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PTC Therapeutics and CHDI Foundation Announce a Collaboration on a Small-Molecule Therapeutic for Huntington's Disease

SOUTH PLAINFIELD, N.J., April 4, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) and the CHDI Foundation, Inc. (CHDI) today announced a research collaboration to advance PTC's Huntington's disease program. The program is optimizing small-molecule compounds—identified using PTC's splicing technology platform—that decrease the production of huntingtin protein. The current compounds have been shown to be orally bioavailable in animals, blood-brain barrier penetrant, and effective in decreasing the amount of huntingtin protein in a mouse model that has the expanded human *huntingtin* transgene.

"We are excited to collaborate with CHDI to advance our small-molecule huntingtin-lowering program for the treatment of Huntington's disease," said Stuart W. Peltz, Ph.D., president and chief executive officer of PTC Therapeutics. "Finding a potential treatment for Huntington's disease fits with our mission to focus on treatments for disorders that have an urgent need for therapeutic options. We are proud that this program utilizes our proprietary splicing platform developed utilizing cutting edge technology to advance the identification of new therapeutics for patients suffering from rare diseases."

"After following PTC's Huntington's disease small-molecule program for several years, we are pleased to start working with the company more directly," said Robi Blumenstein, president of CHDI Management. "It's great that PTC's pre-mRNA splicing expertise has been applied to Huntington's disease with promising results. A pill that lowers the amount of huntingtin protein and treats the underlying cause of the disease holds the promise of improving the quality of life of people with Huntington's disease and their families. We look forward to contributing to and accelerating this program."

The discovery and development of small-molecule huntingtin-expression inhibitors has been enabled by the PTC pre-mRNA splicing platform, a technology that previously has been successfully used to identify the SMN2 splicing modifier, RG7916. PTC, F. Hoffmann-La Roche AG, and the SMA Foundation have a collaboration using RG7916, which is currently in pivotal clinical trials for the treatment of SMA. The splicing technology platform is also being used in a number of other programs, including a program in late-stage chemical optimization, in collaboration with the Massachusetts General Hospital, to identify correctors of IKBKAP splicing to treat familial dysautonomia.

About Huntington's Disease

Huntington's disease (HD) is a rare inherited neurodegenerative disorder caused by a mutation in the *huntingtin* gene. The disorder results in behavioral, cognitive, and motor impairments. These symptoms progressively reduce an individual's quality of life, and ultimately lead to death within 15 to 25 years of overt clinical motoric onset. Each child of a parent with a mutation in the *huntingtin* gene has a 50% chance of inheriting the mutation. It is estimated that around one person in 10,000 carries the mutated huntingtin gene. Current HD therapies manage the severity of the symptoms, but there are currently no therapeutics approved that slow the progression of the disease.

About CHDI

CHDI Foundation, Inc., is a privately funded nonprofit biomedical research organization that is exclusively dedicated to rapidly developing therapies that slow the progression of Huntington's disease. As a collaborative enabler, CHDI seeks to bring the right partners together to identify and address critical scientific issues and move drug candidates to clinical evaluation as quickly as possible. CHDI scientists work closely with a network of more than 700 researchers in academic and industrial laboratories and clinical sites around the world in the pursuit of these novel therapies, providing strategic scientific direction to ensure that our common goals remain in focus. More information about CHDI can be found at www.chdifoundation.org.

About PTC Therapeutics

PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder products and has a global commercial footprint. This success is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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Forward Looking Statements:

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements, other than those of historical fact, contained in this release, are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; timing, results and conduct of PTC's collaboration with CHDI; PTC's alternative splicing program; timing and results of PTC's collaboration with Roche and the SMA Foundation; timing and results of PTC's collaboration with Massachusetts General Hospital; PTC's strategy, future operations, future financial position, future revenues or projected costs; and objectives of management.

Other forward-looking statements may be identified by the words "plan," "guidance," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions. PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including the factors discussed in the "Risk Factors" section of PTC's most recent Annual Report on Form 10-K as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors. The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this release except as required by law.

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