Voyager Therapeutics Announces Second Quarter 2018 Financial Results and Corporate Highlights

August 7, 2018

Clinical data updates and regulatory feedback for VY-AADC during the quarter support planned pivotal program for Parkinson's disease

Pipeline programs progressing with updated preclinical data from ALS and Huntington's disease programs expected during the fourth quarter of this year

Conference call scheduled for today at 4:30 p.m. EDT

CAMBRIDGE, Mass., Aug. 07, 2018 (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 second quarter 2018 results, recent progress and corporate updates and will host a conference call and webcast today at 4:30 p.m. EDT to discuss these results.

"The significant progress made during the second quarter with our lead program VY-AADC for Parkinson's disease and our pipeline programs positions Voyager to potentially achieve important milestones for the remainder of this year and into 2019," said Andre Turenne, president and chief executive officer of Voyager Therapeutics. "These include enrolling our pivotal program for VY-AADC for Parkinson's disease and providing longer-term results from the Phase 1 trials as well as presenting preclinical data on our programs targeting ALS SOD-1 and Huntington's disease. In addition, we continue to advance our collaboration with AbbVie targeting vectorized monoclonal antibodies directed against tau for the treatment of Alzheimer's disease and other neurodegenerative diseases."

Recent Clinical and Preclinical Program Highlights

VY-AADC for Parkinson's disease:

- VY-AADC granted Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA) for Parkinson's disease in patients with motor fluctuations that are refractory to medical management. RMAT designation is an expedited program for the advancement and approval of regenerative medicine products, including gene therapy products. The designation includes all the benefits of the FDA's Fast Track and Breakthrough Therapy designation programs, with the ability for early interactions with the agency to discuss any surrogate or intermediate endpoints to support the potential acceleration of approval and satisfy post-approval requirements. RMAT designation for VY-AADC was granted based on clinical data from the ongoing Phase 1b trial with VY-AADC in patients with Parkinson's disease.

- Based on written feedback from a Type C meeting with the FDA, Voyager announced plans to submit for review a biologics license application (BLA) for VY-AADC based on the nonclinical and clinical safety and efficacy data from the pivotal program, including plans to submit a BLA based on data from the randomized, placebo-controlled Phase 2 trial alone, or if needed, from the randomized, placebo-controlled Phase 3 trial.

- Achieved positive interim results from a Phase 1 posterior trajectory trial that achieved enhanced coverage of the putamen, reduced surgical times, and improvements in patients' motor function at six months that were consistent with improvements achieved from patients in Cohorts 2 and 3 at the same time point in Voyager's Phase 1b trial with VY-AADC. The posterior approach will serve as the preferred surgical route of administration for the pivotal program. Voyager plans to present data from the full cohort of eight patients from the Phase 1 posterior trajectory trial at future scientific and medical conferences.

- Pivotal program progressing with twenty-four clinical trial sites (including neurosurgical and neurology patient referral sites) selected for participation in the Phase 2 randomized, placebo-controlled trial with institutional review board submission and site activation underway. During the remainder of the year, Voyager plans to provide updates on the enrollment status of this trial.

Preclinical programs for ALS SOD-1, Huntington's disease, Friedreich's ataxia and tau for Alzheimer's and other neurodegenerative diseases:

- During the second quarter, Voyager continued to advance its multiple preclinical programs towards clinical trials through further vector optimization and exploration of optimal routes of administration. At the American Society of Gene and Cell Therapy (ASGCT) May 16-19, 2018, in Chicago, Ill., Voyager presented results for VY-SOD101, which targets the

superoxide dismutase 1 gene (SOD1), the first mutant gene discovered to be causal for the development of amyotrophic lateral sclerosis (ALS). The results demonstrated that a one-time administration of VY-SOD101 lowered SOD1 mRNA levels by 78% in the spinal cord motor neurons of non-human primates. Additional new data at this year's ASGCT meeting included tolerability data in non-human primates for VY-HTT01 for Huntington's disease, along with previous data with VY-HTT01 that demonstrated a 54% suppression of huntingtin (HTT) mRNA in the non-human primate putamen after a single administration. Efforts to further optimize delivery for ALS and Huntington's disease programs are underway, and Voyager plans to provide results from these efforts at scientific conferences during the fourth quarter of this year.

Management Updates

Voyager recently announced the appointment of Andre Turenne as president and chief executive officer of Voyager and member of the Voyager Board of Directors. Mr. Turenne joins Voyager with extensive strategic business development and commercial leadership experience, including nearly 12 years at Genzyme and Sanofi where he most recently served as Sanofi's senior vice president, global head, business development and licensing responsible for partnering activities across all of Sanofi's business units.

Voyager today announced the resignation of Bernard Ravina, M.D., M.S., Voyager's chief medical officer, as he pursues a new career opportunity at a private biotechnology company. Voyager has initiated a retained search for this role. Dr. Ravina will continue to serve as a Clinical Advisor to Voyager. Steven M. Paul, M.D., Voyager's former president and chief executive officer, will continue to serve on Voyager's Board of Directors and as a member of Voyager's Science & Technology Committee and as a Senior Advisor to Voyager.

