# Voyager Therapeutics Announces Fourth Quarter and Full Year 2020 Financial Results and Corporate Updates

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- *Expects to Provide Complete Response to FDA Requests on IND Application for VY-HTT01 for Huntington's Disease in the First Half of 2021*

- *Continues to Progress Pipeline and Platform Activities with Expected Presentations of Data in the First Half of 2021*

CAMBRIDGE, Mass., Feb. 25, 2021 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ: VYGR), a clinical-stage gene therapy company focused on developing life-changing treatments for patients suffering from severe neurological diseases, today reported its fourth quarter and full year 2020 financial results and provided corporate updates.

"We have made significant progress readying our lead wholly owned program, VY-HTT01 for Huntington's disease, for clinical trials," said Andre Turenne, President and CEO of Voyager. "We have also continued to advance our preclinical portfolio, including our innovative vectorized antibodies, and have achieved highly promising results with our TRACER platform to identify viral capsids that can cross the blood-brain barrier. With these developments, we look forward to initiating a new phase in our mission to deliver transformative therapies for those affected with severe neurological diseases."

**Recent Corporate Updates**

- The Investigational New Drug (IND) application for VY-HTT01 for the treatment of Huntington's disease remains on clinical hold by the U.S. Food and Drug Administration (FDA) pending the resolution of additional information requests regarding specific chemistry, manufacturing and controls (CMC) topics, including drug device compatibility and drug substance and product characterization. Voyager plans to provide its complete response to the FDA in the first half of 2021 and to initiate its clinical evaluation of VY-HTT01 subject to and upon resolution of the clinical hold and the clearance of the IND application.

- In December 2020, Voyager published a peer-reviewed manuscript in *Molecular Therapy Methods & Clinical Development* entitled "Rapid Evolution of Blood-Brain Barrier-Penetrating AAV Capsids by RNA-Driven Biopanning" which describes the foundational proof-of-concept experiments for Voyager's proprietary TRACER™ platform. Voyager is leveraging the TRACER platform to facilitate the selection of AAV capsids with significantly improved blood-brain barrier crossing and cell-specific transduction properties for therapeutic applications.

- The RESTORE-1 Phase 2 clinical trial for VY-AADC (NBIb-1817) remains on FDA clinical hold. The program was placed on clinical hold in December 2020 and followed the submission by Neurocrine Biosciences, Inc. of an IND Safety Report related to the observation of magnetic resonance imaging abnormalities in RESTORE-1 study participants. The clinical implications of this observation are currently unknown and are being evaluated. The Data Safety Monitoring Board (DSMB) has requested additional imaging data and clinical assessments.

- In February 2021, Voyager announced that Neurocrine decided to terminate that portion of the collaboration agreement related to the VY-AADC (NBIb-1817) program, effective August 2, 2021. Voyager intends to support Neurocrine, the study sponsor and IND holder, on ongoing matters related to the completion of imaging and clinical assessments requested by the DSMB and the provision of other information requested by the FDA for the RESTORE-1 Phase 2 clinical trial.

- In January 2021, Voyager hired Robin Swartz as Senior Vice President of Program Management and Patient Engagement. Robin brings more than twenty years of experience across the industry serving most recently as Vice President, Patient and Product Services for Rare Diseases at Sanofi Genzyme.

**Selected 2020 Corporate Highlights**

- 14 data presentations covering multiple program and pipeline activities at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting in May 2020.

- 4 publications in peer-reviewed journals on our efforts in Huntington's disease, Parkinson's disease, gene therapy delivery and manufacturing.

- Presentation at the Movement Disorder Society (MDS) Virtual Congress in September 2020 of three-year data of VY-AADC

(NBIb-1817) in a dose-escalating Phase 1b study and two-year data in a Phase 1b study using a posterior trajectory.

- Completion and occupancy of a 30,000 square foot, state-of-the-art process research and development facility in Lexington, Massachusetts, to enable manufacturing of AAV gene therapy vectors at laboratory and pilot scale.

- Appointment of Nancy Vitale as an independent director to Voyager's Board of Directors.

- Appointments of leaders across key functional areas, including Maria Lopez-Bresnahan as Senior Vice President, Head of Translational Medicine and Clinical Development; Michelle Quinn Smith as Chief Human Resources Officer; and Diana M. Collazo, Ph.D., J.D. as Chief Patent Counsel.

**Selected Anticipated 2021 Corporate Milestones**

- Voyager plans to provide a complete response to the additional requests from the FDA regarding the IND application for VY-HTT01 in the first half of 2021. Voyager expects to initiate clinical evaluation of VY-HTT01 subject to and upon resolution of the clinical hold and the clearance of the IND application.

- Voyager expects to provide preclinical data on its early pipeline progress as well as its novel capsid discovery efforts in non-human primates at scientific meetings and other presentations in the first half of 2021.

- Voyager plans to determine the potential path forward for the VY-AADC (NBIb-1817) program based on the additional information being collected by Neurocrine in response to the DSMB requests.

**Fourth Quarter and Full Year 2020 Financial Results**

- **Collaboration Revenues:** Voyager had collaboration revenue of $6.5 million for the fourth quarter of 2020 and $171.1 million for the year ended December 31, 2020, compared to collaboration revenue of $32.7 million and $104.4 million, respectively, for the same periods of 2019. The decrease in collaboration revenue in the fourth quarter of 2020 compared to the same period in 2019 was largely due to a reduction of revenue related to research services and cost reimbursements from the collaborations with Neurocrine and AbbVie. Full year 2020 revenue includes $105.2M related to the recognition of remaining deferred revenue for AbbVie upon the termination of the collaboration. All research services related to the AbbVie collaborations were completed prior to the fourth quarter of 2020.

