



Voyager Therapeutics Reports First Quarter 2019 Financial Results and Corporate Highlights

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FDA grants orphan-drug designation for VY-HTT01 for Huntington's disease

Begins strategic collaboration with Neurocrine Biosciences on gene therapy programs for Parkinson's disease and Friedreich's ataxia

Announces Board of Directors leadership change

CAMBRIDGE, Mass., May 07, 2019 (GLOBE NEWSWIRE) – Voyager Therapeutics, Inc. (NASDAQ: VYGR), a clinical-stage gene therapy company focused on developing life-changing treatments for severe neurological diseases, today reported its first quarter 2019 financial results, program progress and corporate updates.

"The first quarter was an eventful period for Voyager as we announced new collaborations, provided updates to our pipeline programs, and advanced our discovery efforts focused on novel gene therapy capsids that have the potential to cross the blood-brain barrier and target specific cells within the brain after a single, systemic administration," said Andre Turenne, president and chief executive officer of Voyager. "During the remainder of the year, our focus will remain on advancing our pipeline aimed at transformative therapies for patients living with devastating neurological diseases."

Recent Corporate and Program Highlights

- In January 2019, Neurocrine Biosciences, Inc. and Voyager announced a strategic collaboration focused on the development and commercialization of two gene therapy programs, VY-AADC for Parkinson's disease and VY-FXN01 for Friedreich's ataxia, as well as two other development programs to be determined. This collaboration combines Neurocrine's expertise in neuroscience, drug development and commercialization with Voyager's innovative gene therapy programs targeting severe neurological diseases. The collaboration became effective in March 2019.
- Announced Phase 1 trial (PD-1102) results for VY-AADC from eight patients with Parkinson's disease who participated in an open-label trial to evaluate the safety and efficacy of VY-AADC and to further assess the posterior (i.e., from the back of the head) surgical delivery approach. These Phase 1 results were presented at the 2019 Annual Meeting of the American Academy of Neurology. The results demonstrated that the posterior trajectory can serve as an additional surgical delivery approach. The results also provide further evidence to the findings from a separate Phase 1b trial (PD-1101) indicating that increased coverage of the putamen with VY-AADC leads to increases in AADC enzyme activity and improvements in motor function and quality of life in patients with Parkinson's disease – with less need for oral levodopa medication.
- Announced new preclinical data in multiple presentations at the Annual Meeting of the American Society of Gene and Cell Therapy. This preclinical data related to Voyager's vectorized antibody program directed against tau for the potential treatment of Alzheimer's disease, its TRACER™ system to discover adeno-associated virus (AAV) capsids with blood-brain barrier crossing and cell-specific transduction properties, VY-SOD102 targeting a monogenic form of amyotrophic lateral sclerosis called SOD1, and VY-HTT01 targeting Huntington's disease.
- VY-HTT01 granted Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA) for the treatment of Huntington's disease. The ODD Program provides orphan status to drugs and biologics intended for the safe and effective treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the U.S.

Board of Directors Leadership Change

Voyager announced that immediately following its 2019 Annual Meeting of Stockholders, scheduled for June 13, 2019, Michael Higgins, currently a member of the Board, will succeed Mark Levin as Board chairman. Mr. Levin, if re-elected at the Annual Meeting, will continue to serve as a member of the Board. Mr. Higgins has served as a member of the Board since July 2015.

First Quarter 2019 Financial Results

For the first quarter of 2019, Voyager reported:

A GAAP net loss of \$27.2 million, or \$0.81 per share, for the first quarter ended March 31, 2019, compared to a GAAP net loss of \$19.9 million, or \$0.63 per share, for the same period in 2018.

Collaboration revenues of \$5.2 million for the first quarter of 2019 compared to \$0.9 million for the first quarter of 2018. The increase in collaboration revenues for the first quarter of 2019 reflects the increase in revenue related to research services performed by Voyager under existing collaborations with Sanofi Genzyme and AbbVie, revenue related to research services from new collaborations with AbbVie and Neurocrine, and the reimbursement of costs related to the collaboration with Neurocrine.

Research and development (R&D) expenses of \$24.8 million for the first quarter ended March 31, 2019 compared to R&D expenses of \$14.9 million for the same period in 2018. The increase in R&D expenses related primarily to an increase associated with preclinical programs, personnel and

facility costs to support the advancement of the VY-AADC program into the RESTORE-1 Phase 2 clinical trial, and gene therapy platform initiatives for new capsid development.

