



Voyager Therapeutics Announces Fourth Quarter and Full Year 2019 Financial Results and Corporate Updates

March 3, 2020

Protocol amendment being implemented for ongoing RESTORE-1 trial of VY-AADC (NB1b-1817) for Parkinson's disease; plan to initiate RESTORE-2 trial in 2H 2020

Update on VY-HTT01 for Huntington's disease preclinical program expected in 2Q 2020

Strong financial position with ~\$282M of cash at the end of 2019 and expected runway into mid-2022

CAMBRIDGE, Mass., March 03, 2020 (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 fourth quarter and full year 2019 financial results, program progress and corporate updates.

"In 2019, we took important steps toward our vision of establishing Voyager as the leader in neurological gene therapy, including forming our strategic collaboration with Neurocrine Biosciences, expanding our partnership with AbbVie, and further progressing our wholly-owned and partnered programs," said Andre Turenne, President and CEO of Voyager. "Turning to 2020, we are excited to continue this momentum across all programs. These initiatives include presenting longer-term data from the Parkinson's disease program, advancing our Huntington's disease program, and further leveraging our novel capsid research and expertise in vector engineering and delivery toward additional pipeline programs."

Recent Corporate Highlights and Program Outlook

VY-AADC for Parkinson's Disease

- Based upon FDA feedback, the protocol of the RESTORE-1 clinical trial of VY-AADC is being amended to enroll approximately 85 patients, randomized 2:1 to receive either VY-AADC treatment or a sham surgical procedure (compared to 1:1 prior randomization). There are no changes to the primary or key secondary endpoints, but certain exploratory endpoints are being eliminated from this registrational trial. Neurocrine Biosciences and Voyager expect to update trial enrollment timelines following implementation of the protocol amendment at the trial sites.
- Neurocrine Biosciences and Voyager plan to initiate the global RESTORE-2 trial in the second half of 2020.
- VY-AADC is also being evaluated in an ongoing Phase 1 development program designed to establish safety and optimize dosing and delivery. Neurocrine Biosciences and Voyager expect to present final three-year data on all three cohorts (15 total patients) of the PD-1101 Phase 1b trial, as well as two-year data from the PD-1102 Phase 1 posterior trajectory trial (8 total patients), at medical conferences in 2020.

VY-HTT01 for Huntington's Disease

- Voyager is currently engaged in the ongoing conduct and review of preclinical studies for its Huntington's disease program, VY-HTT01, and expects to provide an update on the program in the second quarter of 2020, including plans to file an IND application.
- Voyager also plans to initiate a prospective observational study of patients with late prodromal and early manifest Huntington's disease in mid-2020. The longitudinal study will evaluate the clinical and biological evolution of peri-manifest Huntington's disease patients, including clinical, neuroimaging, molecular, and digital biomarker outcomes. Patients participating in the observational study may also be eligible for later enrollment in the VY-HTT01 Phase 1 clinical trial.

Early Pipeline and Platform

- Voyager continues to advance its earlier-stage research programs, including wholly owned efforts and efforts with collaboration partners AbbVie and Neurocrine Biosciences. These initiatives include the Friedreich's ataxia program, the SOD1 ALS program, vectorized antibody programs, and discovery efforts on new research programs and novel AAV capsids.

Corporate Updates

- Voyager has recently made key appointments of leaders across several functional areas. These include the appointments of Jennifer Hunt as Senior Vice President of Development Operations, Paul Cox, as Vice President of Investor Relations, Juliana Muscat, Ph.D., as Vice President of Quality, and Gan Wei, Ph.D., as Vice President of Technical Development.

Anticipated Upcoming Milestones

VY-AADC for Parkinson's Disease:

- Present 3-year results from PD-1101 trial at medical conference (2020)
- Present 2-year results from PD-1102 trial at medical conference (2020)
- Initiate RESTORE-2 registration trial (2H 2020)

VY-HTT01 for Huntington's Disease:

- Provide update on program and IND-enabling studies (2Q 2020)
- Initiate prospective observational study (mid-2020)
- Present additional results from preclinical studies (2H 2020)

Early Pipeline and Platform:

- Provide progress updates on Friedreich's ataxia program, new discovery programs, as well as vectorized antibody and novel capsid efforts (2020)

