Voyager Therapeutics Reports Fourth Quarter and Full Year 2017 Financial Results

March 14, 2018

VY-AADC for advanced Parkinson's disease on track for start of planned global, pivotal Phase 2-3 program during the middle of this year

Two IND filings expected from the ALS, Huntington's disease and Friedreich's ataxia programs for 2019

Recent AbbVie collaboration successfully leveraged Voyager's gene therapy platform and expertise and strengthened the balance sheet to extend cash runway into early 2020

CAMBRIDGE, Mass., March 14, 2018 (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 2017 financial results, provided corporate highlights, goals and financial guidance and will host a conference call and webcast today at 8:00 a.m. ET to discuss these results.

"Our strong performance in 2017 was underscored by advancing our lead program VY-AADC for advanced Parkinson's disease including successfully manufacturing our gene therapy product candidate using our baculovirus/Sf9 cell process and commercial-scale GMP runs, completing our dose-ranging Phase 1b clinical trial as well as our posterior trajectory trial to select both an optimal dose and delivery protocol for the planned pivotal trial, and reporting longer-term Phase 1b data with clinically meaningful and durable responses with this one-time treatment," said Steven Paul, M.D., president and chief executive officer of Voyager Therapeutics. "This performance sets us up to initiate a planned pivotal program in the middle of this year, moving this potential treatment closer to the hundreds of thousands of advanced Parkinson's disease patients who require better control of their motor function in order to improve their quality of life. Our preclinical pipeline targeting other severe neurological diseases continues to advance towards the clinic and with our recently announced collaboration with AbbVie, we continue to successfully leverage our gene therapy platform and expertise towards an exciting new area of vectorized immunotherapy initially to deliver monoclonal antibodies directed against tau for the treatment of Alzheimer's disease and related neurodegenerative diseases."

2017 and Recent Key Pipeline and Corporate Highlights

- Announced updated results to the ongoing, dose-ranging Phase 1b program of VY-AADC for advanced Parkinson's disease. At our mid-dose studied in Cohort 2 at 18 months, patients had a mean increase of approximately five hours a day of on-time without any dyskinesia and experienced 65% less off-time, making this the likely dose for the pivotal Phase 2-3 program.

- Investigational New Drug (IND) application cleared by the Food and Drug Administration (FDA) for VY-AADC in January 2018, allowing the Company to formally initiate clinical trial sites and screen and begin dosing patients for its pivotal Phase 2-3 program for advanced Parkinson's disease. As part of this IND, the chemistry, manufacturing, and controls (CMC) section included data demonstrating comparability between VY-AADC produced under good manufacturing process (GMP) using Voyager's baculovirus/Sf9 manufacturing process and VY-AADC produced using a mammalian cell system consisting of triple-transfection of human embryonic kidney (HEK293) cells similar to that used in Voyager's Phase 1b clinical trial.

- Initiated a separate Phase 1 trial and successfully dosed seven patients with advanced Parkinson's disease with a posterior (i.e., back of the head) infusion trajectory of VY-AADC. A posterior trajectory better aligns the infusion of VY-AADC with the anatomical structure of the putamen and resulted in higher total volume of coverage of the putamen and shorter total procedure time compared to Cohorts 1 through 3 from the ongoing Phase 1b trial that employed a transfrontal, or top of the head, delivery approach into the putamen.

- In February 2018, FDA granted VY-AADC Fast Track designation for advanced Parkinson's disease. Fast Track designation is granted for programs that demonstrate the potential to address an unmet medical need for a serious or life-threatening disease and is intended to facilitate development and expedited regulatory review, including the ability to submit completed sections of a Biologics License Application for a "rolling" review or submission.

- Gained worldwide development and commercial rights to VY-AADC for the treatment of advanced Parkinson's disease. Voyager is on track to dose the first patient in its planned global, pivotal Phase 2-3 program during mid-2018.

