



Voyager Therapeutics Announces First Quarter 2021 Financial Results and Corporate Updates

May 10, 2021

- On track to initiate Phase 1/2 trial of VY-HTT01 for Huntington's disease in the fourth quarter of 2021

- 8 presentations at American Society of Gene and Cell Therapy (ASGCT) conference, including new data on next generation blood brain barrier penetrant capsids

- Virtual investor and analyst event to be held in July 2021

CAMBRIDGE, Mass., May 10, 2021 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ: VYGR), a clinical-stage gene therapy company focused on developing life-changing treatments for patients suffering from serious neurological diseases, today reported its first quarter 2021 financial results, program progress and corporate updates.

"We are very pleased with the progress we've made in the first few months of this year, including receiving IND clearance for our VY-HTT01 program for Huntington's disease as well as presenting important new results at the ASGCT conference from our next generation, blood brain barrier penetrant AAV capsid program which opens up numerous potential opportunities in CNS diseases," said Andre Turenne, President and CEO of Voyager. "We look forward to providing updates on these and other programs at our upcoming investor event in July and over the coming months, particularly as we prepare to initiate our planned VYTAL Phase 1/2 clinical trial in Huntington's disease later this year."

Recent Corporate Highlights and Pipeline Updates

VY-HTT01 for Huntington's Disease

- In April 2021, the Company announced FDA clearance of its Investigational New Drug (IND) application for VY-HTT01, the Company's gene therapy candidate for the treatment of Huntington's disease, and plans to initiate the VYTAL Phase 1/2 clinical trial of VY-HTT01 at multiple US sites in the fourth quarter.
- The planned VYTAL Phase 1/2 clinical trial is designed as a randomized, open-label study with a concurrent delayed-start control. The trial will evaluate the safety and tolerability of VY-HTT01 in patients with early manifest Huntington's disease. Secondary endpoints include disease biomarkers and clinical outcome measures.
- Huntington's disease is caused by toxic gain-of-function mutations in the huntingtin, or HTT, gene. Preclinical data for VY-HTT01 have shown strong reduction in mutant HTT protein in transgenic animal models of Huntington's disease, resulting in improvement in disease phenotype. Additional data in non-human primates have demonstrated widespread distribution of VY-HTT01 across the striatum and cortex, which are core areas of disease pathology, and robust and durable reduction of HTT mRNA and protein.
- The Company expects to initiate the planned VYTAL Phase 1/2 clinical trial by the end of 2021 and anticipates providing initial topline safety, tolerability and biomarker data in 2022.

Novel AAV Capsid Discovery Program

- Voyager has developed a series of novel adeno-associated virus (AAV) capsids which, following intravenous administration, achieve up to 1000-fold higher RNA expression in the brain and 100-fold higher expression in the spinal cord of non-human primates than AAV9, the current natural AAV serotype with the best ability to cross the blood brain barrier. The Company believes these capsids may allow for significantly enhanced gene delivery to specific types of cells in the brain at lower doses.
- The Company believes its TRACER™ technology and novel capsid discoveries have the potential to support a significant number of CNS programs, both for the Company's own development pipeline and for the enablement of programs by other companies, through potential future licensing and collaboration arrangements. The TRACER platform has the potential to be flexibly applied to discover capsids with improved tropism to other tissues and to overcome other limitations of current AAV capsids.
- The Company plans to present new data on these novel blood brain barrier penetrant capsids at the ASGCT conference on May 11, 2021 (see detail below).

VY-AADC (NB1b-1817) for Parkinson's Disease

- As previously announced, Neurocrine Biosciences provided notice of its decision to terminate that portion of the

Company's collaboration agreement related to the VY-AADC (NB1b-1817) program for Parkinson's disease, effective August 2, 2021. Voyager intends to support Neurocrine, the clinical study sponsor and IND holder for the RESTORE-1 clinical trial of VY-AADC (NB1b-1817), on ongoing matters related to the completion of imaging and clinical assessments requested by the trial's Data Safety and Monitoring Board (DSMB). Although Neurocrine remains the study sponsor of the RESTORE-1 clinical trial, the underlying technology licenses for the VY-AADC program will revert to the Company in accordance with the collaboration agreement on August 2, 2021.

- The Company continues to believe that the VY-AADC program holds significant promise for Parkinson's disease patients as evidenced by the positive multi-year safety and efficacy data from the two Phase 1b clinical trials presented in September 2020 at the MDS Virtual Congress. However, as a result of the prioritization of new programs enabled by novel capsids in the Company's preclinical portfolio and its focus on the clinical efforts for the VY-HTT01 program to treat Huntington's disease, the Company has determined that it will not advance the VY-AADC program on its own following the termination of that portion of the collaboration agreement. Instead, the Company expects to turn the future development and commercialization of VY-AADC over to a partner once the potential path forward for the program is determined.

Corporate Updates and Other Anticipated Milestones

Upcoming Presentations and Updates

- The Company recently announced that it will have eight presentations and posters at the ASGCT 24th Annual Meeting, taking place May 11-14, 2021. Oral presentations are expected to highlight data from Voyager's novel capsid, preclinical and manufacturing efforts. The Company also intends to host an Industry Sponsored Symposium titled "Advancing AAV Gene Therapy for CNS Disease," at this year's ASGCT Meeting. Details of the presentation schedule can be found [here](#).
- At an upcoming virtual investor and analyst event planned for July 2021, the Company expects to provide additional updates on multiple platform and program advances on the planned VYTAL Phase 1/2 clinical trial for Huntington's disease, the novel capsid program, and the preclinical pipeline.

