December 8, 2016

PTC Therapeutics Receives France's 2016 Prix Galien for Translarna™

- Award recognizes scientific innovation of first approved drug for underlying cause of nonsense mutation Duchenne muscular dystrophy -

SOUTH PLAINFIELD, N.J., Dec. 8, 2016 /PRNewswire/-- PTC Therapeutics Inc. (NASDAQ: PTCT), today announced that the company received France's 2016 Prix Galien for Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in the Medicines "Rare Disease" category. The award recognizes the scientific innovation represented by Translarna's ability to target the underlying cause of nmDMD and the impact the drug is having on patients. The award was presented at the annual gala and ceremony in Paris, France.

"We appreciate the recognition by the Prix Galien committee," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "This is a prestigious honor and very meaningful to the PTC team that worked over the last eighteen years to discover and develop new therapies targeting genetic disorders due to a nonsense mutation. The result, Translarna, was the first therapy ever approved for an underlying cause of Duchenne muscular dystrophy."

The Prix Galien was established in France in 1970 by French pharmacist Roland Mehl to recognize and promote significant advances in pharmaceutical research. The award is among the industry's highest accolades. The French award committee includes 17 eminent members from the scientific, medical, and academic communities of France.

About Translarna™ (ataluren)
Translarna, discovered and developed by PTC Therapeutics, Inc., is a protein restoration therapy designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. The resulting disorder is determined by which protein cannot be expressed in its entirety and is no longer functional, such as dystrophin in Duchenne muscular dystrophy. Translarna is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. Translarna is an investigational new drug in the United States . The development of Translarna has been supported by grants from Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation); Muscular Dystrophy Association; FDA's Office of Orphan Products Development; National Center for Research Resources; National Heart, Lung, and Blood Institute; and Parent Project Muscular Dystrophy.

About PTC Therapeutics
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 and oncology. 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.

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: the future expectations, plans and prospects for PTC; the timing and outcome of PTC's regulatory process, including the final determination by the European Commission with respect to renewal of the marketing authorization in the European Economic Area (EEA) for Translarna for the treatment of nmDMD; the clinical utility and potential advantages of Translarna; PTC's ability to continue to supply Translarna to patients across Europe and in other territories; PTC's strategy, future operations, and the objectives of management. Other forward-looking statements may be identified by the words "will," "plan," "anticipate," "believe," "expect," "intend," "may," "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: PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the European Commission determines to approve the renewal of such authorization and whether the EMA determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; the final design of the new nmDMD trial that PTC will undertake pursuant to the specific obligation associated with the marketing authorization (following renewal) and PTC's ability to enroll, fund and conduct such trial; the outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD; 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. There are no guarantees that Translarna will receive full regulatory approval in any territory or maintain its current marketing authorization in the EEA, or prove to be commercially successful in general, or specifically with respect to the treatment of nmDMD.

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.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/ptc-therapeutics-receives-frances-2016-prix-galien-for-translarna-300375265.html

SOURCE PTC Therapeutics, Inc.