- Entered into an exclusive strategic collaboration and option agreement with AbbVie to develop and commercialize vectorized antibodies directed against tau for the treatment of Alzheimer's disease and other neurodegenerative diseases,

combining Voyager's gene therapy platform with AbbVie's monoclonal antibody expertise, global clinical development and commercial capabilities. The research period is underway for each company to identify up to five antibodies for inclusion in the collaboration. Voyager received $69 million upfront cash payment and is eligible to receive potentially up to $155 million in preclinical and Phase 1 option payments as well as development and regulatory milestone payments and royalties.

- Further strengthened the balance sheet with approximately $127 million of additional capital raised from the upfront cash payment from the AbbVie collaboration and a follow-on public equity offering in November, extending Voyager's expected ability to fund its operating expenses and capital expenditure requirements into early 2020.

- Announced plans during 2018 for Steven Paul, M.D., to transition from president and chief executive officer to executive science advisor where he will focus on preclinical discovery research and portfolio development including progressing the AbbVie collaboration. In addition to the executive science advisor position, Dr. Paul will continue to serve on Voyager's Board of Directors, and as a member of Voyager's Science & Technology Committee.

- Strengthened the management team with the additions of Matthew P. Ottmer as Chief Operating Officer and Luis Maranga, Ph.D. as Chief Technical Operations Officer. Mr. Ottmer brings to Voyager more than 18 years of biotechnology industry experience, including executive leadership of business operations, product development, and commercialization across multiple therapeutic areas and all stages of development. Dr. Maranga joins Voyager with over 20 years of biotechnology industry experience, focused on bioprocess development, CMC, GMP, process validation and regulatory submissions, and facilities management including extensive work with the baculovirus/Sf9 expression system.

Corporate Goals and 2018 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 devastating diseases. During 2018, Voyager plans to achieve the following corporate goals towards fulfilling this commitment:

- During the second quarter of this year, complete a Type C meeting with the Office of Tissues and Advanced Therapies division of the FDA's Center for Biologics Evaluation and Research and incorporate feedback from this meeting into the Phase 2-3 pivotal program for VY-AADC for advanced Parkinson's disease.

- During the second quarter of this year, provide six-month safety and motor function data from the Phase 1 trial of VY-AADC using the posterior infusion trajectory. Seven patients have been dosed using this trajectory as the likely preferred surgical approach for the Phase 2-3 pivotal program.

- During mid-2018, dose the first patient in the planned Phase 2-3 pivotal program for advanced Parkinson's disease. Neurosurgical and neurology clinical trial sites have been identified and are being activated through institutional review board processes, after which, patient referral, screening and dosing can proceed.

- During the second half of 2018, provide longer-term safety, biomarker, motor function and quality of life data from Cohorts 1-3 and from patients in the posterior trajectory trial of VY-AADC for advanced Parkinson's disease.

- Advance multiple preclinical programs towards clinical trials through further vector optimization and exploration of additional routes of administration, to support filing two IND applications from the ALS SOD1, Huntington's disease, and Friedreich's ataxia programs during 2019.

- Continue to identify, evaluate and progress collaborative business development opportunities for certain Voyager programs, technology platform capabilities, or both.

- Based on the Company's current operating plan, Voyager expects to end 2018 with cash, cash equivalents and marketable debt securities of approximately $125 million to $135 million, which includes the $69 million upfront payment from AbbVie, and projects that its existing cash, cash equivalents and marketable debt securities will be sufficient to fund operating expenses and capital expenditure requirements into early 2020.

Fourth Quarter and Full Year 2017 Financial Results

For the fourth quarter and full year of 2017, Voyager reported:

A GAAP net loss of $11.8 million, or $0.40 per share, for the fourth quarter ended December 31, 2017, compared to a GAAP net loss of $14.7 million, or $0.57 per share, for the same period in 2016. The Company reported a GAAP net loss of $70.7 million, or $2.64 per share, for the full year ended December 31, 2017, compared to a net loss of $40.2 million, or $1.59 per share, for the same period in 2016.

