



## Voyager Therapeutics Announces Third Quarter 2020 Financial Results and Corporate Updates

11/09/20

CAMBRIDGE, Mass., Nov. 09, 2020 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ: VYGR) today reported its third quarter 2020 financial results, program progress and corporate updates.

"During the third quarter, we took important steps to advance our lead programs for Parkinson's disease and Huntington's disease with the presentation of positive long-term clinical results and the filing of an IND, respectively. Together with our partner, Neurocrine, we are currently focused on resuming enrollment in the RESTORE-1 clinical trial for Parkinson's disease pending the requested review and assessment of patient imaging data by the DSMB. For our Huntington's disease program, the FDA has provided clarity regarding the additional information it is requesting pursuant to our IND filing. We plan to work with the FDA to respond to these requests to allow for an IND clearance and the start of VY-HTT01's clinical evaluation," said Andre Turenne, President and CEO of Voyager. "We look forward to continuing our progress on these two important programs, along with our broadening AAV gene therapy portfolio to treat severe neurological diseases."

### Recent Program and Corporate Highlights

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

- Voyager and Neurocrine Biosciences (Neurocrine) are developing VY-AADC (NB1b-1817) as a one-time AAV-based gene therapy encoding the gene for human AADC that is designed to help produce the AADC enzyme in brain cells where it can convert levodopa to dopamine. VY-AADC (NB1b-1817) is administered into the brain using magnetic resonance imaging (MRI)-facilitated targeted delivery.
- In September 2020, Voyager and Neurocrine presented new positive long-term, three-year data from the PD-1101 Phase 1b trial, and two-year data from the PD-1102 trial, demonstrating that a one-time treatment with VY-AADC (NB1b-1817) showed sustained improvement in motor function including greater mean ON time without troublesome dyskinesia, reduction in Unified Parkinson's Disease Rating Scale (UPDRS) Part III scores, and reduction in the amount of Parkinson's disease medications in these patients. The data were presented at the MDS Virtual Congress 2020.
- In November 2020, the Data Safety Monitoring Board (DSMB) for the RESTORE-1 Phase 2 clinical trial reviewed certain patient imaging data from the ongoing trial and recommended a pause in the dosing of patients in RESTORE-1 pending review by the DSMB of additional data. As previously announced, trial sites participating in RESTORE-1 are not currently screening and enrolling patients as a result of the COVID-19 pandemic. In response to the DSMB's recommendation, Voyager and Neurocrine have decided to delay the planned resumption of patient screening in the RESTORE-1 trial until the DSMB is able to complete its evaluation. The DSMB is expected to consider additional patient data before year-end. Neurocrine is preparing an expedited safety report that will be submitted to the FDA within the 15-day reporting window.

#### VY-HTT01 for Huntington's Disease

- Voyager is developing VY-HTT01 as a one-time AAV-based gene therapy treatment designed to knock down expression of the HTT gene. Voyager's approach is focused on delivering VY-HTT01 directly into the brain and targeting a reduction of the levels of HTT protein in the striatum and cortex to potentially slow the progression of both motor and cognitive symptoms.
- In September 2020, Voyager submitted an IND application for VY-HTT01 in Huntington's disease, and in October, Voyager was notified that the IND had been placed on clinical hold pending the resolution of certain CMC information requests. Voyager recently received written feedback from the FDA requesting additional information on specific CMC topics, including drug-device compatibility and drug substance and product characterization. Voyager plans to work closely with the agency to resolve the additional information request in a timely manner.
- Following clearance of the IND by the FDA, Voyager expects to begin a Phase 1b clinical trial of VY-HTT01 in Huntington's disease patients.

#### Novel AAV Capsid Discovery Program

- Voyager continues to progress its efforts in the discovery and engineering of novel AAV capsids with the potential to overcome the limitations of existing capsids, including greater blood-brain barrier (BBB) penetrance following IV administration. Voyager is leveraging its proprietary TRACER™ platform to facilitate the selection of AAV capsids with significantly improved BBB crossing and cell-specific transduction properties.
- Voyager is currently engaged in advanced non-human primate studies to further characterize and select novel AAV capsids with improved properties offering the potential for therapeutic applications in neurological disorders.

#### Recent Corporate Updates

- Voyager recently made key appointments of leaders across several functional areas. These include the appointments of Michelle Quinn Smith as Chief Human Resources Officer, Diana M. Collazo, Ph.D., J.D. as Chief Patent Counsel and Claire Sampson, Ph.D. as Vice President, Global Regulatory Affairs.
- Voyager recently appointed Nancy Vitale as an independent director to its Board of Directors, effective as of September 15, 2020. Ms. Vitale brings more than 25 years of business and human resources experience to Voyager's Board. She is a former Senior Vice President and Chief Human Resource Officer at Genentech, a member of the Roche Group.

### Third Quarter 2020 Financial Results

- **Collaboration Revenues:** Voyager recorded collaboration revenue of \$117.8 million for the third quarter of 2020, compared to collaboration revenue of \$20.4 million for the same period of 2019. The increase in collaboration revenue was a result of the termination of the AbbVie collaborations in August 2020 and the subsequent recognition of all remaining deferred amounts under the agreements.
- **Net Income:** Net income was \$85.6 million for the third quarter of 2020, compared to a net loss of \$15.0 million for the same period of 2019.
- **R&D Expenses:** Research and development expenses were \$25.0 million for the third quarter of 2020, compared to \$29.8 million for the same period in 2019. The decrease in R&D expenses was primarily related to lower external costs for services supporting Voyager's clinical and preclinical pipeline programs.
- **G&A Expenses:** General and administrative expenses were \$8.3 million for the third quarter of 2020, compared to \$8.5 million for the same period in 2019. The decrease in G&A expenses was primarily attributable to a decrease in professional fees supporting Voyager's pipeline programs.
- **Cash Position:** Cash, cash equivalents and marketable debt securities as of September 30, 2020 were \$200 million.

