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Phase 1 Data from Spinal Muscular Atrophy Program to be Presented at the 2015 American Academy of Neurology Annual Meeting

WASHINGTON, April 20, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Phase 1 clinical data from the company's joint development program with Roche and the SMA Foundation in spinal muscular atrophy (SMA) will be presented at the 2015 American Academy of Neurology 67th Annual Meeting. The late-breaking abstract titled "SMN2 splicing modifier RG7800 shows dose-dependent increase of full length SMN2 mRNA in first-in-human study" will be presented as part of the Emerging Science session on April 22, 2015.

As previously disclosed, findings from the Phase 1 study indicated that RG7800, an investigational oral therapy for SMA, showed a favorable safety profile and was well tolerated at all dose levels studied. In addition, proof of mechanism was demonstrated by a dose-dependent effect on Survival Motor Neuron 2 (SMN2) splicing towards the production of full length SMN2 mRNA. The Phase 1 study was a single ascending dose, placebo-controlled, double-blind study in 48 healthy volunteers testing single oral doses from 0.5 to 90 mg.

"The ability to demonstrate proof of mechanism in this Phase 1 study was a major achievement both for our SMA collaboration as well as PTC's alternative splicing platform," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Given its mechanism of action, RG7800 targets the underlying cause of the disorder, and has the potential to restore functional SMN protein levels in the nervous system, muscle, and other tissues throughout the body. We are encouraged by the progress that is being made in the ongoing Phase 2 Moonfish study in SMA patients that was initiated late last year and look forward to the results of this study in 2016."

SMA is a genetic neuromuscular disease caused by a missing or defective SMN1 gene, which results in reduced levels of SMN protein. The homologous SMN2 gene is predominantly spliced to a shortened mRNA, and only produces small amounts of SMN protein. Insufficient levels of SMN protein are responsible for the loss of motor neurons within the spinal cord leading to muscle atrophy and death in infants and toddlers in its most severe form. It is estimated that this devastating disease affects 1 in every 11,000 children born. There are no marketed therapies for SMA.

RG7800 is an orally available small molecule being investigated for its ability to selectively modify the splicing of the SMN2 gene, which is present both in healthy individuals and SMA patients, towards the production of full length mRNA. Preclinical studies in animal models of SMA demonstrated an increase in functional full length SMN protein with significant efficacy benefits on survival and motor function.

The SMA program was initially developed by PTC Therapeutics in partnership with the SMA Foundation. The SMA Foundation was established in 2003 to accelerate the development of a treatment for SMA. In November 2011, Roche gained an exclusive worldwide license to the PTC / SMA Foundation program. The development of RG7800 is being executed by Roche and overseen by a joint steering committee with members from PTC, Roche, and the SMA Foundation.

About PTC Therapeutics, Inc.

PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA is copied from DNA through the transcription process. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has discovered all of its compounds currently under development using its proprietary technologies. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For more information on the company, please visit our website www.ptcbio.com.

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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, including statements regarding the future expectations, plans and prospects for PTC, our collaboration in SMA with Roche and the SMA Foundation, including the timing and conduct of the Phase 2 Moonfish study, and our expectations with respect to RG7800, are forward-looking statements. Other forward-looking statements may be identified by the words "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "look forward," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions. Our actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements we make as a result of a variety of risks and uncertainties, including those related to the initiation and conduct of clinical trials and studies, availability of data from clinical trials and studies, expectations for regulatory approvals, our scientific approach and general development progress, and the factors discussed in the "Risk Factors" section of our 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 included in this press release represent PTC's views only as of the date of this press release and we do 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.

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/phase-1-data-from-spinal-muscular-atrophy-program-to-be-presented-at-the-2015-american-academy-of-neurology-annual-meeting-300068286.html>

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