



Voyager Therapeutics Provides Second Quarter 2016 Investor Update

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VY-AADC01 Phase 1b Study for Advanced Parkinson's Disease On Track to Report Six-Month Safety, Motor Function, and Biomarker Data from Cohorts 1 and 2 by Year-End 2016

Pipeline Advances with Selection of Lead Clinical Candidates for Multiple Pipeline Programs by Late 2016 or Early 2017

CAMBRIDGE, Mass., Aug. 11, 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 second quarter 2016 financial results and highlighted recent pipeline and corporate achievements, as well as expected upcoming milestones.

"Voyager's mission to become the leading gene therapy company focused on severe diseases of the CNS further advanced this quarter with the progress of our lead program, VY-AADC01 for advanced Parkinson's disease, and the progress of our additional programs in earlier stages of development," said Steven Paul, MD, president and chief executive officer of Voyager Therapeutics. "During the second quarter, VY-AADC01 was successfully administered at a second clinical trial site and we are in the process of opening more clinical trial sites. In addition, our preclinical pipeline programs continue to advance with the selection of lead clinical candidates underway."

Second Quarter Pipeline and Corporate Highlights

- Completed enrollment in Cohort 2 in the Phase 1b clinical trial of VY-AADC01 in patients with advanced Parkinson's disease and recently announced dosing of the first patient in the third cohort in this ongoing trial of up to 20 patients. In Cohorts 1 and 2, patients received a single administration of VY-AADC01 at a total dose of up to 7.5×10^{11} vector genomes (vg) and 1.5×10^{12} vg, respectively. Patients recently began enrolling in Cohort 3 and will receive up to a three-fold higher total dose (4.5×10^{12} vg) than Cohort 2. A final, additional cohort (Cohort 4) could increase the total dose to approximately 6-fold higher than Cohort 2. The Company remains on track to provide six-month data on safety, motor function, and biomarkers from patients in Cohort 1 and 2, as well as preliminary data from some patients in Cohort 3, by the end of this year and will report longer-term safety and clinical data from this trial next year as the program advances.
- Presented interim surgical results from the Phase 1b study of VY-AADC01 in patients with advanced Parkinson's disease on June 22 at the 20th International Congress of Parkinson's Disease and Movement Disorders in Berlin, Germany. The interim surgical data presented at the conference demonstrated the use of real-time, intra-operative MRI-guided delivery to allow the surgical teams to visualize the delivery of VY-AADC01, administer higher infusion volumes, and achieve greater coverage of the putamen, the brain region that the Company is targeting with its gene therapy program. Increased infusion volumes of VY-AADC01 in Cohort 2 resulted in a higher average coverage of the overall putamen compared to Cohort 1 and substantially greater coverage of the putamen than has been achieved in previous gene therapy trials using a similar vector.
- Advanced multiple preclinical programs towards selection of lead clinical candidates by late 2016 or early 2017, with the goal of filing an Investigational New Drug (IND) application for VY-SOD101 for a monogenic form of ALS in the fourth quarter of 2017.
- Announced two new preclinical programs at the Company's R&D day in April; VY-TAU01 and VY-NAV01, which are focused on the molecular targets tau and Nav1.7, respectively. Voyager owns worldwide rights to both programs.
 - VY-TAU01 is an adeno-associated virus 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 of Nav1.7 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 addictive potential associated with many current treatments for severe, chronic pain.

Second Quarter 2016 Financial Results and Guidance

For the second quarter ended June 30, 2016, Voyager reported a GAAP net loss of \$9.3 million, or \$0.37 per share, compared to a GAAP net loss of \$6.7 million, or \$5.94 per share, for the same period in 2015.

Research and development (R&D) expenses increased to \$10.5 million for the second quarter ended June 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 \$2.9 million for the second quarter ended June 30, 2016 compared to \$2.4 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 \$204.0 million as of June 30, 2016. Voyager continues to expect to end 2016 with cash, cash equivalents and marketable securities of \$160.0 million and 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. EDT. 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 60529341. 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 worldwide². 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 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 ago³. 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 dopamine⁴. 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 promoter 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 AAV (adeno-associated virus) gene therapy through innovation and investment in vector engineering and optimization, manufacturing and dosing and delivery techniques. The Company's pipeline is focused on severe CNS diseases in need of effective new therapies, including advanced Parkinson's disease, a monogenic form of amyotrophic lateral sclerosis (ALS), Friedreich's ataxia, Huntington's disease, spinal muscular atrophy (SMA), 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](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 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 the 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)

Statement of Operations Items:	Three Months Ended		Six Months Ended	
	June 30,	June 30,	June 30,	June 30,
	2016	2015	2016	2015
Collaboration revenue	\$ 3,720	\$ 4,884	\$ 8,550	\$ 7,460

Operating expenses:

Research and development	10,484	6,455	19,216	11,978
General and administrative	2,854	2,396	6,419	4,277
Total operating expenses	13,338	8,851	25,635	16,255
Operating loss	(9,618)	(3,967)	(17,085)	(8,795)
Total other income (expense)	283	72	562	(9,677)
Net loss	(9,335)	(3,895)	(16,523)	(18,472)
GAAP charges related to pre-IPO preferred stock	—	(2,851)	—	(4,087)
Net loss attributable to common stockholders	<u>\$ (9,335)</u>	<u>\$ (6,746)</u>	<u>\$ (16,523)</u>	<u>\$ (22,559)</u>

Selected Balance Sheet Items:	June 30, December 31,	
	2016	2015
Cash, cash equivalents and marketable securities	\$ 203,993	\$ 224,345
Total assets	\$ 209,815	\$ 229,457
Accounts payable and accrued expenses	\$ 5,503	\$ 4,042
Deferred revenue	\$ 47,184	\$ 54,982
Total stockholders' equity	\$ 155,704	\$ 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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