding options and does not include the potential purchase of any ordinary shares in this offering by these persons). This significant concentration of share ownership may adversely affect the trading price for our ordinary shares because investors often perceive disadvantages in owning stock in companies with controlling shareholders. As a result, these shareholders, if they acted together, could significantly influence or even unilaterally approve matters requiring approval by our shareholders, including the election of directors and the approval of mergers or other business

combination transactions. The interests of these shareholders may not always coincide with our interests or the interests of other shareholders.

Our principal shareholders, chief executive officer and directors currently ownover 80% of our outstanding ordinary

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If you purchase our ordinary shares in this offering, you will incur immediate and substantial dilution in the book value of your shares.

The initial public offering price is substantially higher than the net tangible book value per share of our ordinary shares. Investors purchasing ordinary shares in this offering will pay a price per share that substantially exceeds the net tangible book value of our ordinary shares. As a result, investors purchasing ordinary shares in this offering will incur immediate dilution of $8.37 per share, based on an initial public offering price of $11.00 per share, and our pro forma net tangible book value as of December 31, 2013. In addition, as of the date of this prospectus, options to purchase 411,630 of our ordinary shares, excluding 45,000 options granted to an employee at an exercise price equal to the offering price per share of this offering, at a weighted average exercise price of $0.02 per share were outstanding. The exercise of these options would result in additional dilution. As a result of this dilution, investors purchasing shares in this offering may receive significantly less than the purchase price paid in this offering in the event of liquidation. For more information, please refer to the section of this prospectus entitled Dilution.

Sales of a substantial number of our ordinary shares in the public market by our existing shareholders could cause our share price to fall.

Sales of a substantial number of our ordinary shares in the public market or the perception that these sales might occur, could depress the market price of our ordinary shares and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales may have on the prevailing market price of our ordinary shares. Substantially all of the shares owned by our existing shareholders and option holders are subject to lock-up agreements with the underwriters of this offering that restrict the shareholders ability to transfer our ordinary shares for six months from the date of this prospectus. Substantially all of our outstanding shares will become eligible for unrestricted sale upon expiration of the lockup period, as described in the section of this prospectus entitled Shares Eligible for Future Sale. In addition, shares issued or issuable upon exercise of options and warrants vested as of the expiration of the lock-up period will be eligible for sale at that time. Sales of shares by these shareholders could have a material adverse effect on the trading price of our ordinary shares.

Our management will have broad discretion in the use of the net proceeds from this offering and may allocate the net proceeds from this offering in ways that you and other shareholders may not approve.

Our management will have broad discretion in the use of the net proceeds, including for any of the purposes described in the section entitled Use of Proceeds, and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. The failure of our management to use these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our shareholders.

If securities or industry analysts do not publish or cease publishing research or reports about us, our business or our market, or if they adversely change their recommendations or publish negative reports regarding our business or our shares, our share price and trading volume could decline.

The trading market for our ordinary shares will be influenced by the research and reports that industry or securities analysts may publish about us, our business, our market or our competitors. We do not have any control over these analysts and we cannot provide any assurance that analysts will cover us or provide favorable coverage. If any of the analysts who may cover us adversely change their recommendation regarding our shares, or provide more favorable relative recommendations about our competitors, our share price would likely decline. If any analyst who may cover us were to cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause our share price or trading volume to decline.

If securities or industry analysts do not publish or cease publishing researchor reports about us, our business or ou

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Because we do not intend to declare cash dividends on our ordinary shares in the foreseeable future, shareholders must rely on appreciation of the value of our ordinary shares for any return on their investment.

We have never declared or paid cash dividends on our ordinary shares. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends in the foreseeable future. As a result, only appreciation of the price of our ordinary shares, if any, will provide a return to investors in this offering.

The requirements associated with being a public company will require significant company resources and management attention.

Following this offering, we will become subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, the listing requirements of the securities exchange on which our ordinary shares is traded, and other applicable securities rules and regulations. The Exchange Act requires that we file periodic reports with respect to our business and financial condition and maintain effective disclosure controls and procedures and internal control over financial reporting. In addition, subsequent rules implemented by the SEC and The NASDAQ Stock Market may also impose various additional requirements on public companies. As a result, we will incur additional legal, accounting and other expenses that we did not incur as a nonpublic company, particularly after we are no longer an emerging growth company as defined in the JOBS Act. Further, the need to establish the corporate infrastructure demanded of a public company may divert management s attention from implementing our development plans. We have made changes to our corporate governance standards, disclosure controls and financial reporting and accounting systems to meet our reporting obligations. The measures we take, however, may not be sufficient to satisfy our obligations as a public company, which could subject us to delisting of our ordinary shares, fines, sanctions and other regulatory action and potentially civil litigation.

The JOBS Act and our status as a foreign private issuer will allow us to postpone the date by which we must comply with some of the laws and regulations intended to protect investors and to reduce the amount of information we provide in our reports filed with the SEC, which could undermine investor confidence in our company and adversely affect the market price of our ordinary shares.

For so long as we remain an emerging growth company as defined in the JOBS Act, we intend to take advantage of certain exemptions from various requirements that are applicable to public companies that are not emerging growth companies including:

the provisions of the Sarbanes-Oxley Act requiring that our independent registered public accounting firm provide an attestation report on the effectiveness of our internal control over financial reporting;
the say on pay provisions requiring a non-binding shareholder vote to approve compensation of certain executive officers and the say on golden parachute provisions requiring a non-binding shareholder vote to approve golden parachute arrangements for certain executive officers in connection with mergers and certain other business combinations of the Dodd-Frank Act and some of the disclosure requirements of the Dodd-Frank Act relating to compensation of our chief executive officer;

Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. This means that an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We are electing to delay such adoption of new or revised accounting standards. As a result, our financial statements may not be comparable to companies that comply with the public company effective date.

the requirement to provide detailed compensation discussion and analysis in proxy statements and reports filed under the Exchange Act, and instead provide a reduced level of disclosure concerning executive compensation;

The JOBS Act and our status as a foreign private issuer will allow us topostpone the date by which we must comply

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our ability not to comply with new accounting principles that do not apply to public companies until such accounting principles become applicable to private companies;
any rules that may be adopted by the Public Company Accounting Oversight Board, or PCAOB, requiring mandatory audit firm rotation or a supplement to the auditor s report on the financial statements; and
our ability to furnish two rather than three years of income statements and statements of cash flows in various required filings.

We intend to take advantage of these exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least $1.0 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our ordinary shares that is held by non-affiliates exceeds $700 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Our status as a foreign private issuer also exempts us from compliance with certain SEC laws and regulations and certain regulations of The NASDAQ Stock Market, including the proxy rules, the short-swing profits recapture rules, and certain governance requirements such as independent director oversight of the nomination of directors and executive compensation.

We cannot predict if investors will find our ordinary shares less attractive because we may rely on these exemptions. If some investors find our ordinary shares less attractive as a result, there may be a less active trading market for our ordinary shares, and our share price may be more volatile and may decline.

Risks Related to Israeli Law and Our Operations in Israel

Our headquarters and other significant operations are located in Israel and, therefore, our results may be adversely affected by political, economic and military instability in Israel.

Our executive offices are located in Tel-Aviv, Israel. In addition, the majority of our officers and directors are residents of Israel. Accordingly, political, economic and military conditions in Israel may directly affect our business. Since the establishment of the State of Israel in 1948, a number of armed conflicts have taken place between Israel and its neighboring countries. Any hostilities involving Israel or the interruption or curtailment of trade between Israel and its trading partners could adversely affect our operations and results of operations. During July 2014 and November 2012, Israel was engaged in an armed conflict with a militia group and political party which controls the Gaza Strip, and during the summer of 2006, Israel was engaged in an armed conflict with Hezbollah, a Lebanese Islamist Shiite militia group and political party. These conflicts involved missile strikes against civilian targets in various parts of Israel, including areas in which our employees and some of our consultants are located, and negatively affected business conditions in Israel. Any armed conflicts, terrorist activities or political instability in the region could adversely affect business conditions and could harm our results of operations and could make it more difficult for us to raise capital. Parties with whom we do business may sometimes decline to travel to Israel during periods of heightened unrest or tension, forcing us to make alternative arrangements when necessary in order to meet our business partners face to face. In addition, the political and security situation in Israel may result in parties with whom we have agreements involving performance in Israel claiming that they are not obligated to perform their commitments under those agreements pursuant to force majeure provisions in such agreements.

Our commercial insurance does not cover losses that may occur as a result of an event associated with the security

situation in the Middle East. Although the Israeli government has in the past covered the reinstatement value of certain damages that were caused by terrorist attacks or acts of war, we cannot assure you that this government coverage will be maintained, or if maintained, will be sufficient to compensate us fully for damages incurred. Any losses or damages incurred by us could have a material adverse effect on our business. Any armed conflicts or political instability in the region would likely negatively affect business conditions generally and could harm our results of operations.

Our headquarters and other significant operations are located in Israel and,therefore, our results may be adversely

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Further, in the past, the State of Israel and Israeli companies have been subjected to economic boycotts. Several countries still restrict business with the State of Israel and with Israeli companies. These restrictive laws and policies may have an adverse impact on our operating results, financial conditions or the expansion of our business.

Our operations may be disrupted as a result of the obligation of management or key personnel to perform military service.

Our male employees and consultants in Israel, including members of our senior management, may be obligated to perform one month, and in some cases longer periods, of annual military reserve duty until they reach the age of 40 (or older, for citizens who hold certain positions in the Israeli armed forces reserves), and, in the event of a military conflict, may be called to active duty. In response to increases in terrorist activity, there have been periods of significant call-ups of military reservists. It is possible that there will be similar large-scale military reserve duty call-ups in the future. Our operations could be disrupted by the absence of a significant number of our officers, directors, employees and consultants. Such disruption could materially adversely affect our business and operations.

Exchange rate fluctuations between the U.S. dollar and the New Israeli Shekel currencies may negatively affect our earnings.

We incur expenses both in U.S. dollars and New Israeli Shekels, but our financial statements are denominated in U.S. dollars. As a result, we are exposed to the risks that the New Israeli Shekel may appreciate relative to the U.S. dollar, or, if the New Israeli Shekel instead devalues relative to the U.S. dollar, that the inflation rate in Israel may exceed such rate of devaluation of the New Israeli Shekel, or that the timing of such devaluation may lag behind inflation in Israel. In any such event, the U.S. dollar cost of our operations in Israel would increase and our U.S. dollar-denominated results of operations would be adversely affected. We cannot predict any future trends in the rate of inflation in Israel or the rate of devaluation (if any) of the New Israeli Shekel against the U.S. dollar.

Provisions of Israeli law and our amended and restated articles of association may delay, prevent or otherwise impede a merger with, or an acquisition of, our company, which could prevent a change of control, even when the terms of such a transaction are favorable to us and our shareholders.

Israeli corporate law regulates mergers, requires tender offers for acquisitions of shares above specified thresholds, requires special approvals for transactions involving directors, officers or significant shareholders and regulates other matters that may be relevant to such types of transactions. For example, a merger may not be consummated unless at least 50 days have passed from the date on which a merger proposal is filed by each merging company with the Israel Registrar of Companies and at least 30 days have passed from the date on which the shareholders of both merging companies have approved the merger. In addition, a majority of each class of securities of the target company must approve a merger. Moreover, a tender offer for all of a company s issued and outstanding shares can only be completed if the acquirer receives positive responses from the holders of at least 95% of the issued share capital. Completion of the tender offer also requires approval of a majority of the offerees that do not have a personal interest in the tender offer, unless, following consummation of the tender offer, the acquirer would hold at least 98% of the Company s outstanding shares. Furthermore, the shareholders, including those who indicated their acceptance of the tender offer, may, at any time within six months following the completion of the tender offer, claim that the consideration for the acquisition of the shares does not reflect fair market value, and petition an Israeli court to alter the consideration for the acquisition, unless the acquirer stipulated in its tender offer that a shareholder that accepts the offer may not seek such appraisal rights.

Furthermore, Israeli tax considerations may make potential transactions unappealing to us or to our shareholders whose country of residence does not have a tax treaty with Israel exempting such shareholders from Israeli tax. See Taxation Israeli Tax Considerations for additional information.

Our amended and restated articles of association that will be in effect immediately prior to the consummation of this offering will also contain provisions that could delay or prevent changes in control or changes in our management without the consent of our Board of Directors. These provisions will include the following:

Provisions of Israeli law and our amended and restated articles of associationmay delay, prevent or otherwise impe

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no cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates; and

the exclusive right of our Board of Directors to elect a director to fill a vacancy created by the expansion of the Board of Directors or the resignation, death or removal of a director, which prevents stockholders from being able to fill vacancies on our Board of Directors.

It may be difficult to enforce a judgment of a United States court against us and our officers and directors and the Israeli experts named in this prospectus in Israel or the United States, to assert United States securities laws claims in Israel or to serve process on our officers and directors and these experts.

We were incorporated in Israel. Substantially all of our executive officers and directors reside outside of the United States, and all of our assets and most of the assets of these persons are located outside of the United States. Therefore, a judgment obtained against us, or any of these persons, including a judgment based on the civil liability provisions of the U.S. federal securities laws, may not be collectible in the United States and may not necessarily be enforced by an Israeli court. It also may be difficult for you to affect service of process on these persons in the United States or to assert U.S. securities law claims in original actions instituted in Israel. Additionally, it may be difficult for an investor, or any other person or entity, to initiate an action with respect to United States securities laws in Israel. Israeli courts may refuse to hear a claim based on an alleged violation of United States securities laws reasoning that Israel is not the most appropriate forum in which to bring such a claim. In addition, even if an Israeli court agrees to hear a claim, it may determine that Israeli law and not United States law is applicable to the claim. If United States law is found to be applicable, the content of applicable United States law must be proven as a fact by expert witnesses, which can be a time consuming and costly process. Certain matters of procedure will also be governed by Israeli law. There is little binding case law in Israel that addresses the matters described above. As a result of the difficulty associated with enforcing a judgment against us in Israel, you may not be able to collect any damages awarded by either a United States or foreign court. See Enforceability of Civil Liabilities for additional information on your ability to enforce a civil claim against us and our executive officers or directors named in this prospectus.

Your rights and responsibilities as a shareholder will be governed by Israeli law which differs in some material respects from the rights and responsibilities of shareholders of U.S. companies.

The rights and responsibilities of the holders of our ordinary shares are governed by our amended and restated articles of association and by Israeli law. These rights and responsibilities differ in some material respects from the rights and responsibilities of shareholders in typical U.S.-based corporations. In particular, a shareholder of an Israeli company has certain duties to act in good faith and fairness towards the Company and other shareholders, and to refrain from abusing its power in the Company. See Management Approval of Related Party Transactions under Israeli Law Shareholder Duties for additional information. There is limited case law available to assist us in understanding the nature of this duty or the implications of these provisions. These provisions may be interpreted to impose additional obligations and liabilities on holders of our ordinary shares that are not typically imposed on shareholders of U.S. corporations.

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Some of the statements made under Prospectus Summary, Risk Factors, Use of Proceeds, Management s Discussion and Analysis of Financial Condition and Results of Operations, Business and elsewhere in this prospectus constitute forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as may, will, should, expects, plans, anticipates, believes, estimates, predicts, potential, intends, or conti these terms or other comparable terminology.

Forward-looking statements include, but are not limited to, statements about:

our expectations regarding expansion of the OPMD clinical study;

our expectations regarding the timing of commencing clinical studies with SCA3, SBMA, BB-FA, BB-OTC and BBrm1;

the likelihood of regulatory approvals for our product candidates;

the commercial launch and future sales of our product candidates or any other future products or product candidates;

our ability to achieve favorable pricing for our product candidates;

the potential for our drug candidate product to receive designation as an orphan drug and implications if it does not receive such designation;

our expectations regarding the commercial supply of our product candidates;

third-party payor reimbursement for our product candidates;

our estimates regarding anticipated expenses, capital requirements and our needs for substantial additional financing;

the ultra-rare diseases patient market size and market adoption of our candidate products by physicians and patients;

the timing, cost or other aspects of the commercial launch of our product candidates;

the timing of commencement, duration and cost of clinical trials for our candidate products or whether such trials will be conducted at all;

completion and receiving favorable results of clinical trials for our candidate products;

issuance of patents to us by the USPTO and other governmental patent agencies;

the development and approval of the use of our candidate products for additional indications other than ultra-rare diseases; and

our expectations regarding licensing, acquisitions and strategic operations.

These statements are only current predictions and are subject to known and unknown risks, uncertainties, and other factors that may cause our or our industry s actual results, levels of activity, performance or achievements to be materially different from those anticipated by the forward-looking statements. We discuss many of these risks in this prospectus in greater detail under the heading Risk Factors and elsewhere in this prospectus. You should not rely upon forward-looking statements as predictions of future events.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance, or achievements. Except as required by law, we are under no duty to update or revise any of the forward-looking statements, whether as a result of new information, future events or otherwise, after the date of this prospectus.

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USE OF PROCEEDS

We expect to receive approximately $31.9 million in net proceeds from the sale of 3,200,000 ordinary shares offered by us in this offering (approximately $36.8 million if the underwriters exercise their over-allotment option in full) at an initial public offering price of $11.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

We currently expect to use the net proceeds from this offering for:

completing our clinical program for OPMD, estimated at $6,000,000;
completing our Phase 2 and pivotal clinical study for SCA3, estimated at $3,500,000;
Initiating and completing our Phase 2/3 clinical study for SBMA, estimated at $4,200,000;
completing our pre-clinical program for BB-FA and Phase 1 clinical study, estimated at $4,500,000;
completing our pre-clinical program for BB-OTC and initiation and completion of Phase 1 and 2A-2B clinical studies, estimated at $7,800,000;
Initiating and completing our Phase 1-2 clinical programs for SMA, estimated at $2,200,000;
Other Indications, estimated at $500,000;
Premarketing activity for OPMD, estimated at $1,500,000; and
the remainder for personnel-related costs, preclinical research, working capital, and other general corporate purposes.

The amounts and timing of our actual expenditures will depend upon numerous factors, including the progress of our development and commercialization efforts, the status of and results from our clinical trials, whether or not we enter into strategic collaborations or partnerships, and our operating costs and expenditures. Accordingly, our management will have significant flexibility in applying the net proceeds of this offering.

We have no current understandings, commitments or agreements with respect to any material acquisition of or investment in any technologies, products or companies.

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DIVIDEND POLICY

We have never declared or paid any cash dividends on our ordinary shares and do not anticipate paying any cash dividends in the foreseeable future. Payment of cash dividends, if any, in the future will be at the discretion of our Board of Directors and will depend on then-existing conditions, including our financial condition, operating results, contractual restrictions, capital requirements, business prospects and other factors our Board of Directors may deem relevant.

The Israeli Companies Law imposes further restrictions on our ability to declare and pay dividends. See Description of Share Capital Dividend and Liquidation Rights for additional information.

Payment of dividends may be subject to Israeli withholding taxes. See Taxation Israeli Tax Considerations for additional information.

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CAPITALIZATION

The following table sets forth our cash and cash equivalents and our capitalization as of December 31, 2013:

on an actual basis;

on a pro forma basis to also give effect to the sale of 1,065,076 ordinary shares on January 1, 2014 at the price of $0.95 per share and to the sale of 782,537 ordinary shares on February 6, 2014 at the price of $6.07 per share, as if the sale of the shares in each case had occurred on December 31, 2013.

on a pro forma, as adjusted, basis to also give effect to the sale of 3,200,000 ordinary shares in this offering at the initial public offering price of $11.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, at the closing of the offering, as if the sale of the shares in each case had occurred on December 31, 2013.

You should read this table in conjunction with the sections titled Selected Financial Data and Management s Discussion and Analysis of Financial Condition and Results of Operations and our financial statements and related notes included elsewhere in this prospectus.

	December 31, 2013		
(in thousands, except share and per share data)	Actual	Pro forma (unaudited)	Pro Forma as adjusted (unaudited)
Ordinary shares of NIS 0.01 par value 16,613,139 and 15,102,854 shares authorized at December 31, 2013 and 2012, respectively; 9,182,867 and 7,551,427 issued and outstanding shares at December 31, 2013 and 2012 respectively; 11,030,480 shares pro forma as of December 31, 2013 (unaudited)	24	29	38
Additional paid-in capital	1,551	6,926	38,768
Deficit accumulated during the development stage	(1,400)	(1,400)	(1,400)
Total shareholders equity	175	5,555	37,406
Total capitalization	$175	$ 5,555	$ 37,406

The outstanding share information above excludes:

403,110 shares issuable upon the exercise of share options outstanding as of December 31, 2013; and

53,789 shares reserved as of January 26, 2014 for future grants under our equity incentive plan.

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DILUTION

If you invest in our ordinary shares, you will experience immediate and substantial dilution to the extent of the difference between the initial public offering price of our ordinary shares and the pro forma as adjusted net tangible book value per share of our ordinary shares immediately after the offering.

Our historical net tangible book value per share is determined by dividing our total tangible assets, less total liabilities, by the actual number of outstanding ordinary shares. The historical net tangible book value of our ordinary shares as of December 31, 2013 was $175,000, or $0.02 per share.

On a pro forma basis, giving effect to the sale of 1,065,076 ordinary shares on January 1, 2014 at a price of $0.95 per share and to the sale of 782,537 ordinary shares on February 6, 2014 at a price of $6.07 per share, our historical net tangible book value of our ordinary shares as of December 31, 2013 would have been $5,555,000, or $0.50 per share.

The pro forma as adjusted net tangible book value of our ordinary shares as of December 31, 2013 was $37,405,484, or $2.63 per share. The pro forma as adjusted net tangible book value gives effect to the sale of ordinary shares in this offering at the initial public offering price of $11.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. The difference between the initial public offering price and the pro forma as adjusted net tangible book value per share represents an immediate dilution of $8.37 per share to new investors purchasing ordinary shares in this offering.

The following table illustrates this dilution on a per share basis to new investors:

Initial public offering price per share		$ 11.00
Historical net tangible book value per share before this offering, as of December 31, 2013		0.02
Pro forma increase in net tangible book value per share		0.48
Pro forma net tangible book value per share as of December 31, 2013		0.50
Increase in net tangible book value per share attributable to new investors in this offering	$ 2.13	
Pro forma as adjusted net tangible book value per share after offering		2.63
Dilution in pro forma tangible book value per share to new investors		8.37

If the underwriters over-allotment option to purchase additional shares from us is exercised in full, and based on the initial public offering price of $11.00 per share, the pro forma as adjusted net tangible book value per share after this offering would be approximately $2.88 per share, the increase in the pro forma net tangible book value per share attributable to new investors would be approximately $2.38 per share and the dilution to new investors purchasing shares in this offering would be approximately $8.12 per share.

The table below summarizes as of December 31, 2013, on the pro forma as adjusted basis described above, the number of ordinary shares we issued and sold, the total consideration we received and the average price per share (1) paid by our existing shareholders and (2) to be paid by new investors purchasing our ordinary shares in this offering at the initial public offering price of $11.00 per share, before deducting underwriting discounts and commissions and estimated offering expenses payable by us.

	Shares Purchased	Total Consideration, net

	Number	Percent	Amount	Percent	Average Price Per Share
Existing shareholders	11,030,480	77.5 %	$ 6,666,000	15.9 %	$ 0.60
New investors	3,200,000	22.5 %	35,200,000	84.1	11.00
Total	14,230,480	100 %	$ 41,866,000	100 %	$ 2.94

The number of ordinary shares outstanding immediately after this offering is based on 9,182,867 ordinary shares outstanding as of December 31, 2013 and gives effect to the pro forma transactions of the sale of 1,065,076 ordinary shares on January 1, 2014 at the price of $0.95 per share and to the sale of 782,537 ordinary shares on February 6, 2014 at the price of $6.07 per share, as if the sale of the shares in each case had occurred on December 31, 2013.

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The foregoing table excludes:

- 403,110 shares issuable upon the exercise of share options outstanding as of December 31, 2013; and
- 53,789 shares reserved as of January 26, 2014 for future grants under our equity incentive plan.

To the extent any of the outstanding options as of December 31, 2013 are exercised, there will be further dilution to new investors. To the extent all of such outstanding options had been exercised as of December 31, 2013, the pro forma as adjusted net tangible book value per share after this offering would be $2.56 and the total dilution per share to new investors would be $8.44.

To the extent that new options are granted under our equity benefit plans, there will be further dilution to investors purchasing ordinary shares in this offering.

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SELECTED FINANCIAL DATA

The following table summarizes our financial data. We have derived the following statements of operations data for the years ended December 31, 2013 and 2012 and the balance sheet data as of December 31, 2013 from our audited financial statements included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that may be expected in the future. The following summary financial data should be read in conjunction with Management s Discussion and Analysis of Financial Condition and Results of Operations and our financial statements and related notes included elsewhere in this prospectus.

	Years ended December 31,	
(in thousands of U.S. dollars, except share and per share amounts)	2013	2012
Statements of Operations Data:		
Research and development expenses	$732	$140
General and administrative expenses	416	86
Financial expense (income), net	(3)	3
Deemed dividend	26	
Loss attributable to holders of ordinary shares	1,171	229
Weighted average number of ordinary shares used in computing basic and diluted net loss per share	8,423,018	7,551,427

	Year Ended December 31, 2013		
(in thousands of U.S. dollars)	Actual	Pro forma (unaudited)[1]	Pro forma, as adjusted (unaudited)[2]
Balance Sheet Data:			
Total long-term assets	$ 7	$ 7	$ 7
Total current liabilities	131	131	131
Shareholders equity	175	5,555	37,406

(1) Gives effect to the sale of 1,065,076 ordinary shares on January 1, 2014 at the price of $0.95 per share and to the sale of 782,537 ordinary shares on February 6, 2014 at the price of $6.07 per share, as if the sale of the shares in each case had occurred on December 31, 2013.

(2) Gives effect to the sale of 3,200,000 ordinary shares in this offering at the initial public offering price of $11.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, as if the sale had occurred on December 31, 2013.

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MANAGEMENT S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis should be read in conjunction with our financial statements and related notes included elsewhere in this prospectus. This discussion and other parts of the prospectus contain forward-looking statements based upon current expectations that involve risks and uncertainties. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under Risk Factors and elsewhere in this prospectus.

Introduction

We are a development-stage biopharmaceutical company focused on the identification, licensing, acquisition, development and commercialization of drugs for rare and ultra-rare genetic and metabolic diseases. We seek to identify therapeutic platforms that offer solutions for several diseases that share a common pathophysiological mechanism. We focus on diseases with severe and debilitating manifestations, where the unmet medical need is clear, the biological mechanism of action is understood and for which there is no satisfactory treatment. Since our inception in 2012, we have developed and in-licensed potential treatments for six diseases, one of which is in a Phase 2/3 clinical trial which, if the results are positive, we believe could be a pivotal trial, another one which is in Phase 2 clinical studies and an additional two of which we expect will be in mid/late stage clinical studies by late 2014 or early 2015. We believe, based on discussions with the FDA, that if the Phase 2/3 trial shows positive results, the data could be considered pivotal data, considering, among other things, the applicable FDA guidance for the industry called Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products.

Our current drug candidate pipeline has been either in-licensed from academic institutions or developed internally. Our strategy is based on risk diversification through multiple therapeutic platforms, diversified clinical and pre-clinical stages of our programs, and diversified diseases addressed. We use strict selection criteria of our pipeline platforms and cost-efficient drug development. This allows us to pursue multiple programs in parallel with the goal of promptly delivering safe and effective therapies to patients in dire need.

To date, we have not generated revenue from the sale of any product, and we do not expect to generate significant revenue unless and until we obtain marketing approval of, and commercialize our product candidates. As of December 31, 2013, we had an accumulated deficit of $1.4 million. Our financing activities are described below under Liquidity and Capital Resources.

Financial Overview

Operating Expenses

Our current operating expenses consist of two components research and development expenses, and general and administrative expenses.

Research and Development Expenses

Our research and development expenses consist primarily of cost of third party clinical consultants and expenses related to conducting clinical and preclinical trials, salaries and related personnel expenses, share-based compensation expenses, travel expenses and other research and development expenses.

We expect that our research and development expenses will materially increase as we plan to initiate clinical activity and prepare to conduct clinical trials in the near future.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries, share-based compensation expense, professional service fees for accounting, legal, bookkeeping and facilities, travel expenses and other general and administrative expenses.

We expect our general and administrative expenses, such as accounting and legal fees, to increase after we become a U.S. public company, and we expect increases in the number of our executive, accounting and administrative personnel due to the anticipated growth of our Company.

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Financial Expense and Income

Financial expense and income consist of bank fees and other transactional costs and exchange rate differences.

Critical Accounting Policies and Estimate

We describe our significant accounting policies more fully in Note 2 to our financial statements for the year ended December 31, 2013. We believe that the accounting policies below are critical in order to fully understand and evaluate our financial condition and results of operations.

We prepare our financial statements in accordance with accounting principles generally accepted in the United States (U.S. GAAP).

The preparation of the financial statements in conformity with U.S. GAAP requires management to make estimates, judgments and assumptions. Our management believes that the estimates, judgments and assumptions used are reasonable based upon information available at the time they are made. These estimates, judgments and assumptions can affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the dates of the financial statements, and the reported amounts of expenses during the reporting period. Actual results could differ from those estimates.

JOBS Act

On April 5, 2012, the U.S. Congress enacted the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. This means that an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We are electing to delay such adoption of new or revised accounting standards. As a result, our financial statements may not be comparable to companies that comply with the public company effective date.

Subject to certain conditions set forth in the JOBS Act, as an emerging growth company, we intend to rely on other exemptions, including without limitation, (i) providing an auditor s attestation report on our system of internal controls over financial reporting pursuant to Section 404 and, (ii) complying with any requirement that may be adopted by the PCAOB regarding mandatory audit firm rotation or a supplement to the auditor s report providing additional information about the audit and the financial statements (auditor discussion and analysis). These exemptions will apply for a period of five years following the completion of our initial public offering or until we are no longer an emerging growth company.

Stock-Based Compensation and Fair Value of Ordinary Shares

We account for stock-based compensation in accordance with ASC 718, Compensation Stock Compensation, which requires companies to estimate the fair values of equity-based payments awards on the date of grant using an option-pricing model. The value of the stock options is recognized as an expense over the requisite service periods in our statement of operations. We recognize compensation expenses for the value of our awards granted based on the accelerated method over the requisite service period of each of the awards.

The fair value of the ordinary shares was based on the application of Option-Pricing Method (OPM). The first step in performing a valuation using OPM involves estimating the fair value of the total shareholders equity (capital instruments). As part of our analysis, we used recent investment rounds, respectively to the option valuation dates, in our shares in order to evaluate the fair value of our total shareholders equity.

Under the option-pricing method, we estimated the fair value of the ordinary shares as the net value of a series of call options, representing the present value of the expected future returns to the ordinary shareholders. Essentially, the rights of the ordinary shareholders are equivalent to a call option on any value of the Company above the respective preferred shareholders liquidation preferences, with adjustment to account for the rights retained by the preferred shareholders related to their share in any value above the values at which they would convert to ordinary shares. Thus, the ordinary shares were valued by estimating the value of its share in each of these call option rights. As all options were granted at par value and the exercise price is negligible, the fair value of the options is equal to the share price.

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Results of Operations

	Year ended December 31,	
	2013	2012
	(in thousands US$)	
Research and development expenses	$ 732	$ 140
General and administrative expenses	416	86
Operating loss	1,148	226
Financial Expense (income), net	(3)	3
Loss	$ 1,145	$ 229
Deemed dividend	26	
Loss attributable to holders of ordinary shares	$ 1,171	$ 229

Comparison of the Year Ended December 31, 2013 to the Year Ended December 31, 2012

Research and development expenses

The following table discloses the breakdown of research and development expenses for the last two fiscal years.

	December 31,	
	2013	2012
	(in thousands US$)	
Cost to third-party clinical consultants and expenses related to conducting clinical and preclinical trials	$470	$ 75
Salaries and related personnel	171	34
Share-based compensation	44	
Travel	31	8
Other	16	23
Total	$732	$ 140

Our research and development expenses for the year ended December 31, 2013 amounted to $732,000, representing an increase of $592,000, or 423%, compared to $140,000 for the year ended December 31, 2012. The increase was primarily attributable to an increase of expenses related to third party preclinical consultants and other expenses related to conducting preclinical trials in an amount of $395,000 and to an increase of salaries and related personnel expenses in an amount of $137,000 reflecting an increase in the number of employees engaged in research and development related activities from one to three, in addition an increase of stock based compensation expenses of $44,000 in 2013 from $0 in 2012.

Project expenses by project

	December 31,		Change
	2013	2012	%
	(in thousands US$)		

Cabaletta projects:			
OPMD	$ 407	$	100
SCA3	39		100
SBMA	22		100
mPRT projects:			
Freiedrich s Ataxia	75	64	17
OTC def	94	64	47
Read-through project:			
SMA	48	12	300
Other costs	47		100
Total	$ 732	$ 140	

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Our research and development expense is highly dependent on the development phases of our projects and therefore fluctuates highly from year to year.

The variances in expense between the year ended December 31, 2013 and the corresponding period in 2012 are mainly due to the following projects:

Cabaleta projects. Our expenses related to OPMD, SCA3 and SBMA for the year ended December 31, 2013 amounted to $407,000, $39,000 and $22,000, respectively. These projects were initiated in 2013; therefore, there were no expenses in 2012.
mPRT projects. Our expenses related to Freiedrich s Ataxia and OTC def for the year ended December 31, 2013 amounted to $75,000 and $94,000, respectively, representing an increase of $11,000 and $30,000, or 17% and 47%, respectively, compared to $64,000 for the year ended December 31, 2012. The increase was primarily attributable to an increase in the number of employees involved in this project from one to two and to an increase in the activity.
Read-through project. Our expenses related to SMA for the year ended December 31, 2013 amounted to $48,000, representing an increase of $36,000, or 300%, compared to $12,000 for the year ended December 31, 2012. The increase was primarily attributable to an increase in the number of employees involved in this project from one to two and to the initiation of pre-clinical trials.
Other costs. We incurred costs associated with our research and development that are allocated to projects that were terminated.

General and administrative expenses

Our general and administrative expenses totaled $416,000 for the year ended December 31, 2013, an increase of $330,000, or 384%, compared to $86,000 for the year ended December 31, 2012. The increase resulted primarily from an increase of $201,000 in share-based compensation expenses, an increase of payroll in an amount of $48,000 reflecting an increase of payroll to our employees, and an increase of $43,000 in professional services.

Operating loss

As a result of the foregoing, our operating loss for the year ended December 31, 2013 was $1,148,000, as compared to an operating loss of $226,000 for the year ended December 31, 2012, an increase of $922,000, or 408%.

