Voyager Therapeutics Reports Third Quarter 2017 Financial Results and Corporate Highlights

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VY-AADC for advanced Parkinson's disease on track for start of global, pivotal phase 2-3 program

Integration of novel capsid and delivery optimization efforts expected to result in two IND filings from the ALS, Huntington's disease and Friedreich's ataxia programs now planned for 2019

Voyager further strengthens manufacturing and program teams with key leadership hires

CAMBRIDGE, Mass., Nov. 02, 2017 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ:VYGR), a clinical-stage gene therapy company focused on developing life-changing treatments for severe neurological diseases, today reported its third quarter of 2017 financial results and corporate highlights and will host a conference call and webcast today at 4:30 p.m. EDT to discuss these results.

"During the third quarter, we delivered strong progress with our Parkinson's disease program including gaining worldwide rights, we continued to develop our pipeline programs, and we further progressed our novel capsid discovery efforts," said Steve Paul, M.D., president and chief executive officer of Voyager Therapeutics. "Our global, pivotal program for VY-AADC remains on track, and we are excited to bring this important program one step closer to patients. In addition, we are encouraged by our recent data with our novel capsids and optimized dosing paradigms. Our innovative work in these areas has the potential to yield best-in-class programs and our updated timeline on our preclinical programs reflects a strong emphasis on optimizing capsid and vector delivery and moving only highly-targeted and differentiated candidates into the clinic."

Recent Clinical and Preclinical Program Highlights and Updates

VY-AADC for advanced Parkinson's disease:

During the third quarter, Voyager provided updated results to the ongoing Phase 1b trial with VY-AADC for advanced Parkinson's disease and recently presented the results at the European Society of Gene and Cell Therapy (ESGCT) on October 18, 2017, in Berlin, Germany, and at the Michael J. Fox Foundation meeting on October 30, 2017 in New York City. The results demonstrated durable, dose-dependent and time-dependent improvements across multiple measures of patients' motor function after a one-time administration of VY-AADC, and with meaningfully lower doses of oral levodopa. These measures include patient-reported diaries, Parkinson's disease rating scales, and activities of daily living.

During the third quarter, investigators dosed a fourth patient in a separate Phase 1 trial designed to further optimize the intracranial delivery of VY-AADC. This ongoing Phase 1 trial explores a posterior, or back of the head, delivery approach, compared to Cohorts 1 through 3 from the ongoing Phase 1b trial that used a transfrontal, or top of the head, delivery approach into the putamen. A posterior approach better aligns the infusion of VY-AADC with the anatomical structure of the putamen to potentially reduce the total procedure time and increase the total coverage of the putamen.

Administration of VY-AADC with this posterior approach was well-tolerated in all four patients dosed since the start of the trial. No serious adverse events were reported, and all patients were discharged from the hospital the day after surgery. The posterior approach resulted in greater average putaminal coverage (approximately 50%) and reduced average surgical times by two to three hours compared with the transfrontal approach of Cohorts 1 through 3. Voyager continues to expect to enroll additional patients in this trial prior to the start of the pivotal Phase 2-3 program.

Voyager recently gained worldwide development and commercial rights to VY-AADC for the treatment of advanced Parkinson's disease. Voyager remains on track to begin the global, pivotal Phase 2-3 program for VY-AADC and dose the first patient during the second quarter of 2018. Voyager will continue to follow patients from Cohorts 1 through 3, and those in the posterior trajectory trial, and plans to report updated results from these trials during the first quarter of 2018, prior to the start of patient enrollment in the pivotal program.

Integration of novel capsid and delivery optimization efforts now resulting in two investigational new drug (IND) applications expected to be filed from the ALS, Huntington's disease and Friedreich's ataxia programs in 2019

VY-SOD101 for ALS (SOD1). Earlier in the year, Voyager selected 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, we believe 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 third quarter of 2017, Voyager conducted preclinical studies with additional routes of administration in large animal models, including studies with its novel AAV capsids yielding markedly improved bio-distribution and pharmacology with the potential for substantially improved efficacy. Voyager now plans to further investigate these novel capsids and additional routes of administration before filing an IND application, previously planned for late 2017 or early 2018. These studies, together with the ongoing efforts described below on the Huntington's disease and Friedreich's ataxia programs, are expected to support two IND filings from these programs during 2019.