General and administrative (G&A) expenses of \$9.7 million for the first quarter of 2019 compared to G&A expenses of \$7.2 million for the same period in 2018. The increase in G&A expenses was primarily due to an increase in consulting and professional fees, and personnel and facility costs to support the advancement of Voyager's pipeline programs, platform and manufacturing capabilities.

Cash, cash equivalents, and marketable debt securities as of March 31, 2019 were \$358.5 million. Based on the Company's current operating plan, Voyager expects to end 2019 with cash, cash equivalents and marketable debt securities of approximately \$280 million to \$290 million, with full-year 2019 operating expenses expected to range from \$130 million to \$140 million that includes amounts reimbursable under the Neurocrine collaboration. Voyager continues to project that its cash, cash equivalents and marketable debt securities will be sufficient to fund operating expenses and capital expenditure requirements to mid-2022.

About Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company focused on developing life-changing treatments for severe neurological diseases. Voyager is committed to advancing the field of AAV gene therapy through innovation and investment in vector engineering and optimization, manufacturing, and dosing and delivery techniques. Voyager's wholly-owned and partnered pipeline focuses on severe neurological diseases in need of effective new therapies, including Parkinson's disease, a monogenic form of ALS called SOD1, Huntington's disease, Friedreich's ataxia, Alzheimer's disease, and other neurodegenerative diseases related to defective or excess aggregation of tau and alpha-synuclein proteins in the brain. Voyager has strategic collaborations with Sanofi Genzyme, AbbVie and Neurocrine. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager is headquartered in Cambridge, Massachusetts. For more information on Voyager, please visit the company's website at www.voyagertherapeutics.com or follow @VoyagerTx on Twitter and [LinkedIn](https://www.linkedin.com/company/voyager-therapeutics).

Voyager Therapeutics® is a registered trademark, and TRACER™ is a trademark, of Voyager Therapeutics.

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," "undoubtedly," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify forward-looking statements. For example, all statements Voyager makes regarding the initiation, timing, progress, activities, goals and reporting of results of its preclinical programs and clinical trials and its research and development programs, the potential benefits and future operation of the collaboration agreements with Sanofi Genzyme, AbbVie and Neurocrine, including any potential future payments thereunder, its ability to advance its AAV-based gene therapies 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 platform and its TRACER system, its ability to perform under existing collaborations with, among others, Sanofi Genzyme, AbbVie and Neurocrine and to add new programs to its pipeline, and the regulatory pathway of, and the timing or likelihood of its regulatory filings and approvals for, any of its product candidates, anticipated financial results, including Voyager's available cash and cash equivalents at the end of 2019, the receipt by Voyager of revenues from collaboration partners during 2019 and Voyager's operating expenses during 2019, are forward looking. All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager believes such forward looking statements 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 Voyager expected. Such risks and uncertainties include, among others, those related to the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory communications, submissions and approvals; the continued development of the gene therapy platform and its TRACER system; Voyager's scientific approach and general development progress; the sufficiency of cash resources; the possibility or the timing of the exercise of development, commercialization and license options under collaborations, and the availability or commercial potential of Voyager's product candidates. These statements are also subject to a number of material risks and uncertainties that are described in Voyager's most recent Annual Report on Form 10-K filed for the year ended December 31, 2018 with the Securities and Exchange Commission, as updated by its subsequent filings with the Securities and Exchange Commission. All information in the press release is as of the date of this press release, and any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise this information or any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

Selected Financial Information

(amounts in thousands, except share and per share data)
(Unaudited)

Statement of Operations Items:

	Three Months Ended	
	March 31,	
	2019	2018
Collaboration revenue	\$ 5,197	\$ 942
Operating expenses:		
Research and development	24,831	14,853
General and administrative	9,659	7,182
Total operating expenses	34,490	22,035
Operating loss	(29,293)	(21,093)
Total other income	2,123	987
Loss before income taxes	(27,170)	(20,106)
Income tax benefit	—	180
Net loss	\$ (27,170)	\$ (19,926)

Net loss per share, basic and diluted	\$ (0.81)	\$ (0.63)
Weighted-average common shares outstanding, basic and diluted	<u>33,353,061</u>	<u>31,759,870</u>

Selected Balance Sheet Items:

	<u>March 31,</u> <u>2019</u>	<u>December 31,</u> <u>2018</u>
Cash, cash equivalents, and marketable debt securities	\$ 358,499	\$ 155,806
Total assets	\$ 411,349	\$ 177,029
Accounts payable and accrued expenses	\$ 11,537	\$ 10,826
Deferred revenue	\$ 262,049	\$ 113,046
Total stockholders' equity	\$ 100,694	\$ 46,446

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