First Quarter 2021 Financial Results

- **Collaboration Revenues:** Collaboration revenue was \$6.5 million for the first quarter of 2021, compared to collaboration revenue of \$18.1 million for the same period of 2020. The decrease in collaboration revenue was largely due to a reduction of revenue related to research services and cost reimbursements from the collaborations with Neurocrine and AbbVie. The collaborations with AbbVie were terminated in August 2020.
- **Net Loss:** Net loss was \$21.6 million for the first quarter of 2021, compared to a net loss of \$24.3 million for the same period of 2020.
- **R&D Expenses:** Research and development expenses were \$22.3 million for the first quarter of 2021, compared to \$32.3 million for the same period in 2020. The decrease in R&D expenses was primarily related to lower manufacturing and external clinical expenses for the VY-AADC program.
- **G&A Expenses:** General and administrative expenses were \$9.7 million for the first quarter of 2021, compared to \$10.2 million for the same period in 2020. The decrease in G&A expenses was primarily related to lower intellectual property expenses.
- **Cash Position:** Cash, cash equivalents and marketable debt securities as of March 31, 2021 were \$153.1 million.

About Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company focused on developing life-changing treatments for serious 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 serious neurological diseases for which effective new therapies are needed. Voyager has a strategic collaboration with Neurocrine Biosciences. 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, please visit www.voyagertherapeutics.com or follow [@VoyagerTx](#) on Twitter and [LinkedIn](#).

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 of the planned VYTAL Phase 1/2 clinical trial of VY-HTT01 in Huntington disease in the fourth quarter of 2021 and the release of data from the planned VYTAL clinical trial in 2022; Voyager's continuing efforts in the discovery and engineering of novel AAV capsids; the presentation of data at conferences demonstrating the characteristics and performance of novel AAV capsids developed by Voyager; the continuing progress by Voyager in developing new novel AAV capsids; the ability of Voyager's novel capsid discoveries to support a significant number of central nervous system programs and the ability of Voyager's novel capsid discoveries to support programs sponsored by third parties by means of licensing or collaboration arrangements; the completion of imaging and clinical assessments by Neurocrine Biosciences for participants of the RESTORE-1 clinical study; the decision of Voyager to not advance on its own the VY-AADC program for Parkinson's disease; Voyager's ability to identify and attract parties to participate in research and development collaborations, including any potential collaboration for the VY-AADC program; Voyager's initiation, timing, progress, activities, goals and reporting of the results of its preclinical programs and its research and development programs; the potential benefits, timing and future operation of the collaboration agreement with Neurocrine Biosciences; Voyager's ability to add new programs to its pipeline; Voyager's ability to operate its research and development activities efficiently and effectively; and the utility and value of Voyager's patent portfolio 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, the ability of Neurocrine Biosciences and Voyager to obtain imaging data for patients treated as part of the RESTORE-1 clinical trial; the ability of Voyager to effectively present its novel capsid data by means of virtual conference proceedings in response to the COVID-19 health crisis; the ability of Voyager to progress its research and engineering program for novel capsids, and to procure sufficient numbers of non-human primates to conduct non-human primate studies; the interest of third parties in Voyager's novel capsids, and the ability of Voyager to negotiate and complete licensing or collaboration agreements on terms acceptable to Voyager and such third parties; with respect to the VYTAL clinical trial, the ability of Voyager to successfully establish clinical sites, to engage investigators, to recruit patients meeting eligibility requirements, to manage COVID-19 restrictions and to generate clinical outcomes and data in a timely manner; the ability of Voyager to manage the business disruptions resulting from the COVID-19 health crisis; the ability of Voyager to create research and development programs combining sufficient levels of scientific interest and applied expertise to be attractive in recruiting and maintaining researchers and scientists; the outcomes and consequences associated with regulatory communications, submissions, approvals and non-approvals; Voyager's scientific approach and general development progress; the ability to attract and retain talented contractors and employees; the ability to create and protect intellectual property; the sufficiency of cash resources; the possibility or the timing of the exercise of development, commercialization, license and other 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 Annual Report on Form 10-K for the year ended December 31, 2020, filed 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.

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Selected Financial Information

(\$-amounts in thousands, except per share data)

(Unaudited)

Statement of Operations Items:

Collaboration revenue	
Operating expenses:	
Research and development	
General and administrative	
Total operating expenses	
Operating loss	
Total other income	
Net loss	
Net loss per share, basic and diluted	
Weighted-average common shares outstanding, basic and diluted	

Three Months Ended	
March 31,	
2021	2020
\$ 6,501	\$ 18,067
22,346	32,294
9,744	10,206
32,090	42,500
(25,589)	(24,433)
3,940	170
\$ (21,649)	\$ (24,263)
\$ (0.58)	\$ (0.66)
37,501,065	36,963,255

Selected Balance Sheet Items

Cash, cash equivalents, and marketable debt securities
Total assets
Accounts payable and accrued expenses
Deferred revenue
Total stockholders' equity

March 31,	December 31,
2021	2020
\$ 153,050	\$ 174,782
\$ 237,717	\$ 261,584
\$ 13,712	\$ 14,839
\$ 40,057	\$ 43,817
\$ 136,208	\$ 154,320

Source: Voyager Therapeutics, Inc.