



Voyager Therapeutics Reports Fourth Quarter and Full Year 2018 Financial Results and Corporate Highlights

February 26, 2019

Initiated RESTORE-1 Phase 2 trial for VY-AADC for Parkinson's disease

Began IND-enabling preclinical studies for ALS-SOD1 and Huntington's disease programs

Recently announced strategic collaborations with funding for certain programs and significant upfront and potential near-term payments while retaining commercial optionality and allowing further investment in fully-retained pipeline

CAMBRIDGE, Mass., Feb. 26, 2019 (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 2018 financial results, provided corporate highlights, goals and financial guidance and will host a conference call and webcast today at 4:30 p.m. EST to discuss these results.

"Since the beginning of 2018, we advanced our lead and pipeline programs, further developed our vectorized antibody platform, and entered into key enabling collaborations," said Andre Turenne, president and chief executive officer of Voyager Therapeutics. "The recently announced strategic collaborations provide us considerable expertise from world-class partners and allow us to maintain commercial flexibility while strengthening our capital resources. As we plan for 2019 and beyond, these developments position the company well to achieve our goal of becoming the leading, fully-integrated gene therapy company focused on severe neurological diseases."

2018 and Recent Corporate Highlights

- Entered into a strategic development and commercialization collaboration with Neurocrine Biosciences for VY-AADC for Parkinson's disease, for VY-FXN01 for Friedreich's ataxia and two additional programs to be determined.
- Formed two separate, exclusive, global strategic collaborations with AbbVie to develop and commercialize vectorized antibodies. The first collaboration is directed against tau for the potential treatment of Alzheimer's disease and other tauopathies. The second collaboration targets pathological species of alpha-synuclein for the potential treatment of Parkinson's disease and other diseases characterized by the abnormal accumulation of misfolded alpha-synuclein protein (synucleinopathies).
- Expect to strengthen the balance sheet with approximately \$230 million of additional capital from the upfront cash payments from the recent AbbVie and Neurocrine collaborations, including \$50 million from the sale of shares of Voyager's common stock to Neurocrine. In addition to the upfront payments, the AbbVie collaborations potentially include up to \$460 million in preclinical and Phase 1 option payments as well as potential development, regulatory, and commercial milestone payments. For the Neurocrine collaboration for VY-AADC for Parkinson's disease and VY-FXN01 for Friedreich's ataxia, Voyager will receive reimbursement of all development costs until the programs reach the point of Voyager's option to co-commercialize in the U.S. under a cost- and profit-sharing arrangement or grant Neurocrine full U.S. commercial rights in exchange for milestone payments and royalties.
- Appointed new members to the executive management team including Andre Turenne as Chief Executive Officer, Omar Khwaja, M.D., Ph.D., as Chief Medical Officer, Robert Hesslein as General Counsel, and Allison Dorval as Chief Financial Officer.
- Enhanced the engagement with The Michael J. Fox Foundation for Parkinson's Research (MJFF) to include participation in the Parkinson's Progression Markers Initiative (PPMI) and the Parkinson's Disease Education Consortium (PDEC), expanding Voyager's commitment to the Parkinson's disease community by supporting high quality educational programs and ongoing research.
- Entered into strategic manufacturing collaborations with Brammer Bio and Fujifilm Diosynth Biotechnologies to provide Voyager the ability and flexibility to transfer its manufacturing expertise across multiple high-quality vendors to support the development of its pipeline programs and increase capacity and scale of its gene therapies.

