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PTC Therapeutics Provides Update on Health Canada Review of Translarna™ (ataluren) for the Treatment of Duchenne Muscular Dystrophy

SOUTH PLAINFIELD, N.J., March 14, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the company intends to submit the results of the recently completed Phase 3 ACT DMD study for review by Health Canada as part of the New Drug Submission (NDS) for Translarna™ (ataluren) to treat nonsense mutation Duchenne muscular dystrophy (nmDMD). In order to submit this additional data for review, PTC will withdraw the current NDS from Health Canada and resubmit the NDS with the ACT DMD results. As a result, the company no longer expects Health Canada review of the NDS in the first half of 2016.

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 and Canada. 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 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, the more severe form of the disorder, 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 a nonsense mutation is the cause of DMD in approximately 13 per cent of patients.

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.

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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 strategy and process, including as it relates to PTC's current or future re-submission of a New Drug Submission (NDS) with Health Canada for Translarna™ (ataluren) to treat nonsense mutation Duchenne muscular dystrophy (nmDMD) as well as it relates to PTC's submissions with the FDA, EMA and other regulatory bodies outside of these territories and related regulatory reviews; PTC's ability to maintain its current marketing authorizations or obtain and maintain additional marketing authorizations; PTC's ability to work with the FDA to resolve the matters set forth in the Refuse to File letter PTC received in connection with its NDA for Translarna for the treatment of nmDMD; the price of Translarna for the treatment of nmDMD in territories where PTC is or may be authorized to market Translarna; 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; the timing, results and conduct of PTC's clinical trials and studies of Translarna for the treatment of nmCF and other indications, including statements regarding the timing of initiation, evaluation, enrollment and completion of the trials and studies and the period during which the results of the trials and studies will become available; 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 Health Canada, the FDA, the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD or PTC's other clinical trials; expectations for regulatory approvals, including PTC's ability to make regulatory submissions with Health Canada and regulators in other territories, in a timely manner (or at all), the period during which the outcome of regulatory reviews will become available, 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 timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD, including whether PTC is required to perform additional clinical and non-clinical trials at significant cost and whether such trials, if successful, may enable FDA review of a NDA submission; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna; whether patients and healthcare professionals may be able to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome; 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 ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the EEA, which is subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; PTC's ability to obtain full marketing authorization in the EEA or obtain or maintain marketing authorizations in territories outside the EEA; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD; PTC's ability to fulfill any additional obligations, including with respect to further trials or studies relating to cost-effectiveness, obtaining licenses or satisfying requirements for labor and business practices, in the territories in which it may obtain regulatory approval, including the United States, EEA and other territories; the initiation, conduct and availability of data from clinical trials and studies; PTC's scientific approach and general development progress; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; the outcome of ongoing or future clinical trials or studies; PTC's ability to establish and maintain arrangements with manufacturers, suppliers, distributors and production and collaboration partners on favorable terms; the sufficiency of PTC's cash resources and PTC's ability to obtain adequate financing in the future for PTC's foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Annual Report on Form 10-K 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-provides-update-on-health-canada-review-of-translarna-ataluren-for-the-treatment-of-duchenne-muscular-dystrophy-300235219.html

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