



## Voyager Therapeutics Provides Fourth Quarter and Full Year 2015 Investor Update

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*Top-line Clinical Proof-of-Concept Data for VY-AADC01 for Advanced Parkinson's Disease On Track for Release in the Fourth Quarter of 2016*

*Strong Cash Position Projected to Support Operations into 2019*

CAMBRIDGE, Mass., March 17, 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 2015 fourth quarter and full year financial results, and also provided corporate highlights and upcoming milestones.

"2015 was certainly a year of execution on all fronts and placed Voyager in an excellent position heading into 2016," said Steven Paul, M.D., president and chief executive officer of Voyager Therapeutics. "We are poised to continue to execute across our product pipeline and make meaningful development advances as we translate the promise of gene therapy into innovative therapies for patients suffering from severe CNS disorders. Most notably, we continue to be on track to deliver top-line proof-of-concept clinical results for our Parkinson's disease program, VY-AADC01, before the end of the year. We also anticipate making significant progress toward the clinic for our key preclinical programs."

### 2015 Key Achievements and Corporate Highlights

- Reported encouraging interim clinical results in October 2015 from the ongoing Phase 1b trial of VY-AADC01 in advanced Parkinson's disease. Also in October, the investigational new drug application (IND) for VY-AADC01 was successfully transferred from the University of California, San Francisco to Voyager.
- Announced a transformative strategic collaboration with Sanofi Genzyme in February 2015. Under the agreement, Voyager received a \$100.0 million upfront commitment, including \$65.0 million in upfront cash, a \$30.0 million upfront equity investment and an in-kind commitment of \$5.0 million, and is eligible to receive total future potential option and milestone payments of \$745.0 million. Per the collaboration agreement, Voyager retained U.S. rights to its VY-AADC01, VY-FXN01 and VY-HTT01 product programs. In addition, Voyager retained worldwide rights to VY-SOD101 for a monogenic form of amyotrophic lateral sclerosis (ALS), which is outside the scope of the collaboration agreement.
- Continued to strengthen the overall organization, including key additions to the Voyager leadership team and board of directors. In the middle of 2015, the company appointed three industry and scientific leaders to its board of directors: Perry Karsen, former chief executive officer of the Celgene Cellular Therapeutics division of Celgene Corporation; Michael Higgins, former chief operating officer and chief financial officer of Ironwood Pharmaceuticals, Inc. and entrepreneur-in-residence at Polaris Partners; and Steven Hyman, M.D., director of the Stanley Center for Psychiatric Research at the Broad Institute of Harvard and MIT.
- Closed multiple additional financings to support advancement of Voyager's product pipeline and engine, including a \$60 million Series B financing in April 2015 and a \$73 million (net proceeds) initial public offering in November 2015.
- Ended 2015 in a strong financial position with approximately \$224 million in cash, cash equivalents and marketable securities.

### Product Pipeline Update and Upcoming Milestones

- Enrollment in the Phase 1b clinical trial of VY-AADC01 in patients with advanced Parkinson's disease remains on track and Voyager expects to report top-line human proof-of-concept data in the fourth quarter of 2016. These results will include six-month follow up data from the first 10 patients (cohorts 1 and 2) enrolled in this trial.
- As planned, a second clinical trial site for VY-AADC01 has been opened and is actively enrolling patients at the University of Pittsburgh in Pittsburgh, Pa. The initial clinical trial site at the University of California, San Francisco also continues to enroll patients.
- GMP production activities for VY-AADC01 were initiated in early 2016 in collaboration with MassBiologics of the University of Massachusetts Medical School, utilizing their new manufacturing facility in Fall River, Mass. MassBiologics is a U.S. Food and Drug Administration (FDA) licensed manufacturer of vaccines and biologics, including viral vectors and monoclonal antibodies.
- Voyager's four preclinical programs, VY-SOD101 for a monogenic form of ALS, VY-FXN01 for Friedreich's ataxia, VY-HTT01 for Huntington's disease and VY-SMN101 for spinal muscular atrophy, all continued to advance, with Voyager's next IND submission targeted in 2017.

### Upcoming Events & Presentations

- Alliance for Regenerative Medicine's 4<sup>th</sup> Annual Cell & Gene Therapy Investor Day, New York City, March 22<sup>nd</sup>
- Voyager R&D Day, New York City, April 29<sup>th</sup>

### Financial Results and Guidance

For the fourth quarter ended December 31, 2015, Voyager reported a net loss of \$8.8 million, or \$0.67 per share, compared to a net loss of \$5.2 million, or \$6.58 per share, for the same period in 2014. Net loss was \$38.3 million, or \$9.14 per share, for the year ended December 31, 2015, compared to a net loss of \$17.7 million, or \$27.83 per share, for the same period in 2014.

Research and development (R&D) expenses were \$9.2 million for the fourth quarter ended December 31, 2015, compared to \$3.0 million for the same period in 2014. 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. R&D expenses were \$27.7 million for the year ended December 31, 2015, compared to \$8.9 million for the same period in 2014.

General and administrative (G&A) expenses were \$3.2 million for the fourth quarter ended December 31, 2015, compared to \$1.5 million for the same period in 2014. The increase was largely due to expenditures in G&A personnel associated with the growth of the company, including expenses related to operating as a public company. G&A expenses were \$9.9 million for the year ended December 31, 2015, compared to \$5.5 million for the same period in 2014.

Cash, cash equivalents and marketable securities as of December 31, 2015 were approximately \$224 million, compared to \$161 million on September 30, 2015. Cash, cash equivalents and marketable securities as of December 31, 2015 included total net proceeds of approximately \$73 million from the company's initial public offering of common stock and the underwriters' exercise of their over-allotment option in November 2015.

Based on the company's current operating plan, Voyager expects to end 2016 with cash, cash equivalents and marketable securities of approximately \$160 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.

## 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 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.

## 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) 489-6517 for domestic callers or +1 (929) 387-3945 for international callers, and referencing conference ID number 51816666. 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](http://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 and spinal muscular atrophy (SMA). 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](http://www.voyagertherapeutics.com). Follow Voyager on [LinkedIn](#).

## Selected Financial Information

(amounts in thousands)

(Unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
Statement of Operations Items:	2015	2014	2015	2014
Collaboration revenue	\$ 4,937	\$ —	\$ 17,334	\$ —
Operating expenses:				
Research and development	9,220	2,960	27,679	8,898
General and administrative	3,157	1,536	9,909	5,469
Total operating expenses	12,377	4,496	37,588	14,367
Operating loss	(7,440)	(4,496)	(20,254)	(14,367)
Total other income (expense), net	157	(194)	(9,418)	(1,950)
Net loss	(7,283)	(4,690)	(29,672)	(16,317)
GAAP charges related to pre-IPO preferred stock	(1,534)	(507)	(8,618)	(1,366)

Net loss attributable to common stockholders	<u>\$ (8,817)</u>	<u>\$ (5,197)</u>	<u>\$(38,290)</u>	<u>\$(17,683)</u>
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**Selected Balance Sheet Items:**

	<b>December 31,</b>	
	<b>2015</b>	<b>2014</b>
Cash, cash equivalents and marketable securities	\$ 224,345	\$ 7,035
Total assets	\$ 229,457	\$ 11,497
Accounts payable and accrued expenses	\$ 4,042	\$ 2,196
Deferred revenue	\$ 54,982	\$ -
Redeemable convertible preferred stock	\$ -	\$ 21,979
Total stockholders' equity (deficit)	\$ 169,074	\$(20,830)

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