Voyager Therapeutics Reports Fourth Quarter and Full Year 2016 Financial Results and Corporate Highlights

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Lead program VY-AADC01 for advanced Parkinson's disease on track to report 6-month data from Cohort 3 and longer-term data from Cohorts 1 and 2 in mid-year 2017

VY-AADC01 Phase 1b data accepted as emerging (late-breaking) oral presentation at the upcoming American Academy of Neurology (AAN) meeting

Three investigational new drug (IND) applications planned within the next 24 months for gene therapy pipeline programs targeting devastating CNS diseases

CAMBRIDGE, Mass., March 15, 2017 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ:VYGR), a clinical-stage gene therapy company developing life-changing treatments for severe diseases of the central nervous system (CNS), today reported its fourth quarter and full year 2016 financial results, and provided corporate highlights, goals and financial guidance.

"Voyager's exceptional performance and accomplishments during 2016 created strong momentum for the company for 2017 and beyond as we continue to focus our efforts on developing gene therapies for devastating diseases of the CNS," said Steven Paul, M.D., president and chief executive officer of Voyager Therapeutics. "The positive interim Phase 1b data we provided late last year for VY-AADC01 for advanced Parkinson's disease provided proof-of-concept that a one-time, targeted delivery of a gene therapy was well tolerated and could enhance patients' sensitivity to levodopa while at the same time generate durable, dose-related, and clinically meaningful improvements in patients' motor function. Additional data from this trial mid-year from more patients for longer duration will inform the design of a double-blind, placebo-controlled trial expected to start later this year. Our pipeline programs are rapidly progressing with three INDs planned within the next 24 months for our monogenic ALS, Huntington's disease, and Friedreich's ataxia programs. During the year, we allocated capital wisely towards our manufacturing capabilities and vector engineering platform providing us with a strong foundation for the transformative years ahead."

2016 and Recent Key Pipeline and Corporate Highlights

Lead program highlights:

- In early December, reported positive interim results at six months from the Phase 1b trial of VY-AADC01 for advanced Parkinson's disease demonstrating that real-time, intra-operative accurate MRI-guided delivery of escalating doses of VY-AADC01 were well tolerated, increased coverage of the putamen, increased aromatic L-amino acid decarboxylase (AADC) enzyme activity, enhanced response to levodopa, and generated durable, dose-related, clinically meaningful improvements in various measures of patients' motor function. This was especially evident at the higher dose in Cohort 2 with improved motor symptoms as measured by part three of the United Parkinson's Disease Rating Scale (UPDRS-III) off medication and on medication scores, and patient-reported diary hours, suggesting higher peak effects and a longer duration of action of levodopa. These effects were maintained and in some patients improved at 12 months of follow-up.

- Announced acceptance of the interim Phase 1b data for VY-AADC01 as an emerging (late-breaking) oral presentation at the upcoming AAN Annual Meeting being held April 22-28, 2017, in Boston, MA. The interim Phase 1b data will be presented during the Emerging Science Session taking place Tuesday, April 25, from 5:45 p.m. to 6:30 p.m. EDT.

- Reported the relationship between surgical coverage of the region of the brain (the putamen) targeted with VY-AADC01 and AADC enzyme activity that is responsible for converting levodopa to dopamine in the putamen using [18F] fluorodopa (or F-Dopa) positron emission tomography (PET). The results demonstrated that surgical coverage of the putamen with VY-AADC01 was highly correlated with change in AADC enzyme activity measured by F-Dopa PET ($r=0.81$, $p<0.05$).

- Completed enrollment of five patients in Cohort 3 and reported greater average coverage of the putamen in Cohort 3 (42%) compared to Cohort 2 (34%) with similar infusion volumes and Cohort 1 (21%) with a lower infusion volume. All five patients from Cohort 3 were discharged from the hospital within two days after completing surgery.

Pipeline program highlights:

- Selected VY-SOD101 as a clinical candidate for the treatment of ALS due to mutations in the superoxide dismutase 1 gene (SOD1) and progressed potential lead candidates for VY-HTT01 for Huntington's disease, and VY-FXN01 for Friedreich's ataxia.