VY-HTT101 for Huntington's disease. Voyager previously selected VY-HTT01 as a clinical candidate for the treatment of Huntington's disease. VY-HTT01 is composed of an AAV1 capsid and proprietary transgene that harnesses the RNAi pathway to selectively knock down the production of HTT mRNA. Direct delivery of VY-HTT01 to the brain with a one-time administration could potentially slow or halt the progression of Huntington's disease. Lead selection data presented at this year's ESGCT conference showed that a single intraparenchymal infusion of VY-HTT01 into the non-human primate putamen (a disease-relevant region of the brain) resulted in 54% suppression of HTT mRNA. The extent of HTT mRNA suppression, as well as the high precision and efficiency of primary microRNA processing and tolerability in the non-human primate supported the selection of VY-HTT01 as the lead clinical candidate. Preclinical pharmacology and toxicology studies are underway with VY-HTT01 to support the expected filing of an IND.

VY-FXN01 for Friedreich's ataxia (FA). Data presented at the International Ataxia Research Conference in September in Pisa, Italy in a transgenic mouse model of FA demonstrated that a one-time intravenous (IV) post-symptomatic dosing of an AAV vector composed of a novel AAV capsid and a frataxin transgene, together with intracerebral dosing also delivering a frataxin transgene, led to a rapid halting and reduction of FA disease progression in multiple tests including three functional tests of motor behavior and one electrophysiological test. In addition, increasing IV vector doses with the same novel capsid together with a fixed dose of the intracerebral vector led to a dose-dependent rescue of the FA phenotype. Additional preclinical studies are underway at Voyager including steps to identify a lead clinical candidate for the treatment of FA during 2018.

Voyager strengthens leadership of manufacturing and Friedreich's ataxia program teams

Hired Luis Maranga, Ph.D., as Chief Technical Operations Officer. Dr. Maranga joins Voyager with over 20 years of biotechnology industry experience, focused on bioprocess development, chemistry, manufacturing and controls (CMC), good manufacturing process (GMP), process validation and regulatory submissions, and facilities management including extensive work with the baculovirus/Sf9 expression system. Dr. Maranga most recently served as Vice President, Biologics Development at Bristol-Myers Squibb Company (BMS), where he was responsible for all process and analytical development, and clinical manufacturing from post-discovery to commercial launch for BMS's biologics programs, and where he also held previously the roles of General Manager, Devens, MA, Biologics Commercial Operations, Site Head for Hopkinton, MA site, and Executive Director, Biologics Manufacturing Sciences and Technology. Prior to BMS, Luis served as Head, Technical Development, Viral Vaccines at Novartis, and held senior R&D roles for biologics and vaccines at AstraZeneca/MedImmune, and Merck & Co. Dr. Maranga is an expert on biologics, vaccines, and gene therapy CMC development and manufacturing. Dr. Maranga holds a Ph.D., in Chemical Engineering from Universidade Nova de Lisboa, and a B.S. in Chemical Engineering from the Instituto Superior Tecnico both in Portugal.

Hired Massimo Pandolfo, M.D., as Medical Lead for Friedreich's ataxia program. Dr. Pandolfo is a pioneer in the field of neurogenetics and on Friedreich's ataxia, having led the team that identified the Friedreich ataxia gene in 1996 and has since been at the forefront of basic, translational and clinical research on Friedreich's ataxia. Dr. Pandolfo has published more than 200 peer-reviewed articles in the field of neurogenetics and of clinical and translational neuroscience. He is the coordinator of European Friedreich's Ataxia Consortium for Translational Studies (EFACTS), a board member of the European Ataxia Study Group (ASG) and a member of National Ataxia Foundation (U.S.) scientific advisory board. In addition to his seminal work on Friedreich's ataxia, Dr. Pandolfo has contributed to the clinical and genetic study of other inherited ataxias, of the genetics and pharmacogenetics of epilepsy, of several rare inherited neurological diseases, and of the genetic predisposition to stroke. He is Fellow of the American Academy of Neurology (AAN), member of the AAN Science Committee, and co-chair of the European Academy of Neurology Scientific Panel on Translational Neurology.

