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PTC Announces Translarna™ Access Program In Duchenne Muscular Dystrophy for Siblings of Patients Participating in PTC Clinical Trials

SOUTH PLAINFIELD, N.J., March 3, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that it will make Translarna available for siblings of patients in certain open label PTC clinical trials for nonsense mutation Duchenne muscular dystrophy (nmDMD). Translarna will be made available to these siblings in advance of commercial availability in their regions, after a treating physician and the patient or guardian make a clinical decision to use Translarna, and consistent with any other applicable regulatory requirements.

"For families with more than one child suffering from nonsense mutation Duchenne muscular dystrophy, having only one child enrolled in our clinical trials with access to Translarna, can cause great distress for parents," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Knowing this, we worked to find a solution to ensure those siblings who were not eligible to participate in the trials can still have similar access. We are eager to roll out this program and are committed to providing access to Translarna for all who may benefit."

Kate Bushby, Professor of Neuromuscular Genetics at Newcastle University said, "Though Duchenne muscular dystrophy is a rare disorder, the nature of an inheritable genetic disease means that there is an increased likelihood that siblings will be born with the same mutation. Translarna is the first treatment option available to treat the underlying cause of nonsense mutation DMD. It is exciting to see PTC's commitment to the patients contributing towards our understanding of the natural history of Duchenne and developing Translarna as a treatment for nmDMD."

"Families of children with Duchenne have wanted companies to be responsive in the case of siblings," added Pat Furlong, Founding President of Parent Project Muscular Dystrophy. "With this program, PTC, once again, demonstrates itself as a champion for the Duchenne community."

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 has received conditional marketing authorization in the European Economic Area for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. 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

Patients, Patients' Families, Investigators and Patient Organizations:
Diane Goetz
+1 (908) 912-9256 or (866) 282-5873
patientinfo@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 are forward-looking statements. Other forward-looking statements may be identified by the words "will," "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "project," "target," "potential," "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 scientific approach and general development progress and the factors discussed in the "Risk Factors" section of PTC's most recent filed Form 10-K or Form 10-Q filed with the Securities & Exchange Commission. 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/ptc-announces-translarna-access-program-in-duchenne-muscular-dystrophy-for-siblings-of-patients-participating-in-ptc-clinical-trials-300044303.html

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