# Neurocrine Biosciences and Voyager Therapeutics Form Strategic Development and Commercialization Collaboration for Parkinson's Disease and Friedreich's Ataxia

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*Collaboration leverages strengths and efforts of both companies towards developing and commercializing life-changing treatments for severe neurological diseases*

*Neurocrine Biosciences gains development and commercialization rights to gene therapy programs VY-AADC for Parkinson's disease, VY-FXN01 for Friedreich's ataxia and two additional programs to be determined*

*Voyager receives $165 million upfront, along with funding for ongoing development of each program, and up to $1.7 billion in potential development, regulatory and commercial milestone payments*

SAN DIEGO and CAMBRIDGE, Mass., Jan. 29, 2019 (GLOBE NEWSWIRE) -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) and Voyager Therapeutics, Inc. (NASDAQ: VYGR), today announced the formation of a strategic collaboration focused on the development and commercialization of Voyager's gene therapy programs, VY-AADC for Parkinson's disease and VY-FXN01 for Friedreich's ataxia, as well as rights to two programs to be determined. This collaboration combines Neurocrine Biosciences' expertise in neuroscience, drug development and commercialization with Voyager's innovative gene therapy programs targeting severe neurological diseases.

Neurocrine Biosciences and Voyager will each host a company conference call and webcast to discuss the collaboration. Full webcast details are provided below.

"We are excited to collaborate with Voyager to advance our shared mission to discover and develop medicines that can benefit the lives of people with serious neurological disorders," said Kevin Gorman, Ph.D., chief executive officer of Neurocrine Biosciences. "The partnership with Voyager allows us to expand our clinical development pipeline addressing neurological disorders, leverage Voyager's expertise in CNS-focused gene therapy, and develop potential treatments for diseases, such as Parkinson's disease and Friedreich's ataxia, which have significant unmet clinical needs."

"Neurocrine Biosciences is an ideal partner with its proven expertise developing and commercializing treatments for movement disorders and other neurological diseases," said Andre Turenne, president and chief executive officer of Voyager Therapeutics. "This is a transformational collaboration for Voyager that greatly enhances our efforts towards becoming the leading, fully-integrated gene therapy company focused on severe neurological diseases while allowing us to continue to invest in our additional pipeline programs and platform. We are tremendously excited to collaborate with the talented and dedicated team at Neurocrine Biosciences to further advance these programs."

**Collaboration Details and Financial Terms**

Under the terms of the agreement, Neurocrine Biosciences has agreed to pay Voyager $165 million in cash including a $115 million upfront payment and a $50 million equity investment at a Voyager per share price of $11.96. Voyager will also receive funding from Neurocrine Biosciences for all costs incurred on these collaboration programs as described below. In addition, Voyager may be entitled to earn up to $1.7 billion in development, regulatory and commercial milestone payments across the four programs.

Under terms of the agreement for VY-AADC for Parkinson's disease:

- Neurocrine Biosciences has agreed to fund the clinical development of the Phase 2-3 pivotal program for VY-AADC. After the data readout of the Phase 2 RESTORE-1 trial, Voyager has the option to either: (1) co-commercialize VY-AADC with Neurocrine Biosciences in the U.S. under a 50/50 cost- and profit-sharing arrangement and receive milestones and royalties based on ex-U.S. sales, or (2) grant Neurocrine Biosciences full global commercial rights in exchange for milestone payments and royalties based on global sales.

Under terms of the agreement for VY-FXN01 for Friedreich's ataxia:

- Neurocrine Biosciences has agreed to fund the development through the Phase 1 clinical trial of VY-FXN01. After the data readout of the Phase 1 trial, Voyager has the option to either: (1) co-commercialize VY-FXN01 with Neurocrine Biosciences in the U.S. under a 60/40 cost- and profit-sharing arrangement, or (2) grant Neurocrine Biosciences full U.S. commercial rights in exchange for milestone payments and royalties based on U.S. sales. Sanofi Genzyme retains an option for ex-U.S. rights to VY-FXN01 following the data readout of the Phase 1 trial.

Under terms of the agreement for the two programs to be determined:

- Neurocrine Biosciences has agreed to fund the development of these programs to be determined and Voyager will have the right to earn milestone payments and royalties based on global sales.

The effectiveness of the collaboration agreement and the closing of the sale and issuance of Voyager common stock described above are subject to certain conditions including the expiration or termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and other customary closing conditions.

Centerview Partners served as Neurocrine Biosciences' financial advisor and Cooley LLPserved as Neurocrine Biosciences' legal counsel for the collaboration. Wilmer Cutler Pickering Hale and Dorr LLP served as legal counsel for Voyager.

**Conference Call Information**

Neurocrine Biosciences will host a conference call and webcast today at 8:00 a.m. EST. The live call may be accessed by dialing (866) 342-8588 (U.S.) or (203) 518-9865 (International) using the conference ID: NBIX.

Voyager will host a conference call and webcast today at 8:45 a.m. EST. The live call may be accessed by dialing (877) 851-3834 and referencing conference ID number 3181907.

A live audio webcast of the conference calls will be available online in the Investors section on Neurocrine Biosciences' website at www.neurocrine.com, and from the Investors & Media section of Voyager Therapeutics' website at www.voyagertherapeutics.com. A replay of the webcasts will be available on the website approximately one hour after the conclusion of each event and will be archived for approximately one month.