Financial expense and income

We recognized financial income of $3,000 for the year ended December 31, 2013, compared to financial expenses of $3,000 for the year ended December 31, 2012.

Loss

As a result of the foregoing, our loss for the year ended December 31, 2013 was $1,145,000, as compared to $229,000 for the year ended December 31, 2012, an increase of $916,000 or 400%.

Liquidity and Capital Resources

Overview

Since our inception through December 31, 2013, we have funded our operations principally with $1.3 million from the issuance of ordinary and preferred shares. As of December 31, 2013, we had $270,000 in cash and cash equivalents. Subsequent to the year end, on January 1, 2014 in connection with the share purchase agreement dated June 2013, we issued 1,065,076 ordinary shares for a total consideration of $1,012,000. In addition, on February 6, 2014 we issued 782,537 ordinary shares to investors for a total consideration of $4,368,000, net.

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The table below presents our cash flows for the years 2013 and 2012:

	Years Ended December 31,	
	2013	2012
	(in thousands US$)	
Operating activities	$ (865)	$ (149)
Investing activities	(2)	
Financing activities	991	295
Net increase in cash and cash equivalents	$ 124	$ 146

Operating Activities

Net cash used in operating activities of $0.9 million during the year ended December 31, 2013 was primarily used for payment of $0.6 million for clinical trials and other third party expenses and an aggregate of $0.2 million in salaries and related personnel expenses. The remaining amount of $0.1 was for travel, patent, rent and other miscellaneous expenses. Net cash used in operating activities of $0.1 million during the year ended December 31, 2012 was primarily used for salary payments and for clinical trials and other third party expenses.

Investing Activities

Net cash used in investing activities during 2013 primarily reflected an increase in property and equipment. In 2012, we had no investment activity.

Financing Activities

Net cash provided by financing activities in the year ended December 31, 2013 consisted of approximately $1 million of net proceeds from issuance of ordinary shares. Net cash provided by financing activities in the year ended December 31, 2012 consisted of approximately $0.3 million of net proceeds from issuance of shares.

Current Outlook

We have financed our operations to date primarily through proceeds from sales of our shares. We have incurred losses and generated negative cash flows from operations since inception. To date, we have not generated any revenue from the sale of products and we do not expect to generate revenues from sale of our products in the next few years.

Our independent registered public accounting firm s report to our financial reports for the fiscal year ended December 31, 2013, states that there is a substantial doubt that we will be able to continue as a going concern. Furthermore, according to our estimates, based on our budget, if we are not successful in obtaining additional capital resources, there is a substantial doubt that we will be able to continue our activities after December 31, 2014. Even if we are able to raise funds in the offering contemplated herein, we believe that we will need to raise additional funds before we generate positive cash flow from operations.

As of December 31, 2013, our cash, cash equivalents totaled $0.3 million. On January 1, 2014 we issued 1,065,076 ordinary shares for a total consideration of $1,012,000. In addition, on February 6, 2014 we issued 782,537 ordinary shares to investors for a total consideration of $4,368,000, net. We believe that our existing cash resources and the net proceeds from the current offering will be sufficient to fund our projected cash requirements approximately through

the conclusion of 2015. Nevertheless, we will require significant additional financing in the future to fund our operations if and when we obtain regulatory approval and commercialize our drugs. We currently anticipate that, assuming consummation of the current offering, we will utilize approximately $21.0 million for clinical trial activities over the course of the next 30 months. We also anticipate utilizing approximately between $1 million to $3 million for capital expenditures over such 30-month period, which consists primarily of expenditures for the manufacture of our drug candidate for use in clinical trials and supporting preclinical studies required for obtaining approval to conduct such clinical studies. Our future capital requirements will depend on many factors, including:

the progress and costs of our preclinical studies, clinical trials and other research and development activities;

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the scope, prioritization and number of our clinical trials and other research and development programs;

the costs and timing of obtaining regulatory approval for our drug candidates;

the costs of filing, prosecuting, enforcing and defending patent claims and other intellectual property rights;

the costs of, and timing for, strengthening our manufacturing agreements for production of sufficient clinical and commercial quantities of our drug candidates;

the potential costs of contracting with third parties to provide marketing and distribution services for us or for building such capacities internally;

the costs of acquiring or undertaking the development and commercialization efforts for additional, future therapeutic applications of our drug candidates;

the magnitude of our general and administrative expenses; and

any cost that we may incur under current and future in- and out-licensing arrangements relating to our drug candidates.

Until we can generate significant recurring revenues, we expect to satisfy our future cash needs through the net proceeds from the current offering, debt or equity financings, or by out-licensing applications of our drug candidates. We cannot be certain that additional funding will be available to us on acceptable terms, if at all. If funds are not available, we may be required to delay, reduce the scope of, or eliminate research or development plans for, or commercialization efforts with respect to, one or more applications of our drug candidates. This may raise substantial doubts about the Company s ability to continue as a going concern.

Contractual Obligations

The following table summarizes our significant contractual obligations at December 31, 2013:

	Total	Less than 1 year	1 3 years	4 5 years	More than 5 years
	(in thousands US$)				
Operating leases:					
Facility	4	4			
Motor Vehicles	39		39		

During the year ended December 31, 2013, we engaged with few service providers and vendors. However, we do not deem such engagements as significant compared with our current financial resources.

Off-Balance Sheet Arrangements

We currently do not have any off-balance sheet arrangements.

Quantitative and Qualitative Disclosure About Market Risk

We are exposed to market risks in the ordinary course of our business. Market risk represents the risk of loss that may impact our financial position due to adverse changes in financial market prices and rates. Our market risk exposure is primarily a result of foreign currency exchange rates.

Foreign Currency Exchange Risk

Our results of operations and cash flow are subject to fluctuations due to changes in foreign currency exchange rates. Certain of our expenses are denominated in New Israeli Shekels. Our results of operations and cash flow are, therefore, subject to fluctuations due to changes in foreign currency exchange rates and may be adversely affected in the future due to changes in foreign exchange rates. Approximately 40% of our expenses are denominated in New Israeli Shekel. Changes of 5% and 10% in the U.S. Dollar / New Israeli Shekel exchange rate will increase/decrease the operation expenses by 2% and 4%, respectively. We do not hedge our foreign currency exchange risk. In the future, we may enter into formal currency hedging transactions to decrease the risk of financial exposure from fluctuations in the exchange rates of our principal operating currencies. These measures, however, may not adequately protect us from the material adverse effects of such fluctuations.

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BUSINESS

Overview

We are a development-stage biopharmaceutical company focused on the identification, licensing, acquisition, development and commercialization of drugs for rare and ultra-rare genetic and metabolic diseases. We seek to identify therapeutic platforms that offer solutions for several diseases that share a common pathophysiological mechanism, which are the functional changes that accompany a particular syndrome or disease. We focus on diseases with severe and debilitating manifestations, where the unmet medical need is clear, the biological mechanism of action is understood and for which there is no satisfactory treatment. Since our inception in 2012, we have developed and in-licensed potential treatments for six diseases, one of which in a Phase 2/3 clinical trial which, if the results are positive, we believe could be a pivotal trial, another one which is in Phase 2 clinical study and an additional two of which we expect will be in mid/late stage clinical studies by late 2014 or early 2015. We have not yet submitted any investigational new drug applications for our products to the FDA. We believe, based on discussions with the FDA, that if the Phase 2/3 trial shows positive results, the data could be considered pivotal data, considering, among other things, the applicable FDA guidance for the industry called Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products.

Our current drug candidate pipeline has been either in-licensed from academic institutions or developed internally. Our strategy is based on risk diversification through multiple therapeutic platforms, diversified clinical and pre-clinical stages of our programs, and diversified diseases addressed. We use strict selection criteria of our pipeline platforms and cost-efficient drug development. This allows us to pursue multiple programs in parallel with the goal of promptly delivering safe and effective therapies to patients in dire need.

In order to implement our strategy we are following three principles:

Strict selection criteria of our platforms and clinical programs;
Risk diversification through multiple platforms, clinical programs and stages of development; and
Variable lean cost structure.

We believe that our strategy and special business operation model is especially suitable for commercializing effective and safe products in a timely and cost efficient manner.

Strict selection criteria of our platforms and clinical program.

We target rare and ultra-rare diseases that:

cause severely shortened life expectancy or severe debilitation;
do not have an approved and effective therapy currently available;
have a drug candidate that we believe will either be lifesaving or significantly ameliorate the disease course;
have tangible and validated tests where initial efficacy can be demonstrated in a reasonable time frame to show initial efficacy; and
preferably have validated biomarkers and where there is availability of a natural history study.

As part of this selection process, we:

estimate the scope and availability of research tools for the preclinical studies required;
limit the diseases selected to those in which the full pre-clinical tool kit is available or can be obtained through

collaboration with patients organizations;
estimate the size and structure of the clinical studies that will be required and confine our selections to fit what we believe will be a cost effective program that will not require unreasonable financial resources;
select diseases in which the regulatory path to approval is clear and well defined;

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strictly evaluate the strength of the intellectual property that we license for further development;
estimate the required chemistry, manufacturing and control development program;
exclude drug candidates that are known to have any safety issues and prefer, when possible and feasible to repurposed drugs; and
part of our screening and selection criteria involves the key opinion leaders and the advocacy group for the specific disease we are considering, and our decision to move forward is often taken in consensus with them.

Because these diseases are rare or ultra-rare, no prenatal diagnosis is widely offered that might reduce the incidence of the diseases. Optimally, our target diseases have geographic clusters around a small number of clinics with sufficient number of patients to avoid large multicenter studies. Due to our location in Israel we focus on such diseases for which we can find a disease cluster in Israel where we can start a proof of concept study promptly.

Risk diversification through multiple platforms, clinical programs and stages of development.

Drug development is a complex and unpredictable process with multiple risks. We believe that a biopharmaceutical company such as ours should diversify the inherent risk associated with drug development. Drug development occurs in preclinical and clinical stages, each of which must satisfy separate criteria with different risks, including safety and efficacy.

We have begun to diversify risk by initially focusing on three therapeutic platforms, each of them can be applied to several diseases. In each case, we have identified a lead program, but we believe we have the opportunity to benefit from the shorter and less expansive route to additional indications based on the same platform. In addition there are different risks associated with the development of a small molecule drug, a biological drug, or a repurposed drug. Our portfolio is comprised of all three therefore balancing the systematic risk associated with it.

Variable lean cost structure.

We believe in keeping a small, select and highly professional core team at our Company while relying on experienced service providers and CROs to result in an efficient and time saving development process. We carefully screen our service providers and CROs for their expertise in the specific project required and work with them closely to complete the task assigned to them efficiently and professionally. This business operation model enables us to develop multiple programs while avoiding the exorbitant costs associated with learning curve, setup times, and high fixed costs associated with in-house development.

Our current pipeline is based on three platforms:

Cabaletta is a mutant protein stabilizing platform based on a small repurposed molecules, Trehalose, which is currently used as an excipient for IV protein drugs. We are currently conducting a Phase 2/3 clinical trial which, if the results are positive, we believe could be a pivotal trial to assess its efficacy and safety in treating Oculopharyngeal Muscular Dystropy (OPMD), and a Phase 2 clinical trial to assess its efficacy and safety in treating Machado Joseph disease, or SCA3. We believe, based on discussions with the FDA, that if the Phase 2/3 trial shows positive results, the data could be considered pivotal data, considering, among other things, the applicable FDA guidance for the industry called Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products. Subject to regulatory allowance, we plan to conduct an additional Phase 2 study in 2015 aimed at Kennedy s disease, or SBMA. Mutant unstable cellular proteins are the cause of several genetic diseases known as PolyA/PolyQ, including OPMD, SBMA and SCA3. These pathological proteins aggregate within cells, eventually leading to cell death. Our data to date from preclinical studies from both cells and animal models, indicates that our Cabaletta platform has the potential to prevent mutant protein aggregation in humans.

mPRT is a mitochondrial protein replacement platform that is based on biological components that we synthesize in bacteria. Mitochondria are cell components that supply chemical energy for normal cell functioning. This platform is currently in preclinical development for two diseases: Friedrich s Ataxia and Ornithine Transcarbamylase Deficiency. These diseases are among over 100 genetic

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diseases that are caused by a missing or mutant protein that has critical role in the normal mitochondrial function. While lysosomal protein replacement platforms have been successful in replacing a missing protein in lysosomes, we believe that our mPRT platform is the first one to be successful in replacing mitochondrial proteins. Our product candidates are new fusion proteins that are a combination of the replaced protein fused with two additional sequences that facilitate its transport through biological membranes.

BBrm is our read-through platform based on a small repurposed molecule. The platform enables the read-through (or bypassing) of genetic defects called nonsense mutations or stop codons that interfere with normal protein formation and cause disease. Based on our preclinical data, we believe that this drug platform has the potential to treat six different diseases caused by nonsense mutation. Subject to regulatory approval, we plan to conduct Phase 2 clinical studies in late 2014 for our lead indication in Spinal Muscular Atrophy (SMA).

The following table summarizes our product candidate pipeline:

Product Candidates Overview

Cabaletta for the treatment of Oculopharyngeal Muscular Dystropy (OPMD)

Cabaletta is our proprietary intravenous (IV) solution of Trehalose for the treatment of OPMD. OPMD is an inherited myopathy, which is a muscle disease caused by a primary defect in muscle cells, characterized by dysphagia (difficulty in swallowing), the loss of muscular strength and weakness in multiple parts of the body. As the dysphagia becomes more severe, patients become malnourished, lose significant weight, become dehydrated and suffer from repeated incidents of aspiration pneumonia. These last two are often the cause of death.

Trehalose is naturally occurring and is well known for its protein-stabilizing properties. It is used in several biological systems, such as freeze drying of red blood cells and the preservation of organs for donation and as a protein stabilizer in IV pharmaceutical products. Trehalose is approved by the FDA as a GRAS (Generally Recognized As Safe) food ingredient and is listed in the U.S. National Formulary, which is a compendium of public pharmacopeial standards, which are directions for the identification of compound medicines, as well as in Europe and Japan. It is a disaccharide chemical chaperone, which is a chemical molecule comprised of two sugar components that stabilizes the folding of proteins and buffers abnormal protein aggregation, that protects against pathological processes in cells. It has been shown to prevent pathological aggregation of proteins within cells in several diseases associated with abnormal cellular-protein aggregation as well as acting as an autophagy enhancer. Autophagy is the basic catabolic mechanism that involves cell degradation of unnecessary or dysfunctional cellular components. Autophagy, if regulated,

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ensures the synthesis, degradation and recycling of cellular components. Trehalose has been documented as demonstrating significant efficacy in preclinical animal models of OPMD and other PolyA/PolyQ diseases.

Trehalose is not effective when given orally since it is almost completely metabolized in the small intestine into glucose and often causes diarrhea and flatulence after ingestion of more than 50g. We have developed a proprietary IV solution of Trehalose, which we call Cabaletta™, to circumvent the breakdown of Trehalose in the gastrointestinal tract, and to enable therapeutic doses of Trehalose to reach the muscles.

We are currently conducting a Phase 2/3 trial of Cabaletta to treat OPMD; if the results are positive, we believe this could be a pivotal trial. We believe, based on discussions with the FDA, that if the Phase 2/3 trial shows positive results, the data could be considered pivotal data, considering, among other things, the applicable FDA guidance for the industry called Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products.

The study is planned to include 40 60 patients in 3 centers: in Israel, Canada and the USA. All patients will be treated with 30g Trehalose IV weekly for the first 6 months. After 6 months, patients will be randomized into a treatment arm that will continue with the weekly IV 30g regimen and a non treatment control group that will be followed up under the same protocol for an additional 12 months. As of July 30, 2014, fourteen patients have been treated. If the trial is successful, we are planning to submit a new drug application, or NDA, to the FDA.

Cabaletta for the treatment of spinocerebellar ataxia type 3 (SCA3) (Machado Joseph disease)

SCA3, also known as Machado Joseph disease, is the most common disease among the cerebellar ataxias, which are a group of genetic diseases that are characterized by memory deficits, spasticity, difficulty with speech and swallowing, weakness in arms and other muscular disorders. Symptoms can begin in early adolescence and get worse over time. Eventually SCA3 leads to paralysis, and severe cases can lead to an early death in the fourth decade of life. SCA3 is incurable, and there is currently no approved treatment for the disease.

SCA3 is caused by a mutation in the DNA that leads to the creation of a pathological protein Ataxin 3. Ataxin 3 is unstable, meaning that it aggregates within the cells and eventually leads to cell death.

Multiple reported studies in cell models have shown that Trehalose, both as an anti-mutant protein aggregation agent and as an autophagy enhancer, is able to reduce protein aggregates and improve cell survival in several spinocerebellar ataxias including SCA3 cells. Additional animal studies show that activation of autophagy may be beneficial in alleviating disease symptoms. We recently started our Phase 2 clinical trial of Cabaletta to treat SCA3 in Israel.

Cabaletta for the treatment of Spino Bulbar Cerebellar Ataxia (SBMA *Kennedy s disease)*

SBMA, also known as Kennedy s disease, is characterized by the degeneration and loss of lower motor neurons in the brainstem and spinal cord. Patients present with weakness in muscle function, including severe difficulty in swallowing and suffer from repeated aspiration pneumonia, and other symptoms. SBMA is caused by an abnormal androgen receptor (AR) protein. Like other PolyA/PolyQ diseases, the abnormal protein is unstable and aggregates within cells, eventually leading to cell death. Patients suffering from SBMA suffer from progressive neuromuscular deterioration that can end up in extreme disability and repeated aspiration pneumonia. There is currently no approved therapy for SBMA.

Studies in cell models have shown that Trehalose in its capacity both as an anti-mutant protein aggregation and as an autophagy enhancer is able to reduce protein aggregates and improve cell survival in SBMA cells. Additional studies show that activation of autophagy may be beneficial in alleviating disease symptoms in animal models.

We have entered into an agreement with NINDS (a group within the U.S. National Institutes of Health, or NIH) pursuant to which NINDS will conduct animal studies on SBMA mouse models to validate the potential efficacy of Cabaletta. NINDS will conduct the study using its own resources, and we will supply the research material. If the animal studies are successful, and subject to regulatory allowance, we plan to commence a Phase 2/3 clinical trial.

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Mitochondrial protein replacement platform (mPRT)

Mitochondrial disorders are diseases, for which no cure has been found, that stem from a missing or mutated critical enzyme or protein in the cell organelle called mitochondria. Our fusion proteins, comprised of TAT-MTS(het)-replacement proteins, help move proteins across biological membranes to facilitate the creation of mature proteins inside the mitochondria. Our replacement proteins are able to enter human cells and mitochondria and replace the damaged or missing proteins in the mitochondria. Our breakthrough platform technology demonstrated efficacy in several mitochondrial protein deficiencies in cells and animal models. Currently, Frataxin for Friedrich s Ataxia (BB-FA) is our most advanced mPRT preclinical program, followed by Ornithine Transcarbamylase (BB-OTC) for Ornithine Transcarbamylase Deficiency (OTCD).

Our studies demonstrated the superiority of our proprietary approach of using a heterologous (none native) mitochondrial targeting sequence (MTS) as part of our fusion proteins in four different aspects: expression by bacteria, cell penetration, mitochondrial processing and anchorage of target proteins, and rescue of cells in oxidative stress.

We have an exclusive worldwide and royalty-bearing license to commercialize, develop and use the technology, including certain patent applications, from the Hebrew University in Jerusalem. These patent applications cover our unique approach of using homologous and heterologous MTS in the fusion proteins.

BB-FA for the treatment of Friedrich s Ataxia

Friedreich s Ataxia is an inherited disease characterized by progressive deterioration of muscles and nerves, resulting in gait disturbance (Ataxia), cognitive impairment, progressive heart disease and diabetes. Patients are usually diagnosed in the first or second decade of life, and are typically wheelchair-bound within 15 years of diagnosis. Most do not survive beyond the fourth decade of life. In many cases the cause of death is myopathic heart disease. The underlying causes of Friedrich s Ataxia are reduced levels of Frataxin a protein responsible for iron-sulphor clusters in the mitochondria that are critical for the mitochondria activity. Our preclinical data demonstrated successful placement of Frataxin into the mitochondria and in the treatment of oxidative stress in Friedreich s Ataxia patients cells. We are advancing the Friedreich s Ataxia program through its preclinical development throughout 2014.

BB-OTC for the treatment of ornithine transcarbamylase deficiency (OTCD)

OTCD is the most common disorder among urea cycle disorders a group of rare genetic diseases characterized by body s inability to detoxify ammonia. Ammonia is a toxic breakdown product of proteins. OTCD is caused by a mutated and ineffective form of the enzyme, ornithine transcarbamylase that is part of the urea cycle complex in the mitochondria. As a result, the normal breakdown of ammonia is disrupted and toxic ammonia accumulates in the blood causing severe damage to the brain and other vital organs. Newborn males affected with OTCD may suffer devastating hepatic coma in the first few days after birth, and survivors typically suffer from severe cognitive, mental and metabolic disorders and growth retardation. Many do not survive the first decade of life. We are using our mitochondrial protein replacement platform to replace this enzyme in the mitochondria. Our preclinical data indicates that our OTC fusion protein is well able to be transferred into the mitochondria and be processed in it. We are advancing the BB-OTC program through its preclinical development during 2014.

BBrm our read-through platform

BBrm is our family of small molecule non-glycosides repurposed drugs for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation (stop codon). The platform enables

the read-through (deactivation) of the genetic defects that stop synthesis of normal full length proteins. Deactivation of disease-causing nonsense mutation can alleviate the symptoms of genetic diseases caused by these mutations. Our BBrm family of repurposed drugs is able to induce translational readthrough, restoring full-length functional proteins in diseases where the nonsense mutation results in truncated ineffective proteins. We licensed the exclusive worldwide commercial rights for our BBrm technology for stop codon inhibition of orphan diseases from the Tel Aviv University in January 2014.

BBrm1 for the treatment of Spinal Muscular Atrophy (SMA)

Using our BBrm platform, we developed a unique therapeutic candidate for SMA. SMA is the leading genetic cause of infantile death and is caused by the loss of a functional Survival Motor Neuron 1 (SMN1).

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The disease is manifested by loss of muscle mass and mobility as well as severe compromise of vital functions such as respiration. Another protein called SMN2 is a nearly identical copy of SMN1, differentiated only by a silent, single-nucleotide mutation within the DNA. SMN2 partially compensates for the dysfunction of SMN1, however the small amount of the functional protein that is produced from the SMN2 gene is not able to fully compensate for the loss of SMN1. Prior independent studies proposed that read-through agents, such as aminoglycosides, can induce the read-through of the stop codon located in the SMN2- protein, thus elongating the SMN2 and creating a full length functional protein, that can compensate for the non-functioning SMN1 and alleviate the disease. This approach was also successfully tested in SMA animal models. Nonetheless, chronic administration of aminoglycosides was found to be associated with prohibitive toxicity.

Nevertheless, drug-induced read-through of premature stop codons remains a promising approach to elevate active protein expression from the SMN2 gene which is an ideal therapeutic target as it is found in all SMA patients. Our family of repurposed FDA-approved non-glycosides molecules (BBrm) induced significantly higher levels of full length functional SMN2 protein. These molecules are ineffective if administered orally or intravenously since they do not penetrate into the brain to create high enough therapeutic levels within nerve cells. Our BBrm1 product can be injected directly into the central nervous system (CNS), creating adequate drug concentration. This method of administration has the added benefit of further protection against off label use. We plan to continue our preclinical development of BBrm1 for SMA through 2014, and, if successful and subject to regulatory approval, expect to start a Phase 2 study late in 2014 or early in 2015.

Our current product candidate pipeline has been either in-licensed from academic institutions or developed internally. Where possible, our strategy is to acquire and retain global commercialization rights to our products to maximize long-term value. Over time, we intend to build our own commercial organization, which we believe will be of modest size due to the relatively small number of specialists who treat patients with rare and ultra-rare diseases.

The diseases which we are addressing have devastating consequences on the patient s health, quality of life and life expectancy. In addition these diseases create significant burdens on the patient s family and care takers as well as on the public health resources. In all the diseases we are addressing, patients cannot be offered an alternative therapy or the current solutions are inadequate in their abilities to change the course of the disease. We believe that prompt and efficient drug development can be of substantial benefit to the patients who are suffering from these incurable diseases.

We have assembled an experienced team, of employees, consultants, service providers and Board of Directors with extensive drug development and commercialization capabilities, particularly in the orphan drug area.

Our Strategy

Our strategy is to identify, acquire, license, develop, and commercialize novel products for the treatment of rare and ultra-rare diseases in the United States, the European Union, and select international markets, with the goal of becoming a leading rare disease biotechnology company. Our current product candidate pipeline has been either in-licensed from academic institutions or developed internally. Where possible, our strategy is to acquire, license and retain global commercialization rights to our products to maximize long-term value. Over time, we intend to build our own commercial organization, which we believe will be of modest size due to the relatively small number of specialists who treat patients with rare and ultra-rare diseases.

The patients we seek to treat have diseases with limited or no treatment options, and their lives and well-being are highly dependent upon our efforts to develop new therapies. We strive to build a company that is faster, better and

smarter about advancing multiple product candidates through approval.

The critical components of our business strategy include the following:

Focus on rare and ultra-rare diseases with significant unmet medical need. There are numerous rare and ultra-rare metabolic genetic diseases that currently have no approved drug therapy and for

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which no therapies, to our knowledge, are currently in development. Patients suffering from these diseases often have a high unmet medical need with significant morbidity and/or mortality. We are focused on developing and commercializing therapies for multiple such indications.

Focus on diseases and therapies with clear mechanisms of action. We also focus on diseases that have biology and root causes that are well understood. For example, several of our product candidates are replacement therapies for a single deficient enzyme or substrate in the body. We believe that developing drugs that directly impact known disease pathways will increase the probability of success of our development programs.

Leverage our experience and relationships to in-license promising product candidates. Our management and board has strong relationships with key opinion leaders in the metabolic genetic field, as well as a history of success in the development and commercialization of therapies for rare and ultra-rare genetic diseases. Accordingly, we enjoy excellent access to many in-licensing opportunities. Most of our current product candidates are in-licensed from academic institutions. We believe these parties have agreed to license product candidates to us because they are confident in our drug development capabilities and experience in bringing rare disease therapies to market.

Develop and commercialize multiple product candidates in parallel. Clinical studies for rare and ultra-rare diseases can often be smaller, fewer in number, and less expensive than those for larger market indications. Development of multiple programs in the metabolic genetics field also generates organizational efficiencies and economies of scale. As a result of these efficiencies, we can feasibly develop multiple clinical-stage product candidates in parallel, resulting in a more diversified portfolio that provides multiple opportunities to create value.

Focus on excellent and rapid clinical and regulatory execution. We believe that building a successful and sustainable rare disease-focused company requires very specific expertise in the areas of patient identification, clinical study design and conduct, and regulatory strategy. We have assembled a team with a successful track record in managing global clinical development activities in an efficient manner, and with multinational experience in obtaining regulatory approvals for rare disease products.

Seek to retain global commercialization rights to product candidates. We intend to seek and retain global commercialization rights to our product candidates whenever possible to maximize the potential value of our product portfolio. Our plan is to establish our own commercial organization in major pharmaceutical markets and develop a network of third-party distributors in smaller markets. We believe this commercial organization can be modest and targeted due to the relatively small number of specialists who typically treat patients with the diseases to be addressed by our product candidates. As a result, we do not expect that we will require pharmaceutical partners for commercialization of our product candidates, although we may consider partnering for certain territories or indications or for other strategic purposes.

Product Candidates Disease background, rationale for treatment, development plan and market potential

Oculopharyngeal Muscular Dystropy (OPMD)

Disease background:

OPMD is a rare inherited myopathy characterized by dysphagia (difficulty in swallowing), the loss of muscular strength and weakness in multiple parts of the body. Patients typically suffer from severe dysphagia, ptosis (eye lid drooping), tongue atrophy, lower limb proximal weakness, dysphonia (altered and weak voice), limitation in looking upward, facial muscle weakness and upper limb proximal weakness. The disease is most often diagnosed in the fifth-sixth decade of life and progresses throughout the patient s life. As the dysphagia becomes more severe, patients become malnourished, lose significant weight, become dehydrated and suffer from repeated incidents of aspiration pneumonia. These last two are often the cause of death.

OPMD is one of a larger group of diseases called tri-nucleotide repeat disease that are associated with the presence of an abnormal cellular protein, that aggregates in the cells eventually causing cell death. In

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OPMD the mutant protein PABPN1 was found to be correlated with disease severity in animal models and was identified within the typical cellular protein aggregates-the intranuclear inclusion body (INI) that are the diagnostic hallmark of the disease.

There is no medical treatment or, to our knowledge, potential cure for OPMD. Current therapeutic strategies are confined to surgical interventions that have limited efficacy and need to repeated often while the progressive loss of muscle s contractility continue relentlessly.

Rationale for treatment:

The naturally occurring disaccharide Trehalose is well known for its protein-stabilizing properties. It is used extensively in many applications as a stabilizer of frozen food, in freeze drying of biological systems and cells, as a stabilizer of therapeutic parenteral proteins and as an excipient in tablets and IV solutions. Trehalose has been shown to prevent pathological aggregation of proteins within cells in several diseases associated with mutant cellular-protein aggregation such as PolyA/PolyQ and taupathy diseases (for example OPMD, Huntington s disease, Spinocerebellar ataxia, Parkinson and Alzheimer disease and more). Recent studies demonstrated that Trehalose enhances autophagy a natural mechanism of debris-clearance within cells.

Trehalose was found to be effective in cell studies and in a mouse model of OPMD. In this disease there are characteristic intracellular aggregations of the abnormal protein PABPN1 (INI). Animal studies showed a direct correlation between reduction of mutant PABPN1 aggregates (INI) in cells and reduced cell death. Trehalose effectively reduced the aggregation and toxicity of mutant PABPN1 proteins in OPMD cell models. Furthermore, treatment of an OPMD mouse model with Trehalose resulted in the attenuation of muscle weakness, decreased aggregate formation and a reduced number of TUNEL-positive nuclei in skeletal muscle fibers.

Like all disaccharides, Trehalose is metabolized at the epithelial brush border of the intestine into two D-glucose molecules. Less than 0.5% of ingested Trehalose is absorbed into the blood stream where it is further metabolized by the liver and kidney. Oral Trehalose in amounts exceeding 40 50 g per day causes diarrhea, bloating and abdominal pain. To achieve therapeutic amounts of Trehalose in the muscle cells, it is necessary to circumvent the massive metabolism in the gastrointestinal tract. Cabaletta is our proprietary IV

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formulation of Trehalose developed for the treatment of diseases characterized by unstable mutant protein aggregates causing cellular damage and eventually cell death.

Trehalose was approved by the FDA, EMEA and several other authorities as a GRAS food ingredient and is also registered on the US National Formulary, as well as in Europe and Japan. Its safety in preclinical studies was extensively researched and validated.

Clinical development plan:

Our clinical plan will include eventually three centers that will look at the safety and efficacy of Trehalose in OPMD patients across centers in Israel and North America.

The study is planned to include 40 60 patients in 3 centers, in Israel, Canada and the USA. All patients will be treated with 30g Trehalose IV weekly for the first 6 months. After 6 months, patients will be randomized into a treatment arm that will continue with the weekly IV 30g regimen and a non treatment control group that will be followed up under the same protocol for an additional 12 months. As of July 30, 2014, fourteen patients have been treated. If the trial is successful, we are planning to submit a new drug application, or NDA, to the FDA. Endpoints in the trial include (i) penetration aspiration scale based on video fluoroscopy, (ii) muscle strength, and (iii) quality of life.

The market potential for OPMD:

The prevalence of OPMD is estimated at 1:100,000. The disease is more prevalent among people of French Canadian origin residing in Canada and in the U.S., and among Hispanics in New Mexico, Arizona and the other south western states of the U.S. and in Israel. The prevalence of OPMD among French Canadians in the Montreal, Canada area is 1:1,000. There are estimated 4,000 5,000 patients in Canada. In Israel, the prevalence of OPMD among Bukharean Jews is 1:600. There are estimated 1,200 1,500 patients in Israel. It has been reported to occur in 33 countries in the world. In the United States, the estimated number of patients is less than 6,000.

Spinocerebellar Ataxia type 3 (Machado Joseph disease)

Disease background:

Spinocerebellar ataxia Type 3 (SCA3), also known as Machado-Joseph disease, is a dominantly inherited ataxia, and is the most common disease among the cerebellar ataxias, also known as SCAs. SCA3 is characterized by memory deficits, clumsiness in movements in the arms and legs, unstable gait, difficulty with speech and swallowing, impaired eye movements that may be accompanied by double vision or bulging eyes, and lower limb spasticity. In most individuals with SCA3, symptoms typically begin in the third to fifth decade of life but can start as early as young childhood or as late as 70 years of age. The cause of death is often aspiration pneumonia.

SCA3 is a caused by a repeat expansion in the DNA code causing the creation of an abnormal and unstable cellular protein Ataxin 3. Typically the longer the expansion, the more severe the disease which may manifest earlier in life and exert a broader range of neurological symptoms. Cellular aggregations of Ataxin 3 inclusion bodies are found in SCA3 patients brain and nerve cells.

There is no medical treatment for SCA3 and current approaches are focused on alleviating disease symptoms and supportive care.

Rationale for treatment and development plan:

It has been established that enhancement of autophagy in SCA3 is an effective approach that reduces intracellular aggregates and increase cell survivals. Trehalose was found to be effective in SCA3 cells as a stabilizer of the mutant Ataxin 3 and as an enhancer of autophagy. Animal studies to further prove the concept are now ongoing. We recently started our Phase 2 clinical trial of Cabaletta to treat SCA3 in Israel. This randomized placebo controlled trial will treat patients for one year. We intend to enroll approximately ten to twelve patients.

Market potential:

The prevalence of SCA3 is estimated as 1 2 cases per 100,000 people. The prevalence of the disease is highest among people of Portuguese/Azorean descent. For example, among immigrants of Portuguese ancestry

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in New England, the prevalence is around one in 4,000, and the highest prevalence in the world, about one in 140, occurs on the small Azorean island of Flores. There are an estimated 4,000 to 6,000 patients in the United States, and an estimated 2,000 patients in each of Portugal and Brazil. SCA3 has been reported in over a dozen European countries with a few effected families mentioned in each of the reported case studies.

