



Voyager Therapeutics Provides First Quarter 2016 Investor Update

May 12, 2016

Enrollment Completed in 2nd Cohort of Ongoing Phase 1b Clinical Trial of VY-AADC01 in Advanced Parkinson's Disease

VY-AADC01 Interim Phase 1b Results to be Presented at 20th International Congress of Parkinson's Disease & Movement Disorders in June 2016

CAMBRIDGE, Mass., May 12, 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 first quarter 2016 financial results and provided an update on its advanced Parkinson's disease program, VY-AADC01. The Company also highlighted recent pipeline and corporate achievements, as well as expected upcoming milestones.

"Voyager is off to a great start in 2016. Today, we announced the completion of enrollment of the second patient cohort in the ongoing Phase 1b trial of VY-AADC01. We will present interim results, specifically surgical coverage data for the putamen from cohorts 1 and 2, as well as safety data, at a major medical conference next month. Importantly, we remain on track to deliver top-line, proof-of-concept clinical results for VY-AADC01 in the fourth quarter of this year," said Steven Paul, M.D., president and chief executive officer of Voyager Therapeutics. "In addition, we recently hosted our first R&D Day, where we reviewed our lead programs in advanced Parkinson's disease and ALS, and unveiled two new programs, VY-TAU01 for Alzheimer's disease and frontotemporal dementia, and VY-NAV01 for severe, chronic pain. We continue to evaluate additional opportunities to maximize the value of our robust AAV product engine."

VY-AADC01 Updates

- Voyager completed enrollment of the second cohort (five patients) in the ongoing Phase 1b dose-ranging safety clinical trial of VY-AADC01 in patients with advanced Parkinson's disease. The first patient was dosed at the second clinical trial site at the University of Pittsburgh, Pennsylvania. The initial clinical trial site at the University of California, San Francisco, California, continues to enroll patients.
- In June 2016, the Company will present surgical coverage and safety data from the first 10 patients (cohorts 1 and 2) enrolled in this trial at the 20th International Congress of Parkinson's Disease & Movement Disorders being held in Berlin, Germany June 19-23, 2016.
- Voyager remains on track to report top-line, human proof-of-concept data in the fourth quarter of 2016. These results will include six-month follow up clinical efficacy, biomarker and safety data from cohorts 1 and 2.

Recent Pipeline & Corporate Highlights

- In April 2016, Voyager announced two new preclinical pipeline programs, VY-TAU01 and VY-NAV01, which are focused on the molecular targets tau and Nav1.7, respectively.
 - Voyager is developing an AAV vectorized version of an anti-tau monoclonal antibody, VY-TAU01, 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 is targeting the knockdown of Nav1.7 in the sensory neurons of the spinal cord 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.
- Voyager remains on track to file an Investigational New Drug (IND) application in late 2017 for its next most advanced pipeline program, VY-SOD101 for a monogenic form of ALS.
- Voyager expanded its leadership team with the appointment of Jim Olesen, Ph.D., as vice president of intellectual property and legal affairs, and Matt Osborne as head of investor relations and corporate communications.

Upcoming Events and Presentations

- Piper Jaffray GenomeRx Symposium, New York City, May 17th
- UBS Global Healthcare Conference, New York City, May 23rd
- Jefferies Healthcare Conference, New York City, June 7th

- 20th International Congress of Parkinson's Disease & Movement Disorders, Berlin, Germany, June 22nd

First Quarter 2016 Financial Results and Guidance

For the first quarter ended March 31, 2016, Voyager reported a net loss of \$7.2 million, or \$0.29 per share, compared to a net loss of \$15.8 million, or \$15.79 per share, for the same period in 2015.

Research and development (R&D) expenses were \$8.7 million for the first quarter ended March 31, 2016, compared to \$5.5 million for the same period in 2015. The increase was largely due to expenditures in advancing development of Voyager's pipeline and product engine, including increased R&D personnel costs associated with the growth of the Company.

General and administrative (G&A) expenses were \$3.6 million for the first quarter ended March 31, 2016, compared to \$1.9 million for the same period in 2015. The increase was largely due to expenditures in G&A personnel associated with the growth of the Company and expenses related to operating as a public company.

Cash, cash equivalents and marketable securities as of March 31, 2016 were approximately \$214.0 million, compared to \$224.0 million on December 31, 2015.

Based on the Company's current operating plan, Voyager continues to expect to end 2016 with cash, cash equivalents and marketable securities of approximately \$160.0 million and projects that its existing cash, cash equivalents and marketable securities will be sufficient to fund operating expenses and capital expenditure requirements into 2019.

Conference Call

Voyager will host a conference call and webcast today at 8:30 a.m. ET. 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 98309172. 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 Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company developing life-changing treatments for severe diseases of the central nervous system. 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](#).

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 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 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.

Selected Financial Information

(amounts in thousands)

(Unaudited)

Statement of Operations Items:	Three Months Ended March 31,	
	2016	2015
Collaboration revenue	\$ 4,830	\$ 2,576
Operating expenses:		
Research and development	8,732	5,523
General and administrative	3,565	1,881
Total operating expenses	12,297	7,404

Operating loss	(7,467)	(4,828)
Total other income (expense)	279	(9,749)
Net loss	<u>(7,188)</u>	<u>(14,577)</u>
GAAP charges related to pre-IPO preferred stock	-	(1,236)
Net loss attributable to common stockholders	<u>\$ (7,188)</u>	<u>\$ (15,813)</u>

Selected Balance Sheet Items:	March 31,	December 31,
	2016	2015
Cash, cash equivalents and marketable securities	\$ 213,981	\$ 224,345
Total assets	\$ 219,980	\$ 229,457
Accounts payable and accrued expenses	\$ 4,358	\$ 4,042
Deferred revenue	\$ 50,633	\$ 54,982
Total stockholders' equity	\$ 163,597	\$ 169,074

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