### Financial Guidance

- Based on the Company's current operating plan, Voyager anticipates cash, cash equivalents and marketable debt securities will be between \$150 million and \$170 million at the end of 2020.
- Voyager expects that its cash, cash equivalents and marketable debt securities, as well as amounts expected to be received for reimbursement of development costs from Neurocrine Biosciences, will be sufficient to meet Voyager's projected 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](http://www.voyagertherapeutics.com) or follow [@VoyagerTx](https://twitter.com/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 Voyager's ability to deliver patient imaging data to the DSMB for the RESTORE-1 Phase 2 clinical trial, the review of such data by the DSMB prior to year-end, and pending the DSMB's evaluation of such data the resumption of enrollment in the RESTORE-1 Phase 2 clinical trial; the submission by Neurocrine Biosciences of an expedited safety report relating to the RESTORE-1 clinical trial in a timely manner; Voyager's efforts to work with the FDA to resolve additional information requests relating to the IND application for VY-HTT01, the clearance of the VY-HTT01 IND application by the FDA and the initiation of a Phase 1b clinical trial of VY-HTT01; Voyager's continuing efforts in the discovery and engineering of novel AAV capsids, including progressing non-human primate studies of selected novel capsids; the contributions that will be made to Voyager by key senior level officers and a new member to the Voyager Board of Directors; the timing, progress, activities, goals and reporting of results of Voyager's preclinical programs and clinical trials and its research and development programs; the potential clinical utility of its product candidates; Voyager's ability to add new programs to its pipeline; the regulatory pathway of, and the timing or likelihood of its regulatory filings and approvals for, any of its product candidates; Voyager's anticipated financial results, including Voyager's available cash, cash equivalents and marketable debt securities; Voyager's ability to fund its operating expenses with its current cash, cash equivalents and marketable debt securities through a stated time period; and the ability of Voyager to maintain a high level of business critical activity and maintain a level of scientific leadership during the COVID-19 health crisis are forward looking statements.

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 to provide imaging data to the DSMB for the RESTORE-1 Phase 2 clinical trial, and the ability for the DSMB to complete its evaluation and to resolve questions that may exist regarding such patient data; the ability for Voyager to meet the information requests of, and to resolve questions raised by, the FDA relating to the IND application for VY-HTT01; the ability of Voyager to progress its research and engineering program for novel capsids and to conduct non-human primate studies; the initiation and conduct of preclinical studies and clinical trials; the availability of data from preclinical studies and clinical trials, and the ability to effectively present such data; 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 commercial potential of Voyager's product candidates; the severity and length of the COVID-19 health crisis, the imposition of governmental controls and guidance addressing the COVID health crisis; and the financial and human resources available to Voyager to manage the COVID-19 health crisis. 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.

**Investors:**

Paul Cox  
VP, Investor Relations  
857-201-3463  
[pcox@vygr.com](mailto:pcox@vygr.com)

**Media:**

Sheryl Seapy  
W2Opure  
949-903-4750  
[sseapy@purecommunications.com](mailto:sseapy@purecommunications.com)

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

	<b>Three Months Ended</b>		<b>Nine Months Ended</b>	
	<b>September 30,</b>		<b>September 30,</b>	
<b>Statement of Operations Items:</b>	<b>2020</b>	<b>2019</b>	<b>2020</b>	<b>2019</b>
Collaboration revenue	\$ 117,843	\$ 20,433	\$ 164,591	\$ 71,717
Operating expenses:				
Research and development	25,039	29,777	86,757	83,184
General and administrative	8,277	8,463	26,721	26,444
Total operating expenses	33,316	38,240	113,478	109,628
Operating income (loss)	84,527	(17,807)	51,113	(37,911)
Total other income	1,084	2,801	1,554	6,888
Net income (loss)	\$ 85,611	\$ (15,006)	\$ 52,667	\$ (31,023)
Net income (loss) per share, basic	\$ 2.30	\$ (0.41)	\$ 1.42	\$ (0.87)
Net income (loss) per share, diluted	\$ 2.27	\$ (0.41)	\$ 1.40	\$ (0.87)
Weighted-average common shares outstanding, basic	37,242,504	36,742,993	37,079,242	35,581,408
Weighted-average common shares outstanding, diluted	37,672,328	36,742,993	37,500,155	35,581,408

	<b>September 30,</b>	<b>December 31,</b>
	<b>2020</b>	<b>2019</b>
<b>Selected Balance Sheet Items</b>		
Cash, cash equivalents, and marketable debt securities	\$ 200,018	\$ 281,533
Total assets	\$ 269,757	\$ 354,760
Accounts payable and accrued expenses	\$ 25,051	\$ 25,586
Deferred revenue	\$ 45,671	\$ 194,493
Total stockholders' equity	\$ 166,236	\$ 99,512



Source: Voyager Therapeutics, Inc.