



Axovant Announces Third Fiscal Quarter 2018 Financial Results and Corporate Updates

February 7, 2019

- Expanded pipeline with two investigational gene therapy programs licensed from the University of Massachusetts Medical School for the treatment of GM1 gangliosidosis, Tay-Sachs and Sandhoff diseases
- First two patients dosed in SUNRISE-PD phase 2 trial of AXO-Lenti-PD for Parkinson's disease and initial patient dosed with AXO-AAV-GM2; data from both programs expected in March 2019
- Axovant Sciences Ltd. intends to change its name to Axovant Gene Therapies Ltd. and ticker symbol to "AXGT" to reflect its exclusive focus on gene therapies

BASEL, Switzerland, Feb. 07, 2019 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ: **AXON**), a clinical-stage company developing innovative gene therapies, today provided financial results and corporate updates for its third fiscal quarter and nine months ended December 31, 2018.

"With the addition of new investigational gene therapies for the treatment of GM1 gangliosidosis, Tay-Sachs, and Sandhoff diseases, we are excited about having built a deep pipeline of potentially transformative gene therapies addressing serious conditions. Over the last year, Axovant has become leaner and more cutting-edge in its scientific approach, but we are no less ambitious in our drive to make a difference for patients," said Pavan Cheruvu, M.D., Chief Executive Officer of Axovant. "We look forward to continuing this momentum with a rich set of clinical development milestones this quarter, including the first data readouts in our AXO-Lenti-PD and AXO-AAV-GM2 programs next month, and additional milestones across our pipeline throughout 2019."

Key Highlights and Development Updates

- In-licensed two programs, AXO-AAV-GM1 and AXO-AAV-GM2, for GM1 gangliosidosis, Tay-Sachs and Sandhoff diseases from the University of Massachusetts (UMass) Medical School in December 2018. The programs aim to restore enzymes that are deficient in these diseases by introducing a functional copy of the defective genes.
- First patient dosed with AXO-AAV-GM2 in an investigator-initiated study with initial data expected in March 2019. Plan to dose first patient with AXO-AAV-GM1 in the first half of calendar year 2019.
- Two patients were dosed with AXO-Lenti-PD in the SUNRISE-PD trial for patients with Parkinson's disease. Initial data is expected in March 2019.
- FDA confirmed that studies previously conducted using first generation ProSavin® may be considered part of a single development program with AXO-Lenti-PD, including the preclinical data and over 10 years of clinical data of ProSavin. The FDA also provided feedback on the design of the SUNRISE-PD phase 2 study of AXO-Lenti-PD and agreed that current manufacturing and quality control plans were adequate for the clinical program.
- Hired five new senior leaders with decades of collective gene therapy expertise in key areas such as clinical development, vector optimization and delivery, regulatory affairs, manufacturing and commercialization. Assembled a Scientific Advisory Board of pre-eminent leaders in gene therapy to provide strategic guidance across all development programs.
- Shankar Ramaswamy, MD, was named Chief Business Officer and is responsible for the identification, evaluation, and negotiation of transactions for new gene therapy pipeline assets and other business development opportunities. He was most recently Senior Vice President of Business Development and Operations.
- Axovant Sciences plans to change its name to Axovant Gene Therapies and its stock symbol to "AXGT" to reflect its exclusive focus on the development and commercialization of innovative gene therapies. The company's name change is expected to take effect in March 2019. Common stock will begin trading under a new ticker symbol "AXGT" at the opening of trading on February 14, 2019. The former ticker symbol "AXON" will remain effective through market close on February 13, 2019.
- Completed \$30 million equity financing in December 2018 led by Deerfield Management Company, Sphera Funds Management and Roivant Sciences.

Third-Quarter Financial Summary

For the third fiscal quarter ended December 31, 2018, research and development expenses were \$21.5 million, of which \$10.0 million was attributable to the upfront licensing fee paid to UMass Medical School and \$1.9 million was attributable to non-cash, share-based compensation expense, net of forfeitures. General and administrative expenses for the third fiscal quarter ended December 31, 2018 were \$10.9 million, of which \$2.6 million was non-cash, share-based compensation expense. Net loss for the quarter ended December 31, 2018 was \$34.3 million, or \$0.27 per share.

Nine-Months Financial Summary

For the nine months ended December 31, 2018, research and development expenses were \$80.4 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, \$10.0 million was attributable to the upfront licensing fee paid to UMass Medical School and \$3.3 million was attributable to non-cash, share-based compensation expense, net of forfeitures. General and administrative expenses for the nine months ended December 31, 2018 were \$33.3 million, of which \$9.6 million was attributable to non-cash, share-based compensation expense. Net loss for the nine months ended December 31, 2018 was \$120.0 million, or \$1.01 per share.