Spino Bulbar muscular atrophy (SBMA)

Disease background:

SBMA, also known as Kennedy s disease, is a rare inherited X linked disease, characterized by the degeneration and loss of lower motor neurons in the brainstem and spinal cord resulting in progressive muscle weakness, atrophy, and fasciculation. The disease is typically manifested in the fourth or fifth decade of life. Affected patients also have signs of androgen insensitivity such as gynecomastia (large male breasts), reduced fertility, and testicular atrophy. As the disease progresses, disability is increased until the patient is wheelchair bound. Involvement of the bulbar musculature may be expressed as difficulty in chewing, swallowing, and speaking. Postural tremor and tremor in the upper extremities usually begins late in the course of the illness. Patient may also suffer from sensory disorders as well as symptoms related to endocrinopathy, particularly diabetes.

Rationale for treatment and development plan:

SBMA is caused by an expansion of the CAG trinucleotide repeat in exon 1 of the human androgen receptor (*AR*) gene. A disease causing protein a mutant AR have been identified in patient s cells, where it aggregates both in the cytoplasm and in the nucleus. Several studies demonstrated the ability of Trehalose as a protein stabilizer and an autophagy enhancer to protect SBMA cells from the toxic effects of the mutant AR protein. We are now conducting an a study in animal models, in collaboration with the NIH. We plan to continue our preclinical development and if successful, expect to be in Phase II study in 2015 in the United States.

Market potential:

The estimated incidence of SBMA in the U.S. is approximately 1 case in 40,000 men, or approximately 3,775 patients. There is a general impression that SBMA may be under-diagnosed, owing in part to misdiagnosis and to the mild symptoms exhibited by some patients. The estimated prevalence in the rest of the world (Europe, Japan, Australia) is similar. Some regions, such as western Finland and Japan, may have a higher prevalence. There are an estimated 10,000 patients in Europe, 1,500 patients in Japan and 300 patients in Australia.

Friedrich s Ataxia:

Disease background:

Friedreich s ataxia is a rare inherited disease that causes muscle and nerve damage. It usually begins in childhood and is caused by progressive degeneration of the spinal cord and peripheral nerves. The cerebellum the part of the brain that coordinates balance and movement, also degenerates. This damage results in clumsy, unsteady movements and gait and impaired sensory functions. The disease also causes severe heart disease, spinal deformity and diabetes. Typically patients are diagnosed within the first two decades of life and may become wheelchair bound within 15 years of diagnosis. Life expectancy is severely shortened and most patients do not live beyond the fourth decade of life.

Friedreich s ataxia is caused by a defect (mutation) in a gene labeled FXN. As a result a protein called Frataxin is mutated and dysfunctional. Fratxin plays a pivotal role in the normal mitochondrial metabolism: Without a normal level of Frataxin, certain cells in the body (especially peripheral nerve, spinal cord, brain and heart muscle cells) cannot effectively produce energy, and buildup toxic byproducts leading to what is called oxidative stress. Frataxin deficiency may also lead to increased levels of iron in the mitochondria. When the excess iron reacts with oxygen, free radicals can be produced. Although free radicals are essential molecules in the body s metabolism, they can also destroy cells and harm the body.

Rationale for treatment and development plan:

Unlike successful lysosomal Enzyme-Protein Replacement Therapy (ERT)- mitochondrial ERT has yet to be established. We have developed a successful mitochondrial PRT platform based on a recombinant protein

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containing targeting signals (TAT-MTS-protein). Our novel platform was the first proven approach towards correction of mitochondrial protein deficiency diseases. Based on our approach, PRT therapy has been studied in FA mouse model with some success, using the native mitochondrial targeting sequence (MTS) of Frataxin (MTSfa). We have made a scientific leap by replacing the native MTS with a heterologous MTS (one that is native to another protein). Our approach was tested in preclinical studies and was found to be highly superior in four major parameters: The expression of the fusion protein by bacterial cells, the penetration of the protein into FA patient s cells, the breakout of the fusion protein into its components leaving the Frataxin protein in the mitochondria, and finally, the correction of the pathological oxidative stress of the cells.

Better expression by bacteria

Protein	Final concentration (mg)	Amount purified from 0.5 bacterial culture (mg)
TAT-MTSfra-FRA	0.2	0.6 - 0.7
TAT-MTScs-FRA	1.0	3 - 4
TAT-MTSorf-FRA	1.0	3 - 4
TAT-MTSlad-FRA	0.8	2.4 - 3.2

Higher efficiency in cell rescue

We continue our preclinical development of our BB-FA candidate and will advance into full preclinical regulatory route later in 2014. We expect to start clinical studies late in 2015 in the United States and Europe.

Market potential for BB-FA:

Although rare, Friedreich s ataxia is the most common form of hereditary ataxia, affecting about 1 in every 50,000 people in the United States. It is estimated that the number of Friedreich s ataxia patients in the U.S. is about 6,000, with approximately 15,000 patients worldwide.

Ornithine transcarbamylase deficiency

Disease background:

Ornithine transcarbamylase deficiency (OTCD) is the most common urea cycle disorder, a group of rare genetic metabolic disorders characterized by the body s inability to detoxify ammonia. OTCD is caused by a mutated and ineffective form of the enzyme ornithine transcarbamylase (OTC).

OTC is one of the critical enzymes that participate in the Urea cycle a metabolic process that helps the body get rid of ammonia, the toxic breakdown product of proteins. As a result, the normal breakdown of ammonia is disrupted and toxic ammonia accumulates in the blood causing severe damage to the brain and other vital organs, especially in the highly vulnerable nervous system.

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Ornithine transcarbamylase deficiency often becomes evident in the first few days of life, however it can present at middle age. The typical initial symptoms of a child with hyperammonemia are failure to feed, loss of thermoregulation with a low core temperature, and somnolence. Symptoms progress from somnolence to lethargy and coma. Abnormal posturing and encephalopathy are often related to the degree of central nervous system swelling and pressure upon the brain stem. In cases where OTC enzyme production is low or non-existent, death can occur within the first days of life. Complications from ornithine transcarbamylase deficiency may include developmental delay and mental retardation. Progressive liver damage, skin lesions, and brittle hair may also be seen. Other symptoms include irrational behavior (caused by encephalitis), mood swings, and poor performance in school. In milder (or partial) urea cycle enzyme deficiencies, ammonia accumulation may be triggered by illness or stress at almost any time of life, resulting in multiple mild elevations of plasma ammonia concentration.

Rationale for treatment and development plan:

Loss of OTC blocks the normal metabolism of ammonia into the less toxic urea that can be cleared from the blood by the kidneys. Replacement of the missing or mutated protein can restore the normal function of the urea cycle and prevent the toxic accumulation of ammonia. Based on our PRT platform, we have developed a fusion protein that is comprised of TAT-MTS (het)-OTC. We are developing it towards proof of concept in human cells and in animal models. We continue our preclinical development and expect to start a full preclinical regulatory phase in mid 2014. We expect to start Phase I study late in 2015 in the United States and Europe.

Market potential for BB-OTC:

The incidence of OTCD is 1 in 70,000, with approximately 4,000 patients in the United States, of which 2,000 males suffer from the severe lethal form. This incidence may be an underestimation due to under diagnosis. In some affected individuals, signs and symptoms of ornithine transcarbamylase may be less severe, and may not appear until later in life. Some female carriers become symptomatic later in life in times of metabolic stress. Despite milder presentations in adulthood, hyperammonemia, encephalopathy, cerebral edema, and death can occur. On February 1, 2013, the FDA approved RAVICTI® (glycerol phenylbutyrate) Oral Liquid for use as a nitrogen-binding agent for chronic management of adult and pediatric patients 2 years

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of age or older with urea cycle disorders (UCDs) who cannot be managed by dietary protein restriction and/or amino acid supplementation alone. In clinical studies, RAVICTI was shown to be noninferior to sodium phenylbutyrate, and kept ammonia at safe levels throughout the day and night and over the long-term (1 year). Despite RAVCITI s effectiveness in lowering blood ammonia levels it has some significant limitations in that it is not approved for patients suffering from liver or kidney disease and many OTCD patients have some disturbance in liver or kidney functions. In addition, over 10% of RAVCITI users suffer from diarrhea, bloating, and abdominal pain. There exists a place for a therapy that will prevent the accumulation of ammonia in the first place.

Spinal Muscular atrophy

Disease background:

Spinal Muscular Atrophy (SMA) is a neuromuscular disorder for which there is no available therapy. SMA is the number one autosomal recessive cause of infantile death. SMA is caused by loss or mutation of the survival motor neuron 1 gene, SMN1, while the nearly identical copy gene, SMN2 is retained. In contrast to SMN1, most SMN2 transcripts lack exon 7. This alternatively spliced transcript, |gD7-SMN, translates into a prematurely terminated (truncated) protein that is rapidly degraded. It has been established that read-through of the stop codon in exon 8 of the |gD7-SMN2 protein increases the amount of full-length SMN2 a protein that can significantly attenuate disease severity in SMA patients.

Rational for therapy and development plan:

Drug-induced read-through of the premature termination codon enables full-length translation of SMN, thus serving as an ideal therapeutic target as it is found in all SMA patients. This approach was successfully tested in SMA cells and animal models using a known family of antibiotics called aminoglycosides. Unfortunately, chronic administration of aminoglycosides is impossible due to their well-known prohibitive toxicity.

Our family of FDA-approved molecules (BBrm) are non-glycosides that were found to be highly superior to aminoglycosides in enabling read-through and enabling the expression of full length functional SMN2 and rescue of cells.

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We continue our preclinical development of our BBrm program and expect to start Phase II clinical study by the end of 2014 or beginning of 2015 in Israel to be expanded to the United States and Europe in 2015.

Market potential for SMA:

SMA has a carrier frequency of one in every 35 people, affecting every one in 6,000 live births. It is estimated that some 30,000 children suffer from SMA in the U.S. and in similar numbers in Europe.

There are several drugs in development for SMA; however, there is no approved therapy for this disease.

Competition

The commercialization of new drugs is competitive, and we may face worldwide competition from individual investigators, major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, nutraceutical companies, and ultimately biosimilar and generic companies. Our competitors may develop or market therapies that are more effective, safer, or less costly than any that may be commercialized by us, or may obtain regulatory approval for their therapies more rapidly than we may obtain approval for ours.

The acquisition or licensing of pharmaceutical products is also very competitive, and a number of more established companies, which have acknowledged strategies to license or acquire products, may have competitive advantages as may other emerging companies taking similar or different approaches to product acquisitions. These established companies may have a competitive advantage over us due to their size, cash flows, and institutional experience.

With respect to our programs in Cabaletta for OPMD, although we are not aware of any other products currently in clinical development for the treatment of OPMD, it is possible that competitors may produce, develop, and commercialize therapeutics, or utilize other approaches such as gene therapy, to treat OPMD. An academic based study testing the use of injected myoblasts has completed Phase 2 study.

With respect to our programs in Cabaletta for SCA3, although we are not aware of any other products currently in clinical development for the treatment of SCA3, it is possible that competitors may produce, develop, and commercialize therapeutics, or utilize other approaches such as gene therapy, to treat SCA3. Note that in the last few years several academic researches where conducted to explore the efficacy of approved drugs such as Lithium, Varenicline (Chantix), riluzol, Dalfampridine.

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With respect to our programs in Cabaletta for SBMA, Novartis is planning to conduct a Phase 2 study for its insulin resistance drug candidate BVS857 in SBMA patients. This will be a two year study that will look at the size of patients muscle as an endpoint. Although we are not aware of any other products currently in clinical development for the treatment of SBMA, it is possible that competitors may produce, develop, and commercialize therapeutics, or utilize other approaches such as gene therapy to treat SBMA.

Although we believe that Cabaletta should be considered a drug and that only insignificant amounts of Trehalose can be absorbed through an oral administration it is possible that other companies or individuals may attempt to use food grade Trehalose as a substitute, and others may attempt to sell the product via a nutraceutical or food pathway. We believe that following the approval of our patent applications, if approved, we will be well protected in our intellectual property from the use of Trehalose as an IV product.

With respect to our programs in protein replacement platform although we are not aware of any other similar products currently in clinical development for the treatment of Friedrich s Ataxia, there are a number of competitors trying to develop and commercialize therapeutics, or utilize other approaches such as mitochondria protecting agents, anti-oxidants, HDAC inhibitors, cell therapy or gene therapy, to treat Friedrich s Ataxia. The table below describes, to our knowledge, the current therapeutic approaches in different stages of development. To our knowledge, none of these product candidates have received regulatory approval in the U.S. or Europe.

With respect to the table above, BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced on February 5, 2014, that it had acquired Repligen Corporation s histone deacetylase inhibitor (HDACi) library and related intellectual property. Potential applications of the HDACi portfolio include Friedreich s ataxia and other neurological disorders.

With respect to our programs in protein replacement platform although we are not aware of any other similar products currently in clinical development for the treatment of Ornithine transcarbamylase deficiency,

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there are a number of clinical approaches currently used to treat OTCD patients in emergency situation: Intravenous administration of sodium benzoate, arginine, and sodium phenylacetate might be beneficial for patients in hepatic coma; however, they can only be administered in a large medical facility setting with close laboratory monitoring available. Intravenous sodium benzoate and phenylacetate (Ammonul) was approved in the United States in February 2005.

Glycerol phenylbutyrate (Ravicti) is a pre-prodrug that undergoes metabolism to form phenylacetate. Results of a Phase III study comparing ammonia control in adults showed glycerol phenylbutyrate was non-inferior to sodium phenylbutyrate. In a separate study involving young children ages 2 months through 5 years, glycerol phenylbutyrate resulted in a more evenly distributed urinary output of PAGN over 24 hours and accounted for fewer symptoms from accumulation of phenylacetate. Ravicti was approved by the FDA early in 2013 (Hyperion).

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With respect to our program in BBrm- read through platform there are a number of competitors developing read through molecules, both small molecules (PTC) and exon- skipping RNA based platform (ISIS, Prosensa, Sarepta). Although not all have focused so far on SMA it is possible that they will develop in the future programs that will attempt to utilize their technology for SMA. In addition it is possible that competitors may produce, develop, and commercialize therapeutics, or utilize other approaches such as gene therapy, cell therapy or bone marrow transplantation to treat SCA3. The table below outlines, to our knowledge, the different approaches for developing drugs for SMA.

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We believe that our read-through platform could be synergistic to most technologies outlined above since most are focused on either generally enhancing the total production of SMN2 through exon skipping, or focused on the downstream effects of lack of SMN1. Many of our competitors have substantially greater financial, technical, and human resources than we have. Additional mergers and acquisitions in the pharmaceutical industry may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances made in the commercial applicability of technologies and greater availability of capital for investment in these fields. Our success will be based in part on our ability to build and actively manage a portfolio of drugs that addresses unmet medical needs and creates value in patient therapy.

License Agreements

We have entered into two license agreements with respect to two of our three platform technologies. On December 19, 2011, we entered into a Research and Exclusive License Agreement with Yissum Research Development Company of the Hebrew University in Jerusalem Ltd., whereby we licensed exclusively two patent applications covering our mitochondrial protein replacement platform. One patent application covers the use of TAT-MTS-Protein for protein replacement in mitochondrial diseases. The second patent application covers the use of heterologous MTS in a fusion protein for treatment of mitochondrial protein deficiency diseases. These patent applications, if issued, will expire in 2029 and 2033, respectively. In addition, on January 1, 2014 we entered into an Exclusive License Agreement with Ramot at Tel Aviv University Ltd. for the use, development and commercialization of our-read through platform.

Intellectual Property and Patents and Proprietary Rights

The proprietary nature of, and protection for, our product candidates, processes, and know-how are important to our business. Our success depends in part on our ability to protect the proprietary nature of our product candidates, technology, and know-how, to operate without infringing on the proprietary rights of others, and to prevent others from infringing our proprietary rights. We seek patent protection in the United States and internationally for our product candidates and other technology. Our policy is to patent or in-license the technology, inventions and improvements that we consider important to the development of our business. In addition to patent protection, we intend to use other means to protect our proprietary rights, including pursuing marketing or data exclusivity periods, orphan drug status, and similar rights that are available under regulatory provisions in certain countries, including the United States, Europe, Japan, and China. See U.S. Government Regulation Orphan Designation and Exclusivity, U.S. Government Regulation Pediatric Studies and Exclusivity, U.S. Government Regulation Patent Term Restoration, U.S. Government Regulation Biosimilars and Exclusivity, U.S. Government Regulation Abbreviated New Drug Applications for Generic Drugs, U.S. Government Regulation Hatch-Waxman Patent Certification and the 30-Month Stay, and European Union/Rest of World Government Regulation Orphan Designation and Exclusivity below for additional information.

We also rely on trade secrets, know-how, and continuing innovation to develop and maintain our competitive position. We cannot be certain that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents granted to us in the future will be commercially useful in protecting our technology.

We seek regulatory approval for our products in disease areas with high unmet medical need, great market potential, and where we have a proprietary position through patents covering various aspects of our products, such as composition, dosage, formulation, use, and manufacturing process, among others. Our success depends on an intellectual property portfolio that supports our future revenue streams and erects barriers to our competitors. We are maintaining and building our patent portfolio through filing new patent applications, prosecuting existing applications,

and licensing and acquiring new patents and patent applications.

Despite these measures, any of our intellectual property and proprietary rights could be challenged, invalidated, circumvented, infringed or misappropriated, or such intellectual property and proprietary rights may not be sufficient to permit us to take advantage of current market trends or otherwise to provide competitive advantages. For more information, please see Risks Related to our Intellectual Property.

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As of July 30, 2014, we own and/or are the exclusive licensee of five pending U.S. patent applications and corresponding patents and patent applications internationally. With respect to any patents that may issue in the United States and Europe, we may also be entitled to obtain a patent term extension to extend the patent expiration date. For example, in the United States, we can apply for a patent term extension of up to five years for one of the patents covering a product once the product is approved by the FDA. The exact duration of the extension depends on the time we spend in clinical studies as well as getting a new drug application approval from the FDA. The patent portfolios for our three leading platform and product candidates as of July 30, 2014 are summarized below.

Cabaletta

We have filed two patent applications that relate to the use of Trehalose for the treatment of PolyA/PolyQ diseases and Taupathies. The patent applications are directed to a novel therapeutic regime using parenteral administration of Trehalose, thereby achieving higher bioavailability and therapeutic efficacy in the treatment of myopathic and neurodegenerative diseases associated with abnormal protein aggregation, specifically polyalanine or polyglutamine expansion protein and tauopathies disorders such as OPMD, SCA, SBMA, Huntington and more.

The expiring patent terms for such pending patent applications in the United States would be 2034. We intend to pursue marketing and orphan drug exclusivity periods that are available to us under regulatory provisions in certain countries.

In addition we have received orphan drug designation for the use of Trehalose in OPMD patients.

mPRT

We have filed two patent applications and have licensed exclusively two patent applications from the Hebrew University in Israel that relate to our mitochondrial protein replacement platform. The two licensed patent applications are directed to the use of TAT-MTS-Protein for protein replacement in mitochondrial diseases (See License Agreements). The other two patent applications filed by us are directed to the use of heterologous MTS in a fusion protein for treatment of mitochondrial protein deficiency diseases. These patent applications, if issued, will expire in 2029 and 2033 respectively.

Read-through

We are the exclusive licensee of one PCS and one U.S. patent application covering our-read through platform that we licensed from Tel Aviv University (See License Agreements section above). We also filed one U.S. patent application that relates to our read-through platform. The licensed PCT patent application and the patent application that we filed are directed to a method of treatment for orphan genetic neurodegenerative and neurodevelopmental diseases through non-systemic administration and specific injectable formulations.

The expiration dates on these patents are 2027 and 2033 respectively.

Trademarks

We have filed with the USPTO an intent to use application for the trademark CABALETTA in International Class 5 for certain pharmaceutical preparations.

Other

We rely upon unpatented trade secrets, know-how, and continuing technological innovation to develop and maintain our competitive position. We seek to protect our ownership of know-how and trade secrets through an active program of legal mechanism including assignments, confidentiality agreements, material transfer agreements, research collaborations, and licenses.

Manufacturing

We currently contract with third parties for the manufacturing and testing of our product candidates for preclinical studies and clinical studies and intend to do so in the future. We do not own or operate manufacturing facilities for the production of clinical quantities of our product candidates. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. The use of contracted

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manufacturing and reliance on collaboration partners is relatively cost-efficient and has eliminated the need for our direct investment in manufacturing facilities and additional staff early in development. Although we rely on contract manufacturers, we have personnel with extensive manufacturing experience to oversee our contract manufacturers.

To date, our third-party manufacturers have met our manufacturing requirements. We expect third-party manufacturers to be capable of providing sufficient quantities of our product candidates to meet anticipated full scale commercial demands. To meet our projected needs for commercial manufacturing, third parties with whom we currently work might need to increase their scale of production or we will need to secure alternate suppliers. We believe that there are alternate sources of supply that can satisfy our clinical and commercial requirements, although we cannot be certain that identifying and establishing relationships with such sources, if necessary, would not result in significant delay or material additional costs.

The drug substance for Cabaletta is purchased from a third party supplier and drug product for Cabaletta is manufactured by a third party manufacturer. There are other suppliers for pharmaceutical grade Trehalose in the market. We have not yet started the development of our clinical trial material for our pre-clinical programs in mPRT and Read through platform.

Sales and Marketing

We intend to build the commercial infrastructure in the United States and Europe necessary to effectively support the commercialization of all of our product candidates, if and when we believe a regulatory approval of the first of such product candidates in a particular geographic market appears imminent. The commercial infrastructure for orphan products typically consists of a targeted, specialty sales force that calls on a limited and focused group of physicians supported by sales management, medical liaisons, internal sales support, an internal marketing group, and distribution support. One challenge unique to commercializing therapies for rare diseases is the difficulty in identifying eligible patients due to the very small and sometimes heterogeneous disease populations. Our management team is experienced in maximizing patient identification for both clinical development and commercialization purposes in rare diseases.

Additional capabilities important to the orphan marketplace include the management of key accounts such as managed care organizations, group-purchasing organizations, specialty pharmacies, and government accounts. To develop the appropriate commercial infrastructure, we will have to invest significant amounts of financial and management resources, some of which will be committed prior to any confirmation that any of our product candidates will be approved.

Outside of the United States and Europe, where appropriate, we may elect in the future to utilize strategic partners, distributors, or contract sales forces to assist in the commercialization of our products. In certain instances we may consider building our own commercial infrastructure.

Government Regulation

Clinical trials, the drug approval process, and the marketing of drugs are intensively regulated in the United States and in all major foreign countries. Government authorities in the United States (including federal, state, and local authorities) and in other countries, extensively regulate, among other things, the manufacturing, research and clinical development, marketing, labeling and packaging, storage, distribution, post-approval monitoring and reporting, advertising and promotion, pricing, and export and import of pharmaceutical products, such as those we are developing. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal,

state, local, and foreign statutes and regulations require the expenditure of substantial time and financial resources.

U.S. Government Regulation

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and related regulations, and biologics under the FDCA and the Public Health Service Act, or PHSA, and its implementing regulations. FDA approval is required before any new unapproved drug or dosage form, including a new use of a previously approved drug, can be marketed in the United States. Drugs and biologics are also subject to other federal, state and local statutes and regulations. Failure to comply with the applicable United States regulatory requirements at any time during the product development process, approval process

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or after approval may subject an applicant to administrative or judicial sanctions. These sanctions could include the imposition by the FDA or an Institutional Review Board, or IRB, of a clinical hold on trials, the FDA s refusal to approve pending applications or supplements, license suspension or revocation, withdrawal of an approval, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution. Any agency or judicial enforcement action could have a material adverse effect on us.

The FDA and comparable regulatory agencies in state and local jurisdictions and in foreign countries impose substantial requirements upon the clinical development, manufacture and marketing of pharmaceutical products. These agencies and other federal, state and local entities regulate research and development activities and the testing, manufacture, quality control, safety, effectiveness, labeling, storage, distribution, record keeping, approval, advertising and promotion of our products.

The FDA s policies may change and additional government regulations may be enacted that could prevent or delay regulatory approval of our platforms and candidate products or any future product candidates or approval of new disease indications or label changes. We cannot predict the likelihood, nature or extent of adverse governmental regulation that might arise from future legislative or administrative action, either in the United States or abroad.

Marketing Approval

The process required by the FDA before product candidates may be marketed in the United States generally involves the following:

completion of extensive preclinical laboratory tests and preclinical animal studies, all performed in accordance with GLP regulations;
submission to the FDA of an IND which must become effective before human clinical studies may begin and must be updated annually;
approval by an IRB or ethics committee representing each clinical site before each clinical study may be initiated;
performance of adequate and well-controlled human clinical studies to establish the safety and efficacy of the product candidate for each proposed indication;
preparation of and submission to the FDA of an NDA or BLA after completion of all pivotal clinical studies;
potential review of the product application by an FDA advisory committee, where appropriate and if applicable;
a determination by the FDA within 60 days of its receipt of an NDA or BLA to file the application for review;
satisfactory completion of an FDA pre-approval inspection of the manufacturing facilities where the proposed product drug substance is produced to assess compliance with cGMP; and
FDA review and approval of an NDA or BLA prior to any commercial marketing or sale of the drug in the United States.

The testing and approval process requires substantial time and financial resources, and we cannot be certain that any approvals for our candidate products will be granted on a timely basis, if at all.

An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for human studies. The IND also includes results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational new drug. An IND must become effective before human clinical trials may begin. An IND will automatically become effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to the proposed clinical studies. In such a case,

the IND may be placed on

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clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before clinical studies can begin. Accordingly, submission of an IND may or may not result in the FDA allowing clinical studies to commence.

We will need to successfully complete extensive additional clinical trials in order to be in a position to submit a new drug application to the FDA. Our planned future clinical trials for our candidate products may not begin or be completed on schedule, if at all. Clinical trials can be delayed for a variety of reasons, including delays in:

obtaining regulatory approval to commence a study;
reaching agreement with third-party clinical trial sites and their subsequent performance in conducting accurate and reliable studies on a timely basis;
obtaining institutional review board approval to conduct a study at a prospective site;
recruiting patients to participate in a study; and
supply of the drug.

We must reach agreement with the FDA on the proposed protocols for our future clinical trials in the United States. A separate submission apart from any IND application we submit must be made for each successive clinical trial to be conducted during product development. Further, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial before it commences at that site. Informed consent must also be obtained from each study subject. Regulatory authorities, an IRB, a data safety monitoring board or the sponsor, may suspend or terminate a clinical trial at any time on various grounds, including a finding that the participants are being exposed to an unacceptable health risk.

Clinical Studies

Clinical studies involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with current cGCPs which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical studies are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety, and the efficacy criteria to be evaluated. A protocol for each clinical study and any subsequent protocol amendments must be submitted to the FDA as part of the IND. Additionally, approval must also be obtained from each clinical study site s institutional review board, or IRB, before the studies may be initiated, and the IRB must monitor the study until completed. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

Our objective is to conduct additional clinical trials for our candidate products and, if those trials are successful, seek marketing approval from the FDA and other worldwide regulatory bodies.

For purposes of NDA approval, human clinical trials are typically conducted in phases that may overlap.

Phase I. The drug is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
Phase II. This phase involves trials in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
Phase III. This phase involves trials undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population, often at geographically dispersed clinical trial sites. These trials are intended to establish

the overall risk/benefit ratio of the product and provide an adequate basis for product labeling.

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Phase IV. In some cases, the FDA may condition approval of an NDA or BLA for a product candidate on the sponsor s agreement to conduct additional clinical studies after approval. In other cases, a sponsor may voluntarily conduct additional clinical studies after approval to gain more information about the drug. Such post-approval studies are typically referred to as Phase IV clinical studies.
A pivotal study is a clinical study that adequately meets regulatory agency requirements for the evaluation of a drug candidate s efficacy and safety such that it can be used to justify the approval of the product. Generally, pivotal studies are Phase 3 studies, but the FDA may accept results from Phase 2 studies if the study design provides a well-controlled and reliable assessment of clinical benefit, particularly in situations where there is an unmet medical need and the results are sufficiently robust.

The FDA, the IRB, or the clinical study sponsor may suspend or terminate a clinical study at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk.

Additionally, some clinical studies are overseen by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study. We may also suspend or terminate a clinical study based on evolving business objectives and/or competitive climate.

All of these trials must be conducted in accordance with good clinical practice requirements in order for the data to be considered reliable for regulatory purposes.

The clinical study process can take three to ten years or more to complete, and there can be no assurance that the data collected will support FDA approval or licensure of the product. Government regulation may delay or prevent marketing of product candidates or new drugs for a considerable period of time and impose costly procedures upon our activities. We cannot be certain that the FDA or any other regulatory agency will grant approvals for our candidate products or any future product candidates on a timely basis, if at all. Success in early stage clinical trials does not ensure success in later stage clinical trials. Data obtained from clinical activities is not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval.

The NDA Approval Process

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, detailed investigational new drug product information is submitted to the FDA in the form of an NDA or BLA requesting approval to market the product for one or more indications. Under federal law, the submission of most NDAs and BLAs is subject to an application user fee. For fiscal year 2014, the application user fee exceeds $2.1 million, and the sponsor of an approved NDA or BLA is also subject to annual product and establishment user fees, set at $104,060 per product and $554,600 per establishment. These fees are typically increased annually. Applications for orphan drug products are exempted from the NDA and BLA user fees and may be exempted from product and establishment user fees, unless the application includes an indication for other than a rare disease or condition.

An NDA or BLA must include all relevant data available from pertinent preclinical and clinical studies, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product s chemistry, manufacturing, controls, and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational new drug product to the satisfaction of the FDA.

The FDA will initially review the NDA for completeness before it accepts it for filing. The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency s threshold determination that the application is sufficiently complete to permit substantive review. After the NDA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being

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manufactured in accordance with cGMP to assure and preserve the product s identity, strength, quality and purity. The FDA may refer applications for novel drug products or drug products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and, if so, under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Based on pivotal Phase III trial results submitted in an NDA, upon the request of an applicant, the FDA may grant a priority review designation to a product, which sets the target date for FDA action on the application at six months, rather than the standard ten months. Priority review is given where preliminary estimates indicate that a product, if approved, has the potential to provide a significant improvement compared to marketed products or offers a therapy where no satisfactory alternative therapy exists. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

After the FDA completes its initial review of an NDA, it will communicate to the sponsor that the drug will either be approved, or it will issue a complete response letter to communicate that the NDA will not be approved in its current form and inform the sponsor of changes that must be made or additional clinical, nonclinical or manufacturing data that must be received before the application can be approved, with no implication regarding the ultimate approvability of the application.

Before approving an NDA or BLA, the FDA will typically inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications.

Additionally, before approving an NDA, the FDA may inspect one or more clinical sites to assure compliance with GCPs. If the FDA determines the application, manufacturing process or manufacturing facilities are not acceptable, it typically will outline the deficiencies and often will request additional testing or information. This may significantly delay further review of the application. If the FDA finds that a clinical site did not conduct the clinical trial in accordance with GCP, the FDA may determine the data generated by the clinical site should be excluded from the primary efficacy analyses provided in the NDA. Additionally, notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The testing and approval process for a drug requires substantial time, effort and financial resources, and this process may take several years to complete. Data obtained from clinical activities are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The FDA may not grant approval on a timely basis, or at all. We may encounter difficulties or unanticipated costs in our efforts to secure necessary governmental approvals, which could delay or preclude us from marketing our products.

The FDA may require, or companies may pursue, additional clinical trials after a product is approved. These so-called Phase IV studies may be made a condition to be satisfied for continuing drug approval. The results of Phase IV studies can confirm the effectiveness of a product candidate and can provide important safety information. In addition, the FDA now has express statutory authority to require sponsors to conduct post-market studies to specifically address safety issues identified by the agency.

Any approvals that we may ultimately receive could be withdrawn if required post-marketing trials or analyses do not meet the FDA requirements, which could materially harm the commercial prospects for our candidate products.

The FDA also has authority to require a REMS from manufacturers to ensure that the benefits of a drug or biological product outweigh its risks. A sponsor may also voluntarily propose a REMS as part of the NDA submission. The need for a REMS is determined as part of the review of the NDA. Based on statutory standards, elements of a REMS may include dear doctor letters, a medication guide, more elaborate targeted educational programs, and in some cases restrictions on distribution. These elements are negotiated as

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part of the NDA approval, and in some cases if consensus is not obtained until after the Prescription Drug User Fee Act review cycle, the approval date may be delayed. Once adopted, REMS are subject to periodic assessment and modification.

Even if a product candidate receives regulatory approval, the approval may be limited to specific disease states, patient populations and dosages, or might contain significant limitations on use in the form of warnings, precautions or contraindications, or in the form of onerous risk management plans, restrictions on distribution, or post-marketing study requirements. Further, even after regulatory approval is obtained, later discovery of previously unknown problems with a product may result in restrictions on the product or even complete withdrawal of the product from the market. Delay in obtaining, or failure to obtain, regulatory approval for our candidate products, or obtaining approval but for significantly limited use, would harm our business. In addition, we cannot predict what adverse governmental regulations may arise from future U.S. or foreign governmental action.

Expedited Review and Accelerated Approval Programs

A sponsor may seek approval of its product candidate under programs designed to accelerate the FDA s review and approval of NDAs and BLAs. For example, Fast Track Designation may be granted to a drug intended for treatment of a serious or life-threatening disease or condition that has potential to address unmet medical needs for the disease or condition. The key benefits of fast track designation are the eligibility for priority review, rolling review (submission of portions of an application before the complete marketing application is submitted), and accelerated approval, if relevant criteria are met. Based on results of the Phase 3 clinical study(ies) submitted in an NDA or BLA, upon the request of an applicant, the FDA may grant the NDA or BLA a priority review designation, which sets the target date for FDA action on the application at six months after the FDA accepts the application for filing. Priority review is granted where there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition. If criteria are not met for priority review, the application is subject to the standard FDA review period of ten months after FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the accelerated approval program, the FDA may approve an NDA or BLA on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Post-marketing studies or completion of ongoing studies after marketing approval are generally required to verify the drug s clinical benefit in relationship to the surrogate endpoint or ultimate outcome in relationship to the clinical benefit. In addition, the Food and Drug Administration Safety and Innovation Act which was enacted and signed into law in 2012, established the new Breakthrough Therapy designation. A sponsor may seek FDA designation of its product candidate as a breakthrough therapy if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.

FDA Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval,

most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

Drug manufacturers are subject to periodic unannounced inspections by the FDA and state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA

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regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

We rely, and expect to continue to rely, on third parties for the production of clinical quantities of our product candidates, and expect to rely in the future on third parties for the production of commercial quantities. Future FDA and state inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of previously unknown problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved NDA or BLA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA s policies may change, which could delay or prevent regulatory approval of our products under development.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;

fines, warning letters or holds on post-approval clinical studies;

refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product license approvals;

injunctions or the imposition of civil or criminal penalties; or

product seizure or detention, or refusal to permit the import or export of products.

