



## Voyager Therapeutics Announces Third Quarter 2018 Financial Results and Corporate Highlights

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*One-time treatment with VY-AADC demonstrates durable improvements in motor function at 18 months and beyond from ongoing Phase 1b trial*

*Recent preclinical results for Huntington's disease and Amyotrophic Lateral Sclerosis programs support progress towards IND filings*

CAMBRIDGE, Mass., Nov. 07, 2018 (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 third quarter 2018 results, recent progress and corporate updates and will host a conference call and webcast today at 4:30 p.m. EST to discuss these results.

"We made important progress advancing our innovative pipeline in the third quarter as we presented exciting new preclinical data with our Huntington's disease and SOD1 ALS programs and today announced durable improvements in motor function at 18 months and beyond from the ongoing Phase 1b trial with VY-AADC in patients with Parkinson's disease," said Andre Turenne, president and chief executive officer of Voyager Therapeutics. "We are now focused on initiating our placebo-controlled randomized Phase 2 trial with VY-AADC and further advancing our preclinical programs towards INDs in 2019."

### Recent highlights for VY-AADC for Parkinson's disease:

- Today, we announced in a separate press release ([link here](#)) positive longer-term results from the open-label, dose-escalating Phase 1b trial of VY-AADC demonstrating sustained improvements in patients' motor function. Patients in the two highest dose cohorts (Cohorts 3 and 2) experienced mean improvements in good ON time per day of 1.7 hours at 18 months and 2.7 hours at two years, respectively. Having selected a dose for the Phase 2 trial between the two highest dose cohorts from the Phase 1b trial, results from the combined ten patients in Cohorts 2 and 3 demonstrated an increase from baseline in good ON time of 2.4 hours per day at 12 months, the timepoint for the primary endpoint in the Phase 2 trial, and 2.6 hours per day at 18 months, the latest timepoint measured for both cohorts. These results were achieved with clinically meaningful and sustained reductions in daily oral levodopa and related medications at the two highest cohorts.
- As previously announced, in July 2018, Voyager received written Type C meeting feedback from the Food and Drug Administration (FDA) indicating that the results from the Phase 2 randomized, placebo-controlled trial, if it were to meet its primary endpoint and in the absence of major safety concerns, likely may be considered sufficient for acceptance of submission for review of a BLA. In late October 2018, Voyager received an addendum from the FDA to the July 2018 written responses in which the FDA informed the Company that although the data from the Phase 2 trial may support the safety and efficacy of VY-AADC and could be considered in the BLA review, the FDA currently considers the Phase 2 trial as an early phase exploratory study. Voyager plans to engage with the FDA to gain further clarity on their most recent responses, including through a planned Type B meeting later this year and through the additional mechanisms available to the Company under the RMAT designation. Voyager plans to continue to seek and incorporate FDA guidance into the ongoing development plans for VY-AADC.
- 24 clinical trial sites (including neurosurgical and neurology patient referral and management sites) have been selected for participation in the Phase 2 randomized, placebo-controlled trial. Institutional review board approvals, site activation, and patient screening efforts are underway. Voyager expects to announce when the first patient has been dosed.

### Recent highlights for preclinical programs:

At the Congress of the European Society of Gene and Cell Therapy (ESGCT) on October 16-19, 2018, in Lausanne, Switzerland, Voyager presented results achieving significant reductions of disease-causing gene expression in large animals following one-time delivery of VY-HTT01 for Huntington's disease and VY-SOD102 for SOD1 ALS.

