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## **PTC Therapeutics and Massachusetts General Hospital Collaborate on Rare Disease Research**

SOUTH PLAINFIELD, N.J., Dec. 9, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (Nasdaq: PTCT) has entered into a research collaboration with Massachusetts General Hospital (MGH, a Partners Healthcare hospital) for the treatment of rare genetic disorders resulting from pre-mRNA splicing defects. The collaboration reflects both parties' commitment to improving the lives of those who suffer from rare and neglected diseases.

Under the terms of the agreement, PTC gains an exclusive worldwide license to compounds that modulate alternative splicing of the IKBKAP pre-mRNA - the predominant cause of familial dysautonomia (FD), also known as Riley-Day syndrome, a rare life-threatening genetic disorder. PTC will pay an upfront license fee, make payments upon successful completion of certain development and commercialization milestones, as well as royalties on worldwide product sales. PTC will fund collaborative research and licensing activities, as well as be responsible for the development and commercialization of products arising from the collaboration.

"We are excited to collaborate with PTC Therapeutics," said Susan Slaugenhaupt, Scientific Director of the Mass General Research Institute, Professor of Neurology (Genetics) at Harvard Medical School, and the Elizabeth G. Riley and Dan E. Smith, Jr. MGH Research Scholar in the MGH Center for Human Genetic Research. "PTC's leadership in developing treatments for rare diseases and expertise in RNA biology, combined with our innovative research capabilities, makes this a powerful collaboration to carry forward our research program to develop therapies for splicing-related genetic conditions."

Dr. Slaugenhaupt's research has been supported by a grant under the National Institutes of Health (NIH) Blueprint for Neuroscience Research (<http://neuroscienceblueprint.nih.gov>). Early research in her laboratory on splicing therapies was also funded by the Dysautonomia Foundation, Inc.

"We are thrilled to build on the excellent research being conducted at MGH and expand our RNA platform in alternative splicing," said Neil Almstead, Ph.D., Executive Vice President, Research, Pharmaceutical Operations and Technology at PTC Therapeutics. "Dr. Slaugenhaupt's pioneering work into the genetic mechanisms behind familial dysautonomia and other potential diseases present a strong fit for both our expertise in RNA biology and our mission to develop medicines for people with rare and neglected diseases."

PTC has developed a platform technology that enables discovery and development of drugs that modulate pre-mRNA splicing. The most advanced program built on this platform is in clinical development for spinal muscular atrophy in collaboration with Roche and the SMA Foundation.

### **About Familial Dysautonomia**

Familial dysautonomia (FD), also known as Riley-Day syndrome and HSAN type III, is a rare, life-threatening genetic disease that affects the autonomic and sensory nervous systems of children from birth. It affects every major system of the body, causing severe respiratory, cardiovascular, orthopedic, digestive, renal, and vision problems. The most striking symptoms of FD are reduced sensitivity to pain and temperature, and the inability to produce tears. Children with FD suffer from chronic and often debilitating symptoms that prevent them from leading normal lives.

FD is caused by mutations in the IKBKAP gene. Most FD patients are homozygous for a single nucleotide mutation in intron 20 that leads to alternative splicing of exon 20 and low levels of IKAP protein, predominantly in the nervous system. This mutation leads to reduced protein expression in all tissues, with the most significant reduction in neuronal tissue.

For more information about FD, please contact the Dysautonomia Foundation ([www.familialdysautonomia.org](http://www.familialdysautonomia.org)).

### **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, or mRNA, molecule 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](http://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, are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; timing, results and conduct of PTC's collaboration with MGH; PTC's alternative splicing program; 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 Quarterly Report on Form 10-Q 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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