Voyager Therapeutics Reports First Quarter 2017 Financial Results and Corporate Highlights

05/09/17

VY-AADC01 interim Phase 1b data recently presented at American Academy of Neurology and American Association of Neurological Surgeons meetings; program on track to report 6-month data from Cohort 3 and longer-term data from Cohorts 1 and 2 during the third quarter of 2017

VY-SOD101 lead clinical candidate selected and progressing towards IND filing; preclinical pipeline programs targeting devastating neurological diseases progressing towards lead clinical candidate selection

CAMBRIDGE, Mass., May 09, 2017 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ:VYGR), a clinical-stage gene therapy company developing life-changing treatments for severe neurological diseases, today reported its first quarter of 2017 financial results and corporate highlights.

"The first quarter of 2017 reflected solid progress across the entire organization with our lead clinical program for advanced Parkinson's disease, our pipeline of preclinical gene therapy programs, our product engine and our manufacturing platform," said Steven Paul, M.D., president and chief executive officer of Voyager Therapeutics. "For the first time, our investigators presented interim Phase 1b results for VY-AADC01 for advanced Parkinson's disease at two major scientific conferences targeting key physician audiences for this program, and we remain on track to provide additional data from this trial during the third quarter. The pipeline continues to advance towards IND filing for VY-SOD101 for the treatment of ALS and towards lead clinical candidate selections for both our Huntington's disease and Friedreich's ataxia programs. All of this progress is based, in part, on our ability to optimally deliver these therapeutics to the brain or spinal cord, as well as to manufacture high quality GMP vectors at scale."

Recent Program Highlights

VY-AADC01 for advanced Parkinson's disease:

As part of an oral presentation at the recent American Academy of Neurology meeting, investigators presented VY-AADC01 motor symptom data from Cohorts 1 and 2 and new functional and quality of life data as measured by the Unified Parkinson's Disease Rating Scale (UPDRS) Part II and Part IV and the patient-reported 39-item Parkinson's Disease Questionnaire (PDQ-39). Along with improving motor symptoms, VY-AADC01 improved patients' function and quality of life, which are key indicators of the potential clinical impact of this one-time treatment for advanced Parkinson's disease. The company remains on track to report data from Cohorts 1-3 during the third quarter of this year. These data as well as data from the posterior (i.e., back of the head) infusion trajectory study will help inform the design of the double-blind, placebo-controlled (sham surgery) trial planned to begin during the fourth quarter of 2017.

At the recent American Association of Neurological Surgeons Annual Scientific Meeting, also during an oral presentation, investigators reviewed the evolution of the real-time MRI-guided surgical procedure with VY-AADC01 that resulted in increased coverage of the putamen, a region of the brain associated with motor function in Parkinson's disease. These developments helped to increase the average coverage of the putamen from 21% in Cohort 1, to 34% in Cohort 2, and 42% in Cohort 3, as previously reported.

Pipeline program highlights:

Voyager continues to 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. During the first quarter of 2017, Voyager selected VY-SOD101 as a clinical candidate for the treatment of ALS caused by mutations in the superoxide dismutase 1 gene (SOD1). With a single intrathecal (IT) injection, VY-SOD101 has the potential to durably reduce the levels of toxic mutant SOD1 protein in the CNS to slow the progression of disease. Preclinical pharmacology and toxicology studies are now underway to support the filing of an investigational new drug (IND) application for VY-SOD101 during late 2017 or early 2018. This timing update reflects the time required to complete the preclinical toxicology testing and to produce good manufacturing process (GMP) material to support the clinical trial. During the quarter, the company also progressed its preclinical candidates towards selection of lead clinical candidates for VY-HTT01 for Huntington's disease and VY-FXN01 for Friedreich's ataxia.

Upcoming Investor Conference Participation

Voyager's management team members will participate in the following investor conference:

Event: Goldman Sachs Healthcare Conference, Rancho Palos Verdes, CA
When: Wednesday, June 14, 2017, 2:40 p.m. PDT
Presenter: Steven Paul, M.D., president and chief executive officer

Live-streaming webcasts of this presentation 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.

First Quarter 2017 Financial Results and 2017 Guidance

Voyager reported a GAAP net loss of $16.6 million, or $0.65 per share, for the first quarter ended March 31, 2017, compared to a GAAP net loss of $7.2 million, or $0.29 per share, for the same period in 2016.

Collaboration revenues of $1.5 million for the first quarter of 2017 compared to collaboration revenues of $4.8 million for the first quarter of 2016. 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 first quarter of 2017 compared to the same period in 2016 reflects ongoing reassessments of performance periods for individual programs under the collaboration, as well as previously announced deprioritized development of VY-SMN101 for spinal muscular atrophy.

Research and development (R&D) expenses of $14.1 million for the first quarter ended March 31, 2017 compared to $8.7 million for the same period

in 2016. The increase in R&D expenses was largely due to expenditures associated with the development of Voyager's pipeline, and increased personnel and facility costs to support the advancement of the pipeline programs.

General and administrative (G&A) expenses were $4.9 million for the first quarter ended March 31, 2017 compared to $3.6 million for the same period in 2016. 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 March 31, 2017 were $157.7 million. Based on the company's current operating plan, Voyager continues to expect to end 2017 with cash, cash equivalents, and marketable debt securities of approximately $90 million to $100 million and continues to project that its existing cash, cash equivalents, and marketable debt 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 5:30 p.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 18530851. 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 by restoring motor function in patients and improving symptoms following a single administration.

About Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company 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. The company's pipeline focuses on severe neurological diseases in need of effective new therapies, including advanced Parkinson's disease, a monogenic form of ALS, Huntington's disease, Friedreich's ataxia, 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 Annual Report on Form 10-K 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.

[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

Selected Financial Information

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

(Unaudited)

Statement of Operations Items:	Three Months Ended March 31, 2017	2016
Collaboration revenue	$ 1,464	$ 4,830
Operating expenses:		
Research and development	14,072	8,732
General and administrative	4,914	3,565
Total operating expenses	18,986	12,297
Operating loss	(17,522)	(7,467)
Total other income	648	279
Loss before income taxes	(16,874)	(7,188)
Income tax benefit	226	—
Net loss attributable to common stockholders	$ (16,648)	(7,188)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.65)	$ (0.29)
Weighted-average common shares outstanding, basic and diluted	25,791,591	25,076,769

Selected Balance Sheet Items:	March 31, 2017	December 31, 2016
Cash, cash equivalents, and marketable debt securities	$ 157,670	$ 174,418
Total assets	$ 175,090	$ 189,566
Accounts payable and accrued expenses	$ 6,150	$ 7,038
Deferred revenue	$ 40,162	$ 41,582
Total stockholders' equity	$ 122,697	$ 135,922

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