Oral Splicing Modifiers that Systemically Lower Huntington Disease Protein Discovered Through PTC Therapeutics' Innovative Splicing Platform

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-- Results published in Nature Communications --
-- Description of novel splicing mechanism causing inhibition of HTT gene expression --

SOUTH PLAINFIELD, N.J., Dec. 20, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the Nature Communications publication characterizing the novel splicing mechanism induced by compounds identified from the Huntington Disease (HD) program. These splicing modifiers were discovered through PTC's innovative splicing platform and were shown to specifically and selectively lower huntingtin protein through the modulation of pre-messenger RNA splicing.

"The mechanism induced by HD splicing modifiers described in the Nature Communications paper shows an incredibly innovative and novel approach to regulate gene expression to treat diseases," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "The published results show that PTC's splicing platform can be expanded to identify compounds that induce splicing resulting in lower HTT protein levels. This is an important breakthrough that expands the splicing platform's capabilities. While the splicing platform was first used to identify compounds that increase SMN levels to treat SMA patients, this was the first time that splicing modifiers identified caused the inhibition of gene expression, resulting in reduced HTT protein levels. These results show how this important technology can be used to identify compounds that regulate gene expression to potentially treat patients suffering from many different diseases."

HD is a rare, inherited disease that causes the progressive degeneration of nerve cells in the brain, impacting a person's functional abilities. While HD can present at any age, it is most prevalent in people 30 to 50 years old. It affects approximately 45,000 people in the United States. HD is caused by a mutation in the huntingtin gene, which is responsible for creating huntingtin protein (HTT). As time progresses, the mutated huntingtin protein forms clumps in the brain cells, resulting in damaged cells and eventually cell death. There are no treatments for the underlying cause of HD.

About PTC
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to innovate to identify new therapies and can globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. Our mission is to provide access to best-in-class treatments for patients who have little to no treatment options. The company's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. We believe this allows us to maximize value for all our stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Instagram, Facebook, Twitter, and LinkedIn.

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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 contained in this release, other than statements of historic fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data and other matters; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "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 those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; the enrollment, conduct, and results of PTC518 clinical studies for HD; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; and 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.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval, and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory or prove to be commercially successful.

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 press release except as required by law.

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