- At the company's R&D day in April, announced two new preclinical programs; VY-TAU01 and VY-NAV01, which are focused on the molecular targets tau and $Na_V1.7$, respectively, and for which Voyager owns worldwide rights to both programs. VY-TAU01 is an adeno-associated virus (AAV) vectorized version of an anti-tau monoclonal antibody for direct one-time delivery to the CNS. VY-TAU01 could be a potential treatment for severe neurological disorders, such as

frontotemporal dementia and Alzheimer's disease. Based on preclinical data, Voyager believes that this approach could achieve significantly higher levels of the therapeutic anti-tau antibody in the CNS when compared to the systemic administration of an antibody. VY-NAV01 targets the knockdown, or silencing, of $Na_V1.7$ in sensory neurons of the dorsal root ganglia as a potential one-time treatment of certain forms of severe, chronic pain. Such an approach may avoid the dose-limiting side effects associated with the non-selective profile of many current treatments used to treat severe, chronic pain, and achieve a durable clinical benefit following a single administration of the therapy.

Recent corporate highlights:

- A core competency of Voyager is the ability to genetically engineer and optimize capsids to yield vectors with desirable properties such as enhanced tissue specificity and improved delivery of genes to the brain and spinal cord. In September, Voyager expanded its portfolio of AAV capsids through a co-exclusive worldwide license agreement with the California Institute of Technology (Caltech) related to novel AAV capsids with enhanced blood-brain barrier penetration and CNS targeting. In partnership with Dr. Ben Deverman and Professor Viviana Gradinaru at Caltech, Voyager continues to advance this technology which, in preclinical studies, generated AAV variants that broadly transduced the CNS with enhanced efficiency after intravenous injection.

- In September, announced a research collaboration with CHDI Foundation, Inc. (CHDI) to advance Voyager's VY-HTT01 program, an AAV-mediated gene-silencing therapy for Huntington's disease. The collaboration builds upon a previous collaboration between CHDI and Sanofi Genzyme and includes funding from CHDI to help support preparation for and filing of an investigational new drug application, as well as completion of a Phase 1 clinical trial.

- Promoted Dinah Sah, Ph.D., to chief scientific officer. As Voyager's research and preclinical program leader since early 2014, Dr. Sah has overseen and led the research pipeline, driven strong collaboration efforts with external industry and academic groups, and built teams that include research and development. Dr. Sah has planned, organized and successfully led the research leading to clinical candidate nomination for VY-SOD101, as well as the progression of the Huntington's disease and Friedreich's ataxia programs towards lead selection. With her 24-year industry track record that includes in-depth expertise in RNA interference and leadership of multiple programs from research to Phase 1, Dr. Sah will oversee the pipeline programs as they advance from research to early development, as well as platform discovery and translational work.

- Promoted Bernard Ravina, M.D., M.S., to chief medical officer. As Voyager's clinical development leader since early 2014, Dr. Ravina planned and successfully implemented Voyager's Phase 1b clinical trial for VY-AADC01 for advanced Parkinson's disease, identified, opened and expanded clinical trial sites, drove strong patient and physician participation, and generated support and key input from key opinion leaders. With his track record and experience with other neurological diseases and a team that includes clinical operations, medical, patient advocacy and preclinical safety, Dr. Ravina will oversee the pipeline programs as they advance through clinical development.

- Appointed Jane Pritchett Henderson as chief financial officer, and strengthened the Board of Directors with the appointments of Wendy L. Dixon, Ph.D., and Glenn F. Pierce, M.D., Ph.D. Ms. Henderson brings more than 28 years of life sciences industry and banking experience and leadership to Voyager, most recently serving as chief financial and business officer of Kolltan Pharmaceuticals, Inc. Dr. Dixon brings over 35 years of global biopharmaceutical leadership experience where, as a senior executive, she combined her technical and commercial background to direct the development, launch and growth of over 20 new pharmaceutical products, including many highly successful multi-billion dollar global brands across multiple therapeutic areas including oncology, virology, immunology and neurology. Dr. Pierce serves as entrepreneur-in-residence at Third Rock Ventures, having joined the company in 2016 after more than 30 years of research and development experience working with biotechnology companies including Biogen, where he most recently served as chief medical officer leading the hematology, cell and gene therapies division.

Corporate Goals and 2017 Financial Guidance

Voyager remains committed to becoming the leading gene therapy company focused on severe diseases of the CNS with expertise in discovery, development, manufacturing and commercialization of gene therapy products for people living with these devastating diseases. The significant accomplishments achieved during 2016 provide a solid foundation for continued progress during 2017 and beyond, as measured by the planned achievements of the following corporate goals and 2017 financial guidance:

- For VY-AADC01, provide six-month safety, biomarker and motor function data from Cohort 3, as well as longer-term safety and motor function data from Cohorts 1 and 2, from the ongoing Phase 1b trial for advanced Parkinson's disease in mid-2017.