2018 and Recent Program Highlights

- Initiated RESTORE-1, a Phase 2, randomized, double-blind, placebo-controlled trial evaluating the safety and efficacy of VY-AADC for the treatment of Parkinson's disease in patients with motor fluctuations that are refractory to medical management. RESTORE-1 and a planned Phase 3 trial (RESTORE-2) of similar size and design to RESTORE-1 incorporate guidance from the FDA from the Type B meeting to conduct two adequate and well-controlled clinical trials for a large patient population such as Parkinson's disease.
- Announced positive longer-term results from the Phase 1b open-label, dose-escalating trial of VY-AADC. The combined seven of 10 patients in Cohorts 2 and 3 who would have met the eligibility criteria for the RESTORE-1 Phase 2 trial demonstrated a 2.8-hour and 2.5-hour (mean) improvement in good ON time at 12 months and 18 months, respectively. These results were achieved with clinically meaningful and sustained reductions in daily oral levodopa and related medications.
- Presented preclinical data for VY-HTT01 for Huntington's disease and VY-SOD102 for Amyotrophic Lateral Sclerosis (ALS) programs at the Congress of the European Society of Gene and Cell Therapy. One-time delivery of VY-HTT01 generated significant reduction of HTT gene expression in deeper tissues and outer layers of the brain of non-human primates. One-time delivery of VY-SOD102 generated significant reduction of SOD1 gene expression in multiple locations throughout a large animal spinal cord, including an 82% reduction in cervical motor neurons near the injection site.
- Received 510(k) regulatory clearance of V-TAG™ from the Center for Devices and Radiological Health of the FDA. Voyager developed the Variable Trajectory Array Guide, or V-TAG™, a real-time, intra-operative, MRI-compatible neuro-navigational device, as a choice for neurosurgeons in addition to the ClearPoint® System from MRI Interventions, Inc. 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.

Corporate Goals and 2019 Financial Guidance

Voyager is committed to becoming the leading gene therapy company focused on severe neurological diseases with expertise in discovery, development, manufacturing and commercialization of gene therapy products for people living with these diseases. During 2019, Voyager plans to achieve the following corporate and financial goals towards fulfilling this commitment:

- During the second quarter of 2019, provide 12-month safety, biomarker and motor function data from the open-label Phase 1 trial of VY-AADC in patients with Parkinson's disease exploring the posterior (i.e., back of the head) infusion trajectory. The posterior trajectory better aligns the infusion of VY-AADC with the anatomical structure of the putamen and is the recommended infusion trajectory for the RESTORE-1, Phase 2 trial. During 2019, provide longer-term safety, biomarker, motor function and quality of life data from Cohorts 1-3 from the ongoing, open-label, Phase 1b trial of VY-AADC for Parkinson's disease.
- Complete activation of neurosurgical and neurology patient referral and management trial sites and continue to enroll patients in the RESTORE-1 Phase 2, randomized, placebo-controlled trial of VY-AADC for Parkinson's disease.
- Advance VY-HTT01 for Huntington's disease and VY-SOD102 for ALS-SOD1 towards clinical trials. Preclinical pharmacology and toxicology studies are underway to support potential filings of IND applications for both programs later this year.
- Advance Friedreich's ataxia program VY-FXN01 towards identifying a lead candidate and IND-enabling preclinical pharmacology and toxicology studies.
- Continue to identify, evaluate and progress business development opportunities and continue to invest in the discovery programs, vectorized antibody platform and new capsid development.
- Based on the Company's current operating plan, Voyager expects to end 2019 with cash, cash equivalents and marketable debt securities of approximately \$280 million to \$290 million. This includes the anticipated upfront payment of \$165 million and expected reimbursement of development costs from the Neurocrine collaboration, as well as the \$65 million upfront payment from the recently announced AbbVie collaboration. Operating expenses in 2019 are expected to range from \$130 million to \$140 million, including amounts reimbursable under the collaborations. Voyager projects that its cash, cash equivalents and marketable debt securities, including amounts to be received under the recently announced Neurocrine and AbbVie collaborations, will be sufficient to fund operating expenses and capital expenditure requirements into mid-2022.

Fourth Quarter and Full Year 2018 Financial Results

For the fourth quarter and full year of 2018, Voyager reported:

A GAAP net loss of \$22.5 million, or \$0.70 per share, for the fourth quarter ended December 31, 2018, compared to a GAAP net loss of \$11.8 million, or \$0.40 per share, for the same period in 2017, and a GAAP net loss of \$88.3 million, or \$2.75 per share, for the full year ended December 31, 2018, compared to a GAAP net loss of \$70.7 million, or \$2.64 per share, for the same period in 2017.

