



Voyager Therapeutics Provides Update on NB1b-1817 (VY-AADC) Gene Therapy Program

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CAMBRIDGE, Mass., Dec. 22, 2020 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR) today announced that the U.S. Food and Drug Administration (FDA) has notified Neurocrine Biosciences (Nasdaq: NBIX) that it has placed a clinical hold on the RESTORE-1 clinical trial of NB1b-1817 (VY-AADC). As previously announced, trial sites participating in RESTORE-1 had not been screening, enrolling or dosing patients as a result of the COVID-19 pandemic and more recently, as a result of the independent Data Safety Monitoring Board (DSMB)'s request to pause dosing pending its review of additional data. The DSMB has requested additional patient level data from the trial and now plans to review these data in early 2021. The clinical hold follows the submission by Neurocrine Biosciences of an IND Safety Report related to the observation of MRI abnormalities in some RESTORE-1 study participants. The clinical implications of this observation are currently unknown and are being evaluated.

RESTORE-1 is a Phase 2 clinical trial of NB1b-1817 (VY-AADC), an intracerebral AAV-based investigational gene therapy, in development for the treatment of Parkinson's disease. The RESTORE-1 DSMB has been informed of the clinical hold, as have the study investigators and central and local ethics committees. Neurocrine Biosciences and Voyager will work closely with the FDA and the DSMB to determine the next steps for the RESTORE-1 clinical trial.

About Parkinson's Disease and NB1b-1817 (VY-AADC)

Parkinson's disease is a chronic, progressive, and debilitating neurodegenerative disease that affects approximately one million people in the U.S. and ten million people worldwide. It is characterized by a loss of dopamine and neuronal degeneration with a concomitant loss of the aromatic L-amino acid decarboxylase (AADC) enzyme required to synthesize dopamine in the brain, leading to associated impairment in motor, neuropsychiatric, and autonomic functions. Dopamine is a chemical "messenger" that is produced in the brain and is involved in the control of movement. It is made when AADC converts the chemical levodopa to dopamine. As Parkinson's disease progresses, there is less AADC enzyme in parts of the brain where levodopa is converted to dopamine.

NB1b-1817 (VY-AADC) is an investigational recombinant adeno-associated viral (AAV) serotype 2 vector encoding the gene for human AADC that is designed to help produce the AADC enzyme in brain cells where it can convert levodopa to dopamine. NB1b-1817 (VY-AADC) is administered into the brain using intraoperative monitoring with magnetic resonance imaging (MRI)-facilitated targeted delivery.

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 on Voyager Therapeutics, please visit the company's website at www.voyagertherapeutics.com or follow [@VoyagerTx](https://twitter.com/VoyagerTx) on Twitter and [LinkedIn](https://www.linkedin.com/company/voyager-therapeutics).

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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 the ability of Neurocrine and Voyager to gather additional information to further characterize the safety profile of NB1b-1817 (VY-AADC) and to work with the FDA to determine the next steps for the RESTORE-1 clinical trial.

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 ability of Neurocrine and Voyager to complete their evaluation and to meet the information requests of, and to resolve questions raised by, the FDA required to bring an end to the clinical hold on the RESTORE-1 clinical trial. 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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