- For VY-AADC01, initiate a posterior (i.e., back of the head) infusion trajectory. A posterior trajectory aligns the infusion of VY-AADC01 with the anatomical structure of the putamen and could result in a higher total volume of coverage of the putamen. Data from this trial will also help inform the design of the double-blind, placebo-controlled trial planned to begin during the fourth quarter of 2017.

- Initiate a double-blind, placebo-controlled trial for VY-AADC01 for advanced Parkinson's disease during the fourth quarter of 2017.

- Advance multiple preclinical programs towards clinical trials, with the goal of filing three IND applications within the next 24-months for the VY-SOD101, VY-HTT01, and VY-FXN01 programs, including an IND for VY-SOD101 during the fourth quarter of 2017.

- Identify, evaluate and progress collaborative opportunities for certain unpartnered Voyager programs or our technology platform capabilities.

- Based on the company's current operating plan, Voyager expects to end 2017 with cash, cash equivalents and marketable debt securities of approximately $90 million to $100 million and projects that its existing cash, cash equivalents and marketable debt securities will be sufficient to fund operating expenses and capital expenditure requirements into 2019.

Fourth Quarter and Full Year 2016 Financial Results

Voyager reported a GAAP net loss of $14.7 million, or $0.57 per share, for the fourth quarter ended December 31, 2016, compared to a GAAP net loss of $8.8 million, or $0.67 per share, for the same period in 2015. The company reported a net loss of $40.2 million, or $1.59 per share, for the full year ended December 31, 2016, compared to a net loss of $38.3 million, or $9.14 per share, for the same period in 2015.

Collaboration revenues of $2.4 million for the fourth quarter of 2016 compared to collaboration revenues of $4.9 million for the fourth quarter of 2015. Collaboration revenues of $14.2 million for the full year ended December 31, 2016 compared to collaboration revenues of $17.3 million for the full year ended December 31, 2015. Collaboration revenues reflect recognition of payment for research and development services provided by Voyager for various programs under the Sanofi Genzyme collaboration agreement. The decrease in collaboration revenues for the fourth quarter and full year 2016 compared to the same periods in 2015 reflect deprioritized development of VY-SMN101 for spinal muscular atrophy and a reduction of certain services provided by Sanofi Genzyme.

Research and development (R&D) expenses of $12.7 million for the fourth quarter ended December 31, 2016 compared to $9.2 million for the same period in 2015. R&D expenses of $42.2 million for the year ended December 31, 2016 compared to $27.7 million for the same period in 2015. The increase in R&D expenses was largely due to expenditures associated with the development of Voyager's pipeline and product engine, increased facility expenses and personnel costs to support the advancement of the pipeline programs.

General and administrative (G&A) expenses of $3.5 million for the fourth quarter ended December 31, 2016 compared to $3.2 million for the same period in 2015. G&A expenses of $13.3 million for the year ended December 31, 2016 compared to $9.9 million for the same period in 2015. The increase in G&A expenses was primarily due to personnel costs to support Voyager's growth and facility costs.

Cash, cash equivalents, and marketable debt securities as of December 31, 2016 were $174.4 million.

Conference Call Information

Voyager will host a conference call and webcast today at 8:30 a.m. EDT. 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 83618016. 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 Parkinson's Disease and VY-AADC01

Parkinson's disease is a chronic, progressive and debilitating neurodegenerative disease that affects approximately 700,000 people in the U.S. [1] and seven to 10 million people worldwide[2]. It is estimated that up to 15% of the prevalent population with Parkinson's disease, or approximately 100,000 patients in the U.S., have motor fluctuations that are refractory, or not well-controlled, with levodopa. While the underlying cause of Parkinson's disease in most patients is unknown, the motor symptoms of the disease arise from a loss of neurons in the midbrain that produce the neurotransmitter dopamine. Declining levels of dopamine in this particular region of the brain leads to the motor symptoms associated with Parkinson's disease including tremors, slow movement or loss of movement, rigidity, and postural instability. Motor symptoms during the advanced stages of the disease include falling, gait freezing, and difficulty with speech and swallowing, with patients often requiring the daily assistance of a caregiver.