- For VY-HTT01, the use of a novel delivery paradigm targeting both the putamen and thalamus significantly reduced huntingtin, or HTT, gene expression in both deeper tissues (caudate, putamen, and thalamus) and outer layers (cortex) of the brain of adult, non-human primates. Targeting both the putamen and thalamus leverages more extensive and preserved neuronal pathways to the cortex than delivery to the putamen alone, offering the potential of a one-time treatment with VY-HTT01 to address motor, cognitive and behavioral disabilities associated with Huntington's disease. Robust analyses including quantitative measurement in multiple tissue punches and in neurons captured by laser microdissection revealed that VY-HTT01 reduced HTT messenger RNA (mRNA) on average by 68% in the caudate, 67% in the putamen, 73% in the thalamus, and 32% in cortical neurons.
- For VY-SOD102 for amyotrophic lateral sclerosis (ALS) targeting SOD1, a novel delivery paradigm comprising a one-time, intraparenchymal infusion to the cervical region of the spinal cord significantly reduced SOD1 throughout the spinal cord of

the mini-pig, which has a spinal cord similar in length and diameter to the human spinal cord. This novel delivery approach with VY-SOD102 reduced SOD1 mRNA in the spinal cord on average by 70% and 50% in the cervical and thoracic regions, respectively, both regions critical for respiratory function, and 82% near the site of cervical injection. In addition, VY-SOD102 reduced SOD1 mRNA by 22% in the lumbar region.

### **Voyager's V-TAG™ Neuro-Navigational Surgical Device for Gene Therapy Delivery**

Voyager has implemented the use of real-time, intra-operative magnetic resonance imaging (MRI) compatible devices and techniques allowing neurosurgeons more precise delivery of its gene therapy vectors. For the Phase 1b trial of VY-AADC for Parkinson's disease, investigators used an MRI-compatible device called the ClearPoint® System from MRI Interventions, Inc. Voyager also began developing a real-time, intra-operative, MRI-compatible neuro-navigational device, the Variable Trajectory Array Guide, or V-TAG™, as a choice for neurosurgeons in addition to ClearPoint®. In July 2018, Voyager received 510(k) regulatory clearance of V-TAG™ from the Center for Devices and Radiological Health of the FDA. Beyond serving as an additional choice for neurosurgeons in the Parkinson's disease program, V-TAG™ could also be used for Voyager's Huntington's disease and other programs.

### **Management Update**

Voyager promoted Allison Dorval to the position of chief financial officer from her previous position as vice president of finance. She remains the Company's principal finance and accounting officer. Ms. Dorval, a certified public accountant, joined Voyager in June 2017 and has over 20 years of experience in finance and accounting, including 10 years in various leadership roles in the life sciences industry. Prior to joining Voyager, Ms. Dorval served as vice president and controller of Juniper Pharmaceuticals, Inc., as a consultant at Danforth Advisors, and in several roles at Insulet Corporation, including as chief financial officer. Ms. Dorval received a B.S. in Business Administration, with a concentration in Accounting, from the University of Vermont.

### **Third Quarter 2018 Financial Results**

Voyager reported a GAAP net loss of \$20.3 million, or \$0.63 per share, for the third quarter ended September 30, 2018, compared to a GAAP net loss of \$23.3 million, or \$0.89 per share, for the same period in 2017.

Collaboration revenues of \$2.1 million for the third quarter of 2018 compared to \$1.1 million for the third quarter of 2017. Collaboration revenues reflect recognition of payment for research and development services provided by Voyager for various programs under the collaboration agreements with Sanofi Genzyme and AbbVie. The increase in collaboration revenues for the third quarter of 2018 reflects the recognition of revenue related to research services performed under the AbbVie collaboration agreement offset by reductions in revenue recognized under the Sanofi Genzyme collaboration that relate to research and development services as well as the adoption of certain accounting rules related to revenue recognition methodology.

Research and development (R&D) expenses of \$16.6 million for the third quarter ended September 30, 2018 compared to \$19.6 million for the same period in 2017. The decrease in R&D expenses related primarily to a reduction in manufacturing expenses offset by increases in personnel and facility costs to support the advancement of VY-AADC into the Phase 2 trial and expenditures related to support Voyager's preclinical pipeline programs.

