Voyager Therapeutics Announces First Quarter 2020 Financial Results and Corporate Updates

05/06/20

Announced 14 presentations at upcoming American Society of Gene and Cell Therapy (ASGCT) Virtual Annual Meeting highlighting Huntington's disease, ALS SOD1, vectorized antibodies, novel capsids, and manufacturing

Formed new Scientific Advisory Board with industry-leading expertise

Ended Q1 2020 with over $250M of cash and equivalents with expected runway into mid-2022

CAMBRIDGE, Mass., May 06, 2020 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ: VYGR) today reported its first quarter 2020 financial results, program progress and corporate updates.

"Our goal is to develop life-changing therapies for people living with severe neurological disease, and despite the ongoing COVID-19 health crisis, we ended the first quarter of 2020 in a strong position and expect to provide important progress updates across our programs over the course of 2020," said Andre Turenne, President and Chief Executive Officer of Voyager. "As we advance our pioneering work for both rare and common neurological diseases, we are privileged to have assembled a distinguished group of advisors, comprised of some of the world's leaders in genetic medicine and neuroscience. Moving forward, their scientific expertise will be invaluable in supporting our efforts for patients in need."

Recent Corporate Highlights and Program Outlook

VY-AADC (NBIb-1817) for Parkinson's Disease

- The protocol of the RESTORE-1 clinical trial of VY-AADC (NBIb-1817) for Parkinson's disease is being amended to make the previously announced protocol modifications. Patient screening is expected to resume once trial sites are able to accept study participants in the context of COVID-19.
- Voyager and Neurocrine Biosciences continue preparations for the initiation of the RESTORE-2 registrational study in Parkinson's disease planned for the second half of 2020.
- Voyager and Neurocrine Biosciences expect to report final three-year data on all three cohorts (15 total patients) of the PD-1101 Phase 1b trial, as well as two-year data from the PD-1102 Phase 1 posterior trajectory trial (8 total patients), in the second half of 2020.
- Results from an intravenous (IV) levodopa sub-study from the PD-1101 trial were recently published in *Movement Disorders,* in an article titled "Aromatic L-Amino Acid Decarboxylase Gene Therapy Enhances Levodopa Response in Parkinson's Disease." In the sub-study of 13 patients from the trial, VY-AADC (NBIb-1817) administration improved the magnitude, speed of onset, and duration of Unified Parkinson's Disease Rating Scale (UPDRS) motor responses to low and high dose of IV levodopa, across all cohorts.

VY-HTT01 for Huntington's Disease

- Voyager is currently engaged in the ongoing conduct and review of preclinical studies for its Huntington's disease program, VY-HTT01. Pending this review, Voyager is planning for the potential initiation of both a first-in-human Phase 1 study of VY-HTT01 and a prospective observational study of patients with late prodromal and early manifest Huntington's disease. Voyager anticipates providing an update on the program in mid-2020.
- Voyager plans to present updated VY-HTT01 preclinical data at the upcoming ASGCT 23rd Annual Meeting taking place virtually on May 12-15, 2020.

Early Pipeline and Platform

- Voyager continues to advance its earlier-stage research efforts, including wholly-owned and partnered programs with AbbVie and Neurocrine Biosciences. These initiatives include Friedreich's ataxia, SOD1 ALS, vectorized antibodies, novel AAV capsids, and new discovery activities on novel targets.
- Voyager plans to present multiple progress updates on these efforts as well as its manufacturing platform at the upcoming ASGCT Annual Meeting.

Formation of New Scientific Advisory Board

- Voyager recently formed a new Scientific Advisory Board (SAB) of industry leading experts in neuroscience and genetic medicine, including: chairperson **Guangping Gao, Ph.D.**, a co-founder of Voyager and Co-Director of the Li Weibo Institute for Rare Diseases Research, and Director of the Horae Gene Therapy Center and Viral Vector Core, University of Massachusetts Medical School (UMMS); **Beverly Davidson, Ph.D.**, Professor of Pathology and Laboratory Medicine and Professor of Genetics, University of Pennsylvania Perelman School of Medicine, and Director of the Raymond G. Perelman

Center for Cellular and Molecular Therapeutics and Chief Scientific Strategy Officer at Children's Hospital of Philadelphia; **David Liu, Ph.D.**, the Richard Merkin Professor and Vice-Chair of the Faculty at the Broad Institute of Harvard and MIT, and Professor of Chemistry and Chemical Biology at Harvard University; **Dinah Sah, Ph.D.**, Voyager's former Chief Scientific Officer; and **Phillip Zamore, Ph.D.**, a co-founder of Voyager, the Gretchen Stone Cook Professor of Biomedical Sciences and Chair of the RNA Therapeutics Institute, UMMS.

Expansion of Technical Operations and Manufacturing Facilities

- Voyager has entered into a lease agreement to expand its operations with a 32,000 square foot facility located in Lexington, MA. The Company expects the new state-of-the-art facility currently under construction to be ready for occupancy in late 2020. The facility includes both office and lab space and will house the Company's growing manufacturing capabilities to support the current and future pipeline programs.