Collaboration revenues of \$2.0 million for the fourth quarter of 2018 compared to collaboration revenues of \$6.3 million for the fourth quarter of 2017. Collaboration revenues of \$7.6 million for the full year ended December 31, 2018 compared to collaboration revenues of \$10.1 million for the full year ended December 31, 2017. Collaboration revenues reflect recognition of payments for research and development services provided by Voyager for various programs under the Sanofi Genzyme and AbbVie collaboration agreements and can vary based on quarterly assessments of efforts under the collaborations. The decrease in collaboration revenues for the fourth quarter and full year 2018 compared to the same periods in 2017 reflect the one-time recognition of amounts allocated to Sanofi Genzyme's rights in the Parkinson's program in the fourth quarter of 2017 and the January 1, 2018 adoption of the new revenue accounting standards which modified the Company's recognition methodology. These decreases were partially offset by revenue related to the research services performed under the collaboration agreement with AbbVie entered into in February 2018.

Research and development (R&D) expenses of \$16.9 million for the fourth quarter ended December 31, 2018 compared to \$13.3 million for the same period in 2017. R&D expenses of \$64.9 million for the year ended December 31, 2018 compared to \$62.3 million for the same period in 2017. The increase in R&D expenses related primarily to expenditures associated with the development of Voyager's pipeline including costs related to the RESTORE-1 Phase 2 clinical trial for VY-AADC, and increased personnel and facility costs to support the advancement of the pipeline programs.

General and administrative (G&A) expenses of \$8.3 million for the fourth quarter ended December 31, 2018 compared to \$5.4 million for the same period in 2017. G&A expenses of \$33.8 million for the year ended December 31, 2018 compared to \$19.7 million for the same period in 2017. The increase in G&A expenses was primarily due to increased professional fees including consulting and legal costs in addition to personnel and facility costs to support Voyager's growing business.

Cash, cash equivalents, and marketable debt securities as of December 31, 2018 were \$155.8 million.

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 9672838. 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. 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 other tauopathies, neurodegenerative diseases related to defective or excess aggregation of alpha-synuclein protein in the brain including Parkinson's disease and other synucleinopathies, and severe, chronic pain. Voyager has strategic collaborations with Sanofi Genzyme, AbbVie and Neurocrine Biosciences. 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. For more information on Voyager Therapeutics, please visit the company's website at www.voyagertherapeutics.com or follow [@VoyagerTx](https://twitter.com/VoyagerTx) on Twitter and [LinkedIn](https://www.linkedin.com/company/voyagertherapeutics).

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 and future operation of the collaboration agreements with AbbVie and Neurocrine, including any potential future payments thereunder, 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, AbbVie and Neurocrine and to add new programs to its pipeline, its expected cash, cash equivalents, and marketable debt securities at the end of a fiscal period, the sufficiency of such cash resources, projections of its operating expenses, its ability to enter into new partnerships or collaborations, 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, those related to 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, including antitrust approvals related to Voyager's collaborations; the continued development of the gene therapy platform; Voyager's scientific approach and general development progress; the sufficiency of cash resources; the possibility of timing of AbbVie's exercise of its development and license options under its 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 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.

(Unaudited)

	Three Months Ended		Year Ended	
	December 31,		December 31,	
	2018	2017	2018	2017
Statement of Operations Items:				
Collaboration revenue	\$ 2,008	\$ 6,346	\$ 7,619	10,135
Operating expenses:				
Research and development	16,914	13,327	64,905	62,260
General and administrative	8,255	5,366	33,809	19,738
Total operating expenses	25,169	18,693	98,714	81,998
Operating loss	(23,161)	(12,347)	(91,095)	(71,863)
Total other income	629	549	2,627	1,165
Loss before income taxes	(22,532)	(11,798)	(88,468)	(70,698)
Income tax provision (benefit)	—	30	(180)	—
Net loss	\$ (22,532)	\$ (11,828)	\$ (88,288)	\$ (70,698)
Net loss per share, basic and diluted	\$ (0.70)	\$ (0.40)	\$ (2.75)	\$ (2.64)
Weighted-average common shares outstanding, basic and diluted	32,327,241	29,281,071	32,065,781	26,803,711

	December 31,	
	2018	2017
Selected Balance Sheet Items		
Cash, cash equivalents, and marketable debt securities	\$ 155,806	\$ 169,052
Total assets	\$ 177,029	\$ 184,477
Accounts payable and accrued expenses	\$ 10,826	\$ 12,517
Deferred revenue	\$ 113,046	\$ 31,560
Total stockholders' equity	\$ 46,446	\$ 134,051

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