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German Federal Joint Committee Issues Positive Medical Benefit Rating for Translarna™ i Patients with Nonsense Mutation Duchenne Muscular Dystrophy

- Quantifiable Added Benefit for the First Approved Therapy for nmDMD -

SOUTH PLAINFIELD, N.J., May 21, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that the Benefit Assessment by Germany's Federal Joint Committee (G-BA) indicated that Translarna™ (ataluren) provided a benefit for ambulatory patients aged five years and older with nonsense mutation Duchenne Muscular Dystrophy (nmDMD). The G-BA came to the decision that the existing clinical data package presented by PTC provided convincing evidence for the demonstration of a clinically meaningful added benefit. PTC received a 3 in the rating system established under the German pharmaceutical law.

"We are pleased by the positive assessment we received from the G-BA for Translarna. It reflects the medical benefit provided for nonsense mutation DMD patients where there are no other approved therapies," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We appreciate the G-BA's recognition of Translarna's benefit for these patients with such high unmet medical need."

The Federal Joint Committee is a non-state, self-governance body which is comprised of payers, providers, and patient representation who classify the extent of a drug's added benefit based on defined criteria. This assessment is mandatory for any newly introduced drugs into the German healthcare system. PTC Therapeutics anticipates the initiation of pricing discussions with the Statutory Health Insurance, the umbrella organization which represents Germany's sickness funds in the near term. This rating is approved through June 1, 2016, at which time it will be reassessed based upon the results of PTC's global, confirmatory Phase 3 clinical trial in nmDMD. PTC expects top-line data from this Phase 3 trial in the fourth quarter of 2015. While the G-BA assessment may inform pricing negotiations it is important to note that the assessment does not change the current reimbursement status of Translarna, which is reimbursed for all patients under the Translarna approved label in Germany.

ABOUT GERMANY'S FEDERAL JOINT COMMITTEE (G-BA) RATING SYSTEM
The G-BA performs a benefit assessment based on the manufacturer's value dossiers. Ratings are assigned per indication to inform the price negotiations, which are summarized as follows:

1 - Major additional benefit (e.g. cure of disease, significant extension of survival)
2 - Considerable additional benefit (e.g. moderate extension of survival)
3 - Minor additional benefit (e.g. reduction in symptoms)
4 - Additional benefit but not quantifiable
5 - No additional benefit proven
6 - Less benefit than comparator

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.

Abbreviated Prescribing Information:
Indication: Translarna™ (active ingredient: ataluren) is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older. Dosing: Translarna is available as granules for oral suspension in sachets of 125 mg, 250 mg or 1000 mg. The recommended total daily dose of Translarna is 40 mg/kg body weight, split in 3 doses - 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening. Ingredients: Active ingredient: ataluren. Excipients: polydextrose (E1200), macrogol, poloxamer, mannitol (E421),

crospovidone, hydroxyethyl cellulose, artificial vanilla flavor: maltodextrin, artificial flavours and propylene glycol, silica, colloidal anhydrous (E551), magnesium stearate. Contraindications: Translarna is contraindicated in patients with hypersensitivity to the active substance or to any of the excipients. Should not be co-administered with intravenous aminoglycosides, and concomitant use of other nephrotoxic agents is not recommended. Special warnings and precautions for use: Patients must have a nonsense mutation in the dystrophin gene as determined by genetic testing. Patients who do not have a nonsense mutation should not receive ataluren. Patients with renal and hepatic impairments should be closely monitored. In nmDMD patients receiving ataluren, it is recommended that total cholesterol, LDL, HDL, triglycerides, serum creatinine, BUN, and cystatin C be measured on an annual basis, or more frequently as needed based on clinical status. It is also recommended that resting systolic and diastolic blood pressure be monitored every 6 months in nmDMD patients receiving ataluren concomitantly with corticosteroids, or more frequently as needed based on clinical status. Caution should be exercised when ataluren is coadministered with medicinal products that are substrates or inducers of UGT1A9, inhibitors of BCRP, or substrates of OAT1, OAT3, or OATP1B3. Medicinal product subject to medical prescription. Treatment with Translarna should only be initiated by specialist physicians with experience in the management of DMD. Adverse reactions: The most frequent adverse reactions that were very common at the recommended dose were nausea, vomiting, and headache. Common adverse events include: decreased appetite, weight loss, dizziness, high blood pressure, cough, nosebleed, constipation, diarrhoea, wind, regurgitation, stomach discomfort, stomach pain, rash, arm or leg pain, cyst in the kidney, abnormally frequent urination, involuntary urination, abnormal urine colour, fever, tiredness. Authorised for marketing in the European Union - EU/1/13/902/001. Marketing Authorization Holder. PTC Therapeutics International Limited, Fitzwilliam Business Centre, 77 Sir John Rogerson's Quay, Dublin 2, Ireland.

This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions via the national reporting system.

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 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.

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:
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, including statements regarding the future expectations, plans and prospects for PTC; the timing and scope of PTC's commercial and early access program launches; the rate and degree of market acceptance and clinical utility 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 and conduct of PTC's clinical trials and studies of Translarna for the treatment of nonsense mutation DMD, including statements regarding the timing of evaluation and completion of the trials and studies and the period during which the results of the trials and studies will become available; our ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area, which is conditioned upon completion of our Phase 3 confirmatory trial in nmDMD, among other things, and subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; our current and planned regulatory filings, including with the FDA and in the European Union; our strategy, future operations, future financial position, future revenues or projected costs; and objectives of management, are forward-looking statements. 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 its ability to commercialize Translarna in general and specifically as a treatment for nonsense mutation DMD, and 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 countries in the European Economic Area; the initiation, conduct and availability of data from clinical trials and studies; expectations for regulatory approvals; 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. 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/german-federal-joint-committee-issues-positive-medical-benefit-rating-for-translarna-in-patients-with-nonsense-mutation-duchenne-muscular-dystrophy-300087589.html

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