General and administrative (G&A) expenses of \$6.6 million for the third quarter 2018 compared to \$4.9 million for the same period in 2017. The increase in G&A expenses was primarily due to personnel and facility costs to support the advancement of the lead and pipeline programs, platform and manufacturing capabilities.

Cash, cash equivalents, and marketable debt securities as of September 30, 2018 were \$179.6 million. Based on the Company's current operating plan, Voyager expects to end 2018 with total cash, cash equivalents and marketable debt securities above the previously guided range of \$125 million to \$135 million and projects that its existing cash, cash equivalents and marketable debt securities will be sufficient to fund operating expenses and capital expenditure requirements into early 2020.

### **Conference Call Information**

Voyager will host a conference call and webcast today at 4:30 p.m. EST. The live call may be accessed by dialing (877) 851-3834 for domestic callers or +1 (631) 291-4595 for international callers and referencing conference ID number 5999167. A live audio webcast of the conference call will be available online from the Investors & Media section of Voyager's website at [www.voyagertherapeutics.com](http://www.voyagertherapeutics.com). The webcast will be archived for 30 days.

### **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 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, neurodegenerative diseases related to defective or excess aggregation of tau protein in the brain including Alzheimer's disease and severe, chronic pain. Voyager has broad strategic collaborations with Sanofi Genzyme, the specialty care global business unit of Sanofi, AbbVie, and the University of Massachusetts Medical School. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager Therapeutics is headquartered in Cambridge, Massachusetts.

### **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 and reporting of results of its preclinical programs and clinical trials and its research and development programs, 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, its ability to develop manufacturing capability for its products and successfully transition its manufacturing process, its ability to perform under existing collaborations with, among others, Sanofi Genzyme and AbbVie and to add new programs to its pipeline, its ability to enter into new partnerships or collaborations, the sufficiency of its cash resources and the regulatory pathway of, and the timing or likelihood of its regulatory filings and approvals for, any of its product candidates, are forward looking. All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager 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 Voyager 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 communications, submissions

and approvals; the continued development of the gene therapy platform; Voyager's scientific approach and general development progress; 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 with the Securities and Exchange Commission, 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. Voyager 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.

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**Selected Financial Information**  
(\$-amounts in thousands, except per share data)  
(Unaudited)

	Three Months Ended		Nine Months Ended	
	September 30,		September 30,	
<b>Statement of Operations Items:</b>	<b>2018</b>	<b>2017</b>	<b>2018</b>	<b>2017</b>
Collaboration revenue	\$ 2,094	\$ 1,148	\$ 5,611	\$ 3,790
Operating expenses:				
Research and development	16,632	19,561	47,991	48,933
General and administrative	6,609	4,942	25,554	14,372
Total operating expenses	23,241	24,503	73,545	63,305
Operating loss	(21,147)	(23,355)	(67,934)	(59,515)
Total other income	858	9	1,997	615
Loss before income taxes	(20,289)	(23,346)	(65,937)	(58,900)
Income tax benefit	—	—	180	31
Net loss	<u>\$ (20,289)</u>	<u>\$ (23,346)</u>	<u>\$ (65,757)</u>	<u>\$ (58,869)</u>
Net loss per share, basic and diluted	<u>\$ (0.63)</u>	<u>\$ (0.89)</u>	<u>\$ (2.06)</u>	<u>\$ (2.27)</u>
Weighted-average common shares outstanding, basic and diluted	<u>32,191,475</u>	<u>26,164,527</u>	<u>31,977,670</u>	<u>25,968,849</u>

	September 30,	December 31,
	2018	2017
<b>Selected Balance Sheet Items</b>		
Cash, cash equivalents, and marketable debt securities	\$ 179,601	\$ 169,052
Total assets	\$ 197,064	\$ 184,477
Accounts payable and accrued expenses	\$ 9,777	\$ 12,517
Deferred revenue	\$ 115,054	\$ 31,560
Total stockholders' equity	\$ 65,588	\$ 134,051



Voyager Therapeutics, Inc.