



Voyager Therapeutics Announces Second Quarter 2020 Financial Results and Corporate Updates

August 10, 2020

Plans to present three-year Phase 1b results of VY-AADC (NB1b-1817) in patients with Parkinson's disease at the Movement Disorder Society (MDS) Virtual Congress 2020

Expects screening and enrollment in RESTORE-1 trial of VY-AADC (NB1b-1817) to resume during 2H 2020

Intends to submit Investigational New Drug (IND) application of VY-HTT01 for Huntington's disease during 2H 2020

CAMBRIDGE, Mass., Aug. 10, 2020 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ: VYGR) today reported its second quarter 2020 financial results, program progress and corporate updates.

"We anticipate making important progress across our lead programs in the coming months, including presenting long-term clinical results from our Parkinson's disease gene therapy program, resuming patient screening and enrollment in our RESTORE-1 trial for Parkinson's patients, and moving our wholly-owned gene therapy for Huntington's disease toward the clinic with an IND filing," said Andre Turenne, President and CEO of Voyager. "We're also excited to continue momentum on our next generation capsids, which we believe have the potential to substantially enhance and expand the therapeutic potential of AAV gene therapy to treat severe neurological diseases."

Corporate Highlights and Program Outlook

VY-AADC (NB1b-1817) in Parkinson's Disease

- Voyager and Neurocrine Biosciences plan to present the final three-year data on all three cohorts of the PD-1101 Phase 1b trial, as well as two-year data from the PD-1102 Phase 1 trial, at the MDS Virtual Congress 2020 being held in September. In total, these data will represent more than 60 years of patient experience following VY-AADC treatment and will include a range of patient-reported, physician-assessed and objective endpoints.
- Voyager and Neurocrine Biosciences expect patient screening and enrollment to resume at clinical sites in the RESTORE-1 Phase 2 clinical trial during the second half of 2020. Trial sites had temporarily suspended patient screening and enrollment activity in response to the COVID-19 pandemic and the implementation of protocol amendments.
- Voyager and Neurocrine Biosciences plan to initiate the RESTORE-2 Phase 3 global registrational clinical study of VY-AADC (NB1b-1817) in Parkinson's disease during the first half of 2021.
- Voyager and collaborators recently published a review of the evolution of novel techniques to overcome the challenges of surgical delivery of gene therapies to target brain regions, in an article titled "Data-Driven Evolution of Neurosurgical Gene Therapy Delivery in Parkinson's Disease" in the *Journal of Neurology, Neurosurgery and Psychiatry*.

VY-HTT01 in Huntington's Disease

- Huntington's disease is a fatal genetic disease affecting approximately 40,000 people in the U.S. that results in the progressive decline of motor and cognitive functions. There are currently no approved treatments targeting the underlying cause of the disease.
- Voyager is developing VY-HTT01 as a one-time AAV-based gene therapy treatment designed to knock down expression of the *HTT* gene. Voyager's approach is focused on delivering VY-HTT01 directly into the brain and targeting a reduction of the levels of mutated protein in the striatum and cortex to potentially slow the progression of both motor and cognitive symptoms.
- Voyager recently completed IND-enabling preclinical studies and is finalizing an IND application for VY-HTT01 in Huntington's disease, which it expects to file with the U.S. Food and Drug Administration (FDA) in the second half of 2020.
- In non-human primate studies, one-time administration of VY-HTT01 resulted in robust and durable knockdown of *HTT* mRNA and protein, with knockdown stabilization between 6 and 12 months, and widespread distribution of VY-HTT01 vector genome across the striatum and cortex.
- VY-HTT01 treatment demonstrated robust knockdown of *HTT* mRNA and protein in the YAC128 and BACHD transgenic mouse models of Huntington's disease, with significant improvements in motor function. The Company plans to present preclinical data from the IND-enabling studies at a future scientific congress in the first half of 2021.
- Following clearance of the IND by the FDA, the Company expects to begin the first-in-human clinical trial of VY-HTT01 in

Huntington's disease patients.

- Voyager and collaborators recently published an article titled "Clinical Outcomes and Selection Criteria for Prodromal Huntington's Disease Trials" in *Movement Disorders*. The publication analyzed the use of the normalized prognostic index (PIN) score to improve selection of pre-diagnosis Huntington's disease patients for future clinical trials and identified outcome measures that show robust longitudinal change for measuring treatment efficacy and reducing trial size.

Novel AAV Capsid Discovery Program

- Voyager continues to progress its efforts in the discovery and engineering of novel AAV capsids with the potential to overcome the limitations of existing capsids, including greater blood-brain barrier (BBB) penetrance following IV administration. Voyager is leveraging its proprietary TRACER™ platform to facilitate the selection of AAV capsids with significantly improved BBB crossing and cell-specific transduction properties for therapeutic applications.
- Voyager has presented *in vivo* proof-of-concept data for capsids identified using its TRACER platform. Voyager is currently engaged in non-human primate studies to further characterize and select novel AAV capsids with improved properties and potential for therapeutic applications.

Corporate Updates

- Voyager recently appointed Maria Lopez-Bresnahan, MD, MBA, FAAN, as Senior Vice President, Translational Medicine and Clinical Development. Dr. Lopez-Bresnahan brings more than two decades of industry experience in neurological drug development, including prior clinical leadership roles at Alkermes plc, Vertex Pharmaceuticals, and Pfizer Inc.
- Following the recent conclusion of the tau and alpha-synuclein vectorized antibody collaborations with AbbVie, Voyager has regained full rights to the vectorization technology and certain novel vectorized antibodies developed as part of the collaborations.