Dr. Pandolfo is Professor of Neurology and Director of the Laboratory of Experimental Neurology at the Université Libre de Bruxelles (ULB) in Brussels, Belgium and Chief of Neurology at the ULB-Erasme University Hospital in Brussels. Dr. Pandolfo obtained his medical degree from the University of Milan, Italy, where he also completed his residency in Neurology.

Voyager Therapeutics Upcoming R&D Day

Voyager's management team members will review its program highlights at the upcoming R&D day:

When: Thursday, November 16, 2017, 2:00 to 5:00 p.m. EST
Where: New York City

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.

Third Quarter 2017 Financial Results and 2017 Guidance

Voyager reported a GAAP net loss of $23.3 million, or $0.89 per share, for the third quarter ended September 30, 2017, compared to a GAAP net loss of $9.0 million, or $0.35 per share, for the same period in 2016.

Collaboration revenue of $1.1 million for the third quarter of 2017 compared to $3.3 million for the third quarter of 2016. Collaboration revenue reflects recognition of payments for research and development services provided by Voyager for various programs under the Sanofi-Genzyme collaboration agreement. Collaboration revenue, which is subject to variability based on quarterly assessments of expected or anticipated efforts under the collaboration, decreased during the third quarter of 2017 from the prior year period primarily due to a change in the estimated period of performance for reaching proof of principle for certain programs under the collaboration agreement. As a result of Sanofi Genzyme's decision not to exercise its option to the Parkinson's program, Voyager expects to recognize the portion of the agreement consideration allocated to the license option, in addition to the ongoing amounts related to research and development, as collaboration revenue in the fourth quarter of 2017.

Research and development (R&D) expenses of $19.6 million for the third quarter ended September 30, 2017 compared to $10.3 million for the same period in 2016. The increase in R&D expenses related primarily 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 $4.9 million for the third quarter ended September 30, 2017 compared to $3.4 million for the same period in 2016. The increase in G&A expenses was primarily related to personnel costs to support Voyager's growth, patent fees and facility costs.

Total cash, cash equivalents, and marketable debt securities as of September 30, 2017 were $125.6 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 4:30 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 7099088. 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 ten 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 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 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 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 dopamine4. The intrinsic neurons in the putamen, however, do not degenerate in Parkinson's disease5,6. 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 Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company focused on 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.

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 initiate, enroll and complete, clinical trials, the potential clinical utility of its product candidates, its ability to continue to develop its product engine, its ability to add new programs to its pipeline, its 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. Such risks and uncertainties include, among others, those related to the initiation and conduct of preclinical studies and clinical trials, the availability of data from clinical trials and the expectations for regulatory submissions and approvals; the continued development of the product engine; Voyager's scientific approach and general development progress; the availability or commercial potential of Voyager's product candidates; the sufficiency of cash resources; and need for additional financing. 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 September 30,		Nine Months Ended September 30,	
Statement of Operations Items:	**2017**	**2016**	**2017**	**2016**
Collaboration revenue	$ 1,148	$ 3,308	$ 3,790	$ 11,858
Operating expenses:				
Research and development	19,561	10,309	48,933	29,526
General and administrative	4,942	3,370	14,372	9,789
Total operating expenses	24,503	13,679	63,305	39,315
Operating loss	(23,355)	(10,371)	(59,515)	(27,457)
Total other income	9	1,072	615	1,634
Loss before income taxes	(23,346)	(9,299)	(58,900)	(25,823)
Income tax benefit	—	303	31	303
Net loss	$ (23,346)	$ (8,996)	$ (58,869)	$ (25,520)
Net loss per share, basic and diluted	$ (0.89)	$ (0.35)	$ (2.27)	$ (1.01)
Weighted-average common shares outstanding, basic and diluted	26,164,527	25,374,381	25,968,849	25,227,058

Selected Balance Sheet Items:	September 30, 2017	December 31, 2016
Cash, cash equivalents, and marketable debt securities	$ 125,587	$ 174,418
Total assets	$ 140,915	$ 189,566
Accounts payable and accrued expenses	$ 11,404	$ 7,038
Deferred revenue	$ 37,905	$ 41,582
Total stockholders' equity	$ 85,206	$ 135,922

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[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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