Voyager Therapeutics Reports Second Quarter 2017 Financial Results and Corporate Highlights

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Lead program VY-AADC for advanced Parkinson's disease progressing towards pivotal trials

CAMBRIDGE, Mass., Aug. 08, 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 second quarter of 2017 financial results and corporate highlights.

"The second quarter of 2017 was a very productive period for our company," said Steve Paul, M.D., president and chief executive officer of Voyager Therapeutics. "Our Parkinson's disease program continues to advance as we optimize dose and delivery before initiating our pivotal program later this year. Our preclinical pipeline programs also made solid progress toward entering the clinic. In addition, work with our collaborators to further optimize AAV capsids demonstrated exciting progress, including enhanced gene transfer to the brain and spinal cord in preclinical models compared to the historical standard, AAV9. During the quarter, we also made a number of key hires to strengthen our leadership team and solidify our position as the leading gene therapy company focused on severe neurological diseases."

Recent Program Highlights

VY-AADC for advanced Parkinson's disease:

Successfully administered VY-AADC01 to the first patient in a Phase 1 trial with a posterior surgical delivery approach. This posterior (i.e., back of the head) delivery approach aims to further optimize the surgical delivery of VY-AADC01 for advanced Parkinson's disease. A posterior approach into the putamen, the specific region of the brain targeted by Voyager's gene therapy program, could better align the delivery of VY-AADC01 with the anatomical structure of the putamen to potentially reduce the total procedure time and further increase the total coverage of the putamen. The administration of VY-AADC01 with this posterior approach was well-tolerated and no serious adverse events were reported and, similar to many patients in the Phase 1b trial, this patient was discharged from the hospital one day after surgery. Additional patients completed screening and will enroll shortly. Preliminary total procedure time and putaminal coverage data from this posterior trajectory trial will help inform the design of the pivotal Phase 2-3 program planned to initiate during late 2017.

Pipeline program and platform highlights:

Progressed IND-enabling studies for VY-SOD101. Earlier in the year, Voyager announced the selection of VY-SOD101 as a clinical candidate for the treatment of amyotrophic lateral sclerosis (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 central nervous system (CNS) to slow the progression of disease. Preclinical data in large mammals demonstrated that a single IT administration resulted in robust knock-down of SOD1 in motor neurons. During the second quarter, Voyager progressed pharmacology and toxicology studies to support the filing of an investigational new drug (IND) application for VY-SOD101 during late 2017 or early 2018.

Selected VY-HTT01 as a clinical candidate for the treatment of Huntington's disease. Huntington's disease is a fatal, inherited neurodegenerative disease that results in the progressive decline of motor and cognitive functions caused by an expansion mutation in the huntingtin, or HTT, gene. VY-HTT01 is composed of an adeno-associated virus (AAV) capsid and proprietary transgene that harnesses the RNA interference pathway to selectively silence the production of HTT. Direct delivery of VY-HTT01 to the brain with a one-time administration could potentially slow or halt the progression of this uniformly fatal disease. In preclinical models, a single administration of VY-HTT01 was well-tolerated and resulted in robust and widespread knockdown of HTT messenger RNA in disease-relevant regions of the non-human primate central nervous system. The extent of HTT mRNA suppression (greater than 50%) and high precision and efficiency of primary microRNA processing in these preclinical studies supported the selection of VY-HTT01 as our lead clinical candidate.

Announced the publication of new preclinical data from the California Institute of Technology on a second-generation gene therapy capsid demonstrating further enhanced gene transfer to the brain. 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. Recently, the company announced the publication from the California Institute of Technology (Caltech) in Nature Neuroscience of new preclinical data from ongoing efforts of Dr. Benjamin Deverman, Professor Viviana Gradinaru and the Gradinaru Laboratory at Caltech to develop novel AAV capsids that efficiently cross the blood-brain barrier and widely transduce, or transfer, genes into the CNS after intravenous administration. From these efforts, a new, second-generation AAV capsid provided up to a 100-fold increase in the transduction of the CNS in an adult mouse model over the historical standard, AAV9, as compared with the first-generation capsid reported last year by the Gradinaru group that provided a more than 40-fold improvement over AAV9. Key translational studies in non-human primates are underway by Voyager to evaluate these AAV-variant capsids that have the potential to transform the company's ability to deliver gene therapies to the CNS. Voyager obtained a co-exclusive license to the Caltech novel AAV capsids, intellectual property and related technology in September 2016. The license agreement covers all fields of use and includes novel AAV capsids that have demonstrated enhanced crossing of the blood-brain barrier for the potential treatment of CNS diseases following systemic administration of an AAV gene therapy vector.

Strengthened business operations and clinical development teams:

Hired Allison Dorval as vice president of finance. Ms. Dorval brings to Voyager over 20 years of corporate finance and accounting experience, including nine years of biopharmaceutical executive finance experience, most recently at Juniper Pharmaceuticals, Inc., and Insulet Corporation, where she led financial planning, reporting and accounting including processes for Sarbanes-Oxley compliance. Allison is a certified public accountant and began her career in public accounting at PricewaterhouseCoopers, LLP where she held positions of increasing responsibility and scope. Ms. Dorval obtained a B.S. in Business Administration (Accounting concentration) from the University of Vermont.