Anticipated Upcoming Milestones

VY-AADC (NB1b-1817) for Parkinson's Disease:

- Report 3-year results from PD-1101 trial (September 2020)
- Report 2-year results from PD-1102 trial (September 2020)
- Resume patient screening and enrollment in RESTORE-1 Phase 2 clinical trial (2H 2020)
- Initiate RESTORE-2 Phase 3 registrational clinical trial (1H 2021)

VY-HTT01 for Huntington's Disease:

- IND application and Phase 1 trial initiation (update post-FDA clearance)
- Present IND-enabling preclinical results at a scientific congress (1H 2021)

Early Pipeline and Platform:

- Provide progress updates on earlier-stage research programs, including novel capsid discovery efforts

Second Quarter 2020 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$28.7 million for the second quarter of 2020, compared to collaboration revenue of \$46.1 million for the same period of 2019. The decrease in collaboration revenue primarily reflects the termination of the Sanofi-Genzyme Collaboration in June 2019, which resulted in the recognition of \$28.7 million of previously deferred amounts. The reduction in revenue from the termination of the Sanofi Genzyme Collaboration was offset by an increase in revenue related to research services and cost reimbursements from the collaborations with Neurocrine and AbbVie in the second quarter of 2020 compared to the same period of 2019.
- **Net Loss:** Net loss was \$8.7 million for the second quarter of 2020, compared to net income of \$11.2 million for the same period of 2019.
- **R&D Expenses:** Research and development expenses were \$29.4 million for the second quarter of 2020, compared to \$28.6 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.
- **G&A Expenses:** General and administrative expenses were \$8.2 million for the second quarter of 2020, compared to \$8.3 million for the same period in 2019.
- **Cash Position:** Cash, cash equivalents and marketable debt securities as of June 30, 2020 were \$229.7 million.

Financial Guidance

- Based on the Company's current operating plan, Voyager continues to anticipate 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, Friedreich's ataxia, and other severe neurological diseases. For more information, please visit www.voyagertherapeutics.com or follow [@VoyagerTx](https://twitter.com/VoyagerTx) on Twitter and [Linkedin](https://www.linkedin.com/company/voyager-therapeutics).

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 presenting long-term medical data for patients treated in Voyager's Parkinson's disease clinical trials in September, 2020; resuming patient screening in Voyager's RESTORE-1 Phase 2 clinical trial prior to year-end and initiating the RESTORE-2 Phase 3 clinical trial during the first half of 2021; submitting an IND filing to the FDA for VY-HTT01 before year-end; presenting preclinical data from Voyager's VY-H TT01 preclinical studies, and obtaining clearance by the FDA of Voyager's IND filing for VY-HTT01 and initiating a Phase 1b clinical trial thereafter, continuing to progress its novel capsids discovery program, including the conclusion of non-human primate studies to identify novel AAV capsids; meeting selected development milestones for Voyager's programs for Parkinson's disease, Huntington's disease and technology platform research; maintaining a high level of business critical activity during the COVID-19 health crisis; 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 agreement with Neurocrine Biosciences, identifying and attracting parties to participate in research and development collaborations; advancing AAV-based gene therapies into, and successfully initiating, enrolling and completing, clinical trials; the potential clinical utility of its product candidates, continuing to develop its gene therapy platform; adding 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; 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 initiation and conduct of preclinical studies and clinical trials; the availability of data from preclinical studies and clinical trials; the sufficiency of preclinical and clinical data to support applications for additional studies and marketing approval of its drug development candidates; 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 decisions of regulatory authorities, including the FDA's review of Voyager's IND application for VY-HTT01 and other drug development activities; the changing priorities of collaboration partners; 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 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 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 10K, Voyager's Quarterly Reports on Form 10-Q and other reports filed by Voyager with the Securities and Exchange Commission, as may be 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)

Statement of Operations Items:	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2020	2019	2020	2019

Collaboration revenue	\$ 28,681	\$ 46,087	\$ 46,748	\$ 51,284
Operating expenses:				
Research and development	29,423	28,576	61,718	53,407
General and administrative	8,239	8,322	18,444	17,981
Total operating expenses	<u>37,662</u>	<u>36,898</u>	<u>80,162</u>	<u>71,388</u>
Operating (loss) income	(8,981)	9,189	(33,414)	(20,104)
Total other income	300	1,964	470	4,087
Net (loss) income	<u>\$ (8,681)</u>	<u>\$ 11,153</u>	<u>\$ (32,944)</u>	<u>\$ (16,017)</u>
Net (loss) income per share, basic	<u>\$ (0.23)</u>	<u>\$ 0.30</u>	<u>\$ (0.89)</u>	<u>\$ (0.46)</u>
Net (loss) income per share, diluted	<u>\$ (0.23)</u>	<u>\$ 0.29</u>	<u>\$ (0.89)</u>	<u>\$ (0.46)</u>
Weighted-average common shares outstanding, basic	<u>37,029,524</u>	<u>36,610,918</u>	<u>36,996,390</u>	<u>34,990,989</u>
Weighted-average common shares outstanding, diluted	<u>37,029,524</u>	<u>37,941,257</u>	<u>36,996,390</u>	<u>34,990,989</u>

	<u>June 30,</u>	<u>December 31,</u>
Selected Balance Sheet Items	2020	2019
Cash, cash equivalents, and marketable debt securities	\$ 229,670	\$ 281,533
Total assets	\$ 297,455	\$ 354,760
Accounts payable and accrued expenses	\$ 20,199	\$ 25,586
Deferred revenue	\$ 166,767	\$ 194,493
Total stockholders' equity	\$ 76,884	\$ 99,512

Source: Voyager Therapeutics, Inc.