As of December 31, 2018, the Company had \$84.9 million of cash and cash equivalents, working capital of \$46.2 million, and long-term debt of \$28.3 million. Net cash used in operating activities was \$121.5 million for the nine months ended December 31, 2018, which includes the \$30.0 million upfront payment to Oxford BioMedica, \$5.0 million of which was applied as a credit against the process development work and clinical supply that Oxford BioMedica will provide to us, the \$10.0 million upfront payment to Benitec BioPharma and the \$10.0 million upfront payment to UMass Medical School.

About Axovant Sciences

Axovant Sciences is a clinical-stage gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological and neuromuscular diseases. The company's current pipeline of gene therapy candidates targets GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), Parkinson's disease, oculopharyngeal muscular dystrophy (OPMD), amyotrophic lateral sclerosis (ALS) and frontotemporal dementia. Axovant is focused on accelerating product candidates into and through clinical trials with a team of experts in gene therapy development and through external partnerships with leading gene therapy organizations. For more information, visit 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 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 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, Benitec and UMass Medical School, 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; the timing or likelihood of its regulatory filings and approvals, and the timing of its expected name change and ticker symbol change 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 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 February 7, 2019, 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.

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

	Three Months Ended December 31,		Nine Months Ended December 31,	
	2018	2017	2018	2017
Operating expenses:				
Research and development expenses ⁽¹⁾ (includes total share-based compensation expense of \$1,910 and \$2,453 for the three months ended December 31, 2018 and 2017 and \$3,299 and \$14,625 for the nine months ended December 31, 2018 and 2017, respectively)	\$ 21,483	\$ 37,346	\$ 80,403	\$ 119,613
General and administrative expenses ⁽²⁾ (includes share-based compensation expense of \$2,648 and \$8,186 for the three months ended December 31, 2018 and 2017 and \$9,575 and \$26,954 for the nine months ended December 31, 2018 and 2017, respectively)	10,933	18,032	33,309	69,662

Total operating expenses	32,416	55,378	113,712	189,275
Other expenses:				
Interest expense	1,906	1,950	5,808	5,702
Other expense (income)	(78) 550	275	324
Loss before income tax expense	(34,244) (57,878) (119,795) (195,301
Income tax expense	52	24	224	953
Net loss	\$ (34,296) \$ (57,902) \$ (120,019) \$ (196,254
Net loss per common share — basic and diluted	\$ (0.27) \$ (0.54) \$ (1.01) \$ (1.83
Weighted average common shares outstanding — basic and diluted	128,771,900	107,719,476	119,183,117	107,241,043

(1) Includes total costs allocated from Roivant Sciences Ltd. ("RSL"), Roivant Sciences, Inc. ("RSI") and Roivant Sciences GmbH ("RSG") of \$0 and \$409 for the three months ended December 31, 2018 and 2017, respectively, and \$(450) and \$5,667 for the nine months ended December 31, 2018 and 2017, respectively.

(2) Includes total costs allocated from RSL, RSI and RSG of \$698 and \$1,440 for the three months ended December 31, 2018 and 2017, respectively, and \$2,772 and \$4,936 for the nine months ended December 31, 2018 and 2017, respectively.

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

	December 31, 2018	March 31, 2018
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	\$ 84,939	\$ 154,337
Prepaid expenses and other current assets	4,883	2,174
Income tax receivable	1,580	1,751
Total current assets	91,402	158,262
Other non-current assets	3,449	—
Property and equipment, net	1,365	2,524
Total assets	\$ 96,216	\$ 160,786
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$ 1,018	\$ 3,949
Due to RSL, RSI and RSG	693	1,011
Accrued expenses	22,911	31,862
Current portion of long-term debt	20,583	9,753
Total current liabilities	45,205	46,575
Long-term debt	28,251	42,925
Total liabilities	73,456	89,500
Shareholders' equity:		
Common shares, par value \$0.00001 per share, 1,000,000,000 shares authorized, 155,527,771 and 107,788,074 issued and outstanding at December 31, 2018 and March 31, 2018, respectively	2	1
Additional paid-in capital	699,064	628,110
Accumulated deficit	(676,970) (556,951
Accumulated other comprehensive income	664	126
Total shareholders' equity	22,760	71,286
Total liabilities and shareholders' equity	\$ 96,216	\$ 160,786

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Source: Axovant Sciences Ltd.