The FDA strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

Orphan Designation and Exclusivity

The FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making the drug for this type of disease or condition will be recovered from sales in the United States.

Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical study costs, tax advantages, and user-fee waivers. In addition, if a product receives FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity.

Pediatric Studies and Exclusivity

NDAs and BLAs must contain data (or a proposal for post-marketing activity) to assess the safety and effectiveness of an investigational new drug product for the claimed indications in all relevant pediatric populations in order to support dosing and administration for each pediatric subpopulation for which the drug

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is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults or full or partial waivers if certain criteria are met. Discussions about pediatric development plans can be discussed with the FDA at any time, but usually occur any time between the end-of-Phase II meeting and submission of the NDA or BLA. The requirements for pediatric data do not apply to any drug for an indication for which orphan designation has been granted.

Pediatric exclusivity is another type of non-patent exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the five-year and three-year non-patent and orphan exclusivity. This six-month exclusivity may be granted if an NDA or BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical study is deemed to fairly respond to the FDA s request, the additional protection is granted. If reports of FDA-requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot accept or approve another application relying on the NDA or BLA sponsor s data.

Patent Term Restoration

Depending upon the timing, duration, and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Act.

The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product s approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA or BLA, plus the time between the submission date and the approval of that application. Only one patent applicable to an approved product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical studies and other factors involved in the filing of the relevant NDA or BLA.

Biosimilars and Exclusivity

The Patient Protection and Affordable Care Act, or Affordable Care Act, signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCI Act, which created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product. This amendment to the PHSA attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation

that are still being worked out by the FDA.

A reference biologic is granted twelve years of exclusivity from the time of first licensure of the reference product. The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing,

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(ii) eighteen months after approval if there is no legal challenge, (iii) eighteen months after the resolution in the applicant s favor of a lawsuit challenging the biologics patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

Abbreviated New Drug Applications for Generic Drugs

In 1984, with passage of the Hatch-Waxman Act, the U.S. Congress authorized the FDA to approve generic drugs that are the same as drugs previously approved by the FDA under the NDA provisions of the statute. To obtain approval of a generic drug, an applicant must submit an abbreviated new drug application, or ANDA, to the agency. In support of such applications, a generic manufacturer may rely on the preclinical and clinical testing previously conducted for a drug product previously approved under an NDA, known as the reference listed drug, or RLD.

Specifically, in order for an ANDA to be approved, the FDA must find that the generic version is identical to the RLD with respect to the active ingredients, the route of administration, the dosage form, and the strength of the drug. At the same time, the FDA must also determine that the generic drug is bioequivalent to the innovator drug. Under the statute, a generic drug is bioequivalent to an RLD if the rate and extent of absorption of the generic drug do not show a significant difference from the rate and extent of absorption of the listed drug. . . .

Upon approval of an ANDA, the FDA indicates that the generic product is therapeutically equivalent to the RLD and it assigns a therapeutic equivalence rating to the approved generic drug in its publication Approved Drug Products with Therapeutic Equivalence Evaluations, also referred to as the Orange Book. Physicians and pharmacists consider an AB therapeutic equivalence rating to mean that a generic drug is fully substitutable for the RLD. In addition, by operation of certain state laws and numerous health insurance programs, the FDA s designation of an AB rating often results in substitution of the generic drug without the knowledge or consent of either the prescribing physician or patient.

The FDCA provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity. In cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification, in which case the applicant may submit its application four years following the original product approval. The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication.

Hatch-Waxman Patent Certification and the 30-Month Stay

Upon approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant s product or a method of using the product. Each of the patents listed by the NDA sponsor is published in the Orange Book. When an ANDA applicant files its application with the FDA, the applicant is required to certify to the FDA concerning any patents listed for the reference product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval.

Specifically, the applicant must certify with respect to each patent that:

the required patent information has not been filed;
the listed patent has expired;
the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or

the listed patent is invalid, unenforceable or will not be infringed by the new product.

A certification that the new product will not infringe the already approved product s listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicates that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

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If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months after the receipt of the Paragraph IV notice, expiration of the patent, or a decision in the infringement case that is favorable to the ANDA applicant.

European Union/Rest of World Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical studies and any commercial sales and distribution of our products.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical studies or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical study application much like the IND prior to the commencement of human clinical studies. In the European Union, for example, a clinical study application, or CTA, must be submitted for each clinical protocol to each country s national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is accepted in accordance with a country s requirements, the clinical study may proceed.

The requirements and process governing the conduct of clinical studies vary from country to country. In all cases, the clinical studies are conducted in accordance with cGCP, the applicable regulatory requirements, and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational medicinal product under European Union regulatory systems, we must submit a marketing authorization application. The content of the NDA or BLA filed in the United States is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing product licensing, pricing, and reimbursement vary from country to country.

Countries that are part of the European Union, as well as countries outside of the European Union, have their own governing bodies, requirements, and processes with respect to the approval of pharmaceutical products. If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Authorization Procedures in the European Union

Medicines can be authorized in the European Union by using either the centralized authorization procedure or national authorization procedures.

Centralized procedure. The EMA implemented the centralized procedure for the approval of human medicines to facilitate marketing authorizations that are valid throughout the EEA which is comprised of the 28 member states of the European Union plus Norway, Iceland, and Lichtenstein. This procedure results in a single marketing

authorization issued by the EMA that is valid across the EEA. The centralized procedure is compulsory for human medicines that are: derived from biotechnology processes, such as genetic engineering, contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions, and officially designated orphan medicines.

For medicines that do not fall within these categories, an applicant has the option of submitting an application for a centralized marketing authorization to the European Commission following a favorable opinion by the EMA, as long as the medicine concerned is a significant therapeutic, scientific or technical innovation, or if its authorization would be in the interest of public health.

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National authorization procedures. There are also two other possible routes to authorize medicinal products in several European Union countries, which are available for investigational medicinal products that fall outside the scope of the centralized procedure:

Decentralized procedure. Using the decentralized procedure, an applicant may apply for simultaneous authorization in more than one European Union country of medicinal products that have not yet been authorized in any European Union country and that do not fall within the mandatory scope of the centralized procedure.

Mutual recognition procedure. In the mutual recognition procedure, a medicine is first authorized in one European Union Member State, in accordance with the national procedures of that country. Following this, further marketing authorizations can be sought from other European Union countries in a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization.

In some cases, a Pediatric Investigation Plan, or PIP, and/or a request for waiver or deferral, is required for submission prior to submitting a marketing authorization application. A PIP describes, among other things, proposed pediatric studies and their timing relative to clinical studies in adults.

New Chemical Entity Exclusivity

In the European Union, new chemical entities, sometimes referred to as new active substances, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. This data exclusivity, if granted, prevents regulatory authorities in the European Union from referencing the innovator s data to assess a generic (abbreviated) application for eight years, after which generic marketing authorization can be submitted, and the innovator s data may be referenced, but not approved for two years. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies.

Orphan Designation and Exclusivity

In the European Union, the EMA s COMP grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than 5 in 10,000 persons in the European Union Community and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the medicinal product.

In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Exceptional Circumstances/Conditional Approval

Orphan drugs or drugs with unmet medical needs may be eligible for European Union approval under exceptional circumstances or with conditional approval. Approval under exceptional circumstances is applicable to orphan products and is used when an applicant is unable to provide comprehensive data on the efficacy and safety under normal conditions of use because the indication for which the product is intended is encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence, when the present state of scientific knowledge does not allow comprehensive information to be provided, or when it is medically unethical to collect such information. Conditional marketing authorization is applicable to

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orphan medicinal products, medicinal products for seriously debilitating or life-threatening diseases, or medicinal products to be used in emergency situations in response to recognized public threats. Conditional marketing authorization can be granted on the basis of less complete data than is normally required in order to meet unmet medical needs and in the interest of public health, provided the risk-benefit balance is positive, it is likely that the applicant will be able to provide the comprehensive clinical data, and unmet medical needs will be fulfilled. Conditional marketing authorization is subject to certain specific obligations to be reviewed annually.

Accelerated Review

Under the Centralized Procedure in the European Union, the maximum timeframe for the evaluation of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the EMA s Committee for Medicinal Products for Human Use, or CHMP). Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, particularly from the point of view of therapeutic innovation. In this circumstance, EMA ensures that the opinion of the CHMP is given within 150 days, excluding clock stops.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of coverage and reimbursement from third-party payors. Third-party payors include government authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drugs for a particular indication. Moreover, a payor s decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. In order to obtain coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain regulatory approvals. Our product candidates may not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. By way of example, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively, the Healthcare Reform Law, contains provisions that may reduce the profitability of drug products, including, for example, increased rebates for drugs sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual

fees based on pharmaceutical companies share of sales to federal health care programs. Adoption of government controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals.

In the European Community, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed to by the government. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical studies that

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compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on cost containment measures in the United States and other countries has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Other Healthcare Laws and Compliance Requirements

If we obtain regulatory approval for any of our product candidates, we may be subject to various federal and state laws targeting fraud and abuse in the healthcare industry. These laws may impact, among other things, our proposed sales, marketing and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payers that are false or fraudulent;
- HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
- the federal transparency laws, including the federal Physician Payment Sunshine Act, that requires drug manufacturers to disclose payments and other transfers of value provided to physicians and teaching hospitals;
- HIPAA, as amended by HITECH, and its implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payer, including commercial insurers, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

The Healthcare Reform Law broadened the reach of the fraud and abuse laws by, among other things, amending the intent requirement of the federal Anti-Kickback Statute and the applicable criminal healthcare fraud statutes contained within 42 U.S.C. §1320a-7b, effective March 23, 2010. Pursuant to the statutory amendment, a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the Healthcare Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act (discussed below) or the civil monetary penalties statute. Many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare items or services reimbursed by any source, not only the Medicare and Medicaid programs.

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We are also subject to the Foreign Corrupt Practices Act, or FCPA, which prohibits improper payments or offers of payments to foreign governments and their officials for the purpose of obtaining or retaining business.

Safeguards we implement to discourage improper payments or offers of payments by our employees, consultants, and others may be ineffective, and violations of the FCPA and similar laws may result in severe criminal or civil sanctions, or other liabilities or proceedings against us, any of which would likely harm our reputation, business, financial condition and result of operations.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, exclusion from participation in government healthcare programs, such as Medicare and Medicaid and imprisonment, damages, fines and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Labeling, Marketing and Promotion

The FDA closely regulates the labeling, marketing and promotion of drugs. While doctors are free to prescribe any drug approved by the FDA for any use, a company can only make claims relating to safety and efficacy of a drug that are consistent with FDA approval, and the Company is allowed to actively market a drug only for the particular use and treatment approved by the FDA. In addition, any claims we make for our products in advertising or promotion must be appropriately balanced with important safety information and otherwise be adequately substantiated. Failure to comply with these requirements can result in adverse publicity, warning letters, corrective advertising, injunctions and potential civil and criminal penalties. Government regulators recently have increased their scrutiny of the promotion and marketing of drugs.

Pediatric Research Equity Act

The Pediatric Research Equity Act, or PREA, amended the FDCA to authorize the FDA to require certain research into drugs used in pediatric patients. The intent of PREA is to compel sponsors whose drugs have pediatric applicability to study those drugs in pediatric populations, rather than ignoring pediatric indications for adult indications that could be more economically desirable. The Secretary of Health and Human Services may defer or waive these requirements under specified circumstances.

Anti-Kickback and False Claims Laws

In the United States, the research, manufacturing, distribution, sale and promotion of drug products and medical devices are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare & Medicaid Services, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the U.S. Department of Justice, state Attorneys General, and other state and local government agencies. For example, sales, marketing and scientific/educational grant programs must comply with the Anti-Kickback Statute, the False Claims Act, as amended, the privacy regulations promulgated under HIPAA and similar state laws. Pricing and rebate programs must comply with the Medicaid Drug Rebate Program requirements of the Omnibus Budget Reconciliation Act of 1990, as amended, and the Veterans Health Care Act of 1992, as amended. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. All of these activities are also potentially subject to federal and state consumer protection and unfair competition laws.

In the United States, we are subject to complex laws and regulations pertaining to healthcare fraud and abuse, including, but not limited to, the Anti-Kickback Statute, the federal False Claims Act, and other state and federal laws and regulations. The Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf) to knowingly and willfully solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchase, order, or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid.

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The federal False Claims Act prohibits anyone from knowingly presenting, or causing to be presented, for payment to federal programs (including Medicare and Medicaid) claims for items or services, including drugs, that are false or fraudulent, claims for items or services not provided as claimed, or claims for medically unnecessary items or services.

There are also an increasing number of state laws that require manufacturers to make reports to states on pricing and marketing information. Many of these laws contain ambiguities as to what is required to comply with the laws. In addition, as discussed below, beginning in 2013, a similar federal requirement will require manufacturers to track and report to the federal government certain payments made to physicians and teaching hospitals made in the previous calendar year. These laws may affect our sales, marketing, and other promotional activities by imposing administrative and compliance burdens on us. In addition, given the lack of clarity with respect to these laws and their implementation, our reporting actions could be subject to the penalty provisions of the pertinent state, and soon federal, authorities.

Patient Protection and Affordable Health Care Act

In March 2010, the Patient Protection and Affordable Health Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively PPACA, was enacted, which includes measures that have or will significantly change the way health care is financed by both governmental and private insurers. The fees, discounts and other provisions of this law are expected to have a significant negative effect on the profitability of pharmaceuticals.

Many of the details regarding the implementation of PPACA are yet to be determined, and at this time, it remains unclear the full effect that PPACA would have on our business.

Other Regulations

We are also subject to numerous federal, state and local laws relating to such matters as safe working conditions, manufacturing practices, environmental protection, fire hazard control, and disposal of hazardous or potentially hazardous substances. We may incur significant costs to comply with such laws and regulations now or in the future.

Israel

Clinical Testing in Israel

In order to conduct clinical testing on humans in the State of Israel, special authorization must first be obtained from the ethics committee and general manager of the institution in which the clinical studies are scheduled to be conducted, as required under the Guidelines for Clinical Trials in Human Subjects implemented pursuant to the Israeli Public Health Regulations (Clinical Trials in Human Subjects), as amended from time to time, and other applicable legislation. These regulations require authorization by the institutional ethics committee and general manager as well as from the Israeli Ministry of Health, except in certain circumstances, and in the case of genetic trials, special fertility trials and complex clinical trials, an additional authorization of the Ministry of Health s overseeing ethics committee. The institutional ethics committee must, among other things, evaluate the anticipated benefits that are likely to be derived from the project to determine if it justifies the risks and inconvenience to be inflicted on the human subjects, and the committee must ensure that adequate protection exists for the rights and safety of the participants as well as the accuracy of the information gathered in the course of the clinical testing. Since we perform a portion of the clinical

studies on certain of our therapeutic candidates in Israel, we are required to obtain authorization from the ethics committee and general manager of each institution in which we intend to conduct our clinical trials, and in most cases, from the Israeli Ministry of Health.

Employees

As of July 30, 2014, our staff included eight persons, comprised of six full time employees and two officers, who are engaged with the Company as service providers. All of our employees are based in Israel. None of our employees is represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good. Our company uses a cost-effective business model, only retaining top management as company employees or dedicated consultants, and drawing upon other consultants globally to advance our development programs. As of December 31, 2013, our management consisted of our chief executive officer and our chief financial officer.

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Research and Development

We invested $0.1 million and $0.7 million in research and development in the years ended December 31, 2012 and December 31, 2013, respectively.

Property and Facilities

Our headquarters is currently located in Tel Aviv, Israel and consists of approximately 3,390 square feet of leased office space under a lease for a period of three years with an option to extend the lease period for two additional consecutive three year periods. We may require additional space and facilities as our business expands.

Legal Proceedings

We are not currently subject to any material legal proceedings.

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MANAGEMENT

Executive Officers, Key Employees and Directors

Executive Officers and Directors

The following table sets forth information regarding our executive officers and directors as of the date of this prospectus:

Name	Age	Position
Fredric Price	68	Executive Chairman of the Board of Directors
Dr. Dalia Megiddo	63	Chief Executive Officer, Director
Ehud (Udi) Gilboa	48	Chief Financial Officer, Senior Vice President of Operations, Director
Ran Nusbaum	40	Director
Marlene Haffner	72	Director
Gili Cohen[1][2]	48	Director
Prof. Avizohar Argov	67	Chief Medical Officer

(1) Member of our Audit Committee.

(2) Member of our Compensation Committee upon establishment thereof.

The Company intends to add additional members to its Board of Directors following the closing of this offering. These directors will be independent directors and nominees for the purposes of election as external directors as contemplated by the NASDAQ Stock Market rules and Israeli law. The Company s Audit Committee has been established as of the date of this prospectus and the Company will establish a Compensation Committee following the consummation of this offering.

Fredric Price has been Executive Chairman of our Board of Directors since April 2014, having served as our Chairman of the Board of Directors from April 2012 until April 2014. Since 2013, Mr. Price has served as a member of the Advisory Board of FDNA. From 2013 until 2014, he was Executive Chairman of the Board of Directors and from 2008 to 2013 Chairman of the Board of Directors and CEO of Chiasma. From 2004 to 2008, Mr. Price was Executive Chairman of the Board of Directors of Omrix Biopharmaceuticals, from 2006 to 2012 a member of the Board of Directors of Enobia Pharma, from 2007 to 2010 a member of the Board of Directors of Pharmasset, from 2007 to 2011 Executive Chairman of the Board of Directors of Peptimmune, from 2004 to 2005 Executive Chairman of the Board of Directors of Zymenex, from 2000 to 2004 Chairman of the Board of Directors and CEO of BioMarin Pharmaceutical, and from 1994 to 2000 CEO and a member of the Board of Directors of Applied Microbiology. As chairman and/or CEO, he has raised more than $500 million in a variety of securities transactions, led a total of 21 M&A and licensing transactions, built FDA approved facilities and had drugs approved in the US as well as in international markets. His earlier experience includes having been Vice President of Finance and Administration and CFO of Regeneron Pharmaceuticals, the founder of the strategy consulting firm RxFDP, and Vice President of Pfizer Pharmaceuticals with both line and staff responsibilities. Mr. Price is a co-inventor of 13 issued US patents. He received a BA from Dartmouth College and an MBA from the Wharton School of the University of Pennsylvania.

Dr. Dalia Megiddo has been a director and Chief Executive Officer of the Company since its inception. Dr. Megiddo co-founded Alcobra Ltd., a NASDAQ traded company primarily focused on the development and commercialization

of a proprietary drug, MG01CI, to treat Attention Deficit Hyperactivity Disorder in February 2008 and became a Director at that time. She is an entrepreneur and a medical doctor in family medicine. Since 2000, she has been a manager of InnoMed Ventures, an Israeli venture capital fund focused on life sciences. From 2006 to 2010, she was also a manager of 7 Health Ventures, a Israeli venture capital fund. She is also the founder of a number of life science companies. Dr. Megiddo received her M.D. degree from Hebrew University Hadassah Medical School and holds a specialist degree in Family Medicine, and also holds an M.B.A. from the Kellogg-Recanati School of Business.

Udi Gilboa has been a director, Chief Financial Officer and Senior Vice President of Operations of our Company since its inception. In addition, Mr. Gilboa co-founded Alcobra Ltd. in February 2008, became a

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director at that time and served as its Chief Financial Officer and Chief Accounting Officer until May 2014. Mr. Gilboa is the founder and managing partner of Top Notch Capital, a prominent Israeli life sciences investment bank. He is also the founder of a number of medical device and pharmaceutical companies. Mr. Gilboa holds a Bachelor s degree and M.B.A. from Tel Aviv University. Mr. Gilboa serves a director of Insuline Medical Ltd., a public company whose shares are listed for trading on the Tel Aviv Stock Exchange. In addition, he is a director of Samson Neurosciences Ltd.

Ran Nusbaum has been a director in our company since July 1, 2013. Mr. Nusbaum is a managing partner and the co-founder of The Pontifax Group, which established three funds with over 30 portfolio companies. Over the past 10 years, Mr. Nusbaum has been co-managing The Pontifax Group. From 2006 to 2008 he also served as Chief Executive Officer of Biomedix Ltd. and Spearhead Ltd., as well as Chairman of the Board of Nasvax Ltd. Mr. Nusbaum s experience in the life sciences arena coupled with a 10-year experience in the business intelligence field create a unique blend of skills, enabling him to support companies from inception to commercialization. Mr. Nusbaum also serves on the Board of Directors of many of The Pontifax Group s portfolio companies including, Kite pharma Inc., c-CAM Ltd., Insuline Ltd. (a public company), Eloxx, Theracaot, CollPlant (a public company), Protab, Quiet, Fusimab Ltd and as chairman of the board of Ocon.

Dr. Marlene Haffner has been a director in our company since July 1, 2013. Dr. Haffner is a former Director of the Office of Orphan Products Development (OOPD) of the FDA. As OOPD Director she was responsible for the leadership and management of the FDA orphan products development program, the first Orphan Products program in the world. After leaving the FDA, she served as Executive Director, Global Regulatory Policy and Intelligence at Amgen, Inc, and now holds position of Chief Executive Officer at Orphan Solutions and Haffner Associates, services companies for the Orphan Drugs industry. In addition to her consulting activities, Dr Haffner is Adjunct Professor, Department of Preventive Medicine and Biometrics, and Clinical Professor, Department of Medicine, at the F Edward Hébert School of Medicine, Uniformed Services University of the Health Sciences in Bethesda, Maryland. For 36 years she served in the United States Public Health Service beginning her career with the Indian Health Service in Gallup, New Mexico. She received her MD from the George Washington University School of Medicine where she then interned in Internal Medicine. She received further training in internal medicine, dermatology and hematology at the Presbyterian Hospital, New York and at the Albert Einstein College of Medicine, New York. She received an MPH from the Johns Hopkins University Bloomberg School of Public Health. During her Public Health career, she rose to the rank of Rear Admiral in the United States Public Health Service.

Gili Cohen has been a director in our company since July 30, 2014. Mr. Cohen has been a member of the board of directors of Harel Pension Funds Management Ltd. and Harel Atidit Provident Funds Ltd., which are both members of the Harel Insurance Investments and Financial Services Ltd. group, since March 2012. In addition, Mr. Cohen has been a member of the Israel Land Development Co. Ltd., which deals in real estate and investments, since June 2012. He also currently serves as an independent financial consultant and is an economics professor at The College of Management Academic Studies. From 2000 to 2011 Mr. Cohen was the Chief Investments Officer and head of the Investments Department at Excellence Investments Ltd. Mr. Cohen has a degree in economics and geography and an M.B.A., both with honors, from the Hebrew University in Jerusalem. Mr. Cohen serves on our Audit Committee and has agreed to serve on our Compensation Committee.

Prof. Avizohar Argov was elected to act as the Chief Medical Officer of our Company on April 1, 2014. Prof. Argov is a Professor of Neurology & Josephine Frank Kanrich Chair of Neuromuscular Diseases, Hadassah-Hebrew University Medical Center in Jerusalem, Israel, and is an Adjunct Professor in the Department of Neurology/Neurosurgery at the Neurological Institute at McGill University in Montreal, Canada. Prof. Argov is a former member of the executive committees of the World Muscle Society and the European Neurological Society, and is the former Chairman of the European Neurological Society Subcommittee of Muscle & Neuromuscular Disorders, the former President of the

European Neurological Society and the current Chairman of the Israeli Society of Neuromuscular Diseases. Prof. Argov s primary research fields include hereditary inclusion body myopathies, hereditary neuromuscular disorders, particularly in Jewish ethnic clusters, and latrogenic neuromuscular disorders, particularly drug-induced myasthenia. He received his MD from the Hebrew University-Hadassah Medical School in Jerusalem, Israel and was a Resident in Neurology at the Hadassah University Hospital in Jerusalem, Israel. He received training in

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neuromuscular diseases from the Muscular Dystrophy Association in Newcastle upon Tyne, England and further training in biochemistry and biophysics from the University of Pennsylvania in the United States.

Arrangements Concerning Election of Director; Family Relationships

There are no arrangements or understandings with major shareholders, customers, suppliers or others pursuant to which any of our directors or members of senior management were selected as such. In addition, there are no family relationships among our executive officers and directors.

Corporate Governance Practices

As an Israeli company we are subject to various corporate governance requirements under Israeli law relating to such matters as the election of external directors, the appointment of the audit committee, the compensation committee and an internal auditor. These requirements are in addition to the corporate governance requirements imposed by the Listing Rules of the NASDAQ Stock Market and other applicable provisions of U.S. securities laws to which we will become subject upon consummation of this offering and the listing of our ordinary shares on the NASDAQ Global Market. Under the Listing Rules of the NASDAQ Stock Market, a foreign private issuer may generally follow its home country rules of corporate governance in lieu of the comparable requirements of the Listing Rules of the NASDAQ Stock Market, except for certain matters, including (among others) the composition and responsibilities of the audit committee and the independence of its members within the meaning of the rules and regulations of the SEC. For further information, see Risk factors and NASDAQ Listing Rules and Home Country Practices.

Dr. Marlene Haffner and Gili Cohen are independent under Rule 5605(a)(2) to the NASDAQ Listing Rules.

Board Practices

Board of Directors

Under the Israeli Companies Law, setting up the Company s policy and oversight over our business is vested in our Board of Directors. Our Board of Directors may exercise all powers and may take all actions that are not specifically granted to our shareholders or to management. Our executive officers are responsible for our day-to-day management and have individual responsibilities established by our Board of Directors. Our Chief Executive Officer is appointed by, and serves at the discretion of, our Board of Directors, subject to the service agreement that we have entered into with her. All other executive officers are appointed by our Chief Executive Officer, and are subject to the terms of any applicable employment agreements that we may enter into with them.

Under our amended and restated articles of association, which will be effective upon the consummation of this offering, our Board of Directors must consist of at least 5 and not more 11 than directors, including at least 2 external directors required to be appointed under the Israeli Companies Law. Accordingly, at any time, the minimum number of directors (other than the external directors) may not fall below 3. Our Board of Directors will consist of 6 directors upon the consummation of this offering, which will include Gili Cohen as a new director and nominee as external director whose service as director commenced on July 30, 2014, and we intend to add additional directors and a nominee as an external director following the consummation of this offering. In the case of Gili Cohen, his appointment as external director shall be subject to ratification at a meeting of our shareholders to be held no later

than three months following the completion of this offering. We have only one class of directors.

Other than external directors, for whom special election requirements apply under the Israeli Companies Law as detailed below, our directors are each elected at a general meeting of our shareholders and serve for a term of one year. Directors (other than external directors) shall nevertheless be removed prior to the end of their term by the majority of our shareholders at a general meeting of our shareholders or upon the occurrence of certain events, all in accordance with the Israeli Companies Law and our amended and restated articles of association.

In addition, our amended and restated articles of association allow our Board of Directors to appoint directors, other than external directors, to fill vacancies on our Board of Directors, for a term of office equal to the remaining period of the term of office of the directors whose offices have been vacated. External

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directors are elected for an initial term of three years and may be elected for additional three-year terms under the circumstances described below. External directors may be removed from office only under the limited circumstances set forth in the Israeli Companies Law. See External directors.

In accordance with the exemption available to foreign private issuers under NASDAQ rules, we do not intend to follow the requirements of the NASDAQ rules with regard to the process of nominating directors, and instead, will follow Israeli law and practice, in accordance with which our Board of Directors (or a committee thereof, or a certain number of directors serving thereon) is authorized to recommend to our shareholders director nominees for election. Under the Israeli Companies Law and our amended and restated articles of association, nominations for directors may also be added to the agenda of future general meetings, which has yet to have been summoned, upon the request of any one or more shareholders holding at least one percent (1%) of our outstanding voting power. Furthermore, under the Israeli Companies Law either: (a)(i) two directors; or (ii) no less than one quarter of the directors in office; or (b) one or more shareholders holding, in the aggregate, either (i) 5% of our outstanding shares and 1% of our outstanding voting power; or (ii) 5% of our outstanding voting power, may request the Board of Directors to call a general meeting in order to nominate one or more persons for election as directors at a special meeting. However, any such shareholders may make such a nomination only if a written notice of such shareholder s intent to make such nomination has been given to our chairman of the board (or, if we have no chairman of the board, our chief executive officer). Any such notice must include certain information we are required under the Israeli Companies Law to provide to our shareholders, the consent of the proposed director nominee(s) to serve as our director(s) if elected and a declaration signed by the nominee(s) declaring that there is no limitation under the Israeli Companies Law preventing their election and that all of the information that is required under the Israeli Companies Law to be provided to us in connection with such election has been provided.

In addition to its role in making director nominations, under the Israeli Companies Law, our Board of Directors must determine the minimum number of directors who are required to have accounting and financial expertise. Under applicable regulations, a director with accounting and financial expertise is a director who, by reason of his or her education, professional experience and skill, has a high level of proficiency in and understanding of business accounting matters and financial statements, sufficient to be able to thoroughly comprehend the financial statements of the Company and initiate debate regarding the manner in which financial information is presented. In determining the number of directors required to have such expertise, our Board of Directors must consider, among other things, the type and size of our company and the scope and complexity of its operations. Our Board of Directors has determined that our company requires one director with such expertise and that Gili Cohen has such expertise.

External Directors

Under the Israeli Companies Law, the boards of directors of companies whose shares are publicly traded, including companies with shares listed on the NASDAQ Global Market, are required to include at least two members elected to serve as external directors. Gili Cohen is one such candidate, subject to ratification at a meeting of our shareholders to be held no later than three months following the completion of this offering. The other candidate will be elected as an external director after this offering.

The definitions of an external director under the Israeli Companies Law and independent director under NASDAQ listing rules are similar such that it would generally be expected that our two external directors will also comply with the independence requirement under the NASDAQ Stock Market rules. The definition of an external director includes a set of statutory criteria (which are described below) that must be satisfied and for which the candidates must attest to the company, while the definition of an independent director requires the board to consider any factor which would impair the ability of a director to exercise independent judgment. In addition, while external directors serve for a

period of three years pursuant to the requirements of Israeli law, independent directors serve for one year pursuant to the provisions of our amended and restated articles of association. External directors must be elected by a special majority of shareholders who are not controlling shareholders, while independent directors are elected by an ordinary majority.

The Israeli Companies Law provides that external directors must be elected by a majority vote of the shares present and voting at a shareholders meeting, provided that either:

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the majority voted in favor of election includes a majority of the shares held by non-controlling shareholders who do not have a personal interest in the election of the external director (other than a personal interest not deriving from a relationship with a controlling shareholder) that are voted at the meeting, excluding for such purpose any abstentions, which we refer to as a disinterested majority; or

the total number of shares held by non-controlling disinterested shareholders (as described in the previous bullet-point) that voted against the election of the director does not exceed two percent (2%) of the aggregate voting rights in the Company.

The term controlling shareholder is defined in the Israeli Companies Law as a shareholder with the ability to direct the activities of the Company, other than by virtue of being an office holder. A shareholder is in any case deemed to be a controlling shareholder if the shareholder holds 50% or more of the means of control, which include the right to vote at a shareholders meeting and the right to appoint the directors of the Company or its general manager. In connection with approval of certain extraordinary and interested party transactions as well as corporate approval of executive employment and compensation and private placements, by shareholders, any shareholder (or group of shareholders having interest in the same matter being brought for approval) who hold(s) in the aggregate 25% or more of the means of control if no other shareholder holds more than 50% of the voting rights, would be deemed a controlling shareholder.

After an initial term of three years, external directors may be reelected to serve in that capacity for up to two additional three year terms, provided that either (i) (1) his or her service for each such additional term is recommended by one or more shareholders holding in aggregate at least one percent (1%) of the Company s voting rights and is approved at a shareholders meeting by a majority of the shares held by non-controlling shareholders who do not have a personal interest in the election of the external director (other than a personal interest not deriving from a relationship with a controlling shareholder) that are voted at the meeting, excluding for such purpose any abstentions, where the total number of shares held by non-controlling, disinterested shareholders voting for such reelection exceeds two percent (2%) of the aggregate voting rights in the Company, and (2) the external director who has been nominated in such fashion by the shareholders is not a linked or competing shareholder, and does not have or has not had, on or within the two years preceding the date of such person s appointment to serve as another term as external director, any affiliation with a linked or competing shareholder. The term linked or competing shareholder means the shareholder(s) who nominated the external director for reappointment or a material shareholder of the company holding more than 5% of the shares in the company, provided that at the time of the reappointment, such shareholder(s) of the company, the controlling shareholder of such shareholder(s) of the company, or a company under such shareholder(s) of the company s control, has a business relationship with the company or are competitors of the company; the Israeli Minister of Justice, in consultation with the Israeli Securities Authority, may determine that certain matters will not constitute a business relationship or competition with the company; or (ii) his or her service for each such additional term is recommended by the board of directors and is approved at a shareholders meeting by the same disinterested majority required for the initial election of an external director (as described above). The term of office for external directors for Israeli companies traded on certain foreign stock exchanges, including the NASDAQ Global Market, may be further extended, indefinitely, in increments of additional three-year terms, in each case provided that, in addition to reelection in such manner described above, (i) the audit committee and subsequently the board of directors of the Company confirm that, in light of the external director s expertise and special contribution to the work of the board of directors and its committees, the reelection for such additional period is beneficial to the Company, and (ii) prior to the approval of the reelection of the external director, the Company s shareholders have been informed of the term previously served by such nominee and of the reasons why the board of directors and audit committee recommended the extension of such nominee s term. If an external director no longer complies with the applicable requirements, the external director must notify the Company, and his term shall terminate upon such notification. Furthermore, where concerns regarding an external director's compliance with any requirements under Israeli Companies Law, or regarding an external director's breach of any fiduciary duty, have been brought to the Board of Directors attention, the Board of Directors is required to discuss such concerns in its following meeting.

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If the Board of Directors resolves that an external director no longer complies with any requirement for qualification as an external director, or that such external director has breached any fiduciary duty, a special general meeting shall be convened at which the termination of such external director's service shall be included in the agenda.

If an external directorship becomes vacant and there are less than two external directors on the board of directors at the time, then the board of directors is required under the Israeli Companies Law to call a shareholders meeting as soon as possible to appoint a replacement external director.

Each committee of the board of directors that is authorized to exercise the powers of the board of directors must include at least one external director, except that the audit committee and compensation committee must each include all external directors then serving on the board of directors. Under the Israeli Companies Law, external directors of a company are prohibited from receiving, directly or indirectly, any compensation for their services as external directors, other than compensation and reimbursement of expenses pursuant to applicable regulations promulgated under the Israeli Companies Law. Compensation of an external director is determined prior to his or her appointment and may not be changed during his or her term subject to certain exceptions.