- **Net Income/Loss:** Net loss was $15.9 million for the fourth quarter of 2020 and net income was $36.7 million for the year ended December 31, 2020, compared to a net loss of $12.6 million and $43.6 million, respectively, for the same periods of 2019.

- **R&D Expenses:** Research and development expenses were $22.0 million for the fourth quarter of 2020, compared to $36.6 million for the same period in 2019. For the year ended December 31, 2020, R&D expenses were $108.8 million, compared to $119.7 million for the same period of 2019. The decrease in R&D expenses was primarily related to lower external costs for services supporting our clinical and preclinical pipeline programs.

- **G&A Expenses:** General and administrative expenses were $8.3 million for the fourth quarter of 2020, compared to $9.9 million for the same period in 2019. For the year ended December 31, 2020, G&A expenses were $35.0 million, compared to $36.3 million for the same period of 2019. The decrease in G&A expenses was primarily related to a reduction in legal and professional fees.

- **Cash Position:** Cash, cash equivalents and marketable debt securities as of December 31, 2020 were $174.8 million.

**Financial Guidance**

- Based on the Company's current operating plan and excluding any potential financing or business development activities in 2021, Voyager anticipates cash, cash equivalents and marketable debt securities will be between $50 million and $60 million at the end of 2021.

- Voyager expects that its cash, cash equivalents and marketable debt securities, as well as amounts expected to be received as reimbursement of development costs from Neurocrine, will be sufficient to meet Voyager's planned operating expenses and capital expenditure requirements into 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 for which effective new therapies are needed, including Parkinson's disease, Huntington's disease, Friedreich's ataxia, and other severe neurological diseases. For more information on Voyager Therapeutics, please visit the company's website at www.voyagertherapeutics.com or follow @VoyagerTx on Twitter and LinkedIn.

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

**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 ability and timing for completing clinical evaluations and furnishing information to the FDA in connection with the clearance of IND applications, including specifically Voyager's ability to furnish a complete response to the FDA in the first half of 2021 and to initiate its clinical evaluation of VY-HTT01 upon resolution of the clinical hold and the clearance of the IND application; the efforts and progress of collaboration partners to complete assessments and to respond to regulatory requests, including specifically Neurocrine's ability to provide additional imaging data for and to complete clinical assessments of study participants in the VY-AADC (NBIb-1817) program as requested by the DSMB prior to August 2, 2021, and Voyager's intent and ability to support Neurocrine in such efforts; Voyager's ability to determine a path forward for the VY-AADC (NBIb-1817) program; the future operation of the collaboration agreement with Neurocrine Biosciences; Voyager's ability to present preclinical data on its early pipeline progress and novel capsid discovery efforts in non-human primates at scientific meetings and other presentations in the first half of 2021; Voyager's ability to advance AAV-based gene therapies into, and to initiate, enroll and complete, clinical trials; Voyager's ability to continue to develop its novel AAV capsid development program; the regulatory pathway of, and the timing or likelihood of its regulatory filings and approvals for, any of Voyager's product candidates; Voyager's ability to add new programs to its pipeline; Voyager's anticipated financial results, including the receipt by Voyager of revenues or reimbursement payments from collaboration partners; Voyager's operating expenses, available cash, cash equivalents and marketable debt securities and Voyager's ability to fund its operating expenses with its current cash, cash equivalents and marketable debt securities through a stated time period, in each instance 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 severity and length of the COVID-19 health crisis; the initiation and conduct of preclinical studies and clinical trials; the ability and efforts of collaboration partners complete and achieve program objectives; the availability of data from preclinical studies and clinical trials; the expectations for regulatory communications, submissions and approvals; the continued development of the gene therapy platform and Voyager's TRACER system; 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; the ability of Neurocrine to meet the information requests of, and to resolve the questions raised by, the DSMB regarding the RESTORE-1 clinical trial; 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 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)*

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| **Statement of Operations Items:** | **2020** | **2019** | **2020** | **2019** |
| Collaboration revenue | $ 6,537 | $ 32,674 | $ 171,128 | 104,391 |
| Operating expenses: | | | | |
| Research and development | 21,997 | 36,551 | 108,754 | 119,735 |
| General and administrative | 8,270 | 9,891 | 34,991 | 36,335 |
| Total operating expenses | 30,267 | 46,442 | 143,745 | 156,070 |
| Operating (loss) income | (23,730) | (13,768) | 27,383 | (51,679) |
| Total other income | 7,802 | 1,193 | 9,357 | 8,082 |
| Net (loss) income | $ (15,928) | $ (12,575) | $ 36,740 | $ (43,597) |
| | | | | |
| Net (loss) income per share, basic | $ (0.43) | $ (0.34) | $ 0.99 | $ (1.21) |
| Net (loss) income per share, diluted | (0.43) | (0.34) | 0.98 | (1.21) |
| | | | | |
| Weighted-average common shares outstanding, basic | 37,290,259 | 36,838,507 | 37,132,447 | 35,898,266 |
| Weighted-average common shares outstanding, diluted | 37,290,259 | 36,838,507 | 37,348,514 | 35,898,266 |

| | December 31, | |
|---|---|---|
| **Selected Balance Sheet Items** | **2020** | **2019** |
| Cash, cash equivalents, and marketable debt securities | $ 174,782 | $ 281,533 |

| | | | | |
|---|---|---|---|---|
| Total assets | $ | 261,584 | $ | 354,760 |
| Accounts payable and accrued expenses | $ | 14,839 | $ | 25,586 |
| Deferred revenue | $ | 43,817 | $ | 194,493 |
| Total stockholders' equity | $ | 154,320 | $ | 99,512 |

Source: Voyager Therapeutics, Inc.