Voyager Therapeutics Provides Third Quarter 2016 Investor Update

November 10, 2016

VY-AADC01 program for advanced Parkinson's disease on track for interim Phase 1b results in early December

Preclinical pipeline advances along with prioritization

Recent licensing deal increases access to more novel AAV capsids

CAMBRIDGE, Mass., Nov. 10, 2016 (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 third quarter 2016 financial results and highlighted recent pipeline and corporate achievements, as well as expected upcoming milestones.

"Voyager has made significant progress this quarter with its lead and pipeline programs and business development activities," said Steven Paul, M.D., president and chief executive officer of Voyager Therapeutics. "During the third quarter, we continued to enroll patients in our ongoing, dose-ranging Phase 1b trial of VY-AADC01 in advanced Parkinson's disease. We remain on track to report top-line, interim safety and efficacy data from Cohorts 1 and 2 in early December. Our pipeline of AAV gene therapy programs continued to advance during the quarter, and we have expanded our portfolio of novel AAV capsids through a recent licensing agreement with the California Institute of Technology for capsids which have demonstrated in preclinical models markedly enhanced blood-brain barrier penetration for the potential treatment of CNS diseases."

Recent Highlights and Updates

VY-AADC01 for Advanced Parkinson's Disease

In August, investigators dosed the first patient in the third cohort of the ongoing Phase 1b open-label trial of VY-AADC01 in advanced Parkinson's disease. Patients enrolling in Cohort 3 (up to five patients) will receive a single administration of VY-AADC01 at a total dose of up to 4.5×10^{12} vector genomes (vg), representing a three-fold higher total dose than patients in Cohort 2 (1.5×10^{12} vg). Voyager plans to complete Cohort 3 enrollment by early 2017 and remains on track to report six-month data from this cohort in mid-2017. Voyager also remains on track to provide six-month safety, motor function, and biomarker data from patients in Cohort 1 and 2 in early December of this year. Clinical data from Cohorts 1-3 will help inform the design of the placebo-controlled (sham surgery) trial of VY-AADC01 in advanced Parkinson's disease planned to begin in the fourth quarter of 2017.

The goals of the current Phase 1b trial are to assess the safety, biomarker activity, and relevant clinical measures across ascending doses of VY-AADC01 as well as to optimize vector delivery. Cohorts 1-3 employ a transfrontal (i.e., top of the head) trajectory of VY-AADC01 into the putamen. To further optimize delivery, a study exploring a posterior (i.e., back of the head) trajectory is planned. A posterior trajectory aligns the infusion of VY-AADC01 with the anatomical structure of the putamen. Voyager believes this will result in a higher total volume of coverage of the putamen and a higher total dose of VY-AADC01, up to 9.4×10^{12} vg, representing a two-fold higher total dose than patients in Cohort 3 and a six-fold higher total dose than patients in Cohort 2. Voyager is activating additional clinical trial sites this quarter and plans to dose the first patient with this trajectory in the first quarter of 2017. Data from this trial will also help inform the design of the placebo-controlled trial planned to begin in the fourth quarter of 2017.

Preclinical Pipeline Update

During the quarter, Voyager continued to advance its multiple preclinical programs towards selection of lead clinical candidates with the goal of filing an Investigational New Drug (IND) application for VY-SOD101 for a monogenic form of amyotrophic lateral sclerosis (ALS) in the fourth quarter of 2017. Given the progress of VY-SOD101, VY-FXN01 for Friedreich's ataxia, and VY-HTT01 for Huntington's disease, Voyager is prioritizing and allocating its resources towards these and its other pipeline programs and deprioritizing VY-SMN101 for the treatment of spinal muscular atrophy (SMA).

Licensing Activities

In September, Voyager expanded its portfolio of AAV capsids to include novel gene therapy capsids from the California Institute of Technology (Caltech). The worldwide license agreement covers all fields of use and includes novel AAV capsids that have demonstrated up to ninety-fold enhanced blood-brain barrier penetration than AAV9 in mice for the potential treatment of CNS diseases following systemic administration of an AAV gene therapy vector.

In exchange for co-exclusive rights to the novel AAV capsids, intellectual property and related technology, Voyager made an undisclosed upfront payment to Caltech and will further compensate Caltech upon achievement of certain development and regulatory milestones and share royalties upon the potential commercial launch of products utilizing the capsids included in the agreement. With this agreement, Voyager is also collaborating with Ben Deverman, Ph.D. and professor Viviana Gradinaru, Ph.D. at Caltech to continue to advance and extend this technology for application to the treatment of severe human diseases.