The Israeli Companies Law provides that a person is not qualified to serve as an external director if (i) the person is a relative of the controlling shareholder of the Company, or (ii) if that person or his or her relative, partner, employer, another person to whom he or she was directly or indirectly subject, or any entity under the person s control, has or had, during the two years preceding the date of appointment as an external director: (a) any affiliation or other prohibited relationship with the Company, with any person or entity who is a controlling shareholder of the Company at the date of appointment or a relative of such person, or with any entity controlled, during the two years preceding the date of appointment as an external director, by the Company or a controlling shareholder of the Company; or (b) in the case of a company with no controlling shareholder, any affiliation or other prohibited relationship with a person serving, at the date of appointment as external director, as chairman of the board, chief executive officer, a substantial shareholder or the most senior office holder in the Company s finance department.

The term relative is defined as a spouse, sibling, parent, grandparent or descendant; spouse s sibling, parent or descendant; and the spouse of each of the foregoing persons. The term affiliation and the similar types of prohibited relationships include (subject to certain exemptions):

an employment relationship;

a business or professional relationship even if not maintained on a regular basis (excluding insignificant relationships);

control; and

service as an office holder, excluding service as a director in a private company prior to the first offering of its shares to the public if such director was appointed as a director of the private company in order to be nominated to serve as an external director following the initial public offering.

The term office holder is defined under the Israeli Companies Law as the general manager (chief executive officer), chief business manager, deputy general manager, vice general manager, any other person assuming the responsibilities of any of these positions regardless of that person s title, a director, or a manager directly subordinate to the general manager.

In addition, no person may serve as an external director if that person s position or professional or other activities create, or may create, a conflict of interest with that person s responsibilities as a director or otherwise interfere with that person s ability to serve as an external director or if the person is an employee of the Israel Securities Authority or of an Israeli stock exchange. A person may furthermore not continue to serve as an external director if he or she received direct or indirect compensation for his or her role as a director, other than compensation paid or given in

accordance with Israeli Companies Law regulations or amounts paid pursuant to indemnification and/or exculpation contracts or commitments and insurance coverage. Following the termination of an external director s service on a board of directors, such former external director and his

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or her spouse and children may not be provided with direct or indirect benefit by the Company, its controlling shareholder or any entity under its controlling shareholder s control. This includes appointment as an office holder of the Company or a company controlled by its controlling shareholder, employment as an employee, or receipt of professional services for consideration, either directly or indirectly, including through a corporation in his or her control. These restrictions extend for a period of two years with regard to the former external director and his or her spouse or child, and for one year with respect to other relatives of the former external director.

If at the time at which an external director is appointed all members of the board of directors, who are not controlling shareholders or relatives thereof, are of the same gender, the external director must be of the other gender. A director of one company may not be appointed as an external director of another company if a director of the other company is acting as an external director of the first company at such time.

According to the Israeli Companies Law, a person may be appointed as an external director only if he or she has professional qualifications or if he or she has accounting and financial expertise (each, as defined below). In addition, at least one of the external directors must be determined by our Board of Directors to have accounting and financial expertise. However, if at least one of our other directors (i) meets the independence requirements under the Exchange Act, (ii) meets the standards of the NASDAQ Listing Rules for membership on the audit committee, and (iii) has accounting and financial expertise as defined under Israeli law, then neither of our external directors is required to possess accounting and financial expertise as long as both possess other requisite professional qualifications.

A director with accounting and financial expertise is a director who, due to his or her education, experience and skills, possesses an expertise in, and an understanding of, financial and accounting matters and financial statements, in such a manner which allows him or her to understand the financial statements of the Company and initiate a discussion about the presentation of financial data. A director is deemed to have professional qualifications if he or she has any of (i) an academic degree in economics, business management, accounting, law or public service, (ii) an academic degree or has completed other higher education, in the main field of business of the Company or a field relevant for the position, or (iii) at least five years of experience as one of the following, or at least five years accumulated experience as two or more of the following (a) a senior officer in the business management of a company with a significant volume of business, (b) a senior public officer or senior position in the public service, and (c) a senior position in the Company s main line of business.

Our Board of Directors has determined that Gili Cohen has accounting and financial expertise and possesses professional qualifications as required under the Israeli Companies Law.

Leadership Structure of the Board

In accordance with the Israeli Companies Law and our amended and restated articles of association, our Board of Directors is required to appoint one of its members to serve as Chairman of the Board of Directors. Our Board of Directors has appointed Fredric Price to serve as Executive Chairman of the Board of Directors.

Role of Board in Risk Oversight Process

Risk assessment and oversight are an integral part of our governance and management processes. Our Board of Directors encourages management to promote a culture that incorporates risk management into our corporate strategy and day-to-day business operations. Management discusses strategic and operational risks at regular management meetings, and conducts specific strategic planning and review sessions during the year that include a focused discussion and analysis of the risks facing us. Throughout the year, senior management reviews these risks with the

Board of Directors at regular board meetings as part of management presentations that focus on particular business functions, operations or strategies, and presents the steps taken by management to mitigate or eliminate such risks.

Board Committees

Audit Committee

Under the Israeli Companies Law, the board of directors of a public company must appoint an audit committee. The audit committee must be comprised of at least three directors, including all of the external

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directors, one of whom must serve as chairman of the committee. The audit committee may not include the chairman of the board, any director employed by or otherwise providing services on a regular basis to the Company, to a controlling shareholder or to any entity controlled by a controlling shareholder, any director whose main livelihood is dependent on a controlling shareholder, nor a controlling shareholder or a relative thereof.

Under the Israeli Companies Law, the audit committee of a publicly traded company must consist of a majority of unaffiliated directors. An unaffiliated director is defined as either an external director or as a director, classified as an unaffiliated director by the Company, who meets the following criteria:

he or she meets the qualifications for being appointed as an external director, except for (i) the requirement that the director be an Israeli resident (which requirement does not, in any event, apply to external directors at public companies such as ours whose securities have been offered outside of Israel or are listed outside of Israel) and (ii) the requirement for accounting and financial expertise or professional qualifications with respect to the proposed unaffiliated director; and

he or she has not served as a director of the Company for a period exceeding nine consecutive years. For this purpose, a break of less than two years in the service shall not be deemed to interrupt the continuation of the service.

Our Board of Directors intends to adopt an audit committee charter that will set forth the responsibilities of the Audit Committee consistent with the rules of the SEC and the Listing Rules of the NASDAQ Stock Market, as well as subjecting the audit committee charter to the requirements under the Israeli Companies Law, as described below.

Our Audit Committee provides assistance to our Board of Directors in fulfilling its legal and fiduciary obligations in matters involving our accounting, auditing, financial reporting, internal control and legal compliance functions by pre-approving the services performed by our independent accountants and reviewing their reports regarding our accounting practices and systems of internal control over financial reporting. Our Audit Committee also oversees the audit efforts of our independent accountants and takes those actions that it deems necessary to satisfy itself that the accountants are independent of management.

Under the Israeli Companies Law, our Audit Committee is responsible for (i) determining whether there are deficiencies in the business management practices of our company, including in consultation with our internal auditor or the independent auditor, and making recommendations to the Board of Directors to improve such practices and amend such deficiencies where material deficiencies have been revealed, at least one meeting of the Audit Committee is required to be convened, with the presence of our internal auditor or the independent auditor, and without the presence of any members of the Board of Directors who are not members of the Audit Committee (unless their presence is required for the purpose of presenting their position to matters under their responsibility); (ii) determining whether certain related party transactions (including transactions in which an office holder has a personal interest) should be deemed as material or extraordinary, and to approve such transactions (which may be approved according to certain criteria set out by our Audit Committee on an annual basis) (see Approval of related party transactions under Israeli Law), (iii) to establish procedures to be followed in respect of related party transactions with a controlling shareholder (where such are not extraordinary transactions), which may include, where applicable, the establishment of a competitive process for such transaction, under the supervision of the audit committee, or individual, or other committee or body selected by the audit committee, in accordance with criteria determined by the audit committee; (iv) to determine procedures for approving certain related party transactions with a controlling shareholder, which having been determined by the audit committee not to be extraordinary transactions, were also determined by the audit committee not to be negligible transactions; (v) where the Board of Directors approves the working plan of the internal auditor, to examine such working plan before its submission to the Board and propose amendments thereto, (vi) examining our internal controls and internal auditor s performance, including whether the internal auditor has sufficient resources and tools to dispose of its responsibilities, (vii) examining the scope of our auditor s work and compensation and submitting a recommendation with respect thereto to our Board of Directors or shareholders,

depending on which of them is considering the appointment of our auditor, and (viii) establishing procedures for the handling of employees complaints as to the management of our business and the protection to be provided to such

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employees. Our Audit Committee may not approve an action or a related party transaction, or take any other action required under the Israeli Companies Law, unless at the time of approval a majority of the committee s members are present, which majority consists of unaffiliated directors including at least one external director, and it further complies with the committee composition set forth above.

NASDAQ requirements

Under the Nasdaq Marketplace Rules, we are required to maintain an Audit Committee consisting of at least three independent directors, all of whom are financially literate and one of whom has accounting or related financial management expertise.

Our Audit Committee currently consists of Gili Cohen. We will add additional members to our Audit Committee following the consummation of this offering. All members of our Audit Committee will be required to meet the requirements for financial literacy under the applicable rules and regulations of the SEC and the Listing Rules of the NASDAQ Stock Market. Our board of directors has determined that Gili Cohen is an audit committee financial expert as defined by the SEC rules and has the requisite financial experience as defined by the Listing Rules of the NASDAQ Stock Market.

Each member of the Audit Committee is required to be independent as such term is defined in Rule 10A-3(b)(1) under the Exchange Act, which is different from the general test for independence of board and committee members.

Compensation Committee

We intend to rely upon the exemption available to foreign private issuers under the Listing Rules of the NASDAQ Stock Market with respect to the determination of the compensation of our Chief Executive Officer and other executive officers in lieu of forming a compensation committee consisting entirely of independent directors (or the determination of such compensation solely by the independent members of our Board of Directors), and rather form a compensation committee in compliance with the Israeli Companies Law. See NASDAQ Listing Rules and home country practices.

Under the Israeli Companies Law, the board of directors of a public company must appoint a compensation committee. The compensation committee must be comprised of at least three directors, including all of the external directors, which shall be a majority of the members of the compensation committee and one of whom must serve as chairman of the committee. However, subject to certain exceptions, Israeli companies whose securities are traded on stock exchanges such as NASDAQ, and who do not have a controlling party, do not have to meet this majority requirement; provided, however, that the compensation committee meets other Israeli Companies Law composition requirements, as well as the requirements of the non-Israeli jurisdiction where the company s securities are traded. Other than the external directors, the rest of the members of the compensation committee shall be directors who will receive compensation for their role as directors only in accordance with Israeli Companies Law regulations or amounts paid pursuant to indemnification and/or exculpation contracts or commitments and insurance coverage.

The compensation committee may not include the chairman of the board, any director employed by or otherwise providing services on a regular basis to the Company, to a controlling shareholder or to any entity controlled by a controlling shareholder, any director whose main livelihood is dependent on a controlling shareholder, nor a controlling shareholder or a relative thereof.

After the consummation of this offering we will announce the members of our compensation committee, which will

include our two external director nominees.

Under the Israeli Companies Law, our compensation committee is responsible for (i) proposing an office holder compensation policy to the Board of Directors, (ii) propose necessary revisions to the compensation policy and examine its implementation, (iii) determining whether to approve transactions with respect to the terms of office and employment of office holders, and (iv) determining, in accordance with our office holder compensation policy, whether to exempt an engagement with an unaffiliated nominee for the position of chief executive officer from requiring shareholders approval.

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Under the Israeli Companies Law, our compensation policy must generally serve as the basis for corporate approvals with respect to the financial terms of employment or engagement of office holders, including exemption, insurance, indemnification or any monetary payment or obligation of payment in respect of employment or engagement. The compensation policy must relate to certain factors, including advancement of the company s objective, the company s business plan and its long term strategy, and creation of appropriate incentives for office holders. It must also consider, among other things, the company s risk management, size and nature of its operations. The compensation policy must furthermore consider the following additional factors:

The knowledge, skills, expertise, and accomplishments of the relevant office holder;
The office holder s roles and responsibilities and prior compensation agreements with him or her;
The relationship between the terms offered and the average and median compensation of the other employees of the company, including those employed through manpower companies;
The impact of disparities in salary upon work relationships in the company;
The possibility of reducing variable compensation at the discretion of the board of directors; the possibility of setting a limit on the exercise value of non-cash variable equity-based compensation; and
As to severance compensation, the period of service of the office holder, the terms of his or her compensation during such service period, the company s performance during that period of service, the person s contributions towards the company s achievement of its goals and the maximization of its profits, and the circumstances under which the person is leaving the company.

The compensation policy must also include the following principles:

the link between variable compensation and the long term performance and measurable criteria;
the relationship between variable and fixed compensation, and the ceiling for the value of variable compensation;
the conditions under which an office holder would be required to repay compensation paid to him or her if it was later shown that the data upon which such compensation was based was inaccurate and was required to be restated in the company s financial statements;
the minimum holding or vesting period for variable, equity-based compensation; and
maximum limits for severance compensation.

Under the amendment to the Israeli Companies Law, we are required to adopt an office holder compensation policy no later than .

Compensation Committee *Charter*

Our Board of Directors intends to adopt a compensation committee charter setting forth the responsibilities of the committee, subjecting the compensation committee charter to the requirements under the Israeli Companies Law, as described above.

Compensation Committee *NASDAQ Requirements*

We will announce which members of our Compensation Committee are independent under the listing standards of the NASDAQ Global Market. Gili Cohen has agreed to join our Compensation Committee when it is formed.

Nominating Committee

Our Board of Directors does not currently have a nominating committee, as director nominees are presented by our Board of Directors to our shareholders based upon the nominations made by the Board of Directors itself. We intend to rely upon the exemption available to foreign private issuers under the Listing Rules of the NASDAQ Stock Market

from the NASDAQ listing requirements related to independent director

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oversight of nominations to our Board of Directors and the adoption of a formal written charter or board resolution addressing the nominations process. See NASDAQ Listing Rules and home country practices.

Other than with Gili Cohen, we have service contracts with our directors, Ehud Gilboa, Dr. Dalia Megiddo, Fredric Price, Marlene Haffner and with Pontifax (Cayman) III Limited Partnership and Pontifax (Israel) III Limited Partnership, who are affiliated with our Director, Ran Nusbaum. All of the foregoing service contracts have been approved by our shareholders. Please see Certain Relationships and Related Party Transactions Agreements and Arrangements with, and Compensation of, Directors and Executive Officers for a summary of these agreements.

Internal auditor

Under the Israeli Companies Law, the board of directors of an Israeli public company must appoint an internal auditor recommended by the audit committee and nominated by the board of directors. An internal auditor may not be:

a person (or a relative of a person) who holds more than 5% of the Company s outstanding shares or voting rights;
a person (or a relative of a person) who has the power to appoint a director or the general manager of the Company;
an office holder (including a director) of the Company (or a relative thereof); or
a member of the Company s independent accounting firm, or anyone on his or her behalf.

The role of the internal auditor is to examine, among other things, our compliance with applicable law and orderly business procedures.

NASDAQ Listing Rules and Home Country Practices

The Sarbanes-Oxley Act, as well as related rules subsequently implemented by the SEC, requires foreign private issuers, such as us, to comply with various corporate governance practices. In addition, upon the contemplated listing of our ordinary shares on the NASDAQ Global Market, we will need to comply with the Listing Rules of the NASDAQ Stock Market. Under those Listing Rules, we may elect to follow certain corporate governance practices permitted under the Israeli Companies Law in lieu of compliance with corresponding corporate governance requirements otherwise imposed by the Listing Rules of the NASDAQ Stock Market for U.S. domestic issuers.

In accordance with Israeli law and practice and subject to the exemption set forth in Rule 5615 of the Listing Rules of the NASDAQ Stock Market, if we list on the NASDAQ Global Market we intend to follow the provisions of the Israeli Companies Law, rather than the Listing Rules of the NASDAQ Stock Market, with respect to the following requirements:

Distribution of periodic reports to shareholders; proxy solicitation. As opposed to the Listing Rules of the NASDAQ Stock Market, which require listed issuers to make such reports available to shareholders in one of a number of specific manners, Israeli law does not require us to distribute periodic reports directly to shareholders, and the generally accepted business practice in Israel is not to distribute such reports to shareholders but to make such reports available through a public website. In addition to making such reports available on a public website, we plan to make our audited financial statements available to our shareholders at our offices and will only mail such reports to shareholders upon request. As a foreign private issuer, we are generally exempt from the SEC s proxy solicitation rules.

Nomination of our directors. With the exception of our external directors and directors elected by our Board of Directors due to vacancy, our directors are elected by an annual meeting of our shareholders to hold office until the next annual meeting following one year from his or her election. See Management Board Practices Board of Directors. The nominations for directors, which are presented to our shareholders by our Board of Directors, are

generally made by the Board of Directors itself, in accordance with the provisions of our amended and restated articles of association and the Israeli Companies Law. Nominations need not be made by a nominating committee of our

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Board of Directors consisting solely of independent directors, as required under the Listing Rules of the NASDAQ Stock Market. Nominations may also be made by one or more of our shareholders, as provided in our Articles of Association, or under the Israeli Companies Law.

Compensation of officers. Israeli law and our amended and restated articles of association do not require that the independent members of our Board of Directors (or a compensation committee composed solely of independent members of our Board of Directors) determine an executive officer s compensation, as is generally required under the Listing Rules of the NASDAQ Stock Market with respect to the Chief Executive Officer and all other executive officers.

Instead, compensation of executive officers is determined and approved by our Board of Directors and our Compensation Committee, either in consistency with our office holder compensation policy or, in special circumstances, taking into account certain considerations stated in the Israeli Companies Law.

Shareholder approval shall be further required in the event (i) approval by our Board of Directors and our Compensation Committee is not consistent with our office holders compensation policy, or (ii) compensation required to be approved is that of our chief executive officer or an executive officer who is also the controlling shareholder of our company (including an affiliate thereof). Such shareholder approval shall require a majority vote of the shares present and voting at a shareholders meeting, provided either (i) such majority includes a majority of the shares held by non-controlling shareholders who do not have a personal interest in the compensation arrangement that are voted at the meeting, excluding for such purpose any abstentions disinterested majority, or (ii) the total shares held by non-controlling disinterested shareholders voted against the arrangement does not exceed two percent (2%) of the voting rights in our company.

Additionally, approval of the compensation of an executive officer, who is also a director, shall require a simple majority vote of the shares present and voting at a shareholders meeting, if consistent with our office holders compensation policy or a special majority as set forth above if the proposed compensation for the director is not consistent with our office holders compensation policy. Our Compensation Committee may, in special circumstances, approve the compensation of an executive officer (other than a director or a controlling shareholder) despite shareholders objection, based on specified arguments and taking shareholders objection into account. Our Compensation Committee may exempt an engagement with a nominee for the position of chief executive officer, who meets the non-affiliation requirements set forth for an external director, from requiring shareholders approval, if such engagement is consistent with our office holders compensation policy and our Compensation Committee determines based on specified arguments that presentation of such engagement to shareholders approval is likely to prevent such engagement. To the extent that any such transaction with a controlling shareholder is for a period extending beyond three years, approval is required once every three years.

A director or executive officer may not be present when the compensation committee or board of directors of a company discusses or votes upon the terms of his or her compensation, unless the chairman of the compensation committee or board of directors (as applicable) determines that he or she should be present to present the transaction that is subject to approval.

Independent directors. Israeli law does not require that a majority of the directors serving on our Board of Directors be independent, as defined under NASDAQ Listing Rule 5605(a)(2), and rather requires we have at least two external directors who meet the requirements of the Israeli Companies Law, as described above under Management Board Practices External Directors. We are required, however, to ensure that all members of our Audit Committee are independent under the applicable NASDAQ and SEC criteria for independence (as we cannot exempt ourselves from compliance with that SEC independence requirement, despite our status as a foreign private issuer), and we must also ensure that a majority of the members of our Audit Committee are unaffiliated directors as defined in the Israeli Companies Law. Furthermore, Israeli law does not require, nor do our independent directors conduct, regularly

scheduled meetings at which only they are present, which the NASDAQ Listing Rules otherwise require.

Shareholder approval. We will seek shareholder approval for all corporate actions requiring such approval under the requirements of the Israeli Companies Law, rather than seeking approval for

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corporation actions in accordance with NASDAQ Listing Rule 5635. In particular, under this NASDAQ rule, shareholder approval is generally required for: (i) an acquisition of shares/assets of another company that involves the issuance of 20% or more of the acquirer s shares or voting rights or if a director, officer or 5% shareholder has greater than a 5% interest in the target company or the consideration to be received; (ii) the issuance of shares leading to a change of control; (iii) adoption/amendment of equity compensation arrangements; and (iv) issuances of 20% or more of the shares or voting rights (including securities convertible into, or exercisable for, equity) of a listed company via a private placement (and/or via sales by directors/officers/5% shareholders) if such equity is issued (or sold) below the greater of the book or market value of shares. By contrast, under the Israeli Companies Law, shareholder approval is required for, among other things: (i) transactions with directors concerning the terms of their service or indemnification, exemption and insurance for their service (or for any other position that they may hold at a company), for which approvals of the compensation committee, board of directors and shareholders are all required, (ii) extraordinary transactions with controlling shareholders of publicly held companies, which require the special approval described below under Approval of related party transactions under Israeli Law Disclosure of personal interests of controlling shareholders , and (iii) terms of employment or other engagement of the controlling shareholder of the Company or such controlling shareholder s relative, which require the special approval described below under Approval of related party transactions under Israeli Law Disclosure of personal interests of controlling shareholders . In addition, under the Israeli Companies Law, a merger requires approval of the shareholders of each of the merging companies.

Approval of Related Party Transactions under Israeli Law

Fiduciary duties of directors and executive officers

The Israeli Companies Law codifies the fiduciary duties that office holders owe to a company. Each person listed in the table under Management Executive officers and directors is an office holder under the Israeli Companies Law.

An office holder s fiduciary duties consist of a duty of care and a duty of loyalty. The duty of care requires an office holder to act with the level of care with which a reasonable office holder in the same position would have acted under the same circumstances. The duty of loyalty requires that an office holder act in good faith and in the best interests of the Company. The duty of care includes a duty to use reasonable means to obtain:

information on the advisability of a given action brought for his or her approval or performed by virtue of his or her position; and

all other important information pertaining to these actions.

The duty of loyalty requires an office holder to act in good faith and for the benefit of the Company, and includes a duty to:

refrain from any conflict of interest between the performance of his or her duties to the Company and his or her other duties or personal affairs;

refrain from any activity that is competitive with the Company;

refrain from exploiting any business opportunity of the Company to receive a personal gain for himself or herself or others; and

disclose to the Company any information or documents relating to the Company s affairs which the office holder received as a result of his or her position as an office holder.

Disclosure of Personal Interests of an Office Holder

The Israeli Companies Law requires that an office holder promptly disclose to the board of directors any personal interest that he or she may have concerning any existing or proposed transaction with the Company, as well as any substantial information or document with respect thereof. An interested office holder s disclosure must be made promptly and in any event no later than the first meeting of the board of directors at

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which the transaction is considered. A personal interest includes an interest of any person in an act or transaction of a company, including a personal interest of one s relative or of a corporate body in which such person or a relative of such person is a 5% or greater shareholder, director or general manager or in which he or she has the right to appoint at least one director or the general manager, but excluding a personal interest stemming from one s ownership of shares in the Company. A personal interest furthermore includes the personal interest of a person for whom the office holder holds a voting proxy or the interest of the office holder with respect to his or her vote on behalf of the shareholder for whom he or she holds a proxy even if such shareholder itself has no personal interest in the approval of the matter. An office holder is not, however, obliged to disclose a personal interest if it derives solely from the personal interest of a relative of such office holder in a transaction that is not considered an extraordinary transaction. Under the Israeli Companies Law, an extraordinary transaction is defined as any of the following:

a transaction other than in the ordinary course of business;

a transaction that is not on market terms; or

a transaction that may have a material impact on a company s profitability, assets or liabilities.

If it is determined that an office holder has a personal interest in a transaction which is not an extraordinary transaction, approval by the board of directors is required for the transaction, unless the Company s articles of association provide for a different method of approval. Further, so long as an office holder has disclosed his or her personal interest in a transaction, the board of directors may approve an action by the office holder that would otherwise be deemed a breach of duty of loyalty. However, a company may not approve a transaction or action that is adverse to the Company s interest or that is not performed by the office holder in good faith. Approval first by the Company s audit committee and subsequently by the board of directors is required for an extraordinary transaction in which an office holder has a personal interest. Arrangements regarding the compensation, indemnification or insurance of an office holder require the approval of the compensation committee, board of directors and, in certain circumstances, the shareholders, in that order, as described above under NASDAQ Listing Rules and home country practices Compensation of officers and NASDAQ Listing Rules and home country practices Shareholder approval.

Generally, except with respect to non-extraordinary transactions, a person who has a personal interest in a matter which is considered at a meeting of the board of directors or the audit committee may not be present at such a meeting or vote on that matter unless a majority of the directors or members of the audit committee have a personal interest in the matter, or unless the chairman of the audit committee or board of directors (as applicable) determines that he or she should be present in order to present the transaction that is subject to approval. Generally, if a majority of the members of the audit committee and/or the board of directors has a personal interest in the approval of a transaction, then all directors may participate in discussions of the audit committee and/or the board of directors on such transaction and the voting on approval thereof, but shareholder approval is also required for such transaction.

Disclosure of a personal interest is also required of a person who is an interested party with respect to (i) a private placement submitted for approval whereby 20% or more of the company s outstanding share capital prior to the placement is offered, and the payment for which is not only in cash or in tradable securities registered in a stock exchange, or that is not at market terms, and which will result in an increase of the holdings of a shareholder that already holds 5% or more of the company s outstanding share capital or voting rights or will cause any person to become, as a result of the issuance, a holder of more than 5% of the company s outstanding share capital or voting rights, or (ii) that as a result of a private placement submitted for approval will become a controlling shareholder. Such personal interest disclosure requirements also apply to certain shareholders of a public company who have a personal interest in the adoption by the shareholders of certain proposals with respect to (i) certain special tender offers or forced bring along share purchase transactions, (ii) election of external directors, (iii) approval of a compensation policy governing the terms of employment and compensation of office holders, (iv) approval of the terms of employment and compensation of the general manager, (v) approval of the terms of employment and compensation of office holders of the company when such terms deviate from the compensation policy previously approved by the

company s shareholders, and (vi) approving the appointment of either (1) the chairman of the board or his/her relative as the chief executive officer of the company, or (2) the chief executive officer or his/her relative as the chairman

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of the board of directors of the company. If any shareholder casting a vote at a shareholders meeting in connection with such proposals as aforesaid does not notify the company if he, she or it has a personal interest with respect to such proposal, his, her or its vote with respect to the proposal will be disqualified.

Disclosure of Personal Interests of Controlling Shareholders

The disclosure requirements regarding personal interests that apply to directors and executive officers also apply to controlling shareholders, as defined below. The Israeli Companies Law requires a special approval procedure for (1) extraordinary transactions with controlling shareholders, (2) extraordinary transactions with a third party where a controlling shareholder has a personal interest in the transaction, and (3) any transaction with the controlling shareholder or the controlling shareholder s relative regarding terms of service provided directly or indirectly (including through a company controlled by the controlling shareholder) and terms of employment (for a controlling shareholder who is not an office holder). A relative is defined in the Companies Law as spouse, sibling, parent, grandparent, descendant, spouse s descendant, sibling, parent, or the spouse of any of the foregoing.

Such extraordinary transactions with controlling shareholders require the approval of the audit committee or the compensation committee, as applicable, the board of directors and the majority of the voting power of the shareholders present and voting at the general meeting of the company (not including abstentions), provided that either:

- the majority of the shares of shareholders who have no personal interest in the transaction and who are present and voting, vote in favor; or
- shareholders who have no personal interest in the transaction who vote against the transaction do not represent more than two percent of the aggregate voting rights in the company.

Any shareholder participating in the vote on approval of an extraordinary transaction with a controlling shareholder must inform the company prior to the voting whether or not he or she has a personal interest in the approval of the transaction, and if he or she fails to do so, his or her vote will be disregarded.

Further, extraordinary transactions with controlling shareholders, extraordinary transactions with a third party where a controlling shareholder has a personal interest in the transaction, or transactions with a controlling shareholder or his or her relative concerning terms of service or employment need to be re-approved once every three years, provided, however, that with respect to extraordinary transactions with controlling shareholders or extraordinary transaction with a third party where a controlling shareholder has a personal interest in the transaction, the audit committee may determine that the duration of the transaction in excess of three years is reasonable given the circumstances related thereto.

In accordance with regulations promulgated under the Israeli Companies Law, certain defined types of extraordinary transactions between a public company and its controlling shareholder or controlling shareholders are exempt from the shareholder approval requirements if the audit committee and the board of directors determine that the transaction meets the conditions for such type of transaction which are set forth in the regulations. Examples of such transactions are stipulated in the regulations and include transactions being entered in pursuant to a framework arrangement whose terms and conditions and length of validity were established beforehand and was approved as such a framework by the shareholders; an extension of a transaction which was previously approved by the shareholders without any significant change to the terms and conditions; or transactions that can only benefit the company. However, such exemptions will not apply if one or more shareholders holding at least 1% of the issued and outstanding shares or voting rights, objects to the use of these exemptions in writing not later than 14 days from the date the company notifies its shareholders of the adoption by the relevant corporate bodies of the resolution regarding the transaction without shareholder approval in reliance upon such exemption.

In addition, the approval of the audit committee, followed by the approval of the board of directors and the shareholders, is required in order to effect a private placement of securities, in which either (i) 20% or more of the company s outstanding share capital prior to the placement is offered, and the payment for which is not only in cash or in tradable securities registered in a stock exchange, or that is not at market terms, and which will result in an increase of the holdings of a shareholder that already holds 5% or more of the company s outstanding share capital or voting rights or will cause any person to become, as a result of the

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issuance, a holder of more than 5% of the company s outstanding share capital or voting rights or (ii) a person will become a controlling party in the company.

A controlling shareholder is defined in the Israeli Companies Law for purposes of the provisions governing related party transactions and office holder compensation as a person with the ability to direct the actions of a company, or a person who holds 25% or more of the voting power in a public company if no other shareholder owns more than 50% of the voting power in the company, but excluding a person whose power derives solely from his or her position as a director of the company or any other position with the company. Any two or more persons holding voting rights in the company, who each have a personal interest in the approval of the same such transaction, shall be deemed to be one holder with respect thereto.

Arrangements regarding the terms of engagement and compensation of a controlling shareholder who is an office holder, and the terms of employment of a controlling shareholder who is an employee of the Company, require the approval of the compensation committee, board of directors and, generally, the shareholders, in that order, as described above under NASDAQ Listing Rules and home country practices Compensation of officers .

Shareholder Duties

Pursuant to the Israeli Companies Law, a shareholder has a duty to act in good faith and in a customary manner toward the Company and other shareholders and to refrain from abusing his or her power in the Company, including, among other things, in voting at the general meeting of shareholders and at class shareholder meetings with respect to the following matters:

an amendment to the Company s articles of association;
an increase of the Company s authorized share capital;
a merger;
approval of interested party transactions and acts of office holders that require shareholder approval; or
approval of the compensation policy.

In addition, a shareholder also has a general duty to refrain from discriminating against other shareholders.

Certain shareholders have a further duty of fairness toward the Company. These shareholders include any controlling shareholder, any shareholder who knows that it has the power to determine the outcome of a shareholder vote or a shareholder class vote and any shareholder who has the power to appoint or to prevent the appointment of an office holder of the Company or other power towards the Company. The Israeli Companies Law does not define the substance of this duty of fairness, except to state that the remedies generally available upon a breach of contract will also apply in the event of a breach of the duty to act with fairness.

Exculpation, Insurance and Indemnification of Directors and Officers

Under the Israeli Companies Law, a company may not exculpate an office holder from liability for a breach of the duty of loyalty. An Israeli company may exculpate an office holder in advance from liability to the Company, in whole or in part, for damages caused to the Company as a result of a breach of duty of care but only if a provision authorizing such exculpation is included in its articles of association. Our amended and restated articles of association include such a provision. The company may not exculpate in advance a director from liability arising out of a prohibited dividend or distribution to shareholders.

Under the Israeli Companies Law and the Israeli Securities Law, a company may indemnify, or undertake in advance to indemnify, an office holder for the following liabilities and expenses, imposed on office holder or incurred by office holder due to acts performed by him or her as an office holder, provided its articles of association include a provision authorizing such indemnification:

financial liability incurred by or imposed on him or her in favor of another person pursuant to a judgment, including a settlement or arbitrator s award approved by a court. However, if an

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undertaking to indemnify an office holder with respect to such liability is provided in advance, then such an undertaking must be limited to events which, in the opinion of the board of directors, can be foreseen based on the Company s activities when the undertaking to indemnify is given, and to an amount or according to criteria determined by the board of directors as reasonable under the circumstances, and such undertaking shall detail the abovementioned foreseen events and amount or criteria;

reasonable litigation expenses, including attorneys fees, incurred by the office holder as a result of an investigation or proceeding instituted against him or her by an authority authorized to conduct such investigation or proceeding, provided that (i) no indictment was filed against such office holder as a result of such investigation or proceeding; and (ii) no financial liability was imposed upon him or her as a substitute for the criminal proceeding as a result of such investigation or proceeding or, if such financial liability was imposed, it was imposed with respect to an offense that does not require proof of criminal intent or as a monetary sanction (the items (i) and (ii) above shall have the meanings ascribed to them in section 260(a)(1a) of the Israeli Companies Law);

reasonable litigation expenses, including attorneys fees, incurred by the office holder or imposed by a court in proceedings instituted against him or her by the Company, on its behalf, or by a third party, or in connection with criminal proceedings in which the office holder was acquitted, or as a result of a conviction for an offense that does not require proof of criminal intent; and

expenses, including reasonable litigation expenses and legal fees, incurred by an office holder in relation to an administrative proceeding instituted against such office holder, or certain compensation payments required to be made to an injured party, pursuant to certain provisions of the Israeli Securities Law.

Under the Israeli Companies Law, a company may insure an office holder against the following liabilities incurred for acts performed by him or her as an office holder if and to the extent provided in the Company s articles of association:

a breach of the duty of loyalty to the Company, provided that the office holder acted in good faith and had a reasonable basis to believe that the act would not harm the Company;

a breach of duty of care to the Company or to a third party; and

a financial liability imposed on the office holder in favor of a third party.