Hired Robert Blood as vice president of legal affairs. Mr. Blood provides counsel across a wide range of legal areas including business development and strategic collaborations, commercial transactions, corporate governance, intellectual property, compliance and matters related to the securities and exchange commission. Rob joins Voyager from AMAG Pharmaceuticals, Inc., where he was vice president of legal affairs, deputy general counsel & chief compliance officer providing legal counsel to support organizational growth while managing risk in compliance with securities, healthcare and privacy laws. Previously, Rob was associate general counsel for EMD Serono and his large firm experience includes Goodwin Procter LLP, and Montgomery, McCracken, Walker & Rhodes, LLP. Rob obtained his J.D. from Northeastern University School of Law, and a B.S. in Foreign Affairs from Georgetown University.

Hired Steven Hersch, M.D., Ph.D., as senior director in clinical development. Dr. Hersch is a leading HD researcher, was a founding member and co-chair of the Huntington Study Group, the leading academic research consortium for HD, and led the development of the Huntington's Disease Society of America's Center of Excellence Program. He is also a professor of neurology at Harvard Medical School and director of the Laboratory of Neurodegeneration and Neurotherapeutics at Massachusetts General Hospital. His work has encompassed the anatomy and pharmacology of the cortex and striatum, pathophysiology of HD, preclinical target identification and therapeutic proof-of-concept studies in transgenic mouse models, biomarker development, natural history and neuroimaging studies of HD patients, clinical measure development, and the conduct of early and late phase clinical trials. Dr. Hersch has over 125 publications to his name and earned Ph.D. and M.D. degrees from Boston University School of Medicine, an M.A. in Medicine from Harvard Medical School, and a B.S. in Interdisciplinary Neuroscience from The American University.

Second Quarter 2017 Financial Results and 2017 Guidance

Voyager reported a GAAP net loss of $18.9 million, or $0.73 per share, for the second quarter ended June 30, 2017, compared to a GAAP net loss of $9.3 million, or $0.37 per share, for the same period in 2016.

Collaboration revenues of $1.2 million for the second quarter of 2017 compared to $3.7 million for the second quarter of 2016. Collaboration revenues reflect recognition of payments for research and development services provided to Voyager for various programs under the Sanofi-Genzyme collaboration agreement. Collaboration revenues, which are subject to variability based on quarterly assessments of expected or anticipated efforts under the collaboration, decreased during the second quarter of 2017 from the prior year period primarily due to ongoing reviews of programs under the collaboration.

Research and development (R&D) expenses of $15.3 million for the second quarter ended June 30, 2017 compared to $10.5 million for the same period in 2016. The increase in R&D expenses was primarily 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 of $4.5 million for the second quarter ended June 30, 2017 compared to $2.9 million for the same period in 2016. The increase in G&A expenses primarily related to personnel costs to support Voyager's growth and facility costs.

Total cash, cash equivalents, and marketable debt securities as of June 30, 2017 were $141.3 million. Based on the company's current operating plan, Voyager continues to expect to end 2017 with total 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 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 62803624. 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-AADC

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 (the putamen) 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-AADC, 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-AADC, 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 Amyotrophic Lateral Sclerosis

Amyotropic Lateral Sclerosis is a rare, rapidly progressive, fatal disease characterized by the degeneration of nerve cells in the spinal cord and brain resulting in severe muscle atrophy with loss of the ability to walk and speak, and premature death. The median survival is approximately three years, and 90 percent of people with ALS die within five years of symptom onset.[7] ALS affects approximately 20,000 people in the U.S., with less than 10,000 new cases identified each year reflecting a high rate of mortality and short survival, relative to other diseases with similar incidences.[8]

Patients with ALS typically develop weakness in one body region (upper or lower limb or bulbar) and then develop symptoms and signs of progressive dysfunction of motor neurons. The majority of ALS cases occur sporadically and with unknown cause, but in approximately 10 percent of patients, the cause is familial and can be linked to an identifiable genetic defect. An estimated 20 percent of familial cases can be attributed to mutations in SOD1, the first mutant gene discovered to be causal for the development of ALS, through a toxic gain of function mechanism leading to motor neuron pathogenesis.[9] Riluzole is the only drug approved by the U.S. Food and Drug Administration for the treatment of ALS. In controlled trials, Riluzole delayed the time to onset of tracheostomy or death by approximately two to three months but did not improve muscle strength or neurological function.

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 laws. 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 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.

Selected Financial Information

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

(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
Statement of Operations Items:	**2017**	**2016**	**2017**	**2016**
Collaboration revenue	$ 1,177	$ 3,720	$ 2,642	$ 8,550
Operating expenses:				
Research and development	15,300	10,484	29,372	19,216
General and administrative	4,516	2,854	9,430	6,419
Total operating expenses	19,816	13,338	38,802	25,635
Operating loss	(18,639)	(9,618)	(36,160)	(17,085)
Total other income (expense)	(42)	283	606	562
Loss before income taxes	(18,681)	(9,335)	(35,554)	(16,523)
Income tax provision	(195)	—	31	—
Net loss	$ (18,876)	$ (9,335)	$ (35,523)	$ (16,523)
Net loss per share, basic and diluted	$ (0.73)	$ (0.37)	$ (1.37)	$ (0.66)

Weighted-average common shares outstanding, basic and diluted	25,946,333	25,228,405	25,869,390	25,152,587

Selected Balance Sheet Items:	June 30, 2017	December 31, 2016
Cash, cash equivalents, and marketable debt securities	$ 141,323	$ 174,418
Total assets	$ 158,414	$ 189,566
Accounts payable and accrued expenses	$ 7,621	$ 7,038
Deferred revenue	$ 39,053	$ 41,582
Total stockholders' equity	$ 105,291	$ 135,922

[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

[7] Sorenson EJ, et al. (2002) Neurology 59:280-282

[8] www.alsa.org

[9] Rosen D, et al. (1993) Nature 362:59-62

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