



## Axovant Announces Second Fiscal Quarter 2018 Financial Results and Corporate Updates

November 7, 2018

- First patient dosed in clinical study of AXO-Lenti-PD, a novel gene therapy for treatment of Parkinson's disease
- Expanded pipeline of gene therapies with in-licensing of AXO-AAV-OPMD for treatment of oculopharyngeal muscular dystrophy (OPMD) and collaboration agreement for five additional neurological gene therapy programs
- Board capabilities expanded with additions of Dr. Frank Torti, Myrtle Potter and Dr. Pavan Cheruvu, CEO
- Mathew Bazley appointed as general counsel

BASEL, Switzerland, Nov. 07, 2018 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ: **AXON**) today provided financial results and corporate updates for its second fiscal quarter ended September 30, 2018.

"We are pleased with the expansion of our gene therapy pipeline and continued evidence of execution with dosing of our first patient in the AXO-Lenti-PD clinical study. Across Axovant's portfolio of innovative gene therapies, we see a promising opportunity to deliver lasting, transformative treatments to patients with serious neurological and neuromuscular diseases," said Pavan Cheruvu, M.D., chief executive officer of Axovant.

"Axovant has built a pipeline of potentially best-in-class gene therapies and continues to advance the manufacturing capacity to support it through development and commercialization. The progress made thus far helps realize our vision to build Axovant into a leader in neurological gene therapies. We look forward to several major milestones in the coming months and 2019, including initial data from the AXO-Lenti-PD clinical study in the first half of the calendar year 2019 and the initiation of a potentially pivotal clinical study for AXO-AAV-OPMD in the second half of calendar year 2019."

### Key Highlights and Development Updates

- Dosed first patient in clinical study of AXO-Lenti-PD for treatment of Parkinson's disease in October 2018. Initial data from the first cohort of patients is expected in the first half of 2019. The ongoing clinical study will evaluate safety and tolerability, as well as collect efficacy data including standard measures of motor function in patients with Parkinson's disease.
- Long-term results from ProSavin® phase I/II study, presented at the Annual Congress of the European Society of Gene and Cell Therapy and published in *Human Gene Therapy*, show favorable safety and tolerability with sustained improvements in motor function observed for up to 6 years.
- Licensed global rights for AXO-AAV-OPMD, a gene therapy for the treatment of oculopharyngeal muscular dystrophy, along with collaboration agreement for development of five additional gene therapy products in neurological disorders based on the Silence-and-Replace platform.
- Presented preclinical data for AXO-AAV-OPMD at the Annual Congress of the European Society of Gene and Cell Therapy.
- Completed engineering run of AXO-AAV-OPMD at 250L scale using a baculovirus-based production system, in anticipation of cGMP manufacturing for planned clinical program.
- Discontinued development plans for RVT-104, a combination of rivastigmine and a peripheral muscarinic receptor antagonist, as a potential treatment for patients with Alzheimer's disease or dementia with Lewy bodies.

### Corporate Update

Mathew Bazley was appointed general counsel of Axovant in October 2018. Mr. Bazley was previously general counsel of The Medicines Company's infectious disease business and deputy general counsel of the company as a whole. Prior to The Medicines Company, he served in multiple roles at Savient Pharmaceuticals, Inc. Prior to Savient, he practiced law in the New York office of Skadden, Arps, Slate, Meagher & Flom LLP. He began his career as an attorney with the U.S. Securities & Exchange Commission in Washington D.C. He received his J.D. and M.B.A. from Emory University and a B.S.B.A. from Georgetown University.

In September 2018, Pavan Cheruvu, M.D., the chief executive officer of Axovant, Frank M. Torti, M.D., and Myrtle S. Potter were appointed to the Board of Directors, with Dr. Torti appointed as chairperson of the Board. The Board also appointed Atul Pande, M.D., a current member of the Board, as lead independent director and Vivek Ramaswamy resigned as a member and chairperson of the Board. Dr. Torti and Ms. Potter are assuming leadership positions on the Board as Mr. Ramaswamy departs to focus on his role as chief executive officer of Roivant Sciences, Inc.