Without derogating from the aforementioned, subject to the provisions of the Israeli Companies Law and the Israeli Securities Law, we may also enter into a contract to insure an office holder, in respect of expenses, including reasonable litigation expenses and legal fees, incurred by an office holder in relation to an administrative proceeding instituted against such office holder or payment required to be made to an injured party, pursuant to certain provisions of the Israeli Securities Law.

Nevertheless, under the Israeli Companies Law, a company may not indemnify, exculpate or insure an office holder against any of the following:

a breach of fiduciary duty, except for indemnification and insurance for a breach of the duty of loyalty to the Company in the event office holder acted in good faith and had a reasonable basis to believe that the act would not prejudice the Company;

a breach of duty of care committed intentionally or recklessly, excluding a breach arising out of the negligent conduct of the office holder;

an act or omission committed with intent to derive unlawful personal benefit; or

a fine, monetary sanction, penalty or forfeit levied against the office holder.

Under the Israeli Companies Law, exculpation, indemnification and insurance of office holders require the approval of the compensation committee, board of directors and, in certain circumstances, the shareholders, as described above under NASDAQ Listing Rules and home country practices Compensation of officers

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Our amended and restated articles of association permit us to exculpate, indemnify and insure our office holders to the fullest extent permitted by the Israeli Companies Law and Israeli Securities Law.

We have obtained directors and officers liability insurance for the benefit of our office holders and intend to continue to maintain such coverage and pay all premiums thereunder to the fullest extent permitted by the Israeli Companies Law and Israeli Securities Law. In addition, we have entered into agreements with each of our office holders undertaking to indemnify them to the fullest extent permitted by Israeli law, including with respect to liabilities resulting from this offering to the extent that these liabilities are not covered by insurance.

Code of Business Conduct and Ethics

We have adopted, effective upon the consummation of this offering, a Code of Business Conduct and Ethics applicable to all of our directors and employees, including our Chief Executive Officer, Chief Financial Officer, controller or principal accounting officer, or other persons performing similar functions, which is a code of ethics as defined in Item 16B of Form 20-F promulgated by the SEC. The full text of the Code of Business Conduct and Ethics is posted on our website at *www.bioblast-pharma.com*. If we make any amendment to the Code of Business Conduct and Ethics or grant any waivers, including any implicit waiver, from a provision of the code of ethics, we will disclose the nature of such amendment or waiver to the extent required by the rules and regulations of the SEC. Under Item 16B of the SEC s Form 20-F, if a waiver or amendment of the Code of Business Conduct and Ethics applies to our principal executive officer, principal financial officer, principal accounting officer or controller and relates to standards promoting any of the values described in Item 16B(b) of such Form 20-F, we will disclose such waiver or amendment in accordance with the requirements of Instruction 4 to such Item 16B.

Compensation of Executive Officers and Directors

The aggregate compensation, including share-based compensation, paid by us to our directors and executive officers with respect to the year ended December 31, 2013 was approximately $0.5 million. This amount does not include business travel, relocation, professional and business association due and expenses reimbursed to office holders, and other benefits commonly reimbursed or paid by companies in our industry. Our current directors and executive officers are not eligible for pension, retirements or similar benefits. Upon the consummation of this offering, each member of our Board of Directors, other than our Executive Chairman of the Board, executive officers and external directors, shall be entitled to an annual service fee of $25,000 plus Value Added Tax (currently at 18%), if applicable, to be paid quarterly in arrears.

As of July 30, 2014, options to purchase 83,579 ordinary shares were issued to Mrs. Marlene Haffner, a director of our Company. Of such outstanding options, options to purchase 27,860 ordinary shares were vested as of January 1, 2014 and May 13, 2014, respectively, with an exercise price of $0.0004 per share. The expiration date of all of these options is May 13, 2023. 27,859 additional options shall vest on May 13, 2015.

As of July 30, 2014, options to purchase 319,531 ordinary shares were issued to Mr. Fredric Price, the Executive Chairman of our Board of Directors. Of such outstanding options, options to purchase 106,510 ordinary shares were vested as of January 1, 2014 and April 24, 2014, respectively, with an exercise price of $0.0004 per share. The expiration date of all of these options is April 24, 2022. 106,511 additional options shall vest on April 24, 2015. In addition, upon the consummation of this offering, options to purchase 206,702 ordinary shares will be issued to Mr. Price at an exercise price equal to the offering price per share of this offering. These options shall vest in accordance with the 2013 Incentive Option Plan. Also upon the consummation of this offering, the annual compensation of Mr. Price shall be $120,000.

We do not have written agreements with any director providing for benefits upon the termination of their employment with our company.

Employment or Service Agreements with Executive Officers; Consulting and Directorship Services Provided by Directors

We have entered into an amended and restated written service agreement with our chief executive officer, Dr. Dalia Megiddo through her wholly-owned company, DM Medica Ltd. This agreement contains provisions standard for a company in our industry regarding non-competition, confidentiality of information and assignment of inventions.

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We have entered into an amended and restated written service agreement with our chief financial officer, Mr. Udi Gilboa, through his wholly owned company Top Notch Consultancy (2009) Ltd. This agreement contains provisions standard for a company in our industry regarding non-competition, confidentiality of information and assignment of inventions.

Under current applicable Israeli employment laws, we may not be able to enforce (either in whole or in part) covenants not to compete and therefore may be unable to prevent our competitors from benefiting from the expertise of some of our former employees. Please see Risk factors Risks Relating to Our Business and Industry. for a further description of the enforceability of non-competition clauses. See Certain Relationships and Related Party Transactions Agreements and Arrangements with, and Compensation of, Directors and Executive Officers for additional information.

2013 Incentive Option Plan

We maintain one equity incentive plan our 2013 Incentive Option Plan, or our 2013 Plan. As of July 30, 2014, a total of 269 shares were reserved for issuance under our 2013 Plan. In addition, options to purchase 456,630 ordinary shares were issued and outstanding. Of such outstanding options, options to purchase 136,500 ordinary shares were vested as of that date, with a weighted average exercise price of $0.0004 per share.

Our 2013 Plan, which was adopted by our Board of Directors on November 13, 2013, provides for the grant of options to our and our affiliates respective directors, employees, office holders, service providers and consultants.

The 2013 Plan is administered by our Board of Directors, which shall determine, subject to Israeli law, the grantees of awards and various terms of the grant. The 2013 Plan provides for granting options in compliance with Section 102 of the Israeli Income Tax Ordinance, 1961, or the Ordinance.

Section 102 of the Ordinance allows employees, directors and officers, who are not controlling shareholders and are considered Israeli residents, to receive favorable tax treatment for compensation in the form of shares or options. Our Israeli non-employee service providers and controlling shareholders may only be granted options under Section 3(9) of the Ordinance, which does not provide for similar tax benefits. Section 102 of the Ordinance includes two alternatives for tax treatment involving the issuance of options or shares to a trustee for the benefit of the grantees and also includes an additional alternative for the issuance of options or shares directly to the grantee. Section 102(b)(2) of the Ordinance, the most favorable tax treatment for grantees, permits the issuance to a trustee under the capital gains track. However, under this track we are not allowed to deduct an expense with respect to the issuance of the options or shares. In order to comply with the terms of the capital gains track, all options granted under the 2013 Plan pursuant and subject to the provisions of Section 102 of the Ordinance, as well as the ordinary shares issued upon exercise of these options and other shares received subsequently following any realization of rights with respect to such options, such as share dividends and share splits, must be granted to a trustee for the benefit of the relevant employee, director or officer and should be held by the trustee for at least two years after the date of the grant.

Options granted under the 2013 Plan will generally vest over four years commencing on the date of grant such that 25% vest after one year and an additional 6.25% vest at the end of each subsequent three-month period thereafter for 36 months. Options that are not exercised within ten years from the grant date expire, unless otherwise determined by the Board or its designated committee, as applicable. In case of termination for reasons of disability or death, the grantee or his legal successor may exercise options that have vested prior to termination within a period of six months from the date of disability or death. If we terminate a grantee s employment or service for cause, all of the grantee s vested and unvested options will expire on the date of termination. If a grantee s employment or service is terminated

for any other reason, the grantee may exercise his or her vested options within 30 days of the date of termination. Any expired or unvested options return to the pool for reissuance.

In the event of a merger or consolidation of our company subsequent to which we shall no longer exist as a legal entity, or a sale of all, or substantially all, of our shares or assets or other transaction having a similar effect on us, then any outstanding option shall be assumed, or an equivalent option shall be

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substituted, by such successor corporation or an affiliate thereof or, in case the successor corporation refuses to assume or substitute the option, our Board of Directors or its designated committee may (a) provide the grantee with the opportunity to exercise the option as to all or part of the shares, vested or otherwise, and (b) specify a period of time, no less than 7 days, following which all outstanding options shall terminate.

Certain relationships and related party transactions

The following is a description of the material terms of those transactions with related parties to which we, or our subsidiaries, are party and which we are required to disclose pursuant to the disclosure rules of the SEC.

We entered into a service agreement, dated July 1, 2013, with Pontifax (Cayman) III Limited Partnership and Pontifax (Israel) III Limited Partnership (together, Pontifax). Under the terms of the service agreement, Pontifax is entitled to a gross monthly consideration of $1,000 plus Value Added Tax (currently at 18%). Pontifax s service engagement is automatically terminated on the second anniversary date of this agreement, and if extended thereafter, terminable by either party upon thirty days prior written notice. The service agreement further contains a customary provision regarding confidentiality of information and explicitly state that all work products shall belong to the Company. Our Director, Ran Nusbaum, is a managing partner of The Pontifax Group, which operates the Pontifax limited partnerships, which invest pro rata in Israeli and Israeli-related high growth pharmaceutical, biotechnology and medical devise companies.

Agreements and Arrangements With, and Compensation of, Directors and Executive Officers

Service Agreement with DM Medica Ltd

We have entered into an amended and restated service agreement, dated April 22, 2014, with our Chief Executive Officer, Dr. Dalia Megiddo, through her wholly owned service providing company, DM Medica Ltd. Subject to the consummation of this offering, under the terms of her service agreement, Dr. Megiddo is entitled to a gross monthly fee of $19,287 plus Value Added Tax (currently at 18%). Dr. Megiddo s service engagement is terminable by either party upon 60 days prior written notice, and contains customary provisions regarding noncompetition, confidentiality of information and assignment of inventions. DM Medica Ltd. is also entitled to a one-time bonus of $90,000 plus Value Added Tax upon consummation of this offering.

Services Agreement with Top Notch Consultancy (2009) Ltd.

We have entered into an amended and restated service agreement, dated April 22, 2014, with our chief financial officer, Mr. Udi Gilboa, through his wholly-owned company Top Notch Consultancy (2009) Ltd. for part time CFO services, effective as of August 1, 2013 and terminable by either party upon 60 days prior written notice, and contains provisions standard for a company in our industry regarding non-competition, confidentiality of information and assignment of inventions. Subject to the consummation of this offering, the monthly amount payable under the agreement is NIS 57,861 plus Value Added Tax (currently at 18%). Top Notch Consultancy (2009) Ltd. is also entitled to a one-time bonus of $80,000 plus Value Added Tax upon consummation of this offering. In addition, effective as of September 10, 2013, we entered into an agreement with Top Notch Consultancy (2009) Ltd. for providing the Company with office space and services for a monthly fee of NIS 14,832 (approximately $4,250), plus Value Added Tax (currently at 18%). Either party may terminate the agreement on 30-days notice.

Engagement Letter with Fredric Price

Fredric Price, our Executive Chairman of the Board of Directors, has been appointed by the Company to its Board of Directors pursuant to an engagement letter agreement, effective April 24, 2012, as amended April 22, 2014 and terminable by either party upon 30 days prior written notice. We granted Mr. Price an aggregate of 319,531 options to purchase our ordinary shares, of which 213,021 have vested as of July 30, 2014. In addition, contingent upon the consummation of this offering, we granted Mr. Price 206,702 options to purchase our ordinary shares at an exercise price equal to the offering price per share of the offering, which options shall vest in accordance with the 2013 Plan.

Engagement Letter with Marlene Haffner

Marlene Haffner, a Director of our Company, has been appointed by the Company to its Board of Directors pursuant to an engagement letter agreement, effective May 13, 2013 and terminable by either party

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upon 30 days prior written notice. We granted Mrs. Haffner an aggregate of 83,579 options to purchase our ordinary shares, of which 55,719 have vested as of July 30, 2014.

Indemnification Agreements

Our amended and restated articles of association permit us to exculpate, indemnify and insure each of our directors and office holders to the fullest extent permitted by the Israeli Companies Law and the Israeli Securities Law. We have also obtained Directors & Officers insurance for each of our officers and directors. For further information, see Management Exculpation, insurance and indemnification of directors and officers.

Participation in this Offering

Certain of our existing investors and their affiliated entities, as well as certain of our directors, have indicated an interest in purchasing up to an aggregate of approximately $10.0 million worth of our ordinary shares in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may sell more, less or no shares in this offering to any of these persons, or any of these persons may determine to purchase more, less or no shares in this offering. The underwriters will receive the same underwriting discounts and commissions on any shares purchased by these persons as they will on any other shares sold to the public in this offering.

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PRINCIPAL SHAREHOLDERS

The following table sets forth information regarding beneficial ownership of our ordinary shares as of the date of this prospectus by:

each person, or group of affiliated persons, known to us to be the beneficial owner of more than 5% of our outstanding ordinary shares;

each of our directors and executive officers; and

all of our directors and executive officers as a group.

Beneficial ownership is determined in accordance with the rules of the SEC, and includes voting or investment power with respect to ordinary shares. Ordinary shares issuable under share options or warrants that are exercisable within 60 days after the date of this prospectus are deemed outstanding for the purpose of computing the percentage ownership of the person holding the options or warrants but are not deemed outstanding for the purpose of computing the percentage ownership of any other person. Percentage of shares beneficially owned before this offering is based on 11,030,480 shares outstanding on the date of this prospectus. The number of ordinary shares deemed outstanding after this offering includes the ordinary shares being offered for sale in this offering but assumes no exercise of the underwriter s over-allotment option and no potential purchase of any ordinary shares in this offering by these shareholders.

As of the date of this prospectus, there were thirty one record holders of our ordinary shares. None of our shareholders has different voting rights from other shareholders. To the best of our knowledge, we are not owned or controlled, directly or indirectly, by another corporation or by any foreign government. We are not aware of any arrangement that may, at a subsequent date, result in a change of control of our Company.

Except as indicated in footnotes to this table, we believe that the shareholders named in this table have sole voting and investment power with respect to all shares shown to be beneficially owned by them, based on information provided to us by such shareholders. Unless otherwise noted below, each shareholder s address is: c/o Bio Blast Pharma Ltd., 37 Dereh Menachem Begin St., Tel Aviv 6522042, Israel.

	No. of Shares Beneficially Owned Prior to this Offering	Percentage Owned Before this Offering	Percentage Owned After this Offering
Holders of more than 5% of our voting securities:			
Udi Gilboa	3,288,694	29.8 %	23.1 %
Dr. Dalia Megiddo	3,288,694	29.8 %	23.1 %
Pontifax[1]	2,229,008	20.2 %	15.7 %
Directors and executive officers who are not 5% holders:			
Gili Cohen			
Fredric Price[2]	227,265	2.0 %	1.6 %
Marlene Haffner[3]	27,857	0.3 %	0.2 %
Prof. Avizohar Argov			
All directors and executive officers as a group (6 persons)	6,832,510	61.9 %	48.0 %

(1)

Comprised of Pontifax (Cayman) III Limited Partnership that holds 709,431 ordinary shares of the Company and Pontifax (Israel) III Limited Partnership who holds 1,519,577 ordinary shares of the Company. These two entities are under common control of and are affiliated with our Director, Ran Nusbaum.

(2) Comprised of 120,752 ordinary shares and 106,513 ordinary shares issuable upon the exercise of outstanding options that are vested within 60 days of the date hereof. The exercise price of these options is $0.0004 per share and the options expire on April 24, 2022.

(3) Comprised of 27,860 ordinary shares issuable upon the exercise of outstanding options that are vested within 60 days of the day hereof. The exercise price of these options is $0.0004 per share and the options expire on May 13, 2023.

Record Holders

As of the date of this prospectus, there were 31 holders of record of our ordinary shares, of which 17 record holders holding approximately 7.26% of our outstanding shares had registered addresses in the United States.

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DESCRIPTION OF SHARE CAPITAL

The following description of our share capital and provisions of our amended and restated articles of association which will be adopted immediately prior to the consummation of this offering are summaries and do not purport to be complete.

General

Ordinary Shares

Immediately prior to the consummation of this offering, effective upon the adoption of our amended and restated articles of association, our authorized share capital will consist of 50,000,000 ordinary shares, par value NIS 0.01 per share, of which 11,030,480 shares will be issued and outstanding.

All of our outstanding ordinary shares are validly issued, fully paid and non-assessable. They are not redeemable and do not have any preemptive rights.

Options

As of July 30, 2014, we had the following options outstanding:

Options issued to the Executive Chairman of the Board. We issued to Mr. Fredric Price, our Executive Chairman of the Board, options to purchase up to 319,531 ordinary shares at an exercise price of $0.0004 per share. These options shall expire on April 24, 2022.
Options issued to one of our board members. We issued to Mrs. Marlene Haffner, a Director of our Company, options to purchase up to 83,579 ordinary shares at an exercise price of $0.0004 per share. These options shall expire on May 13, 2023.
Options issued to one of our employees. We issued to Dr. Hagar Greif, Director of Pre-clinical Studies, options to purchase up to 8,520 ordinary shares at an exercise price of $0.95 per share. These options shall expire on April 1, 2024.

Share History

The following is a summary of the history of our share capital since the Company s inception.

February 2012 Subscription Agreement. On February 13, 2012, we closed the February 2012 Subscription Agreement, pursuant to which we sold an aggregate of 471,964 series A preferred shares, at a price of $0.53 per share.

August 2012 Joinder to a Subscription Agreement. On August 6, 2012, we closed the August 2012 Joinder to a Subscription Agreement, pursuant to which we sold an aggregate of 94,393 series A preferred shares, at a price of $0.53 per share.

June 2013 Share Purchase Agreement. On July 1, 2013, we closed the first closing of the June 2013 Share Purchase Agreement, with the second closing occurred on January 1st, 2014, pursuant to which we sold an aggregate of 2,130,159 of our ordinary shares at a price of $0.95 per share.

Conversion of Preferred Shares to Ordinary Shares. In connection with the June 2013 Share Purchase Agreement, on June 19, 2013, all classes of preferred shares in the Company s share capital, including all preferred shares issued and outstanding, were converted to ordinary shares on a one for one basis, resulting in a one-class capital structure consisting solely of ordinary shares.

February 2014 Private Placement. On February 6, 2014, we closed a private placement, pursuant to which we sold an aggregate of 782,537 of our ordinary shares at a price of $6.07 per share.

Share Options. Since the Company s inception, we have granted options to purchase a total of 456,630 ordinary shares upon the exercise of such share options. None of such options were exercised up to date, and all of the options are still outstanding. In addition, contingent upon consummation of this offering, we have granted options to purchase a total of 206,702 ordinary shares.

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Voting Rights

Holders of our ordinary shares have one vote for each ordinary share held on all matters submitted to a vote before the shareholders at a general meeting.

Transfer of Shares

Our ordinary shares that are fully paid for are issued in registered form and may be freely transferred under our amended and restated articles of association, unless the transfer is restricted or prohibited by applicable law or the rules of a stock exchange on which the shares are traded. The ownership or voting of our ordinary shares by non-residents of Israel is not restricted in any way by our amended and restated articles of association or the laws of the State of Israel, except under certain circumstances for ownership by nationals of some countries that are, or have been, in a state of war with Israel.

Election of Directors

Our ordinary shares do not have cumulative voting rights in the election of directors. As a result, the holders of a majority of the voting power represented at a shareholders meeting have the power to elect all of our directors, subject to the special approval requirements for external directors in accordance with Israeli Companies Law which are described under Management External directors.

Our directors hold office for their scheduled term unless they are removed from office upon the occurrence of certain events, in accordance with the Israeli Companies Law and our amended and restated articles of association. In addition, our amended and restated articles of association allow our Board of Directors to appoint directors to fill vacancies on the Board of Directors to serve for a term of office equal to the remaining period of the term of office of the directors(s) whose office(s) have been vacated. External directors are elected for an initial term of three years, may be elected for additional terms of three years each under certain circumstances, and may be removed from office pursuant to the terms of the Israeli Companies Law. See Management Board practices External directors.

Dividend and Liquidation Rights

We may declare a dividend to be paid to the holders of our ordinary shares in proportion to their respective shareholdings. Under the Israeli Companies Law, dividend distributions are determined by the board of directors and do not require the approval of the shareholders of a company unless the Company s articles of association provide otherwise. Our amended and restated articles of association do not require shareholder approval of a dividend distribution and provide that dividend distributions may be determined by our Board of Directors.

Pursuant to the Israeli Companies Law, the distribution amount is limited to the greater of retained earnings or earnings generated over the previous two years, as such are defined in the Israeli Companies Law, according to our then last reviewed or audited financial reports, provided that the date of the financial reports is not more than six months prior to the date of distribution, or we may distribute dividends that do not meet such criteria only with court approval. Where court approval is required, we will only be permitted to pay a dividend if the court determined that there is no reasonable concern that payment of the dividend will prevent us from satisfying our existing and foreseeable obligations as they become due.

In the event of our liquidation, after satisfaction of liabilities to creditors, our assets will be distributed to the holders of our ordinary shares in proportion to their shareholdings. This right, as well as the right to receive dividends, may be affected by the grant of preferential dividend or distribution rights to the holders of a class of shares with preferential rights that may be authorized in the future.

Exchange Controls

There are currently no Israeli currency control restrictions on remittances of dividends on our ordinary shares, proceeds from the sale of the shares or interest or other payments to non-residents of Israel, except under certain circumstances for shareholders who are subjects of countries that are, or have been, in a state of war with Israel.

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Shareholder Meetings

Under Israeli law, we are required to hold an annual general meeting of our shareholders once every calendar year that must be no later than 15 months after the date of the previous annual general meeting. All meetings other than the annual general meeting of shareholders are referred to as special meetings. Our Board of Directors may call special meetings whenever it sees fit, at such time and place, within or outside of Israel, as it may determine. In addition, the Israeli Companies Law provides that our Board of Directors is required to convene a special meeting upon the written request of (i) any two of our directors or one-quarter of our Board of Directors, or (ii) one or more shareholders holding, in the aggregate, either (a) 5% of our outstanding issued shares and 1% of our outstanding voting power, or (b) 5% of our outstanding voting power.

Subject to the provisions of the Israeli Companies Law and the regulations promulgated thereunder, shareholders entitled to participate and vote at general meetings are the shareholders of record on a date to be decided by the board of directors, which may generally be between four and 40 days prior to the date of the meeting. Furthermore, the Israeli Companies Law requires that resolutions regarding the following matters must be passed at a general meeting of our shareholders:

amendments to our amended and restated articles of association;

the exercise of our Board of Director s powers by a general meeting, if our Board of Directors is unable to exercise its powers and the exercise of any of its powers is required for our proper management;

appointment or termination of our auditors;

appointment of external directors;

approval of acts and transactions involving related parties, as defined by the Israeli Companies Law;

increases or reductions of our authorized share capital; and

a merger.

The Israeli Companies Law and our amended and restated articles of association require that a notice of any annual general meeting or special shareholders meeting be provided to shareholders at least 21 days prior to the meeting and if the agenda of the meeting includes matters upon which shareholders may vote by means of a voting deed, including the appointment or removal of directors, the approval of a compensation policy with respect to office holders, the approval of transactions with office holders or interested or related parties, or an approval of a merger, notice must be provided at least 35 days prior to the meeting.

Under the Israeli Companies Law and our amended and restated articles of association, shareholders are not permitted to take action via written consent in lieu of a meeting.

Voting Rights

Quorum Requirements

The quorum required for our general meetings of shareholders consists of at least two shareholders present in person, by proxy or written ballot who hold or represent between them at least one-third of the total outstanding voting rights. A meeting adjourned for lack of a quorum is generally adjourned to the same day in the following week at the same time and place or to a later time/date if so specified in the summons or notice of the meeting. At the reconvened meeting, any two or more shareholders present in person or by proxy shall constitute a lawful quorum.

Vote Requirements

Our amended and restated articles of association provide that all resolutions of our shareholders require a simple majority vote, unless otherwise required by the Israeli Companies Law or by our amended and restated articles of association. Under the Israeli Companies Law certain actions require a special majority, which may include (i) appointment of external directors, requiring the approval of certain transactions described above under Management Board Practices External Directors , (ii) approval of an extraordinary transaction

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with a controlling shareholder and the terms of employment or other engagement of the controlling shareholder of the Company or such controlling shareholder s relative (even if not extraordinary), requiring the approval described above under Approval of Related Party Transactions under Israeli Law Disclosure of Personal Interests of Controlling Shareholders , (iii) approval of executive officer compensation inconsistent with our office holder compensation policy, compensation of our chief executive officer, or the compensation of an executive officer who is also the controlling shareholder of our company (including an affiliate thereof), all of which require the approval described above under Management NASDAQ Listing Rules and Home Country Practices Compensation of Officers. ; (iv) approving the authorization of the chairman of the board or a relative thereof to assume the role or responsibilities of the chief executive officer, or the authorization of the chief executive officer or a relative thereof to assume the role or responsibilities of the chairman of the board, for periods of no longer than three years each and subject to receipt of the approval of a majority of the shares voting on the matter, providing that either (1) included in such majority are at least two-thirds of the shares of shareholders who are non-controlling parties and do not have a personal interest in the said resolution (excluding for such purpose any abstentions); or (2) the total number of shares of shareholders specified in clause (3) who voted against the resolution does not exceed two percent (2%) of the voting rights in the company; and (v) mergers, certain private placements that will increase certain types of shareholders relative holdings in the company, or certain special tender offers or forced bring along share purchase transactions, all of which require the approval described below under Acquisitions under Israeli Law .

Under our amended and restated articles of association, the alteration of the rights, privileges, preferences or obligations of any class of our share capital requires a simple majority of the class so affected (or such other percentage of the relevant class that may be set forth in the governing documents relevant to such class), in addition to the ordinary majority vote of all classes of shares voting together as a single class at a shareholder meeting.

Further exceptions to the simple majority vote requirement are a resolution for the voluntary winding up, or an approval of a scheme of arrangement or reorganization, of the Company pursuant to Section 350 of the Israeli Companies Law, which requires the approval of the majority of the shareholders in each class of shareholders present at the meeting and who are together the holders of 75% of the voting rights represented at the meeting, in person, by proxy or by voting deed and voting on the resolution.

Israeli law provides that a shareholder of a public company may vote in a meeting and in a class meeting by means of a voting deed in which the shareholder indicates how he or she votes on resolutions relating to the following matters:

appointment or removal of directors;
approval of transactions with office holders or interested or related parties;
approval of a merger;
authorization of the chairman of the board or a relative thereof to assume the role or responsibilities of our chief executive officer, and authorization of our chief executive officer or a relative thereof to assume the role or responsibilities of the chairman of the board;
approval of an arrangement or reorganization of the Company pursuant to Section 350 of the Israeli Companies Law;
approval of the compensation policy with respect to the terms of office and employment of office holders; and
other matters in respect of which there is a provision in the articles of association providing that decisions of the general meeting may also be passed by voting deed or which may be prescribed by Israel s Minister of Justice.

The provision allowing the vote by voting deed does not apply if, to the best knowledge of the Company at the time of calling the general shareholders meeting, a controlling shareholder will hold on the record date for such shareholders meeting, voting power sufficient to determine the outcome of the vote.

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The Israeli Companies Law provides that a shareholder, in exercising his or her rights and performing his or her obligations toward the Company and its other shareholders, including voting at general meetings, must act in good faith and in a customary manner, and avoid abusing his or her power. See Approval of Related Party Transactions under Israeli Law Shareholder Duties above for further detail.

Access to Corporate Records

Under the Israeli Companies Law and our amended and restated articles of association, shareholders are provided access to the following corporate records: minutes of our general meetings; our shareholders register and principal shareholders register, articles of association and financial statements; and any document that we are required by law to file publicly with the Israeli Companies Registrar or the Israel Securities Authority. In addition, shareholders may submit a reasoned request to be provided with any document related to an action or transaction requiring shareholder approval under the approval of related party transaction provisions of the Companies Law. We may deny this request if we believe it has not been submitted in good faith or if such denial is necessary to protect our interest or protect a trade secret or patent.

Modification of Class Rights

The rights attached to any class of shares, such as voting, liquidation and dividend rights, may be amended by adoption of a resolution by the holders of a majority (or a special majority, as may be applicable to the particular matter) of the shares of that class present at a separate class meeting, or otherwise in accordance with the rights attached to such class of shares, as set forth in our amended and restated articles of association.

Acquisitions under Israeli Law

Full Tender Offer

A person wishing to acquire shares of a public Israeli company and who could as a result hold over 90% of the target company s issued and outstanding share capital or voting rights is required by the Israeli Companies Law to make a tender offer to all of the company s shareholders for the purchase of all of the issued and outstanding shares of the company. A person wishing to acquire shares of a public Israeli company and who could as a result hold over 90% of the issued and outstanding share capital or voting rights of a certain class of shares is required to make a tender offer to all of the shareholders who hold shares of the relevant class for the purchase of all of the issued and outstanding shares of that class. If the shareholders who do not accept the offer hold less than 5% of the issued and outstanding share capital and voting rights of the company or of the applicable class, all of the shares that the acquirer offered to purchase will be transferred to the acquirer by operation of law (provided that a majority of the offerees that do not have a personal interest in such tender offer shall have approved it, which condition shall not apply if, following consummation of the tender offer, the acquirer would hold at least 98% of all of the company s outstanding shares and voting rights (or shares and voting rights of the relevant class)). However, shareholders may, at any time within six months following the completion of the tender offer, petition the court to alter the consideration for the acquisition. Even shareholders who indicated their acceptance of the tender offer may so petition the court, unless the acquirer stipulated that a shareholder that accepts the offer may not seek appraisal rights. If the shareholders who did not accept the tender offer hold 5% or more of the issued and outstanding share capital or voting rights of the company or of the applicable class, the acquirer may not acquire shares of the company that will increase its holdings to more than 90% of the company s issued and outstanding share capital or voting rights or 90% of the shares or voting rights of the applicable class, from shareholders who accepted the tender offer.

Special Tender Offer

The Israeli Companies Law provides that an acquisition of a control bloc of shares in a public Israeli company must be made by means of a special tender offer if as a result of the transaction the shareholder could become a holder of 25% or more of the voting rights in the company, unless one of the exemptions in the Israeli Companies Law (as described below) is met. This rule does not apply if there is already another holder of at least 25% of the voting rights in the company. Similarly, the Israeli Companies Law provides that an acquisition of shares in a public company must be made by means of a tender offer if as a result of the acquisition the purchaser could become a holder of more than 45% of the voting rights in the company, if

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there is no other shareholder of the company who holds more than 45% of the voting rights in the company, unless one of the exemptions in the Israeli Companies Law is met. Such exemptions include (a) acquisition of shares issued in the course of a private placement approved by the general meeting of the company as a private placement intended to provide purchaser with holdings of 25% or more of the voting rights in the company, if there is no other shareholder of the company who holds more than 25% of the voting rights in the company, or as a private placement intended to provide purchaser with holdings of more than 45% of the voting rights in the company, if there is no other shareholder of the company who holds more than 45% of the voting rights in the company, (b) acquisition of shares from a holder of 25% or more of the voting rights in the company following which purchaser shall hold 25% or more of the voting rights in the company, or (c) acquisition of shares from a holder of 45% or more of the voting rights in the company following which purchaser shall hold 45% or more of the voting rights in the company.

A special tender offer must be extended to all shareholders of a company but the offeror is not required to purchase shares representing more than 5% of the voting power attached to the company s outstanding shares, regardless of how many shares are tendered by shareholders. A special tender offer may be consummated only if (i) at least 5% of the voting power attached to the company s outstanding shares will be acquired by the offeror and (ii) the number of shares tendered in the offer exceeds the number of shares whose holders objected to the offer (disregarding holders who control the offeror and who have a personal interest in the acceptance of the offer or the holder of 25% or more of the voting rights of the company, any of their relatives, or corporations controlled by any of the above).

If a special tender offer is accepted, then the purchaser or any person or entity controlling it or under common control with the purchaser or such controlling person or entity may not make a subsequent tender offer for the purchase of shares of the target company and may not enter into a merger with the target company for a period of one year from the date of the offer, unless the purchaser or such person or entity undertook to effect such an offer or merger in the initial special tender offer.

Merger

The Israeli Companies Law permits merger transactions between Israeli companies if approved by each party s board of directors and, unless certain requirements described under the Israeli Companies Law are met, by a majority vote of each party s shares, and, in the case of the target company, a majority vote of each class of its shares, voted on the proposed merger at a shareholders meeting called with at least 35 days prior notice.

For purposes of the shareholder vote, unless a court rules otherwise, the merger will not be deemed approved if a majority of the votes of shares represented at the shareholders meeting (disregarding abstentions) that are held by parties other than the other party to the merger, or by any person (or group of persons acting in concert) who holds (or hold, as the case may be) 25% or more of the voting rights or the right to appoint 25% or more of the directors of the other party, vote against the merger, or anyone on such parties behalf, including relatives of such parties and corporations controlled them, vote against the merger. If, however, the merger involves a merger with a company s own controlling shareholder or if the controlling shareholder has a personal interest in the merger, then the merger is instead subject to the same special majority approval that governs all extraordinary transactions with controlling shareholders (as described above in this prospectus under Management NASDAQ Listing Rules and Home Country Practices Shareholder Approval).

If the transaction would have been approved by the shareholders of a merging company but for the separate approval of each class or the exclusion of the votes of certain shareholders as provided above, a court may still approve the merger upon the request of holders of at least 25% of the voting rights of a company, if the court holds that the merger is fair and reasonable, taking into account the value of the parties to the merger and the consideration offered to the

shareholders of the company that have petitioned the court to approve the merger.

Upon the request of a creditor of either party to the proposed merger, the court may delay or prevent the merger if it concludes that there exists a reasonable concern that, as a result of the merger, the surviving company will be unable to satisfy the obligations of any of the parties to the merger, and may further give instructions to secure the rights of creditors.

