Axovant Sciences Ltd.
Form 8-K
July 09, 2018

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **July 8, 2018**

Axovant Sciences Ltd.

(Exact name of registrant as specified in its charter)

Bermuda	**001-37418**	**98-1333697**
(State or other jurisdiction of incorporation)	(Commission File No.)	(I.R.S. Employer Identification No.)

Suite 1, 3rd Floor

11-12 St. James s Square

London SW1Y 4LB, United Kingdom

(Address of principal executive office)

Registrant s telephone number, including area code: **+44 203 318 9708**

(Former name or former address, if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company x

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. x

Item 1.01 **Entry into a Material Definitive Agreement.**

Benitec Biopharma License and Collaboration Agreement

On July 8, 2018, Axovant Sciences Ltd., through its wholly owned subsidiary, Axovant Sciences GmbH, entered into a license and collaboration agreement (the License Agreement) with Benitec Biopharma Limited (Benitec). Pursuant to the License Agreement, we received a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Benitec to develop and commercialize investigational gene therapy BB-301 and related gene therapy products (collectively, the AXO-AAV-OPMD Program) for all diseases and conditions. The AXO-AAV-OPMD Program employs a single vector gene therapy construct which uses DNA directed RNA interference (ddRNAi) that is designed to silence expression of the mutant gene associated with oculopharyngeal muscular dystrophy (OPMD), while simultaneously adding back a copy of the functional version of the same gene to restore normal gene function.

Benitec will perform certain development and manufacturing activities for the AXO-AAV-OPMD Program, and we will reimburse Benitec for its costs incurred, in accordance with an agreed-upon development plan and budget. We are solely responsible, at our expense, for all other activities related to the development and commercialization of products from the AXO-AAV-OPMD Program.

Under the License Agreement, we will also collaborate with Benitec on five additional research plans as part of the Collaboration Programs for other genetic neurological disorders using Benitec technologies. Benitec will perform certain research activities for each Collaboration Program, and we will reimburse Benitec for its costs incurred, in accordance with an agreed-upon research plan and budget. We will receive a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Benitec to develop and commercialize products arising from each Collaboration Program. We are solely responsible, at our expense, for all other activities related to the research, development and commercialization of products arising from the Collaboration Programs.

We have agreed to use commercially reasonable efforts to develop and commercialize products from the AXO-AAV-OPMD Program and Collaboration Programs in certain agreed-upon markets. Benitec has agreed to customary non-compete restrictions limiting its ability to develop or commercialize certain directly-competing gene therapy products.

We will make an upfront payment to Benitec of $10.0 million. In addition, we will be obligated to make payments to Benitec totaling up to (i) for the AXO-AAV-OPMD Program, $67.5 million upon the achievement of specified development and regulatory milestones and $120.0 million upon the achievement of specified sales milestones, and (ii) for each Collaboration Program, $33.5 million upon the achievement of specified development and regulatory milestones and $60.0 million upon the achievement of specified sales milestones.

Benitec will receive 30% of net profits of world-wide sales of products arising from the AXO-AAV-OPMD Program, subject to an agreed minimum amount for such payments. This profit sharing payment will be made for so long as we or our affiliates or sublicensees commercialize such products. We will also pay Benitec a tiered royalty based on yearly aggregate net sales of products arising from each Collaboration Program, subject to specified reductions upon the occurrence of certain events as set forth in the License Agreement. These royalties are required to be paid, on a product-by-product and country-by-country basis, until the latest to occur of the expiration of the last to expire valid claim of a licensed patent covering such product in such country, the expiration of regulatory exclusivity for such product in such country, or ten years after the first commercial sale of such product in such country.

The License Agreement will expire, (i) with respect to the AXO-AAV-OPMD Program, upon the expiration of our obligation to make profit-sharing payments to Benitec as described above, and (ii) with respect to each product from a Collaboration Program, upon the expiration of the royalty payment term described above for such product throughout the world. We may terminate the License Agreement on a program-by-program basis at any time for any reason with prior written notice to Benitec upon 90 days prior notice before the first regulatory approval for a product from the applicable program, or upon 180 days prior notice thereafter. Either party may terminate the License Agreement for the other party s uncured material breach of the Agreement or insolvency. If the License Agreement is terminated with respect to a program, all rights and licenses granted to us with respect to such program cease.

The foregoing description of the License Agreement does not purport to be complete and is qualified in its entirety by reference to the License Agreement, a copy of which we expect to file as an exhibit to our Quarterly Report on Form 10-Q for the quarter ending September 30, 2018.

Silence-and-Replace Technology

The Silence-and-Replace technology platform is designed to produce a long-term restoration of normal gene function and is achieved by combining RNA interference (silence) with gene therapy (replace) in a single administration of a single viral vector construct. This approach is applicable to various genetic diseases, particularly autosomal dominant genetic disorders caused by nucleotide repeat expansion.

Many neurological and muscular diseases are known to result from the erroneous expression of a mutated gene. RNA interference (RNAi) has shown significant potential to silence the expression of these disease-associated genes. Commonly-used RNAi approaches, in which small interfering RNA (siRNA) is introduced directly into the cell, achieve only transient gene silencing and are limited by the requirement for repeated administration and variable concentrations of siRNA over time. To provide lasting gene silencing, the Silence-and-Replace technology employs ddRNAi, in which viral vectors deliver a DNA construct that produces short hairpin RNAs (shRNAs), which are processed by the cell into siRNAs, which then silence the mutated genes.

