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PTC Therapeutics Provides Update on German pricing and Reimbursement Process for Translarna™ (ataluren)

SOUTH PLAINFIELD, N.J., Feb. 26, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the company has had multiple discussions with the German Federal Association of the Statutory Health Insurances (GKV-SV) over the last several months to come to agreement on pricing and reimbursement. Recently, these discussions transitioned into an arbitration process, which did not lead to an acceptable agreement. As a result, PTC is considering delisting Translarna from the German pharmacy ordering system. Under these circumstances, patients and healthcare professionals may be able to access Translarna through a reimbursed importation pathway possible under German law, thus minimizing any access issues for German patients while maintaining a sustainable price.

In May of 2015, Germany's Federal Joint Committee (G-BA), the highest decision-making body in the healthcare sector, granted Translarna an Early Benefit Assessment rating of 3, which signifies that the drug provides a quantifiable added benefit to patients. Additionally, in recognition of its innovative mechanism-of-action, Translarna was awarded the 2015 Prix Galien as the most innovative new product in the Orphan Drug category in Germany.

Translarna received marketing authorization in the European Economic Area (EEA) in August 2014 as the only treatment for the underlying cause of nmDMD, an ultra-rare progressive and irreversible muscle wasting disorder affecting boys and young men. Translarna has been commercially available in Germany since December 1, 2014, and is currently available on a commercial basis to patients in 23 countries.

About Duchenne Muscular Dystrophy

Primarily affecting males, Duchenne muscular dystrophy (DMD) is a progressive muscle disorder caused by the lack of functional dystrophin protein. Dystrophin is critical to the structural stability of skeletal, diaphragm, and heart muscles. Patients with DMD lose the ability to walk as early as age 10 and experience life-threatening lung and heart complications in their late teens and twenties. It is estimated that nonsense mutations account for approximately 13% of DMD cases.

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, 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

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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; the clinical utility and potential advantages of Translarna; the timing and scope of PTC's commercial and early access program launches; the rate and degree of market acceptance of Translarna; PTC's estimates regarding the potential market opportunity for Translarna, including the size of eligible patient populations and PTC's ability to identify such patients; PTC's strategy, 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 "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 those related to: whether patients and healthcare professionals may be able to access Translarna through a reimbursed importation pathway provided under German law, and whether this will minimize any access issues for German patients while maintaining a sustainable price; PTC's ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the EEA, which is subject to ongoing conditions and is also subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD, including its ability to successfully negotiate favorable pricing and reimbursement processes on a timely basis in the countries in which it may obtain regulatory approval, including the United States, EEA and other territories; whether the FDA or the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD and other data with respect to the safety and efficacy of Translarna for the treatment of nmDMD; expectations for regulatory approvals, including PTC's ability to advance regulatory submissions in a timely manner (or at all), adverse decisions by regulatory authorities, other delay or deceleration of the regulatory process, and PTC's ability to meet existing or future regulatory standards with respect to Translarna; the scope of regulatory approvals or authorizations for Translarna (if any), including labeling and other matters that could affect the availability or commercial potential of Translarna; PTC's scientific approach and general development progress; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; 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 release except as required by law.

To view the original version on PR Newswire, visit:<http://www.prnewswire.com/news-releases/ptc-therapeutics-provides-update-on-german-pricing-and-reimbursement-process-for-translarna-ataluren-300227137.html>

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