**About Voyager's VY-AADC Gene Therapy for Parkinson's Disease**

VY-AADC is an investigational gene therapy product designed to deliver the *AADC* gene directly into neurons of the putamen where dopamine receptors are located, bypassing the substantia nigra neurons and enabling the neurons of the putamen to produce the AADC enzyme to convert levodopa into dopamine. With this approach, VY-AADC has the potential to durably enhance the conversion of levodopa to dopamine and provide clinically meaningful improvements by restoring motor function in patients and improving symptoms following a single administration.

The FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation for VY-AADC for the treatment of Parkinson's disease in patients with motor fluctuations who are refractory to medical management. RMAT designation is an expedited program for the advancement and approval of regenerative medicine products, including gene therapy products. RMAT designation was granted based on clinical data from the Phase 1b trial with VY-AADC in patients with Parkinson's disease. During this trial, one-time administrations of VY-AADC demonstrated robust and durable improvements in patients' motor function along with substantial reductions in use of daily oral levodopa and other Parkinson's disease medications. Infusions of VY-AADC have been well-tolerated in this trial with no vector-related serious adverse events reported to date.

Voyager recently initiated the Phase 2 RESTORE-1 trial in patients who have been diagnosed with Parkinson's disease for at least four years, are not responding adequately to oral medications, and have at least three hours of OFF time during the day as measured by a validated self-reported patient diary.

For additional information regarding Voyager's RESTORE-1 Phase 2 clinical trial with its gene therapy VY-AADC for the treatment of Parkinson's disease, please use the following link or email Voyager at clinicaltrials@vygr.com.

**About Voyager's VY-FXN01 for Friedreich's Ataxia**

Friedreich's ataxia (FA) is a rare, severe, inherited neurological disease caused by mutations in the frataxin (FXN) gene leading to decreased expression of FXN, which results in severe sensory impairment, progressive loss of the ability to walk, generalized weakness, and loss of sensation, as well as severe and potentially fatal cardiomyopathy. The typical age of onset is 10 to 12 years with reduced life expectancy between 35 to 45 years of age due to neurological and cardiac complications. The goal of VY-FXN01 is to restore FXN protein levels, with a one-time treatment, to at least 50% of normal in relevant neurons and cardiac myocytes, to slow the progression of disease.

In a preclinical model of FA disease, Voyager's frataxin gene therapy vector durably improved ataxia and sensory function, and rescued the FA phenotype based on multiple functional tests. In physiological and behavioral assays, Voyager's frataxin gene therapy vector demonstrated dose-dependent and durable responses for more than 10 months after a single administration, preventing central and peripheral disease progression. Additional preclinical studies are underway at Voyager including steps to identify a lead clinical candidate for the treatment of FA during 2019.

**About Neurocrine Biosciences**

Neurocrine Biosciences, a San Diego based biopharmaceutical company, is focused on developing treatments for neurological and endocrine related disorders. The company discovered, developed and markets INGREZZA® (valbenazine) capsules, the first FDA-approved product indicated for the treatment of adults with tardive dyskinesia, an involuntary movement disorder. Discovered and developed through Phase II clinical trials by Neurocrine, ORILISSA®(elagolix), the first FDA-approved oral medication for the management of endometriosis with associated moderate to severe pain in over a decade, is marketed by AbbVie as part of a collaboration to develop and commercialize elagolix for women's health. Neurocrine's clinical development programs include opicapone as an adjunctive therapy to levodopa/DOPA decarboxylase inhibitors in Parkinson's disease patients, elagolix for uterine fibroids with AbbVie, valbenazine for the treatment of Tourette syndrome, and NBI-74788 for the treatment of congenital adrenal hyperplasia (CAH). For more information and the latest updates from Neurocrine Biosciences, please visit www.neurocrine.com.

**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 strategic collaborations with Sanofi Genzyme, AbbVie and Neurocrine Biosciences. 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 on Voyager Therapeutics, please visit the company's website at www.voyagertherapeutics.com.

**Neurocrine Biosciences Forward-Looking Statements**

In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements related to: statements related to the potential benefits to be derived from the Voyager Therapeutics collaboration agreement, including any statements related to Voyager's proprietary CNS-focused gene therapy platform and Neurocrine's ability to leverage such platform; Neurocrine's ability to expand its research and development pipeline, and Neurocrine's ability to develop disease modifying and potentially curative treatments for diseases, including Parkinson's disease and Friedreich's ataxia. Among the factors and risks that could cause actual results to differ materially from those indicated in the forward-looking statements are risks that the benefits of the agreement with Voyager may never be realized; risks that the products licensed from Voyager may not obtain regulatory approval from the FDA or such approval may be delayed or conditioned; risks that development activities related to the products licensed from Voyager may not be completed on time or at all; risks associated with the Company's dependence on Voyager for research, development and manufacturing activities; risks that ongoing or future clinical trials may not be successful or replicate previous clinical trial results, or may not be predictive of real-world results or of results in subsequent clinical trials; risks and uncertainties relating to competitive products and technological changes that may limit demand for for products licensed from Voyager; risks that the products licensed from Voyager may be precluded from commercialization by the proprietary rights of third parties; and other risks that are described in the Company's periodic reports filed with the Securities and Exchange Commission, including without limitation the Company's quarterly report on Form 10-Q for the quarter ended September 30, 2018. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.

**Voyager 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, AbbVie and Neurocrine Biosciences 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 regulatory pathway of, and the timing or likelihood of its regulatory filings and approvals for, any of its product candidates, 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 communications, 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.