Collaboration revenues of $6.3 million for the fourth quarter of 2017 compared to collaboration revenues of $2.4 million for the fourth quarter of 2016. Collaboration revenues of $10.1 million for the full year ended December 31, 2017 compared to collaboration revenues of $14.2 million for the full year ended December 31, 2016. Collaboration revenues reflect recognition of payment for research and development services provided by Voyager for various programs under the Sanofi Genzyme collaboration agreement. Collaboration revenues can vary based on quarterly assessments of expected or anticipated efforts under the collaboration. The increase in collaboration revenues for the fourth quarter 2017 compared to the same period in 2016 reflect revenue recognition as a result of Sanofi Genzyme's decision not to exercise its option to the ex-U.S. rights to the Parkinson's disease program and Voyager's recognition of the portion of the agreement allocated to that license option. The decrease in collaboration revenues for the full year 2017 compared to the same period in 2016 reflects the change in the estimated period for reaching development milestones for certain preclinical programs under the collaboration agreement.

Research and development (R&D) expenses of $13.3 million for the fourth quarter ended December 31, 2017 compared to $12.7 million for the same period in 2016. R&D expenses of $62.3 million for the year ended December 31, 2017 compared to $42.2 million for the same period in 2016. The increase in R&D expenses related primarily to expenditures associated with the development of Voyager's pipeline including the ongoing Phase 1 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 $5.4 million for the fourth quarter ended December 31, 2017 compared to $3.5 million for the same period in 2016. G&A expenses of $19.7 million for the year ended December 31, 2017 compared to $13.3 million for the same period in 2016. The increase in G&A expenses was primarily due to personnel and facility costs to support Voyager's pipeline programs.

Cash, cash equivalents, and marketable debt securities as of December 31, 2017 were $169.1 million.

Conference Call Information

Voyager will host a conference call and webcast today at 8:00 a.m. ET. 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 3688788. 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 advanced Parkinson's disease, a monogenic form of ALS called SOD1, Huntington's disease, Friedreich's ataxia, and neurodegenerative diseases related to defective or excess aggregation of tau protein in the brain including Alzheimer's disease and severe, chronic pain. Voyager has broad strategic collaborations with Sanofi Genzyme, the specialty care global business unit of Sanofi, AbbVie, and the University of Massachusetts Medical School. 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, please visit www.voyagertherapeutics.com.

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 its CEO transition plans, the initiation, timing, progress and reporting of results of its preclinical programs and clinical trials and its research and development programs, 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 product engine, 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 and AbbVie and to add new programs to its pipeline, its ability to enter into new partnerships or collaborations, its expected cash, cash equivalents and marketable debt securities at the end of a fiscal period and anticipation for how long expected cash, cash equivalents and marketable debt securities will last, and the timing or likelihood of its regulatory filings and approvals, 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 search for a new CEO; the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory submissions and approvals; the continued development of the product engine; Voyager's scientific approach and general development progress; the availability or commercial potential of Voyager's product candidates; the sufficiency of cash resources; need for additional financing; and the possibility and timing of Voyager's partner's exercise of their options to the collaboration programs. 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.

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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:	**2017**	**2016**	**2017**	**2016**
Collaboration revenue	$ 6,345	$ 2,362	$ 10,135	14,220
Operating expenses:				
Research and development	13,327	12,723	62,260	42,249
General and administrative	5,366	3,481	19,738	13,270
Total operating expenses	18,693	16,204	81,998	55,519
Operating loss	(12,348)	(13,842)	(71,863)	(41,299)
Total other income (loss)	550	(477)	1,165	1,158
Loss before income taxes	(11,798)	(14,319)	(70,698)	(40,141)
Income tax (benefit) provision	(31)	355	—	52
Net loss	$ (11,767)	$ (14,674)	$ (70,698)	$ (40,193)
Net loss per share, basic and diluted	$ (0.40)	$ (0.57)	$ (2.64)	$ (1.59)
Weighted-average common shares outstanding, basic and diluted	29,281,071	25,526,843	26,803,711	25,302,414

	December 31,	
Selected Balance Sheet Items	**2017**	**2016**
Cash, cash equivalents, and marketable debt securities	$ 169,052	$ 174,418
Total assets	$ 184,477	$ 189,566
Accounts payable and accrued expenses	$ 12,517	$ 7,038
Deferred revenue	$ 31,560	$ 41,582
Total stockholders' equity	$ 134,051	$ 135,922

Voyager Therapeutics, Inc.