Anticipated Upcoming Milestones

VY-AADC (NBIb-1817) for Parkinson's Disease:

- Report 3-year results from PD-1101 trial (2H 2020)
- Report 2-year results from PD-1102 trial (2H 2020)
- Initiate RESTORE-2 registration trial (2H 2020)

VY-HTT01 for Huntington's Disease:

- Provide update on program and clinical plans (mid-2020)
- Present additional results from preclinical studies (2H 2020)

Early Pipeline and Platform:

- Provide progress updates on Friedreich's ataxia program, new discovery programs, as well as vectorized antibody and novel capsid efforts (2020)

First Quarter 2020 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of $18.1 million for the first quarter of 2020, compared to collaboration revenue of $5.2 million for the same period of 2019. The increase in collaboration revenue was largely due to efforts related to the Neurocrine Biosciences and AbbVie alpha-synuclein collaborations, which were entered into in the first quarter of 2019.
- **Net Loss:** Net loss was $24.3 million for the first quarter of 2020, compared to a net loss of $27.2 million for the same period of 2019.
- **R&D Expenses:** Research and development expenses were $32.3 million for the first quarter of 2020, compared to $24.8 million for the same period in 2019. The increase in R&D expenses was primarily related to employee-related, external and facility costs to support Voyager's clinical and preclinical pipeline programs, including the VY-AADC (NBIb-1817) and VY-HTT01 programs.
- **G&A Expenses:** General and administrative expenses were $10.2 million for the first quarter of 2020, compared to $9.7 million for the same period in 2019. The increase in G&A expenses was primarily related to employee costs to support the advancement of Voyager's pipeline programs and operations, partially offset by a reduction in legal and other administrative costs.
- **Cash Position:** Cash, cash equivalents and marketable debt securities as of March 31, 2020 were $250.9 million.

Financial Guidance

- Based on the Company's current operating plan, Voyager anticipates cash, cash equivalents and marketable debt securities will be between $150 million and $170 million at the end of 2020.
- Voyager expects that its cash, cash equivalents and marketable debt securities, as well as amounts expected to be received for reimbursement of development costs from Neurocrine Biosciences, will be sufficient to meet Voyager's projected operating expenses and capital expenditure requirements into mid-2022.

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 wholly-owned and partnered pipeline focuses on severe neurological diseases for which effective new therapies are needed, including Parkinson's disease, Huntington's disease, a monogenic form of ALS called SOD1, Friedreich's ataxia, Alzheimer's disease, and other neurodegenerative diseases related to defective or excess aggregation of tau and alpha-synuclein proteins in the brain. Voyager has strategic collaborations with AbbVie and Neurocrine Biosciences. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager is headquartered in Cambridge, Massachusetts. For more information, please visit www.voyagertherapeutics.com or follow @VoyagerTx on Twitter and 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," "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 ability of Voyager to maintain a high level of business critical activity and maintain a level of scientific leadership during the COVID-19 health crisis, the continued operation and effect of the Scientific Advisory Board on Voyager's research and development activities, the completion of construction projects to expand Voyager's facilities and to establish operations in the new facilities, the ability to maintain a Scientific Advisory Board, and to retain as members of the Scientific Advisory Board individuals of recognized scientific prominence, the initiation, timing, progress, activities, goals and reporting of results of its preclinical programs and clinical trials and its research and development programs, the potential benefits, timing and future operation of the collaboration agreements with AbbVie and Neurocrine Biosciences, its ability to identify and attract parties to participate in research and development collaborations, 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 perform under existing collaborations including those with AbbVie and Neurocrine Biosciences, its ability to add new programs to its pipeline, the regulatory pathway of, and the timing or likelihood of its regulatory filings and approvals for, any of its product candidates, its ability to operate its research and development activities efficiently and effectively, the utility and value of Voyager's patent portfolio, and Voyager's anticipated financial results, including Voyager's available cash, cash equivalents and marketable debt securities, the receipt by Voyager of revenues or reimbursement payments from collaboration partners, Voyager's operating expenses, and Voyager's ability to fund its operating expenses with its current cash, cash equivalents and marketable debt securities through a stated time period are forward looking. All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager believes such forward-looking statements 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 severity and length of the COVID-19 health crisis, the imposition of governmental controls and guidance addressing the COVID health crisis, and the financial and human resources available to Voyager to manage the COVID-19 health crisis; the inability of Voyager to create research and development programs combining sufficient levels of scientific interest and applied expertise to be attractive in recruiting and maintaining renown scientists to serve as members of a Scientific Advisory Board; the initiation and conduct of preclinical studies and clinical trials; the availability of data from preclinical studies and clinical trials, and the ability to effectively present such data by means of conference proceedings conducted virtually in response to the COVID-19 health crisis; the expectations for regulatory communications, submissions and approvals; the continued development of the gene therapy platform; Voyager's scientific approach and general development progress; the ability to attract and retain talented contractors and employees; the ability to create and protect intellectual property; the sufficiency of cash resources; the possibility or the timing of the exercise of development, commercialization, license and other options under collaborations; 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 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. All information in the press release is as of the date of this press release, and any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise this information or any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

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Selected Financial Information
($-amounts in thousands, except per share data)
(Unaudited)

	Three Months Ended March 31,	
Statement of Operations Items:	**2020**	**2019**
Collaboration revenue	$ 18,067	$ 5,197
Operating expenses:		
Research and development	32,294	24,831
General and administrative	10,206	9,659
Total operating expenses	42,500	34,490
Operating loss	(24,433)	(29,293)
Total other income	170	2,123
Net loss	$ (24,263)	$ (27,170)
Net loss per share, basic and diluted	$ (0.66)	$ (0.81)
Weighted-average common shares outstanding, basic and diluted	36,963,255	33,353,061

	March 31,	December 31,
Selected Balance Sheet Items	**2020**	**2019**
Cash, cash equivalents, and marketable debt securities	$ 250,931	$ 281,533
Total assets	$ 322,610	$ 354,760

Accounts payable and accrued expenses	$	22,308	$	25,586
Deferred revenue	$	186,150	$	194,493
Total stockholders' equity	$	79,757	$	99,512

Source: Voyager Therapeutics, Inc.