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In addition, a merger may not be consummated unless at least 50 days have passed from the date on which a proposal for approval of the merger was filed by each party with the Israeli Registrar of Companies and at least 30 days have passed from the date on which the merger was approved by the shareholders of each party.

Anti-takeover Measures under Israeli Law

The Israeli Companies Law allow us to create and issue shares having rights different from those attached to our ordinary shares, including shares providing certain preferred rights, distributions or other matters and shares having preemptive rights. As of the closing of this offering, no preferred shares will be authorized under our amended and restated articles of association. In the future, if we do authorize, create and issue a specific class of preferred shares, such class of shares, depending on the specific rights that may be attached to it, may have the ability to frustrate or prevent a takeover or otherwise prevent our shareholders from realizing a potential premium over the market value of their ordinary shares. The authorization and designation of a class of preferred shares will require an amendment to our amended and restated articles of association, which requires the prior approval of the holders of a majority of the voting power attaching to our issued and outstanding shares at a general meeting. The convening of the meeting, the shareholders entitled to participate and the majority vote required to be obtained at such a meeting will be subject to the requirements set forth in the Israeli Companies Law as described above in Voting Rights.

Borrowing Powers

Pursuant to the Israeli Companies Law and our amended and restated articles of association, our Board of Directors may exercise all powers and take all actions that are not required under law or under our amended and restated articles of association to be exercised or taken by our shareholders or other corporate bodies, including the power to borrow money for company purposes.

Changes in Capital

Our amended and restated articles of association enable us to increase or reduce our share capital. Any such changes are subject to the provisions of the Israeli Companies Law and must be approved by a resolution duly passed by our shareholders at a general meeting by voting on such change in the capital. In addition, transactions that have the effect of reducing capital, such as the declaration and payment of dividends in the absence of sufficient retained earnings or profits and, in certain circumstances, an issuance of shares for less than their nominal value, require the approval of both our Board of Directors and an Israeli court.

Transfer Agent

Our transfer agent in the United States will be VStock Transfer, LLC.

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SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, no public market existed for our ordinary shares. Sales of substantial amounts of our ordinary shares following this offering, or the perception that these sales could occur, could adversely affect prevailing market prices of our ordinary shares and could impair our future ability to obtain capital, especially through an offering of equity securities. Assuming that the underwriters do not exercise their over-allotment option with respect to this offering and assuming no exercise of options outstanding following the offering, we will have an aggregate of 14,230,480 ordinary shares outstanding upon completion of this offering. Of these shares, the ordinary shares sold in this offering will be freely tradable without restriction or further registration under the Securities Act, unless purchased by affiliates (as that term is defined under Rule 144 of the Securities Act), who may sell only the volume of shares described below and whose sales would be subject to additional restrictions described below.

The remaining ordinary shares will be held by our existing shareholders. Because substantially all of these shares were sold outside the United States to persons residing outside the United States at the time, they also will be freely tradable without restriction or further registration, except that shares held by affiliates must be sold under Rule 144, and except for the lock-up restrictions described below. Further, substantially all of our outstanding shares are subject to the lock-up agreements.

Lock-up agreements

We, all of our directors and executive officers and holders of substantially all of our outstanding ordinary shares have signed lock-up agreements pursuant to which, subject to certain exceptions, they have agreed not to sell or otherwise dispose of their ordinary shares or any securities convertible into or exchangeable for ordinary shares for a period of 180 days after the date of this prospectus without the prior written consent of Oppenheimer & Co. Inc. and Roth Capital Partners, LLC. In addition, certain holders of options to purchase our shares have entered into similar lock-up agreements.

Rule 144

In general, under Rule 144, beginning 90 days after the date of this prospectus, any person who is not our affiliate and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, and so long as we have filed all reports required by the Exchange Act during the previous 12 months (or such shorter period that were required to file such reports) may sell shares without restriction. In addition, under Rule 144, any person who is not an affiliate of ours and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available.

Beginning 90 days after the date of this prospectus, a person who is our affiliate or who was our affiliate at any time during the preceding three months and who has beneficially owned restricted securities for at least six months, including the holding period of any prior owner other than one of our affiliates, is entitled to sell a number of shares within any three-month period that does not exceed the greater of:

one percent of the number of ordinary shares then outstanding, which will equal 142,305 shares; or
the average weekly trading volume of our ordinary shares on the NASDAQ Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

If an affiliate acquires restricted securities, those securities will also be subject to holding period requirements.

Upon expiration of the applicable lock-up period described above, substantially all of our outstanding ordinary shares will either be unrestricted or will be eligible for sale under Rule 144. We cannot estimate the number of our ordinary shares that our existing stockholders will elect to sell.

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Form S-8 Registration Statements

Following the completion of this offering, we intend to file one or more registration statements on Form S-8 under the Securities Act to register up the ordinary shares issued or reserved for issuance under our 2013 Plan. The registration statement on Form S-8 will become effective automatically upon filing. Ordinary shares issued to individuals upon exercise of a share option and registered under the Form S-8 registration statement will, subject to vesting and lock-up provisions and Rule 144 volume limitations applicable to our affiliates, be available for sale in the open market immediately unless they are subject to the 180-day lock-up, or, if subject to the lock-up, immediately after the applicable lock-up period expires.

TAXATION

The following description is not intended to constitute a complete analysis of all tax consequences relating to the ownership or disposition of our ordinary shares. You should consult your own tax advisor concerning the tax consequences of your particular situation, as well as any tax consequences that may arise under the laws of any state, local, foreign, including Israeli, or other taxing jurisdiction.

ISRAELI TAX CONSIDERATIONS

The following is a summary of the material Israeli income tax laws applicable to us. This section also contains a discussion of material Israeli income tax consequences concerning the ownership and disposition of our ordinary shares. This summary does not discuss all the aspects of Israeli income tax law that may be relevant to a particular investor in light of his or her personal investment circumstances or to some types of investors subject to special treatment under Israeli law. Examples of this kind of investor include residents of Israel or traders in securities who are subject to special tax regimes not covered in this discussion. To the extent that the discussion is based on new tax legislation that has not yet been subject to judicial or administrative interpretation, we cannot assure you that the appropriate tax authorities or the courts will accept the views expressed in this discussion. This summary is based on laws and regulations in effect as of the date of this prospectus and does not take into account possible future amendments which may be under consideration.

General corporate tax structure in Israel

Israeli resident companies, such as the Company, are generally subject to corporate tax at the rate of 26.5% as of 2014.

Capital gains derived by an Israeli resident company are generally subject to tax at the same rate as the corporate tax rate. Under Israeli tax legislation, a corporation will be considered as an Israeli Resident if it meets one of the following: (a) it was incorporated in Israel; or (b) the control and management of its business are exercised in Israel.

Taxation of our Israeli individual shareholders on receipt of dividends

Israeli residents who are individuals are generally subject to Israeli income tax for dividends paid on our ordinary shares (other than bonus shares or share dividends) at a rate of 25%, or 30% if the recipient of such dividend is a substantial shareholder (as defined below) at the time of distribution or at any time during the preceding 12-month period.

As of January 1, 2013, an additional income tax at a rate of 2% is imposed on high earners whose annual income or gain exceeds NIS 811,560.

A substantial Shareholder is generally a person who alone, or together with his relative or another person who collaborates with him on a regular basis, holds, directly or indirectly, at least 10% of any of the means of control of the corporation. Means of control generally include the right to vote, receive profits, nominate a director or an officer, receive assets upon liquidation, or instruct someone who holds any of the aforesaid rights regarding the manner in which he or she is to exercise such right(s), and all regardless of the source of such right.

The term Israeli Resident is generally defined under Israeli tax legislation with respect to individuals as a person whose center of life is in Israel. The Israeli Tax Ordinance New Version, 1961 provides that in

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order to determine the center of life of an individual, account will be taken of the individual s family, economic and social connections, including: (a) place of permanent home; (b) place of residential dwelling of the individual and the individual s immediate family; (c) place of the individual s regular or permanent occupation or the place of his permanent employment; (d) place of the individual s active and substantial economic interests; (e) place of the individual s activities in organizations, associations and other institutions. The center of life of an individual will be presumed to be in Israel if: (a) the individual was present in Israel for 183 days or more in the tax year; or (b) the individual was present in Israel for 30 days or more in the tax year, and the total period of the individual s presence in Israel in that tax year and the two previous tax years is 425 days or more. The presumption in this paragraph may be rebutted either by the individual or by the assessing officer.

Taxation of Israeli Resident Corporations on Receipt of Dividends

Israeli resident corporations are generally exempt from Israeli corporate income tax with respect to dividends paid on our ordinary shares.

Capital Gains Taxes Applicable to Israeli Resident Shareholders

The income tax rate applicable to Real Capital Gain derived by an Israeli individual from the sale of shares which had been purchased after January 1, 2012, whether listed on a stock exchange or not, is 25%. However, if such shareholder is considered a Substantial Shareholder (as defined above) at the time of sale or at any time during the preceding 12-month period, such gain will be taxed at the rate of 30%. As of January 1, 2013, an additional tax at a rate of 2% is imposed on high earners whose annual income or gains exceed NIS 811,560.

Moreover, capital gains derived by a shareholder who is a dealer or trader in securities, or to whom such income is otherwise taxable as ordinary business income, are taxed in Israel at ordinary income rates (26.5 as of 2014 for corporations and up to 50% for individuals).

Taxation of Non-Israeli Shareholders on Receipt of Dividends

Non-Israeli residents are generally subject to Israeli income tax on the receipt of dividends paid on our Shares at the rate of 25% or 30% if such recipient is a substantial shareholder at the time receiving the dividend or on any date in the 12 months preceding such date. If the shares are held by a nominee company, the nominee company or the financial institution will withhold at the source a tax of 25% whether the recipient is a substantial shareholder or not. Otherwise, the withholding at the source will be 25% or 30% in accordance with the above, unless a lower tax rate is provided in a tax treaty between Israel and the shareholder s country of residence.

A non-Israeli resident who receives dividends from which tax was withheld is generally exempt from the duty to file returns in Israel in respect of such income; provided such income was not derived from a business conducted in Israel by the taxpayer, and the taxpayer has no other taxable sources of income in Israel.

For example, under the Convention Between the Government of the United States of America and the Government of Israel with Respect to Taxes on Income, as amended (the U.S.-Israel Tax Treaty), Israeli withholding tax on dividends paid to a U.S. resident for treaty purposes may not, in general, exceed 25%, or 15% in the case of dividends paid out of the profits of a Benefited Enterprise, subject to certain conditions. Where the recipient is a U.S. corporation owning 10% or more of the voting shares of the paying corporation during the part of the paying corporation s taxable year which precedes the date of payment of the dividend and during the whole of its prior taxable year (if any) and the dividend is not paid from the profits of a Benefited Enterprise, and not more than 25% of the gross income of the

paying corporation consists of interest or dividends (other than interest derived from the conduct of banking, insurance, or financing business or interest received from subsidiary corporations, 50% or more of the outstanding shares of the voting stock of which is owned by the paying corporation at the time such dividends or interest is received) the Israeli tax withheld may not exceed 12.5%, subject to certain conditions.

Capital gains income taxes applicable to non-Israeli shareholders.

Non-Israeli resident shareholders are generally exempt from Israeli capital gains tax on any gains derived from the sale, exchange or disposition of our ordinary shares, provided that such gains were not derived

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from a permanent establishment or business activity of such shareholders in Israel. However, non-Israeli corporations will not be entitled to the foregoing exemptions if Israeli residents (i) jointly have a controlling interest of more than 25% in such non-Israeli corporation or (ii) are the beneficiaries of or are entitled to 25% or more of the revenues or profits of such non-Israeli corporation, whether directly or indirectly.

Regardless of whether shareholders may be liable for Israeli income tax on the sale of our ordinary shares, the payment of the consideration may be subject to withholding of Israeli tax at the source. Accordingly, shareholders may be required to demonstrate that they are exempt from tax on their capital gains in order to avoid withholding at source at the time of sale.

Estate and gift tax

Israeli law presently does not impose estate or gift taxes.

EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE PARTICULAR ISRAELI TAX CONSEQUENCES OF PURCHASING, HOLDING, AND DISPOSING OF OUR ORDINARY SHARES, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAWS.

U.S. FEDERAL INCOME TAX CONSEQUENCES

Except as specifically set forth below, the following discussion is limited to the material U.S. federal income tax consequences relating to the purchase, ownership and disposition of our ordinary shares by U.S. Holders (as defined below) that purchase ordinary shares pursuant to the offering and hold such ordinary shares as capital assets. This discussion is based on the U.S. Internal Revenue Code of 1986, as amended, or the Code, U.S. Treasury regulations promulgated thereunder and administrative and judicial interpretations thereof, all as in effect on the date hereof and all of which are subject to change, possibly with retroactive effect. This discussion does not address all of the U.S. federal income tax consequences that may be relevant to specific U.S. Holders in light of their particular circumstances or to U.S. Holders subject to special treatment under U.S. federal income tax law (such as certain financial institutions, insurance companies, broker-dealers and traders in securities or other persons that generally mark their securities to market for U.S. federal income tax purposes, tax-exempt entities, retirement plans, regulated investment companies, real estate investment trusts, certain former citizens or residents of the United States, persons who hold ordinary shares as part of a straddle, hedge, conversion transaction, synthetic security or integrated investment, persons that have a functional currency other than the U.S. dollar, persons that own (or are deemed to own) 10% or more (by voting power or value) of our shares, corporations that accumulate earnings to avoid U.S. federal income tax, partnerships and other pass-through entities, and investors in such pass-through entities). Except as expressly set forth herein, this discussion does not address any U.S. state or local or non-U.S. tax consequences or any U.S. federal estate, gift or alternative minimum tax consequences.

As used in this discussion, the term U.S. Holder means a beneficial owner of ordinary shares that is, for U.S. federal income tax purposes, (i) an individual who is a citizen or resident of the United States, (ii) a corporation (or entity treated as a corporation for U.S. federal income tax purposes) created or organized in or under the laws of the United States, any state thereof, or the District of Columbia, (iii) an estate the income of which is subject to U.S. federal income tax regardless of its source or (iv) a trust (x) with respect to which a court within the United States is able to exercise primary supervision over its administration and one or more United States persons have the authority to control all of its substantial decisions or (y) that has elected under applicable U.S. Treasury regulations to be treated as a domestic trust for U.S. federal income tax purposes.

If an entity treated as a partnership for U.S. federal income tax purposes holds the ordinary shares, the U.S. federal income tax consequences relating to an investment in the ordinary shares will depend in part upon the status and activities of such entity and the particular partner. Any such entity should consult its own tax advisor regarding the U.S. federal income tax consequences applicable to it and its partners of the purchase, ownership and disposition of the ordinary shares.

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Persons considering an investment in the ordinary shares should consult their own tax advisors as to the particular tax consequences applicable to them relating to the purchase, ownership and disposition of the ordinary shares, including the applicability of U.S. federal, state and local tax laws and non-U.S. tax laws.

Distributions

Subject to the discussion below under Passive foreign investment company consequences, a U.S. Holder that receives a distribution with respect to an ordinary share generally will be required to include the amount of such distribution in gross income as a dividend (without reduction for any Israeli tax withheld from such distribution) when actually or constructively received to the extent of the U.S. Holder s pro rata share of our current and/or accumulated earnings and profits (as determined under U.S. federal income tax principles). To the extent a distribution received by a U.S. Holder is not a dividend because it exceeds the U.S. Holder s pro rata share of our current and accumulated earnings and profits, it will be treated first as a tax-free return of capital and reduce (but not below zero) the adjusted tax basis of the U.S. Holder s ordinary shares. To the extent the distribution exceeds the adjusted tax basis of the U.S. Holder s shares, the remainder will be taxed as capital gain. Because we do not account for our income in accordance with U.S. federal income tax purposes, U.S. Holders should expect all distributions to be reported to them as dividends.

The U.S. dollar value of any distribution on the ordinary shares made in NIS generally should be calculated by reference to the exchange rate between the U.S. dollar and the NIS in effect on the date of receipt of such distribution by the U.S. Holder regardless of whether the NIS so received is in fact converted into U.S. dollars at that time. If the NIS so received is converted into U.S. dollars on the date of receipt, such U.S. Holder generally should not recognize currency gain or loss on such conversion. If the NIS so received is not converted into U.S. dollars on the date of receipt, such U.S. Holder generally will have a basis in such NIS equal to the U.S. dollar value of such NIS on the date of receipt. Any gain or loss on a subsequent conversion or other disposition of such NIS by such U.S. Holder generally will be treated as ordinary income or loss and generally will be income or loss from sources within the United States for U.S. foreign tax credit purposes.

Distributions on the ordinary shares that are treated as dividends generally will constitute income from sources outside the United States for foreign tax credit purposes. Such dividends will not be eligible for the dividends received deduction generally allowed to corporate shareholders with respect to dividends received from U.S. corporations. Distributions treated as dividends that are received by non-corporate U.S. Holders are expected to qualify for the 20% reduced maximum tax rate available for dividends received from qualified foreign corporation provided certain holding periods and other requirements are met. We will not be treated as a qualifying foreign corporation, and therefore the reduced maximum tax rate in effect for 2014 described above will not apply, if we are a PFIC for the taxable year in which the dividend is paid or the preceding taxable year (see Passive foreign investment company consequences, below).

Distributions may be subject to Israeli withholding tax see Taxation Israeli tax considerations Taxation of our shareholders Taxation of non-Israeli shareholders on receipt of dividends. Subject to certain conditions and limitations, Israeli taxes withheld from distributions by us may be credited against a U.S. Holder s U.S. federal income tax liability or, alternatively, deducted to determine the U.S. Holder s taxable income. This election to deduct foreign income taxes is made on a year-by-year basis and applies to all foreign taxes paid by a U.S. Holder that year. Dividends paid on the ordinary shares generally will constitute income from sources outside the United States and be categorized as passive category income for U.S. foreign tax credit purposes.

Sale, exchange or other disposition of the ordinary shares

Subject to the discussion below under Passive foreign investment company consequences, a U.S. Holder generally will recognize capital gain or loss for U.S. federal income tax purposes upon the sale, exchange or other disposition of an ordinary share in an amount equal to the difference, if any, between the amount realized (i.e., the amount of cash plus the fair market value of any property received) on the sale, exchange or other disposition and such U.S. Holder s adjusted tax basis in the ordinary share, both amounts determined in U.S. dollars. Such capital gain or loss generally will be long-term capital gain taxable at a reduced rate for non-corporate U.S. Holders (currently a maximum of 20%) or loss if, on the date of sale,

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exchange or other disposition, the ordinary share was held by the U.S. Holder for more than one year. Any capital gain of a non-corporate U.S. Holder that is not long-term capital gain is taxed at ordinary income rates. The deductibility of capital losses is subject to limitations. Any gain or loss recognized from the sale or other disposition of our ordinary shares will generally be gain or loss from sources within the United States for U.S. foreign tax credit purposes.

A U.S. Holder that receives NIS from the sale, exchange or other disposition of ordinary shares will generally realize an amount equal to the U.S. dollar value of the NIS received at the spot rate on the date of sale (or, in the case of cash basis and electing accrual basis U.S. Holders, the settlement date). A U.S. Holder will recognize foreign currency gain or loss to the extent the U.S. dollar value of the amount received at the spot exchange rate on the settlement date differs from the amount realized. A U.S. Holder will have a tax basis in the NIS received equal to its U.S. dollar value on the settlement date. Any gain or loss on a subsequent conversion or other disposition of the NIS will be U.S. source ordinary income or loss.

Passive foreign investment company consequences

In general, a corporation organized outside the United States will be treated as a PFIC in any taxable year in which either (i) at least 75% of its gross income is passive income or (ii) on average at least 50% of its assets is attributable to assets that produce passive income or are held for the production of passive income. Passive income for this purpose generally includes, among other things, dividends, interest, royalties, rents, and gains from commodities transactions and from the sale or exchange of property that gives rise to passive income. Assets that produce or are held for the production of passive income include cash, even if held as working capital or raised in a public offering, marketable securities and other assets that may produce passive income. The average percentage of a corporation s assets that produce or are held for the production of passive income generally is determined on the basis of the fair market value of the corporation s assets at the end of each quarter. This determination is based on the adjusted tax basis of the corporation s assets however, if the corporation is a controlled foreign corporation , that is not a publicly traded corporation for the taxable year. In determining whether a foreign corporation is a PFIC, a proportionate share of the income and assets of each corporation in which it owns, directly or indirectly, at least a 25% interest (by value) is taken into account.

Based on the nature of our business, the projected composition of our income and the projected composition and estimated fair market values of our assets, we cannot rule out that we will be a passive foreign investment company within the meaning of Section 1297 of the Code or the regulations promulgated thereunder in 2013, 2014 or a subsequent year. Nevertheless, because this determination is made annually after the close of each taxable year, because we hold and expect to continue to hold following this offering a substantial amount of cash and cash equivalents, and because the calculation of the value of our assets may be based in part on the value of our ordinary shares, which may fluctuate after this offering and may fluctuate considerably given that market prices of technology companies historically often have been volatile, it is difficult to predict whether we will be a PFIC in any taxable year. Even if we determine that we are not a PFIC after the close of our taxable year, there can be no assurance that the IRS will agree with our conclusion.

If we are a PFIC in any taxable year during which a U.S. Holder owns ordinary shares, such U.S. Holder could be liable for additional taxes and interest charges upon (i) certain distributions by us (generally any distribution paid during a taxable year that is greater than 125 percent of the average annual distributions paid in the three preceding taxable years, or, if shorter, the U.S. Holder s holding period for our ordinary shares), and (ii) any gain recognized on a sale, exchange or other disposition, including a pledge, of the ordinary shares, whether or not we continue to be a PFIC. In these circumstances, the tax will be determined by allocating such distributions or gain ratably over the U.S.

Holder s holding period for the ordinary shares. The amount allocated to the current taxable year (i.e., the year in which the distribution occurs or the gain is recognized) and any year prior to the first taxable year in which we are a PFIC will be taxed as ordinary income earned in the current taxable year. The amount allocated to other taxable years will be taxed at the highest marginal rates in effect for individuals or corporations as appropriate applicable to ordinary income for each such taxable year, and an interest charge, generally that applicable to underpayments of tax, will be added to the tax. If we are a PFIC for any year during which a U.S. Holder holds our ordinary shares, we will

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generally continue to be treated as a PFIC with respect to the holder for all succeeding years during which the U.S. Holder holds ordinary shares even if we cease to meet the requirements for PFIC status.

The tax consequences that would apply if we were a PFIC would be different from those described above if a timely and valid mark-to-market election is made by a U.S. Holder for our ordinary shares. An electing U.S. Holder generally would take into account as ordinary income each year, the excess of the fair market value of our ordinary shares held at the end of the taxable year over the adjusted tax basis of such ordinary shares. The U.S. Holder would also take into account, as an ordinary loss each year, the excess of the adjusted tax basis of such ordinary shares over their fair market value at the end of the taxable year, but only to the extent of the amount previously included in income as a result of the mark-to-market election. The U.S. Holder s tax basis in our ordinary shares would be adjusted to reflect any income or loss resulting from the mark-to-market election. Any gain from a sale, exchange or other disposition of the ordinary shares in any taxable year in which we are a PFIC would be treated as ordinary income and any loss from such sale, exchange or other disposition would be treated first as ordinary loss (to the extent of any net mark-to-market gains previously included in income) and thereafter as capital loss. If, after having been a PFIC for a taxable year, we cease to be classified as a PFIC, the U.S. Holder would not be required to take into account any latent gain or loss in the manner described above and any realized gain or loss would be classified as a capital gain or loss.

A mark-to-market election is available to a U.S. Holder only if the ordinary shares are considered marketable stock . Generally, stock will be considered marketable stock if it is regularly traded on a qualified exchange within the meaning of applicable U.S. Treasury regulations. A class of stock is regularly traded during any calendar year during which such class of stock is traded, other than in de minimis quantities, on at least 15 days during each calendar quarter. Our ordinary shares will be marketable stock as long as they remain listed on the NASDAQ Global Market and are regularly traded. A mark-to-market election will not apply to our ordinary shares for any taxable year during which we are not a PFIC, but will remain in effect with respect to any subsequent taxable year in which we become a PFIC. Such election will not apply to any subsidiary that we own.

The tax consequences that would apply if we were a PFIC would also be different from those described above if a U.S. Holder were able to make a valid qualified electing fund (QEF) election. As we do not expect to provide U.S. Holders with the information required in order to permit a QEF election, prospective investors should assume that a QEF election will not be available.

If we are a PFIC in any taxable year during which a U.S. Holder owns the ordinary shares, such U.S. Holder may also suffer adverse tax consequences under the PFIC rules described above with respect to any lower-tier PFIC in which we have a direct or indirect equity interest.

Each U.S. Holder who is a shareholder of a PFIC must file an annual report containing certain information.

The U.S. federal income tax rules relating to PFICs are complex. Prospective U.S. investors are urged to consult their own tax advisers with respect to the purchase, ownership and disposition of ordinary shares, the consequences to them of an investment in a PFIC, any elections available with respect to our ordinary shares and the IRS information reporting obligations with respect to the purchase, ownership and disposition of ordinary shares.

Certain reporting requirements with respect to payments of Offer Price

U.S. Holders paying more than U.S. $100,000 for ordinary shares generally will be required to file IRS Form 926 reporting the payment of the Offer Price for an ordinary share to us. Substantial penalties may be imposed upon a U.S. Holder that fails to comply. Each U.S. Holder should consult its own tax advisor as to the possible obligation to file IRS Form 926.

Information reporting and backup withholding

Dividends on and proceeds from the sale or other disposition of the ordinary shares may be reported to the IRS unless the U.S. Holder establishes a basis for exemption. Backup withholding may apply to amounts

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subject to reporting if (i) the holder fails to provide an accurate taxpayer identification number or otherwise establish a basis for exemption, or (ii) is described in certain other categories of persons. Backup withholding is not an additional tax.

Any amounts withheld under the backup withholding rules generally will be allowed as a refund or a credit against a U.S. Holder s U.S. federal income tax liability if the required information is furnished by the U.S. Holder on a timely basis to the IRS.

Certain U.S. Holders who are individuals must report information relating to stock of a non-U.S. person, subject to certain exceptions (including an exception for stock held in custodial accounts maintained by a U.S. financial institution). U.S. Holders are urged to consult their tax advisers regarding the effect, if any, of this legislation on their ownership and disposition of ordinary shares.

THE DISCUSSION ABOVE IS A GENERAL SUMMARY. IT DOES NOT COVER ALL TAX MATTERS THAT MAY BE OF IMPORTANCE TO A PROSPECTIVE INVESTOR. EACH PROSPECTIVE INVESTOR IS URGED TO CONSULT ITS OWN TAX ADVISOR ABOUT THE TAX CONSEQUENCES TO IT OF AN INVESTMENT IN ORDINARY SHARES IN LIGHT OF THE INVESTOR S OWN CIRCUMSTANCES.

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UNDERWRITING

We have entered into an underwriting agreement with the underwriters named below. Oppenheimer & Co. Inc. and Roth Capital Partners, LLC are acting as representatives of the underwriters.

The underwriting agreement provides for the purchase of a specific number of shares of ordinary shares by each of the underwriters. The underwriters obligations are several, which means that each underwriter is required to purchase a specified number of ordinary shares, but is not responsible for the commitment of any other underwriter to purchase ordinary shares. Subject to the terms and conditions of the underwriting agreement, each underwriter has severally agreed to purchase the number of ordinary shares set forth opposite its name below:

Underwriter	Number of Shares
Oppenheimer & Co. Inc.	1,440,000
Roth Capital Partners, LLC	1,440,000
BTIG LLC	320,000
Total	3,200,000

The underwriters have agreed to purchase all of the ordinary shares offered by this prospectus (other than those covered by the over-allotment option described below) if any are purchased. Under the underwriting agreement, if an underwriter defaults in its commitment to purchase ordinary shares, the commitments of non-defaulting underwriters may be increased or the underwriting agreement may be terminated, depending on the circumstances. Certain of our directors or their affiliates or related parties have indicated that they may have an interest in purchasing ordinary shares in this offering, which would reduce the number of ordinary shares sold to the general public. However, because indications of interest are not binding agreements or commitments to purchase, these persons or entities may determine not to purchase any ordinary shares in this offering.

The underwriters are offering the ordinary shares subject to various conditions and may reject all or part of any order. The representatives have advised us that the underwriters propose to offer the ordinary shares directly to the public at the public offering price that appears on the cover page of this prospectus. In addition, the representatives may offer some of the ordinary shares to other securities dealers at such price less a concession of $0.462 per share. After the ordinary shares are released for sale to the public, the representatives may change the offering price and other selling terms at various times.

We have granted the underwriters an over-allotment option. This option, which is exercisable for up to 30 days after the date of this prospectus, permits the underwriters to purchase a maximum of 480,000 additional ordinary shares from us to cover over-allotments. If the underwriters exercise all or part of this option, they will purchase ordinary shares covered by the option at the initial public offering price that appears on the cover page of this prospectus, less the underwriting discount. If this option is exercised in full, the total price to public will be $40,480,000, and the total proceeds to us will be $37,646,400, before expenses. The underwriters have severally agreed that, to the extent the over-allotment option is exercised, they will each purchase a number of additional ordinary shares proportionate to the underwriter's initial amount reflected in the foregoing table.

The following table provides information regarding the amount of the discount to be paid to the underwriters by us:

	Per Share

		Total Without Exercise of Over-Allotment Option	Total With Full Exercise of Over-Allotment Option
Public offering price	$ 11.00	$ 35,200,000	$ 40,480,000
Underwriting discounts and commissions	$ 0.77	$ 2,464,000	$ 2,833,600
Proceeds, before expenses, to us	$ 10.23	$ 32,736,000	$ 37,646,400

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We estimate that our total expenses of the offering, excluding the underwriting discounts and commissions, will be approximately $885,000, which includes $50,000 that we have agreed to reimburse the underwriters for the fees and expenses incurred by them in connection with this offering.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

We, our officers and directors and substantially all other stockholders have agreed to a 180 day lock up with respect to 11,030,480 ordinary shares and other of our securities that they beneficially own, including securities that are convertible into ordinary shares and securities that are exchangeable or exercisable for ordinary shares. This means that, subject to certain exceptions, for a period of 180 days following the date of this prospectus, we and such persons may not offer, sell, pledge or otherwise dispose of these securities without the prior written consent of the representatives.

The representatives have informed us that they do not expect discretionary sales by the underwriters to exceed five percent of the shares offered by this prospectus.

Rules of the Securities and Exchange Commission may limit the ability of the underwriters to bid for or purchase shares before the distribution of the shares is completed. However, the underwriters may engage in the following activities in accordance with the rules:

Stabilizing transactions The representative may make bids or purchases for the purpose of pegging, fixing or maintaining the price of the shares, so long as stabilizing bids do not exceed a specified maximum.
Over-allotments and syndicate covering transactions The underwriters may sell more ordinary shares in connection with this offering than the number of ordinary shares than they have committed to purchase. This over-allotment creates a short position for the underwriters. This short sales position may involve either covered short sales or naked short sales. Covered short sales are short sales made in an amount not greater than the underwriters' over-allotment option to purchase additional ordinary shares in this offering described above. The underwriters may close out any covered short position either by exercising their over-allotment option or by purchasing ordinary shares in the open market. To determine how they will close the covered short position, the underwriters will consider, among other things, the price of ordinary shares available for purchase in the open market, as compared to the price at which they may purchase ordinary shares through the over-allotment option. Naked short sales are short sales in excess of the over-allotment option. The underwriters must close out any naked short position by purchasing ordinary shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that, in the open market after pricing, there may be downward pressure on the price of the ordinary shares that could adversely affect investors who purchase ordinary shares in this offering.
Penalty bids If the representative purchases ordinary shares in the open market in a stabilizing transaction or syndicate covering transaction, they may reclaim a selling concession from the underwriters and selling group members who sold those ordinary shares as part of this offering.
Similar to other purchase transactions, the underwriters' purchases to cover the syndicate short sales or to stabilize the market price of our ordinary shares may have the effect of raising or maintaining the market price of our ordinary shares or preventing or mitigating a decline in the market price of our ordinary shares. As a result, the price of the shares of our ordinary shares may be higher than the price that might otherwise exist in the open market. The imposition of a penalty bid might also have an effect on the price of the ordinary shares if it discourages resales of the ordinary shares.

Neither we nor the underwriters makes any representation or prediction as to the effect that the transactions described above may have on the price of the ordinary shares. These transactions may occur on The NASDAQ Global Market or

otherwise. If such transactions are commenced, they may be discontinued without notice at any time.

Electronic Delivery of Preliminary Prospectus: A prospectus in electronic format may be delivered to potential investors by one or more of the underwriters participating in this offering. The prospectus in

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electronic format will be identical to the paper version of such preliminary prospectus. Other than the prospectus in electronic format, the information on any underwriter's web site and any information contained in any other web site maintained by an underwriter is not part of this prospectus.

Determination of Offering Price

Prior to the offering, there has not been a public market for our ordinary shares. Consequently, the public offering price for our ordinary shares has been determined by negotiations between us and the representatives of the several underwriters for this offering. Among the factors to be considered in these negotiations were the prevailing market conditions, our financial information, market valuations of other companies that we and the representatives believe to be comparable to us, estimates of our business potential, the present state of our development and other factors deemed relevant.

We offer no assurances that the public offering price will correspond to the price at which the ordinary shares will trade in the public market subsequent to the offering or that an active trading market for the ordinary shares will develop and continue after the offering.

Notice to Non-U.S. Investors

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive, or a Relevant Member State, an offer to the public of any securities which are the subject of the offering contemplated by this prospectus may not be made in that Relevant Member State except that an offer to the public in that Relevant Member State of any securities may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

(a) to legal entities which are authorized or regulated to operate in the financial markets or, if not so authorized or regulated, whose corporate purpose is solely to invest in securities;

(b) to any legal entity which has two or more of (i) an average of at least 250 employees during the last financial year; (ii) a total balance sheet of more than €43,000,000 and (iii) an annual net turnover of more than €50,000,000, as shown in its last annual or consolidated accounts;

(c) by the representative to fewer than 100 natural or legal persons (other than qualified investors as defined in the Prospectus Directive); or

(d) in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided, that no such offer of securities shall result in a requirement for the publication by us or any representative of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an offer to the public in relation to any securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any securities to be offered so as to enable an investor to decide to purchase any securities, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State and the expression Prospectus Directive means Directive 2003/71/EC and includes any relevant implementing measure in each Relevant Member State.