Fourth Quarter and Full Year 2019 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$32.7 million for the fourth quarter of 2019 and \$104.4 million for the year ended December 31, 2019, compared to collaboration revenue of \$2.0 million and \$7.6 million, respectively, for the same periods of 2018. This increase reflects the recognition of amounts related to the restructuring of the Sanofi Genzyme collaboration in June, and amounts related to the Neurocrine Biosciences and AbbVie alpha-synuclein collaborations, both of which became effective in the first quarter of 2019.
- **Net Loss:** Net loss was \$12.6 million for the fourth quarter of 2019 and \$43.6 million for the year ended December 31, 2019, compared to a net loss of \$22.5 million and \$88.3 million, respectively, for the same periods of 2018.
- **R&D Expenses:** Research and development expenses were \$36.6 million for the fourth quarter of 2019, compared to \$16.9 million for the same period in 2018. For the year ended December 31, 2019, R&D expenses were \$119.7 million, compared to \$64.9 million for the same period of 2018. The increase in R&D expenses was primarily related to both external costs and employee-related costs to support Voyager's clinical and preclinical pipeline programs, including the RESTORE-1 clinical trial for VY-AADC.
- **G&A Expenses:** General and administrative expenses were \$9.9 million for the fourth quarter of 2019, compared to \$8.3 million for the same period in 2018. For the year ended December 31, 2019, G&A expenses were \$36.3 million, compared to \$33.8 million for the same period of 2018. The increase in G&A expenses was primarily related to employee and facility costs to support the advancement of Voyager's pipeline programs and operations.
- **Cash Position:** Cash, cash equivalents and marketable debt securities as of December 31, 2019 were \$281.5 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.

Conference Call Information

Voyager will host a conference call and webcast today at 4:30 p.m. EST. The conference call may be accessed by dialing (877) 851-3834 for domestic callers, or +1 (631) 291-4595 for international callers. Please reference conference ID number 8461408 to join the call. The conference call will be webcast live from the Investors & Media section of Voyager's website at www.voyagertherapeutics.com and will be archived there following the call for 90 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 wholly-owned and partnered pipeline focuses on severe neurological diseases for which effective new therapies are needed, including Parkinson's disease, Huntington's disease, a monogenic form of ALS called SOD1, 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 AbbVie and 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](https://twitter.com/VoyagerTx) on Twitter and [LinkedIn](https://www.linkedin.com/company/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, timing and future operation of the

collaboration agreements with AbbVie and Neurocrine Biosciences, including any potential future payments thereunder, its ability to identify and attract parties to participate in research and development collaborations, 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 perform under existing collaborations including those with AbbVie and Neurocrine Biosciences, its 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, its ability to operate its research and development activities efficiently and effectively, the utility and value of Voyager's patent portfolio, and Voyager's anticipated financial results, including Voyager's available cash, cash equivalents and marketable debt securities, the receipt by Voyager of revenues or reimbursement payments from collaboration partners, Voyager's operating expenses, and Voyager's ability to fund its operating expenses with its current cash, cash equivalents and marketable debt securities through a stated time period 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 preclinical studies and 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; 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 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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Voyager Therapeutics, Inc.
Selected Financial Information
(\$-amounts in thousands, except per share data)
(Unaudited)

Statement of Operations Items:	Three Months Ended		Year Ended	
	December 31,		December 31,	
	2019	2018	2019	2018
Collaboration revenue	\$ 32,674	\$ 2,008	\$ 104,391	7,619
Operating expenses:				
Research and development	36,551	16,914	119,735	64,905
General and administrative	9,891	8,255	36,335	33,809
Total operating expenses	46,442	25,169	156,070	98,714
Operating loss	(13,768)	(23,161)	(51,679)	(91,095)
Total other income	1,193	629	8,082	2,627
Loss before income taxes	(12,575)	(22,532)	(43,597)	(88,468)
Income tax benefit	—	—	—	180
Net loss	\$ (12,575)	\$ (22,532)	\$ (43,597)	\$ (88,288)
Net loss per share, basic and diluted	\$ (0.34)	\$ (0.77)	\$ (1.21)	\$ (2.75)
Weighted-average common shares outstanding, basic and diluted	36,838,507	29,281,071	35,898,266	32,065,781

Selected Balance Sheet Items	December 31,	
	2019	2018
Cash, cash equivalents, and marketable debt securities	\$ 281,533	\$ 155,806
Total assets	\$ 354,760	\$ 177,029
Accounts payable and accrued expenses	\$ 25,586	\$ 10,826
Deferred revenue	\$ 194,493	\$ 113,046
Total stockholders' equity	\$ 99,512	\$ 46,446



Source: Voyager Therapeutics, Inc.