In an autosomal dominant genetic disorder, particularly one caused by nucleotide repeat expansion, silencing of the mutant gene can also lead to silencing of the wild type gene which may be required for normal function. The Silence-and-Replace strategy is designed to address this potential issue by delivering a functional copy of the gene that is re-engineered to be resistant to knockdown. The gene that encodes the functional protein is contained within the same viral vector as the ddRNAi construct.

AXO-AAV-OPMD Program

The AXO-AAV-OPMD Program is an investigational gene therapy being developed as a one-time treatment for OPMD. The Program utilizes an AAV vector to silence the mutant poly-A binding protein N1 (*PABPN1*) gene that causes OPMD and replace with a functional copy of the *PABPN1* gene. The Silence-and-Replace approach aims to knock down the expression of the mutant *PABPN1* gene through ddRNAi, while at the same time express a re-engineered copy of the *PABPN1* gene coding for the functional PABPN1 protein. The gene therapy will be delivered in a single administration directly into target muscle tissue to provide long-term correction of muscle pathology and restoration of function. Depending on the type of AAV vector that we decide to commercialize, it may be necessary for us to use the patented or proprietary technology of one or more third parties to commercialize AXO-AAV-OPMD. If we are unable to use these technologies or obtain licenses from these third parties when needed or on commercially reasonable terms, our ability to commercialize AXO-AAV-OPMD, if approved, would likely be delayed.

Data from mouse models of OPMD showed gene therapy from the AXO-AAV-OPMD Program provided up to 86% inhibition of *PABPN1* expression, while restoring functional *PABPN1* up to 63% of normal levels. The A17 mouse model is a well validated *in vivo* model that is designed to exhibit many of the key pathological features of OPMD patients. The levels of gene silencing and expression achieved in this model coincided with decreased muscle pathology and a restoration of muscle force and muscle weight to near wild-type levels.

We expect to initiate a placebo-controlled clinical study for the investigational AXO-AAV-OPMD Program in 2019. The U.S. Food & Drug Administration and European Commission have granted Orphan Drug Designation to the AXO-AAV-OPMD Program for the treatment of OPMD.

Oculopharyngeal Muscular Dystrophy

OPMD is a muscular disease that is inherited through a primarily autosomal dominant pattern. OPMD is estimated to affect approximately 15,000 people in North America and Europe. The disease generally presents between the ages of 40-70 years old and is characterized primarily by progressive swallowing difficulty (dysphagia), eyelid drooping (ptosis), and weakness of the proximal extremities. Swallowing difficulties can have life-threating consequences, including malnutrition and aspiration pneumonia. As the disease progresses, the dysphagia becomes more severe and other muscles may become involved. There are no products approved for the treatment of OPMD and therefore, treatment options available to patients are limited.

OPMD is caused by mutations in the gene coding for PABPN1, a ubiquitously expressed protein that regulates the processing of messenger RNAs. The normal PABPN1 protein contains ten copies of the amino acid alanine, which forms a polyalanine tract. In OPMD, the mutated *PABPN1* gene has an expansion of alanine-encoding trinucleotide repeats, resulting in an abnormally long polyalanine tract. The protein that forms from the mutated gene is prone to aggregating into insoluble nuclear inclusion bodies which leads to muscle cell pathology and disease progression.

Additional Research Programs

Under our research and development collaboration with Benitec, we will pursue five additional investigational gene therapy research plans as part of Collaboration Programs focused on genetic neurological disorders utilizing Benitec s technologies. We plan to initiate a research plan to develop gene therapy products targeting the *C9orf72* gene which is associated with amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD). In addition, we plan to initiate four other research plans focused on undisclosed genetic neurological disorders.

ALS and FTD are neurological disorders that have been linked to hexanucleotide repeats in the *C9orf72* gene. Thirty to forty percent of familial ALS cases are associated with *C9orf72* mutations and these patients have a progressive muscle weakness resulting from the death of motor neurons in the spinal cord and brain. Patients with FTD associated with *C9orf72* mutations have a progressive brain disorder that affects personality, behavior, language and movement. While the exact role of *C9orf72* mutation is unknown, both expression of the mutated *C9orf72* and lack of functional *C9orf72* are believed to be implicated. We believe Silence-and-Replace gene therapy is a promising approach for the restoration of normal *C9orf72* function and has the potential to deliver lasting benefits for ALS and FTD patients.

Item 7.01 **Regulation FD Disclosure.**

On July 9, 2018, we issued a press release announcing, among other things, the entry into the License Agreement and our conference call to be held at 8:30 a.m., Eastern time, on July 9, 2018.

A copy of the press release and the presentation to be discussed on the conference call are furnished as Exhibit 99.1 and Exhibit 99.2, respectively, to this Current Report on Form 8-K and are incorporated by reference into this Item 7.01. The information furnished under this Item 7.01, including Exhibit 99.1 and Exhibit

99.2, shall not be deemed filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or subject to the liabilities of that section. The information shall not be deemed incorporated by reference into any other filing with the Securities and Exchange Commission made by us, regardless of any general incorporation language in such filing.

Item 9.01 **Financial Statements and Exhibits.**

(d) Exhibits.

Exhibit No.	
99.1	Press release dated July 9, 2018.
99.2	Presentation dated July 9, 2018.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Axovant Sciences Ltd.

Date: July 9, 2018	By:	/s/ Gregory Weinhoff	
		Name:	Gregory Weinhoff
		Title:	Principal Financial Officer