January 23, 2018

Data from RG7916 Programs in SMA to be Presented at the International Scientific Congress on Spinal Muscular Atrophy

SOUTH PLAINFIELD, N.J., Jan. 23, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that updates and preliminary data from ongoing clinical trials of RG7916, an oral survival motor neuron 2 (SMN2) splicing modifier in SMA patients will be presented at the International Scientific Congress on Spinal Muscular Atrophy at Jagiellonian University, Kraków, Poland from January 25-27th. The first interim analysis of survival and real-life experiences from the FIREFISH clinical trial in infants with Type 1 SMA will be presented, as well as poster presentations on SMN protein production and the safety and pharmacodynamic effects from the clinical trials in Type 2/3 SMA patients. The three ongoing clinical trials, FIREFISH, SUNFISH, and JEWELFISH, are being conducted in Type 1 and Type 2/3 SMA patients respectively. SMA is a rare genetic disorder that results in neuromuscular disability beginning in infancy and is the leading inherited cause of mortality in infants and young children. The SMA program is a joint collaboration with Roche and the SMA Foundation.

Presentation Details:

- "FIREFISH, a multi-center, open-label trial to investigate the safety and efficacy of RG7916 in babies with Type 1 SMA: Study update and real-life experience of study implementation" on Saturday, Jan. 27 at 12:00 p.m. CET

Poster Presentation Details:

- "Updated pharmacodynamic and safety data from SUNFISH Part 1, a study evaluating the oral SMN2 splicing modifier RG7916 in patients with Type 2 or 3 spinal muscular atrophy" on Thursday, Jan. 25 at 5:30 p.m. CET
- "Relationship Between Central and Peripheral SMN Protein Increase Upon Treatment with RO7034067 (RG7916)" on Thursday, Jan. 25 at 5:30 p.m. CET
- "Preliminary evidence for pharmacodynamics effects of RG7916 in JEWELFISH, a study in patients with spinal muscular atrophy who previously participated in a study with another SMN2-splicing targeting therapy" on Friday, Jan. 26 at 3:30 p.m. CET

For more information on the conference, visit https://krakow2018.sma-europe.eu

About the SMA Clinical Trials
FIREFISH: An open-label, two-part clinical trial. Part 1 is a dose escalation study in at least 8 infants for a minimum of 4 weeks. The primary objective of Part 1 is to assess the safety profile of RG7916 in infants and determine the dose for Part 2. Part 2 is a single-arm study in approximately 40 infants with Type 1 SMA for 24 months, followed by an open-label extension.

SUNFISH: A double-blind, two-part, placebo-controlled trial. Part 1 enrolled patients with Type 2 or 3 SMA to evaluate safety, tolerability, and PK/PD of several RG7916 dose levels. The pivotal SUNFISH Part 2, in non-ambulant patients with Type 2 or 3 SMA, will evaluate safety and efficacy of the RG7916 dose level selected from Part 1.

JEWELFISH: An exploratory, open-label study to establish the safety and tolerability of RG7916 in people who have previously participated in a study with another therapy targeting SMN2 splicing.

About PTC Therapeutics
PTC is a global biopharmaceutical company focused on the discovery, development, and commercialization of novel medicines using our expertise in RNA biology. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology. PTC has discovered all of its compounds currently under development using its proprietary technologies. Since its founding 20 years ago, PTC's mission has focused on developing treatments to fundamentally change the lives of patients living with rare genetic disorders. The company was founded in 1998 and is headquartered in South Plainfield, New Jersey. For more information on the company, please visit our website www.ptcbio.com.

For More Information:

Investors:
Emily Hill
+ 1 (908) 912-9327
ehill@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167
jbaj@ptcbio.com

Forward Looking Statements:
All statements, other than those of historical fact, contained in this press release, are forward-looking statements, including statements regarding: any advancement of the joint development program in SMA with PTC, Roche, and SMAF, in particular as related to the timing of enrollment, completion and evaluation of the Phase 2 clinical studies of RG7916 in SMA patients and the period during which the results of the studies will become available; the clinical utility and potential advantages of RG7916, including its potential to impact every aspect of the disease; the timing and outcome of PTC's regulatory strategy and process; PTC's strategy, future expectations, plans and prospects, future operations, future financial position, future revenues or projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "potential," "will," "promise," "expect," "plan," "target," "anticipate," "believe," "estimate," "intend," "may," "project," "possible," "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 initiation, enrollment, conduct and availability of data from either the SUNFISH or FIREFISH studies and the outcome of such studies; events during, or as a result of, these studies that could delay or prevent further development of RG7916, including future actions or activities under the SMA joint development program; our expectations for regulatory approvals; PTC's scientific approach and general development progress; and 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.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products, including with respect to PTC's joint development program in SMA with Roche and the SMAF. There are no guarantees that any product candidate under the joint development program will receive 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.

View original content:http://www.prnewswire.com/news-releases/data-from-rg7916-programs-in-sma-to-be-presented-at-the-international-scientific-congress-on-spinal-muscular-atrophy-300587005.html

SOURCE PTC Therapeutics, Inc.

News Provided by Acquire Media