Upcoming Investor Conference Participation

During November, Voyager's management team members will participate in the following investor conferences:

- **Event:** Stifel 2016 Healthcare Conference, New York City
  When: November 15, 2016, 10:15 to 10:55 a.m. EST
  Presenter: Bernard Ravina, M.D., vice president of clinical development

- **Event:** Piper Jaffray Healthcare Conference, New York City
  When: November 30, 2016, 10:00 to 10:55 a.m. EST
  Presenter: Steven Paul, M.D., president and chief executive officer

Live-streaming webcasts of these presentations can be accessed through the Investors & Media section of Voyager's website at www.voyagertherapeutics.com. The webcast will be archived for 30 days after the live event concludes.

Third Quarter 2016 Financial Results and Guidance

For the third quarter ended September 30, 2016, Voyager reported a GAAP net loss of $9.0 million, or $0.35 per share, compared to a GAAP net loss of $6.9 million, or $5.25 per share, for the same period in 2015.

Research and development (R&D) expenses increased to $10.3 million for the third quarter ended September 30, 2016 compared to $6.5 million for the same period in 2015 primarily due to R&D, manufacturing, and personnel costs associated with Voyager's advancing pipeline.

General and administrative (G&A) expenses increased to $3.4 million for the third quarter ended September 30, 2016 compared to $2.5 million for the same period in 2015 primarily due to G&A personnel costs to support Voyager's growth, and expenses related to operating as a public company.

Voyager reported cash, cash equivalents and marketable securities of $191.2 million as of September 30, 2016. Voyager now expects to end 2016 with cash, cash equivalents, and marketable securities of approximately $170.0 to $175.0 million, compared with approximately $160.0 million previously disclosed, due to decreased spending related to pipeline prioritization activities for VY-SMN101 and timing of certain payments for planned research and development expenses and facilities expansion. Voyager continues to project that its existing cash, cash equivalents and marketable securities will be sufficient to fund operating expenses and capital expenditure requirements into 2019.

Conference Call Information

Voyager will host a conference call and webcast today at 8:30 a.m. EST. The conference 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 11101029. A live audio webcast of the conference call and replay 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 percent 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 disease arises 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 aromatic L-amino acid decarboxylase (AADC) enzyme. Neurons in the substantia nigra release dopamine into the putamen where the receptors for dopamine reside. In advanced Parkinson's disease, the neurons in the substantia nigra degenerate and the enzyme AADC is markedly reduced, which limits the brain's ability to convert oral levodopa to dopamine [4]. The neurons in the putamen do not degenerate in Parkinson's disease [5,6]. VY-AADC01, comprised of the adeno-associated virus-2 capsid and a cytomegalovirus promotor to drive AADC transgene expression, is designed to deliver the AADC gene directly into the putamen where the 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 adeno-associated virus (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,” “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, its expected cash, cash equivalents and marketable securities at the end of a fiscal year and anticipation for how long expected cash, cash equivalents and marketable 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)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
Statement of Operations Items:	**2016**	**2015**	**2016**	**2015**
Collaboration revenue	$ 3,308	$ 4,937	$ 11,858	$ 12,397
Operating expenses:				
Research and development	10,309	6,481	29,526	18,459
General and administrative	3,370	2,475	9,789	6,752
Total operating expenses	13,679	8,956	39,315	25,211
Operating loss	(10,371)	(4,019)	(27,457)	(12,814)
Total other income (expense)	1,072	102	1,634	(9,575)
Loss before income taxes	(9,299)	(3,917)	(25,823)	(22,389)
Income tax benefit	303	—	303	—
Net loss	(8,996)	(3,917)	(25,520)	(22,389)
GAAP charges related to pre-IPO preferred stock	—	(2,997)	—	(7,084)
Net loss attributable to common stockholders	$ (8,996)	$ (6,914)	$ (25,520)	$ (29,473)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.35)	$ (5.25)	$ (1.01)	$ (25.36)
Weighted-average common shares outstanding, basic and diluted	25,374,381	1,317,150	25,227,058	1,161,982

	September 30,	December 31,
Selected Balance Sheet Items:	**2016**	**2015**
Cash, cash equivalents, and current marketable securities	$ 191,153	$ 224,345
Total assets	$ 201,984	$ 229,457
Accounts payable and accrued expenses	$ 7,091	$ 4,042
Deferred revenue	$ 43,752	$ 54,982
Total stockholders’ equity	$ 149,124	$ 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

Investor Relations:
Matt Osborne
Head of Investor Relations & Corporate Communications
857-259-5353
mosborne@vygr.com

Media:
Katie Engleman
Pure Communications, Inc.
910-509-3977
Katie@purecommunicationsinc.com

Voyager Therapeutics