### Second-Quarter Financial Summary

For the second fiscal quarter ended September 30, 2018, research and development expenses were \$21.5 million, of which \$10.0 million was attributable to the upfront licensing fee paid to Benitec Biopharma and which includes a \$1.1 million benefit attributable to non-cash, share-based compensation, net of forfeitures. General and administrative expenses for the second fiscal quarter ended September 30, 2018 were \$10.6 million, of which \$3.6 million was non-cash, share-based compensation expense. Net loss for the quarter ended September 30, 2018 was \$33.8 million, or \$0.28 per share.

## First-Half Financial Summary

For the first fiscal half ended September 30, 2018, research and development expenses were \$58.9 million, of which \$25.0 million was attributable to the upfront licensing fee paid to Oxford BioMedica, \$10.0 million was attributable to the upfront licensing fee paid to Benitec Biopharma and \$1.4 million was attributable to non-cash, share-based compensation expense, net of forfeitures. General and administrative expenses for the first half ended September 30, 2018 were \$22.4 million, of which \$6.9 million was attributable to non-cash, share-based compensation expense. Net loss for the six months ended September 30, 2018 was \$85.7 million, or \$0.75 per share.

As of September 30, 2018, the Company had \$90.7 million of cash, working capital of \$47.4 million, and long-term debt of \$33.3 million. Net cash used in operating activities was \$88.7 million for the six months ended September 30, 2018, which includes the \$30.0 million upfront payment to Oxford BioMedica, \$5.0 million of which will be applied as a credit against the process development work and clinical supply that Oxford BioMedica will provide to us, and the \$10.0 million upfront payment to Benitec Biopharma.

## Upcoming Presentation

Pavan Cheruvu, M.D., chief executive officer, will present a corporate overview at the Jefferies 2018 London Healthcare Conference in London, UK on November 14, 2018 at 5:20 p.m. GMT.

A live webcast will be available in the Events section of Axovant's website at [www.axovant.com](http://www.axovant.com). A replay will be available for 30 days following the conference.

## About Axovant Sciences

Axovant is a clinical-stage gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological and neuromuscular diseases such as Parkinson's disease, oculopharyngeal muscular dystrophy (OPMD), amyotrophic lateral sclerosis (ALS), frontotemporal dementia and other indications. For more information, visit [www.axovant.com](http://www.axovant.com).

## Forward-Looking Statements

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as "may," "might," "will," "would," "should," "expect," "plan," "anticipate," "believe," "estimate," "project," "intend," "future," "potential," "continue," or "well-positioned" and other similar expressions are intended to identify forward-looking statements. For example, all statements Axovant makes regarding the initiation, timing, progress, and reporting of results of its preclinical programs, clinical trials, and research and development programs; its ability to advance its small molecule and gene therapy product candidates into and successfully initiate, enroll, and complete clinical trials; the potential clinical utility of its product candidates; its ability to continue to develop its small molecule and gene therapy platforms; its ability to develop and manufacture its products and successfully transition manufacturing processes; its ability to perform under existing collaborations with, among others, Oxford BioMedica and Benitec, and to add new programs to its pipeline; its ability to enter into new partnerships or collaborations; its ability to retain and successfully integrate its leadership and personnel; and the timing or likelihood of its regulatory filings and approvals, are forward-looking. All forward-looking statements are based on estimates and assumptions by Axovant's management that, although Axovant believes to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Axovant expected. Such risks and uncertainties include, among others, the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory submissions and approvals; the continued development of its small molecule and gene therapy product candidates and platforms; Axovant's scientific approach and general development progress; and the availability or commercial potential of Axovant's product candidates. These statements are also subject to a number of material risks and uncertainties that are described in Axovant's most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 7, 2018, as updated by its subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Axovant undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