"Since joining Voyager over four and a half years ago, Bernard played an integral role in advancing the Parkinson's disease program and in leading our medical activities," said Andre Turenne, president and chief executive officer of Voyager Therapeutics. "With VY-AADC entering a robust pivotal program based on the positive Phase 1 clinical trial data and receiving RMAT designation and the positive Type C meeting feedback from the FDA, Bernard's departure occurs during a natural transition point, and we are highly confident in the strong medical team that he has assembled to continue to execute on our priorities. We wish Bernard success in his next endeavor and are very pleased that he will continue to contribute to Voyager as a Clinical Advisor."

Second Quarter 2018 Financial Results

Voyager reported a GAAP net loss of $25.5 million, or $0.80 per share, for the second quarter ended June 30, 2018, compared to a GAAP net loss of $18.9 million, or $0.73 per share, for the same period in 2017.

Collaboration revenues of $2.6 million for the second quarter of 2018 compared to $1.2 million for the second quarter of 2017. Collaboration revenues reflect recognition of payment for research and development services provided by Voyager for various programs under the collaboration agreements with Sanofi Genzyme and AbbVie. The increase in collaboration revenues for the second quarter of 2018 compared to the same period in 2017 primarily reflects the recognition of revenue related to research services performed under the AbbVie collaboration agreement that was announced in February 2018. These amounts were offset primarily by a reduction in revenue recognized under the Sanofi Genzyme collaboration resulting from the adoption of certain accounting rules related to revenue recognition.

Research and development (R&D) expenses of $16.5 million for the second quarter ended June 30, 2018 compared to $15.3 million for the same period in 2017. The increase in R&D expenses related primarily to expenditures associated with increased personnel, facility and external costs to support the advancement of VY-AADC into the randomized, placebo-controlled Phase 2 trial, as well as expenditures related to support Voyager's preclinical pipeline programs.

General and administrative (G&A) expenses of $11.8 million for the second quarter 2018 compared to $4.5 million for the same period in 2017. The increase in G&A expenses was primarily due to personnel costs including an increase in non-cash stock-based compensation expenses and higher professional fees.

Cash, cash equivalents, and marketable debt securities as of June 30, 2018 were $197.0 million. Based on the Company's current operating plan, Voyager continues to expect to end 2018 with total cash, cash equivalents and marketable debt securities of approximately $125 million to $135 million and projects that its existing cash, cash equivalents and marketable debt securities will be sufficient to fund operating expenses and capital expenditure requirements into early 2020.

Conference Call Information

Voyager will host a conference call and webcast today at 4:30 p.m. ET. 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 8498509. 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 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. Voyager's pipeline focuses on severe neurological diseases in need of effective new therapies, including Parkinson's disease, a monogenic form of ALS called SOD1, Huntington's disease, Friedreich's ataxia, neurodegenerative diseases related to defective or excess aggregation of tau protein in the brain including Alzheimer's disease and severe, chronic pain. Voyager has broad strategic collaborations with Sanofi Genzyme, the specialty care global business unit of Sanofi, AbbVie, 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.

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," "would," "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 gene therapy platform, its ability to develop manufacturing capability for its products and successfully transition its manufacturing process, its ability to perform under existing collaborations with, among others, Sanofi Genzyme and AbbVie and to add new programs to its pipeline, its ability to enter into new partnerships or collaborations, the sufficiency of its cash resources 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, the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory submissions and approvals; the continued development of the gene therapy platform; Voyager's scientific approach and general development progress; and the availability or commercial potential of Voyager's product candidates. 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:	**2018**	**2017**	**2018**	**2017**
Collaboration revenue	$ 2,575	$ 1,177	$ 3,517	$ 2,642
Operating expenses:				
Research and development	16,507	15,300	31,360	29,372
General and administrative	11,762	4,516	18,945	9,430
Total operating expenses	28,269	19,816	50,305	38,802
Operating loss	(25,694)	(18,639)	(46,788)	(36,160)
Total other income (expense)	153	(42)	1,140	606
Loss before income taxes	(25,541)	(18,681)	(45,648)	(35,554)
Income tax (provision) benefit	—	(195)	180	31
Net loss	$ (25,541)	$ (18,876)	$ (45,468)	$ (35,523)
Net loss per share, basic and diluted	$ (0.80)	$ (0.73)	$ (1.43)	$ (1.37)
Weighted-average common shares outstanding, basic and diluted	31,976,922	25,946,333	31,868,995	25,869,390

Selected Balance Sheet Items	**June 30, 2018**	**December 31, 2017**
Cash, cash equivalents, and marketable debt securities	$ 196,983	$ 169,052
Total assets	$ 214,663	$ 184,477
Accounts payable and accrued expenses	$ 9,255	$ 12,517
Deferred revenue	$ 117,148	$ 31,560
Total stockholders' equity	$ 81,972	$ 134,051

Investor Relations:
Matt Osborne
Vice President of Investor Relations & Corporate Communications
857-259-5353
mosborne@vygr.com

Media:

Josie Butler
W2O/Pure Communications, Inc.

910-239-5099
jbutler@w2ogroup.com

Voyager Therapeutics, Inc.