There are currently no therapies that effectively slow or reverse the progression of Parkinson's disease. Levodopa remains the standard of care treatment, with its beneficial effects on symptom control having been discovered over 40 years ago[3]. Patients are generally well-controlled with oral levodopa in the early stages of the disease, but become less responsive to treatment as the disease progresses. Patients experience longer periods of reduced mobility and stiffness termed off-time, or the time when medication is no longer providing benefit, and shorter periods of on-time when their medication is effective.

The progressive motor symptoms of Parkinson's disease are largely due to the death of dopamine neurons in the substantia nigra, a part of the midbrain that converts levodopa to dopamine, in a single step catalyzed by the enzyme AADC. Neurons in the substantia nigra release dopamine into the putamen where the receptors for dopamine reside. In advanced Parkinson's disease, neurons in the substantia nigra degenerate and the enzyme AADC is markedly reduced in the putamen, which limits the brain's ability to convert oral levodopa to dopamine [4]. The intrinsic neurons in the putamen, however, do not degenerate in Parkinson's disease [5],[6]. VY-AADC01, comprised of the adeno-associated virus-2 capsid and a cytomegalovirus promoter to drive AADC transgene expression, is designed to deliver the AADC gene directly into neurons of the putamen where dopamine receptors are located, bypassing the substantia nigra neurons and enabling the neurons of the putamen to express the AADC enzyme to convert levodopa into dopamine. The approach with VY-AADC01, therefore, has the potential to durably enhance the conversion of levodopa to dopamine and provide clinically meaningful improvements in motor symptoms following a single administration.

About Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company developing life-changing treatments for severe diseases of the CNS. 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. The Company's pipeline focuses on severe CNS diseases in need of effective new therapies, including advanced Parkinson's disease, a monogenic form of ALS, Friedreich's ataxia, Huntington's disease, frontotemporal dementia, Alzheimer's disease and severe, chronic pain. Voyager has broad strategic collaborations with Sanofi Genzyme, the specialty care global business unit of Sanofi, 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. Follow Voyager on LinkedIn.

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 law. The use of words such as “may,” “might,” “will,” “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 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 complete, clinical trials, its ability to continue to develop its product engine, its ability to add new programs to its pipeline, ability to enter into new partnerships or collaborations, its expected cash, cash equivalents and marketable debt securities at the end of a fiscal year 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. These statements are also subject to a number of material risks and uncertainties that are described in Voyager’s most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission, as updated by its future 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.

Selected Financial Information

($-amounts in thousands, except per share estimates)

(Unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
Statement of Operations Items:	**2016**	**2015**	**2016**	**2015**
Collaboration revenue	$ 2,362	$ 4,937	$ 14,220	17,334
Operating expenses:				
Research and development	12,723	9,220	42,249	27,679
General and administrative	3,481	3,157	13,270	9,909
Total operating expenses	16,204	12,377	55,519	37,588
Operating loss	(13,842)	(7,440)	(41,299)	(20,254)
Total other income (expense)	(476)	157	1,158	(9,418)
Loss before income taxes	(14,318)	(7,283)	(40,141)	(29,672)
Income tax expense	355	—	52	—
Net loss	(14,673)	(7,283)	(40,193)	(29,672)
GAAP charges related to pre-IPO preferred stock	—	(1,534)	—	(8,618)
Net loss attributable to common stockholders	$ (14,673)	$ (8,817)	$ (40,193)	(38,290)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.57)	$ (0.67)	$ (1.59)	(9.14)
Weighted-average common shares outstanding, basic and diluted	25,526,843	13,178,922	25,302,414	4,191,210

	December 31,	
Selected Balance Sheet Items	**2016**	**2015**
Cash, cash equivalents, and marketable debt securities	$ 174,418	$ 224,345
Total assets	$ 189,566	$ 229,457
Accounts payable and accrued expenses	$ 7,038	$ 4,042
Deferred revenue	$ 41,582	$ 54,982
Total stockholders’ equity	$ 135,922	$ 169,074

[1] Willis et al, *Neuroepidemiology*.2010;34:143–151

[2] www.pdf.org/en/parkinson_statistics

[3] Poewe W, et al, *Clinical Interventions in Aging*.2010;5:229-238.

[4] Lloyd, *J Pharmacol Exp Ther*. 1975;195:453-464, Nagatsu, *J Neural Transm Suppl*.2007

[5] Cold Spring Harb Perspect Med 2012;2:a009258

[6] Braak et al, *Cell Tissue Res*.2004;318:121-134

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