## Contacts:

### Media

Lara Yuan  
(646) 802-3585  
[media@axovant.com](mailto:media@axovant.com)

### Investors

Tricia Truehart  
(631) 892-7014  
[investors@axovant.com](mailto:investors@axovant.com)

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**AXOVANT SCIENCES LTD.**  
**Condensed Consolidated Statements of Operations**  
*(Unaudited, in thousands, except share and per share amounts)*

	Three Months Ended September 30,		Six Months Ended September 30,	
	2018	2017	2018	2017
Operating expenses:				
Research and development expenses <sup>(1)</sup>				
(includes total share-based compensation expense (benefit) of \$(1,128) and \$5,916 for the three months ended September 30, 2018 and 2017 and \$1,389 and \$12,172 for the six months ended	\$ 21,502	\$ 38,555	\$ 58,920	\$ 82,267

September 30, 2018 and 2017, respectively)

General and administrative expenses<sup>(2)</sup>

(includes share-based compensation expense of \$3,585 and \$9,424 for the three months ended September 30, 2018 and 2017 and \$6,927 and \$18,768 for the six months ended September 30, 2018 and 2017, respectively)

	10,622	30,112	22,376	51,630
Total operating expenses	32,124	68,667	81,296	133,897
Other expenses:				
Interest expense	1,932	1,878	3,902	3,752
Other expense (income)	(315)	) 131	353	(226)
Loss before income tax expense	(33,741)	) (70,676)	) (85,551)	) (137,423)
Income tax expense (benefit)	94	) (1,590)	) 172	929
Net loss	\$ (33,835)	) \$ (69,086)	) \$ (85,723)	) \$ (138,352)
Net loss per common share — basic and diluted	\$ (0.28)	) \$ (0.64)	) \$ (0.75)	) \$ (1.29)
Weighted average common shares outstanding — basic and diluted	20,863,455	107,593,609	114,362,408	107,000,519

<sup>(1)</sup> Includes total costs allocated from Roivant Sciences Ltd. ("RSL"), Roivant Sciences, Inc. ("RSI") and Roivant Sciences GmbH ("RSG") of \$(3,069) and \$2,257 for the three months ended September 30, 2018 and 2017, respectively, and \$(450) and \$5,258 for the six months ended September 30, 2018 and 2017, respectively.

<sup>(2)</sup> Includes total costs allocated from RSL, RSI and RSG of \$772 and \$1,623 for the three months ended September 30, 2018 and 2017, respectively, and \$2,074 and \$3,496 for the six months ended September 30, 2018 and 2017, respectively.

**AXOVANT SCIENCES LTD.**  
**Condensed Consolidated Balance Sheets**  
*(Unaudited, in thousands)*

	September 30, 2018	March 31, 2018
<b>Assets</b>		
Current assets:		
Cash	\$ 90,726	\$ 154,337
Prepaid expenses and other current assets	4,095	2,174
Income tax receivable	1,530	1,751
Total current assets	96,351	158,262
Other non-current assets	4,324	—
Property and equipment, net	1,513	2,524
Total assets	\$ 102,188	\$ 160,786
<b>Liabilities and Shareholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 1,817	\$ 3,949
Due to RSL, RSI and RSG	2,859	1,011
Accrued expenses	24,315	31,862
Current portion of long-term debt	20,009	9,753
Total current liabilities	49,000	46,575
Long-term debt	33,309	42,925
Total liabilities	82,309	89,500
Shareholders' equity:		
Common shares, par value \$0.00001 per share, 1,000,000,000 shares authorized, 122,175,480 and 107,788,074 issued and outstanding at September 30, 2018 and March 31, 2018, respectively	1	1
Additional paid-in capital	661,980	628,110
Accumulated deficit	(642,674)	) (556,951)
Accumulated other comprehensive income	572	126
Total shareholders' equity	19,879	71,286
Total liabilities and shareholders' equity	\$ 102,188	\$ 160,786

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Source: Axovant Sciences Ltd.