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## **JOINT PROGRAM IN SPINAL MUSCULAR ATROPHY ENTERS FIRST STAGE OF CLINICAL DEVELOPMENT**

SOUTH PLAINFIELD, NJ, JANUARY 22, 2014 - PTC Therapeutics, Inc. (NASDAQ: PTCT), the SMA Foundation, and Roche (SIX: RO, ROG; OTCQX: RHHBY), announced today that their joint research program in Spinal Muscular Atrophy (SMA) has entered the first stage of clinical development aiming to assess safety and tolerability of an oral compound in healthy volunteers. SMA is a genetic neuromuscular disorder that leads to muscle weakness and mobility impairment and is considered the leading genetic cause of death in infants and toddlers.

Luca Santarelli, Head of Neuroscience, Ophthalmology and Rare Diseases at Roche commented: "The compelling science behind this project and the highly synergistic alliance among Roche, PTC Therapeutics and the SMA Foundation are the groundwork for this research program. Although still early in development, we consider every step towards a therapeutic option in SMA of high importance for patients and families affected by this devastating and currently untreatable condition."

SMA is caused by a missing or defective SMN1 gene, which results in reduced levels of the survival motor neuron (SMN) protein. SMN protein plays a critical role in the survival of neuronal cells in the spinal cord responsible for muscle movements. Reduced levels of SMN protein lead to dysfunction and death of the neuronal cells, making muscles smaller and weaker over time, and can even result in death in the most severe forms of the disease. The orally available small molecule compounds in the program are designed to target the underlying cause of the disease by increasing SMN protein levels in the nervous system, muscles, and other tissues. It is estimated that approximately one in 10,000 children are born with this rare genetic disorder. "We are delighted with the achievement of this important milestone," commented Stuart W. Peltz, Ph.D., CEO of PTC Therapeutics, Inc. "The innovative approach developed at PTC has benefited tremendously from the scientific partnership and dedication of both Roche and the SMA Foundation. The rapid progress highlights our combined dedication to advance an investigational medicine for SMA as rapidly as possible."

The SMA program was initiated in 2006 by PTC Therapeutics in collaboration with the SMA Foundation, which provided the majority of its funding prior to Roche joining the collaboration.

"This important achievement may pave the way toward a brighter, more hopeful future for children and adults with SMA," said Loren Eng, President of the SMA Foundation. "While we appreciate that the safety studies in healthy volunteers are an early step and much further development will be required, this is a significant accomplishment, and we are grateful to the commitment of our partners in accelerating this program as rapidly as possible."

In November 2011, Roche had gained an exclusive worldwide license to the SMA program. The initiation of the clinical development triggers a \$7.5 million payment to PTC from Roche.

### **ABOUT PTC THERAPEUTICS, INC.**

PTC Therapeutics is a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are essential to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and which form the basis for collaborations with leading biopharmaceutical companies. For more information on the company, please visit the website [www.ptcbio.com](http://www.ptcbio.com).

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PTC THERAPEUTICS, INC. FORWARD LOOKING STATEMENTS:

Any statements in this press release about future expectations, plans and prospects for PTC, the development of and potential market for PTC's product candidates, our clinical trials for lead compounds targeting SMA, our collaboration in SMA with Roche and the SMA Foundation, our current and planned regulatory submissions, our earlier stage programs, and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Forward-looking statements involve substantial risks and uncertainties that could cause our future results, performance or achievements to differ significantly from those expressed or implied by these forward-looking statements. Such risks and uncertainties include, among others, those related to the initiation and conduct of clinical trials, availability of data from clinical trials, expectations for regulatory approvals, our scientific approach and general development progress, the availability or commercial potential of our product candidates and other factors discussed in the "Risk Factors" in the most recent Quarterly Report, which is on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent PTC's views only as of the date of this press release. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing PTC's views as of any date subsequent to the date of this press release.