Each underwriter has represented, warranted and agreed that:

(a) it has only communicated or caused to be communicated and will only communicate or cause to be communicated any invitation or inducement to engage in investment activity (within the meaning of section 21 of the Financial

Services and Markets Act 2000, or the FSMA, received by it in connection with the issue or sale of any securities in circumstances in which section 21(1) of the FSMA does not apply to us; and

(b) it has complied with and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the securities in, from or otherwise involving the United Kingdom.

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In the State of Israel, the ordinary shares offered hereby may not be offered to any person or entity other than the following:

(a) a fund for joint investments in trust (i.e., mutual fund), as such term is defined in the Law for Joint Investments in Trust, 5754-1994, or a management company of such a fund;

(b) a provident fund as defined in Section 47(a)(2) of the Income Tax Ordinance of the State of Israel, or a management company of such a fund;

(c) an insurer, as defined in the Law for Oversight of Insurance Transactions, 5741-1981, (d) a banking entity or satellite entity, as such terms are defined in the Banking Law (Licensing), 5741-1981, other than a joint services company, acting for their own account or for the account of investors of the type listed in Section 15A(b) of the Securities Law 1968;

(d) a company that is licensed as a portfolio manager, as such term is defined in Section 8(b) of the Law for the Regulation of Investment Advisors and Portfolio Managers, 5755-1995, acting on its own account or for the account of investors of the type listed in Section 15A(b) of the Securities Law 1968;

(e) a company that is licensed as an investment advisor, as such term is defined in Section 7(c) of the Law for the Regulation of Investment Advisors and Portfolio Managers, 5755-1995, acting on its own account;

(f) a company that is a member of the Tel Aviv Stock Exchange, acting on its own account or for the account of investors of the type listed in Section 15A(b) of the Securities Law 1968;

(g) an underwriter fulfilling the conditions of Section 56(c) of the Securities Law, 5728-1968;

(h) a venture capital fund (defined as an entity primarily involved in investments in companies which, at the time of investment, are primarily engaged in research and development or manufacture of new technological products or processes and (ii) involve above-average risk);

(i) an entity primarily engaged in capital markets activities in which all of the equity owners meet one or more of the above criteria; and

(j) an entity, other than an entity formed for the purpose of purchasing ordinary shares in this offering, in which the shareholders equity (including pursuant to foreign accounting rules, international accounting regulations and U.S. generally accepted accounting rules, as defined in the Securities Law Regulations (Preparation of Annual Financial Statements), 1993) is in excess of NIS 50 million.

Any offeree of the ordinary shares offered hereby in the State of Israel shall be required to submit written confirmation that it falls within the scope of one of the above criteria. This prospectus will not be distributed or directed to investors in the State of Israel who do not fall within one of the above criteria.

EXPENSES

We estimate that the total expenses of this offering payable by us, excluding the underwriting discounts and commissions and expenses, will be as follows:

SEC filing fee	$ 5,925
FINRA filing fee	6,950
NASDAQ listing fee	125,000
Transfer agent fees and expenses	5,000
Printer fees and expenses	10,000
Legal fees and expenses	500,000
Accounting fees and expenses	160,000
Miscellaneous	22,125
Total	$ 835,000

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LEGAL MATTERS

Certain legal matters concerning this offering will be passed upon for us by Zysman, Aharoni, Gayer and Sullivan & Worcester LLP, New York, New York. Certain legal matters with respect to the legality of the issuance of the securities offered by this prospectus will be passed upon for us by Zysman, Aharoni, Gayer & Co., Tel Aviv, Israel. As of the date of this prospectus, certain partners with Zysman Aharoni Gayer & Co. Law Offices beneficially own 537,585 ordinary shares. Certain legal matters related to the offering will be passed upon for the underwriters by Mintz, Levin, Cohn, Ferris, Glovsky and Popeo, P.C., New York, New York and Yigal Arnon & Co., Tel Aviv, Israel.

EXPERTS

The financial statements of Bio Blast Pharma Ltd. for its fiscal years ended December 31, 2013 and December 31, 2012 included herein have been audited by Kost Forer Gabbay & Kasierer (a Member of EY Global), independent registered public accounting firm, as set forth in their report thereon, which contains an explanatory paragraph describing conditions that raise substantial doubt about the Company s ability to continue as a going concern as described in Note 1(c) to the financial statements included herein. Such financial statements are included herein in reliance upon such report given on the authority of such firm as experts in accounting and auditing. The address of Kost Forer Gabbay & Kasierer is 3 Aminadav St., Tel-Aviv, Israel 67067.

ENFORCEABILITY OF CIVIL LIABILITIES

We are incorporated under the laws of the State of Israel. Service of process upon us and upon our directors and officers and the Israeli experts named in this prospectus, substantially all of whom reside outside of the United States, may be difficult to obtain within the United States. Furthermore, because substantially all of our assets and substantially all of our directors and officers are located outside of the United States, any judgment obtained in the United States against us or any of our directors and officers may not be collectible within the United States.

We have been informed by our legal counsel in Israel, Zysman, Aharoni, Gayer & Co., that it may be difficult to assert U.S. securities law claims in original actions instituted in Israel. Israeli courts may refuse to hear a claim based on a violation of U.S. securities laws because Israel is not the most appropriate forum to bring such a claim. In addition, even if an Israeli court agrees to hear a claim, it may determine that Israeli law and not U.S. law is applicable to the claim. If U.S. law is found to be applicable, the content of applicable U.S. law must be proved as a fact which can be a time-consuming and costly process. Certain matters of procedure will also be governed by Israeli law.

Subject to specified time limitations and legal procedures, Israeli courts may enforce a United States judgment in a civil matter which, subject to certain exceptions, is non-appealable, including judgments based upon the civil liability provisions of the Securities Act and the Exchange Act and including a monetary or compensatory judgment in a non-civil matter, provided that among other things:

the judgment is obtained after due process before a court of competent jurisdiction, according to the laws of the state in which the judgment is given and the rules of private international law currently prevailing in Israel;

the judgment is final and is not subject to any right of appeal;

the prevailing law of the foreign state in which the judgment was rendered allows for the enforcement of judgments of Israeli courts;

adequate service of process has been effected and the defendant has had a reasonable opportunity to be heard and to present his or her evidence;
the liabilities under the judgment are enforceable according to the laws of the State of Israel and the judgment and the enforcement of the civil liabilities set forth in the judgment is not contrary to the law or public policy in Israel nor likely to impair the security or sovereignty of Israel;
the judgment was not obtained by fraud and do not conflict with any other valid judgments in the same matter between the same parties;

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an action between the same parties in the same matter is not pending in any Israeli court at the time the lawsuit is instituted in the foreign court; and

the judgment is enforceable according to the law of the foreign state in which the relief was granted.

If a foreign judgment is enforced by an Israeli court, it generally will be payable in Israeli currency, which can then be converted into non-Israeli currency and transferred out of Israel. The usual practice in an action before an Israeli court to recover an amount in a non-Israeli currency is for the Israeli court to issue a judgment for the equivalent amount in Israeli currency at the rate of exchange in force on the date of the judgment, but the judgment debtor may make payment in foreign currency. Pending collection, the amount of the judgment of an Israeli court stated in Israeli currency ordinarily will be linked to the Israeli consumer price index plus interest at the annual statutory rate set by Israeli regulations prevailing at the time. Judgment creditors must bear the risk of unfavorable exchange rates.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form F-1 under the Securities Act relating to this offering of our ordinary shares. This prospectus does not contain all of the information contained in the registration statement. The rules and regulations of the SEC allow us to omit certain information from this prospectus that is included in the registration statement. Statements made in this prospectus concerning the contents of any contract, agreement or other document are summaries of all material information about the documents summarized, but are not complete descriptions of all terms of these documents. If we filed any of these documents as an exhibit to the registration statement, you may read the document itself for a complete description of its terms.

You may read and copy the registration statement, including the related exhibits and schedules, and any document we file with the SEC without charge at the SEC s public reference room at 100 F Street, N.E., Room 1580, Washington, DC 20549. You may also obtain copies of the documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street, N.E., Room 1580, Washington, DC 20549. Please call the SEC at 1-800-SEC-0330 for further information on the public reference room. The SEC also maintains an Internet website that contains reports and other information regarding issuers that file electronically with the SEC. Our filings with the SEC are also available to the public through the SEC s website at *http://www.sec.gov*.

Upon completion of this offering, we will be subject to the information reporting requirements of the Exchange Act that are applicable to foreign private issuers, and under those requirements will file reports with the SEC. Those other reports or other information may be inspected without charge at the locations described above. As a foreign private issuer, we will be exempt from the rules under the Exchange Act related to the furnishing and content of proxy statements, and our officers, directors and principal shareholders will be exempt from the reporting and short-swing profit recovery provisions contained in Section 16 of the Exchange Act. In addition, we will not be required under the Exchange Act to file annual, quarterly and current reports and financial statements with the SEC as frequently or as promptly as United States companies whose securities are registered under the Exchange Act. However, we will file with the SEC, within 120 days after the end of each fiscal year, or such applicable time as required by the SEC, an annual report on Form 20-F containing financial statements audited by an independent registered public accounting firm, and will submit to the SEC, on Form 6-K, unaudited quarterly financial information.

We expect to maintain a corporate website at *www.bioblast-pharma.com.* Information contained on, or that can be accessed through, our website does not constitute a part of this prospectus, and we have included our website address in this prospectus solely as an inactive textual reference. We will post on our website any materials required to be so posted on such website under applicable corporate or securities laws and regulations, including, posting any XBRL interactive financial data required to be filed with the SEC, and any notices of general meetings of our shareholders.

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BIO BLAST PHARMA LTD.
(A development stage company)

FINANCIAL STATEMENTS
AS OF DECEMBER 31, 2013
U.S. DOLLARS IN THOUSANDS

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Shareholders and Board of Directors of BIO BLAST PHARMA LTD. (A development stage company)

We have audited the accompanying balance sheets of BIO BLAST PHARMA Ltd. (a development stage company) (the Company) as of December 31, 2013 and 2012, and the related statements of operations, changes in shareholders equity and cash flow for the year ended December 31, 2013, for the period from January 22, 2012 (date of inception) to December 31, 2012 and for the period from January 22, 2012 (date of inception) to December 31, 2013. These financial statements are the responsibility of the Company s board of directors and management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company s internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances but not for the purpose of expressing an opinion on the effectiveness of the Company s internal control over financial reporting. Accordingly, we express no such opinion. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by the board of directors and management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, based on our audits, the financial statements referred to above present fairly, in all material respects, the financial position of the Company as of December 31, 2013 and 2012, and its results of operations and cash flows for the year ended December 31, 2013, for the period from January 22, 2012 (date of inception) to December 31, 2012 and for the period from January 22, 2012 (date of inception) to December 31, 2013, in conformity with generally accepted accounting principles in the United States.

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As more fully described in Note 1c, the Company has incurred losses in the amount of $1,145 thousand during the year ended December 31, 2013, and has a deficit accumulated during the development stage of $1,400 thousand as of December 31, 2013. Its ability to continue to operate is dependent upon obtaining additional financial support. These conditions, among other matters described in Note 1c, raise substantial doubt about the Company s ability to continue as a going concern. The financial statements do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classification of liabilities that may result from the outcome of this uncertainty.

Tel-Aviv, Israel
April 7, 2014

/s/ KOST FORER GABBAY & KASIERER
KOST FORER GABBAY & KASIERER
A Member of EY Global

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BIO BLAST PHARMA LTD.
(A development stage company)

BALANCE SHEETS
U.S. dollars in thousands

	December 31,		Pro Forma financial information as of December 31, 2013* (Unaudited)
	2013	2012	
ASSETS			
CURRENT ASSETS:			
Cash and cash equivalents	$270	$ 146	$ 5,550
Receivables and prepaid expenses	29	10	129
Total current assets	299	156	5,679
LONG-TERM ASSETS:			
Long-term deposit	5		5
Property and equipment, net	2		2
Total long-term assets	7		7
TOTAL ASSETS	$306	$ 156	$ 5,686
LIABILITIES AND SHAREHOLDERS EQUITY			
CURRENT LIABILITIES:			
Trade payables	$46	$ 74	$ 46
Other accounts payable	85	7	85
Total current liabilities	131	81	131
SHAREHOLDERS EQUITY:			
Ordinary shares of NIS 0.01 par value 16,613,139 and 15,102,854 shares authorized at December 31, 2013 and 2012, respectively; 9,182,867 and 7,551,427 issued and outstanding shares at December 31, 2013 and 2012, respectively; 11,030,480 shares pro forma as of December 31, 2013 (Unaudited).	$24	$ 20	$ 29
Preferred shares of NIS 0.01 par value Nil neither authorized nor outstanding as of December 31, 2013. 1,510,285 shares authorized at December 31, 2012; 566,357 issued and outstanding preferred shares at December 31, 2012.		1	
Additional paid- in capital	1,551	283	6,926
Deficit accumulated during the development stage	(1,400)	(229)	(1,400)
Total shareholders equity	$175	$ 75	$ 5,555
TOTAL LIABILITIES AND SHAREHOLDERS EQUITY	$306	$ 156	$ 5,686

* See Note 2.b.

The accompanying notes are an integral part of the financial statements.

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BIO BLAST PHARMA LTD.
(A development stage company)

STATEMENTS OF OPERATIONS
U.S. dollars in thousands, (except share and per share data)

	Year ended December 31,		Period from January 22, 2012 (date of inception) to December 31, 2013
	2013	2012	
Research and development expenses	$732	$140	$ 872
General and administrative expenses	416	86	502
Operating loss	1,148	226	1,374
Financial expenses (income), net	(3)	3	
Loss	$1,145	$229	$ 1,374
Deemed dividend	26		26
Loss attributable to holders of Ordinary shares	$1,171	$229	$ 1,400
Basic and diluted loss per share	$(0.14)	$(0.03)	
Weighted average number of Ordinary shares used in computing basic and diluted loss per share	8,423,018	7,551,427	

The accompanying notes are an integral part of the financial statements.

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BIO BLAST PHARMA LTD.
(A development stage company)

STATEMENTS OF CHANGES IN SHAREHOLDERS EQUITY
U.S. dollars in thousands, except share data

	Ordinary shares		Preferred A shares		Additional paid-in capital	Deficit accumulated during the development stage	Total shareholders equity
	Number	Amount	Number	Amount			
Balance as of January 22, 2012 (date of inception)							
Issuance of Ordinary shares to founders	7,551,427	20			(20)		
Issuance of Preferred A shares, net ($0.53 per share)			566,357	1	294		295
Share based compensation related					9		9
Loss						(229)	(229)
Balance as of December 31, 2012	7,551,427	20	566,357	1	283	(229)	75
Conversion of Preferred A shares into Ordinary shares	566,357	1	(566,357)	(1)			
Deemed Dividend					26	(26)	
Issuance of Ordinary shares, net ($0.95 per share)	1,065,083	3			988		991
Share based compensation related					254		254
Loss						(1,145)	(1,145)
Balance as of December 31, 2013	9,182,867	$24		$	$1,551	$(1,400)	$175

The accompanying notes are an integral part of the financial statements.

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BIO BLAST PHARMA LTD.
(A development stage company)

STATEMENTS OF CASH FLOWS
U.S. dollars in thousands, except share data

	Year ended December 31,		Period from January 22, 2012 (date of inception) to December 31, 2013
	2013	2012	
Cash flows from operating activities			
Loss	$(1,145)	$ (229)	$ (1,374)
Adjustments to reconcile loss to net cash used in operating activities:			
Depreciation	*)		*)
Stock based compensation	254	9	263
Change in operating assets and liabilities:			
Receivables and prepaid expenses	(19)	(10)	(29)
Long-term deposit	(5)		(5)
Trade payables	(28)	74	46
Other accounts payables	78	7	85
Net cash used in operating activities	(865)	(149)	(1,014)
Cash flows from investing activities			
Purchase of property and equipment	(2)		(2)
Net cash used in investing activities	(2)		(2)
Cash flows from financing activities			
Issuance of shares, net	991	295	1,286
Net cash provided by financing activities	991	295	1,286
Increase in cash and cash equivalents	124	146	270
Cash and cash equivalents at the beginning of the period	146		
Cash and cash equivalents at the end of the period	$270	$ 146	$ 270

*) Represents an amount lower than $1.

The accompanying notes are an integral part of the financial statements.

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 1:- GENERAL

a. Bio Blast Pharma Ltd. (the Company) was incorporated in Israel and commenced its operation on January 22, 2012. The Company is an emerging biopharmaceutical company primarily focused on the identification, licensing, acquisition, development and commercialization of drugs for rare and ultra-rare genetic and metabolic diseases. The Company seeks to identify therapeutic platforms that offer solutions for several diseases that share a common pathophysiological mechanism. The Company s objective is to conduct additional clinical trials for its drugs (the Drugs) and, if those trials are successful, seek marketing approval from the U.S. Food and Drug Administration (the FDA) and other worldwide regulatory bodies.

b. The Company is engaged in the research and development of products in the biopharmaceutical filed, has not generated revenue from the sale of any product, and does not expect to generate significant revenue unless and until the obtaining of marketing approval, and commercialize its Drugs. Accordingly, the Company is considered to be in the development stage as defined in ASC 915, Development stage entities .

c. The Company has incurred losses in the amount of $1,145 during the year ended December 31, 2013. The Company has deficit accumulated during the development stage in the amount of $1,400 as of December 31, 2013 and as of that date the accumulated negative cash flow from operating activity is in the amount of $1,014. These conditions raise substantial doubts about the Company s ability to continue as a going concern. The Company s ability to continue to operate is dependent upon raising additional funds to finance its activities. According to the management s estimates, based on the Company s budget, if the Company is not successful in obtaining additional capital resources to maintain its operational activities, there is substantial doubt that the Company will be able to continue its activity until December 31, 2014. The Company is in the process of listing its securities on the NASDAQ Stock Market (the NASDAQ), for the purpose of raising capital to finance its operations. There are no assurances, however, that the Company will be successful in obtaining an adequate level of financing needed for the long-term development and commercialization of its products. The financial statements do not include any adjustments with respect to the carrying amounts of assets and liabilities and their classification that might be necessary should the Company be unable to continue as a going concern.

NOTE 2:- SIGNIFICANT ACCOUNTING POLICIES

The financial statements have been prepared in accordance with accounting principles generally accepted in the United States (U.S. GAAP).

a. Use of estimates:

The preparation of the financial statements in conformity with U.S. GAAP requires management to make estimates, judgments and assumptions. The Company s management believes that the estimates, judgments and assumptions used are reasonable based upon information available at the time they are made. These estimates, judgments and assumptions can affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the dates of the financial statements, and the reported amounts of expenses during the reporting period. Actual

results could differ from those estimates.

b. Unaudited pro forma information:

Unaudited pro forma information as of December 31, 2013 as adjusted for the assumed two significant transactions of issuance of shares by the Company on January 1, 2014 and on February 6, 2014 of 1,065,076 Ordinary shares in an amount of $1,012 and 782,537 Ordinary shares in an

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 2:- SIGNIFICANT ACCOUNTING POLICIES - (continued)

amount of $4,368 net, respectively. No pro forma basic and diluted loss per share has been presented, as these transactions are assumed to occur on December 31, 2013, (see Note 12.4 and Note 12.7).

c. Financial statements in U.S. dollars:

The Company finances its operation in U.S. dollars. The majority of the Company s operations are currently conducted in Israel, a significant part of the Company s expenses are denominated and determined in U.S. dollars. The Company s management believes that the dollar is the currency of the primary economic environment in which the Company operates and expects to continue to operate in the foreseeable future. Thus, the functional and reporting currency of the Company is the U.S. dollar.

Transactions and balances denominated in U.S. dollars are presented at their original amounts. Non-dollar transactions and balances have been remeasured to U.S. dollars in accordance with ASC 830, Foreign Currency Matters , of the Financial Accounting Standards Board (FASB). All transaction gains and losses from remeasurement of monetary balance sheet items denominated in non-dollar currencies are reflected in the statements of operations as financial income or expenses, as appropriate.

d. Cash equivalents:

Cash equivalents are short-term highly liquid investments that are readily convertible to cash with original maturities of three months or less at acquisition.

e. Property and equipment, net:

Property and equipment are stated at cost, net of accumulated depreciation. Depreciation is calculated using the straight-line method over the estimated useful lives of the assets at the following rates:

	%
Computers and electronic equipment	33

The Company s property and equipment are reviewed for impairment in accordance with ASC 360, Property, Plant, and Equipment, whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset to the future undiscounted cash flows expected to be generated by the assets. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the carrying amount of the assets exceeds the fair value of the assets. Assets to be disposed of are reported at the lower of the carrying amount or fair value less costs to sell. In 2013 and 2012, no impairment losses have been identified.

f. Long-term deposits:

Long-term deposits include long-term deposits for motor vehicles under operating leases, presented at their cost.

g. Research and development costs:

Research and development costs are expensed as incurred. Those expenses includes payments to third party clinical consultants, expenses related to conducting clinical trials, salaries and related personnel expenses, travel expenses, and share based compensations expenses to research and development employees.

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 2:- SIGNIFICANT ACCOUNTING POLICIES - (continued)

h. Income taxes:

The Company account for income taxes in accordance with ASC 740, Income Taxes . This topic prescribes the use of the liability method whereby deferred tax assets and liability account balances are determined based on differences between financial reporting and tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. The Company provides a valuation allowance, to reduce deferred tax assets to the amount that is more likely than not to be realized.

The Company implements a two-step approach to recognize and measure uncertain tax positions. The first step is to evaluate the tax position taken or expected to be taken in a tax return by determining if the weight of available evidence indicates that it is more likely than not that, on an evaluation of the technical merits, the tax position will be sustained on audit, including resolution of any related appeals or litigation processes. The second step is to measure the tax benefit as the largest amount that is more than 50% (cumulative basis) likely to be realized upon ultimate settlement. As of December 31, 2013 and 2012 the Company has not recorded a liability for uncertain tax positions.

i. Concentrations of credit risk:

Financial instruments that potentially subject the Company to concentrations of credit risk consist principally of cash and cash equivalents.

Cash and cash equivalents are invested in major banks in Israel. Management believes that the financial institutions that hold the Company s investments are financially sound and, accordingly, minimal credit risk exists with respect to these investments.

The Company has no off-balance-sheet concentration of credit risk such as foreign exchange contracts, option contracts or other foreign hedging arrangements.

j. Fair value of financial instruments:

The Company has no financial instruments that are measured at fair value.

The carrying amounts of cash and cash equivalents, accounts receivable and accounts payable, approximate their fair value due to the short-term maturities of such instruments.

k. Basic and diluted loss per share:

Basic loss per share is computed based on the weighted average number of Ordinary shares outstanding during each year. Diluted loss per share is computed based on the weighted average number of Ordinary shares outstanding during each year plus dilutive potential equivalent Ordinary shares considered outstanding during the year, in accordance

with ASC 260, Earnings per Share.

All outstanding stock options have been excluded from the calculation of the diluted net loss per share because all such securities are anti-dilutive for all periods presented.

l. Accounting for stock-based compensation:

The Company accounts for stock-based compensation in accordance with ASC 718, Compensation Stock Compensation that requires the measurement and recognition of compensation expense based on estimated fair values for all share-based payment awards made to employees, directors. ASC 718 requires companies to estimate the fair value of equity-based payment awards on the date of grant using an option-pricing model. The value of the option award

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 2:- SIGNIFICANT ACCOUNTING POLICIES - (continued)

is recognized as an expense over the requisite service periods in the Company s statement of operations based on the accelerated method.

As all options were granted at par value and the exercise price is negligible, the fair value of the options is equal to the share price.

NOTE 3:- RECEIVABLES AND PREPAID EXPENSES

	December 31,	
	2013	2012
Government authorities	$ 26	$ 10
Prepaid expenses	3	
	$ 29	$ 10

NOTE 4:- PROPERTY AND EQUIPMENT, NET

	December 31,	
	2013	2012
Cost:		
Computers and electronic equipment	$ 2	$ 2
Accumulated depreciation:		
Computers and electronic equipment	*)	*)
Depreciated cost	$ 2	$

*) Represents an amount lower than $1.

Depreciation expenses for the year ended December 31, 2013 were less than $1.

NOTE 5:- OTHER ACCOUNTS PAYABLE

	December 31,	
	2013	2012
Employees and payroll accruals	$ 26	$
Accrued expenses	59	7
	$ 85	$ 7

NOTE 6:- INCOME TAXES

a. Tax rates applicable to the Company:

The Company is incorporated in Israel. Taxable income of Israeli companies is subject to tax at the rate of 25% in 2012 and 2013 and 26.5% in 2014 and onward.

b. Net operating losses carry forward:

The Company has accumulated losses for tax purposes as of December 31, 2013 in the amount of approximately $600 which may be carried forward and offset against taxable income in the future for an indefinite period.

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 6:- INCOME TAXES - (continued)

c. Deferred income taxes:

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of the Company s deferred tax assets are as follows:

	December 31,	
	2013	2012
Operating loss carry forward	$ 166	$ 26
Research and Development expenses	146	29
Net deferred tax asset before valuation allowance	312	55
Valuation allowance	(312)	(55)
Net deferred tax asset	$	$

Management currently believes that since the Company has a history of losses it is more likely than not that the deferred tax regarding the loss carry forward and other temporary differences will not be realized in the foreseeable future.

d. No liability for uncertain tax positions was recorded as a result of implementation of ASC 740.

e. The main reconciling item between the statutory tax rate of the Company and the effective tax rate is the recognition of valuation allowances in respect of deferred taxes relating to accumulated net operating losses carried forward due to the uncertainty of the realization of such deferred taxes.

NOTE 7:- CONTINGENT LIABILITIES AND COMMITMENTS

a. The Company is engaged in an operating lease agreement for its office facilities. Future minimum non-cancelable rental payments under the operating lease are $4. The rent expenses for the year ended December 31, 2013 amounted to $14.

b. On June 2013 the Company entered into an operating lease agreement for its vehicles until 2016. The rent expenses for the year ended December 31, 2013 amounted to $11. Future minimum payments under the lease are as follows:

Year ended December 31,	Total
2014	$ 15
2015	15
2016	9
	$ 39

c. License agreement:

The Company entered into a Research and Exclusive License Agreement with Yissum Research Development Company of the Hebrew University in Jerusalem Ltd., for the use, development and commercialization of the TAT-MTS-Protein for protein replacement in mitochondrial diseases. The consideration to Yissum is composed of tiered low single digit royalties on net sales and a sublicense fee that will not exceed double-digits in the mid to high ten percent range of the sublicense consideration, but, if the sublicense arises from the sales of a product, the sublicense fee shall not be less than a low single digit percent of the gross sales of such product.

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 8:- SHAREHOLDERS EQUITY

a. General:

All Ordinary shares, options, per share data and exercise price included in these financial statements for all periods presented have been retroactively adjusted to reflect the issuance on January 26, 2014 of 6.55-to-one bonus shares (equivalent to a 7.55-for-1 stock split), (see Note 12.2).

b. Share capital:

The Ordinary shares confer upon their holders the right to participate and vote in general shareholders meetings of the Company and to share in the distribution of dividends, if any declared by the Company.

c. Issuances of share:

1. On January 22, 2012, (inception day) the Company issued 7,551,427 Ordinary shares in consideration of their par value.
2. In February 2012, the Company entered into an investment agreement, according to which, the Company issued 471,964 Preferred A shares in consideration of $250. In addition, in August 2012, the Company issued 94,393 Preferred A shares in consideration of $50. The issuance expenses amounted to $5.
3. In June 2013, the Company entered into a share purchase agreement according to which, the Company shall issue a total of 2,130,159 Ordinary shares in consideration of $2,024, in two equal installments. As of December 31, 2013, the Company issued 1,065,083 Ordinary shares in consideration of $991, net of issuance expenses. Subsequent to the balance sheet date, on January 1, 2014, the Company issued 1,065,076 Ordinary shares in consideration for $1,012.

Prior to the closing of the share purchase agreement above and as a condition to it, the Company affected an equity restructuring, under which, all of the Company s Preferred shares (566,357 Preferred A shares) were converted into Ordinary shares at 1:1 ratio. As a result and in accordance with ASC 718-20-35-6, the Company recorded compensation expense in the amount of $183 and a deemed dividend in the amount of $26 in the year ended December 31, 2013.

d. 2013 incentive option plan:

In December 2013, the Company authorized through its 2013 incentive option plan (the 2013 Plan) the grant of options to officers, directors, advisors, management and other key employees. The options granted have a graded vesting schedule of generally four years and expire ten years after the grant date (see Note 12.5).

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 8:- SHAREHOLDERS EQUITY - (continued)

A summary of the Company s options activity (for employees and directors) under the 2013 Plan is as follows:

	Year ended December 31,			
	2013		2012	
	Number of options	Weighted average exercise price	Number of options	Weighted average exercise price
Outstanding at beginning of year	319,531	$ 0.0004		$
Granted	83,579	$ 0.0004	319,531	$ 0.0004
Outstanding at end of year	403,110	$ 0.0004	319,531	$ 0.0004
Vested and expected to vest	403,110	$ 0.0004		
Options exercisable at the end of the period	134,370	$ 0.0004		

As of December 31, 2013, the weighted-average remaining contractual term of the outstanding and exercisable options is 8.53 years; the aggregated intrinsic value of the outstanding and exercisable options is $383 and $128. As of December 31, 2013, the unrecognized compensation cost is $20 and $5 to be recognized in 2014 and 2015, respectively.

e. Options granted to consultants:

The Company did not grant any options to consultants.

f. Share-based payment:

The share based expense recognized in the financial statements is as follows:

	Year ended December 31,		Period from January 22, 2012 (date of inception) to December 31, 2013
	2013	2012	
Research and development	$ 44	$	$ 44
General and administrative expenses	$ 210	$ 9	$ 219
	$ 254	$ 9	$ 263

NOTE 9:- RELATED PARTY BALANCES AND TRANSACTIONS

Balances with related parties:

	December 31,	
	2013	2012
Trade payable[(d)]	$ 1	$
Other accounts payable[(a)(b)(c)]	$ 36	$ 8

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 9:- RELATED PARTY BALANCES AND TRANSACTIONS - (continued)

Related parties expenses:

	Year ended December 31,		Period from January 22, 2012 (date of inception) to December 31, 2013
	2013	2012	
Amounts charged to:*)			
General and administrative expense[(a)(b)(c)(d)]	$ 257	$ 55	$ 312
Research and Development expense[(c)]	$ 99	$ 34	$ 133

*) Including share based compensation expenses for the year ended December 31, 2013 in the amounts of $176.

a. In August, 2012, the Company signed an agreement with a consultant, who is also one of the Company s shareholders and a director, as a contractor to render management, finance and operation services. The Company pays the consultant an amount of $6 per month.

b. The Company signed an agreement with a company owned by one of its related parties. Under the agreement, the related company renders the Company with office services and office lease for a monthly fee in the amount of approximately $4 since September 10, 2013. Each party may terminate the agreement with 30-days notice.

c. An agreement was signed on August 20, 2013 between the Company and one its shareholders, as a contractor to render services related to pre-clinical, clinical, regulatory and intellectual property issues, for an amount of approximately $15 per month.

d. On July 1, 2013 the Company signed an agreement with a consultant, who is also one of the Company s shareholders. The consultant shall provide advisory services. The Company pays the consultant an amount of $1 per month.

NOTE 10:- FINANCIAL EXPENSES (INCOME), NET

	Year ended December 31,		Period from January 22, 2012 (date of inception) to
	2013	2012	

			December 31, 2013
Financial expenses:			
Interest expense	$ 2	$)*	$ 2
Exchange rate		3	3
	2	3	5
Financial income:			
Exchange rate	5		5
	5		5
	$ (3)	$ 3	$

*) Represents an amount lower than $1.

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BIO BLAST PHARMA LTD.
(A development stage company)

NOTES TO FINANCIAL STATEMENTS
U.S. dollars in thousands, except share data

NOTE 11:- BASIC AND DILUTED NET LOSS PER SHARE

The following table sets forth the computation of the Company s basic and diluted loss per Ordinary share:

	Year ended December 31,	
	2013	2012
Net loss attributable to Ordinary shares as reported	$(1,171)	$(229)
Shares used in computing net loss per share of Ordinary shares, basic and diluted	8,423,018	7,551,427
Net loss per share of Ordinary share, basic and diluted	$(0.14)	$(0.03)

For the years ended December 31, 2013 and 2012, all outstanding options have been excluded from the calculation of the diluted net loss per share since their effect was anti-dilutive.

NOTE 12:- SUBSEQUENT EVENTS

1. The Company evaluates events or transactions that occur after the balance sheet date but prior to the issuance of financial statements to identify matters that require additional disclosure. For its financial statements as of December 31, 2013 and for the year then ended, the Company evaluated subsequent events through April 7, 2014, the date that the financial statements were issued. Except as described below, the Company has concluded that no subsequent event has occurred that requires disclosure.
2. On January 26, 2014, the Company s shareholders resolved to issue bonus shares of 6.55-to-one bonus shares (equivalent to a 7.55-for-1 stock split) to all of its Ordinary shares and options holders.
3. During January 2014, the Company s shareholders resolved to increase the Company s authorized shares to 17,000,000 Ordinary shares.
4. On January 1, 2014 and in connection to the share purchase agreement dated June 2013 (see Note 8.c.3), the Company issued 1,065,076 Ordinary shares in consideration of $1,012.
5. During January 2014, the Company s board of directors resolved to increase the Company s options pool reserved for future grant to a total of 456,899.
6. During January 2014, the Company entered into an Exclusive License Agreement with Ramot At Tel Aviv University, for the use, development and commercialization of our-read through platform.
7. On February 6, 2014, the Company issued 782,537 Ordinary shares to private placement investors in consideration of $4,368, net.
8. On April 1, 2014, the Company's board of directors resolved to approve the grant of options to purchase 8,520 ordinary shares of the Company to an employee. The exercise price shall be $0.95 per share.
9. On April 1, 2014, the Company s board of directors resolved the following resolutions, subject to the approval of the general meeting and to the closing of an Initial Public Offering (the IPO):

(i) To issue the chairman of the board 206,702 options to ordinary shares and to pay him an annual compensation fee in an amount of $120.

(ii) To increase the monthly fee of two related party consultants to approximately $17 and $19

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NOTE 12:- SUBSEQUENT EVENTS - (continued)

per month, respectively. In addition, it was resolved to grant the consultants a one-time bonus of $80 and $90, respectively, upon the closing of the IPO (See Note 9.a and 9.c).

(iii) To approve an annual service fee of US$25 to each of the Company s board members, excluding the chairman of the board, any executive of the Company and the external directors.

(iv) To increase the Company s authorized shares to 50,000,000 ordinary shares.

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3,200,000 Shares

Ordinary Shares

PROSPECTUS

Oppenheimer & Co. **Roth Capital Partners**

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