Tegsedi® Receives Innovative Drug Category Pricing in Brazil

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SOUTH PLAINFIELD, N.J., Oct. 11, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Tegsedi® (inotersen) has successfully received Category 1 classification from Câmara de Regulação do Mercado de Medicamentos - CMED (Drug Market Regulation Chamber) in Brazil. Category 1 classification is given to innovative treatments that provide greater efficacy than current standards of care. Category 1 allows for pricing in line with international markets.

Tegsedi has been approved by the Brazilian regulatory agency ANVISA (Agência Nacional de Vigilância Sanitária) for the treatment of Stage 1 or 2 polyneuropathy (nerve damage) in adult patients with hereditary transthyretin-mediated (hATTR) amyloidosis to delay disease progression and improve quality of life. It is the first antisense medicine available for patients in Brazil to address the underlying cause of the disease.

"This is an incredibly important decision in allowing patients access to Tegsedi in Brazil," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "Because of the high Portuguese descent, there is a higher prevalence of hATTR patients in the Brazilian population. The disease has a devastating impact on patients and their caregivers, and we are proud to bring an innovative treatment that provides greater efficacy to these patients."

Studies have proven that Tegsedi significantly improved measures of neuropathic disease progression and quality of life for patients.[1] There are estimated to be 5,000 patients with hATTR amyloidosis in Brazil,[2] which is about 10 percent of all cases worldwide.[3]

About Tegsedi® (inotersen)
Tegsedi is designed to block the production of the TTR protein, which is the underlying cause of hATTR amyloidosis. It is a novel, first-in-class 2'-O-2- methoxyethyl phosphorothioate antisense oligonucleotide and its mechanism of action is distinct from all previous therapies for hATTR amyloidosis. In the Phase 3 NEURO-TTR study, Tegsedi significantly reduced the levels of TTR protein in patients irrespective of mutation type or stage of disease,[1] and significantly improved quality of life.[1] Tegsedi is a once-weekly, self-administered subcutaneous treatment, which enables greater patient independence. Tegsedi is approved in Brazil, Canada, the European Union, and the United States.

Tegsedi, a product of Ionis Pharmaceutical's proprietary antisense technology, is an antisense medicine designed to reduce the production of transthyretin (TTR) protein. PTC Therapeutics is collaborating with Akcea Therapeutics, Inc., an affiliate of Ionis Pharmaceuticals, to commercialize Tegsedi in Latin America. The collaboration leverages PTC's global infrastructure and reflects PTC's successes in bringing innovative drugs to patients in Latin America. Tegsedi® is a trademark of Akcea Therapeutics, Inc.

About Hereditary Transthyretin (hATTR) Amyloidosis
hATTR amyloidosis is a progressive, systemic and fatal inherited disease caused by the abnormal formation of the TTR protein and aggregation of TTR amyloid deposits in various tissues and organs throughout the body, including in peripheral nerves, heart, intestinal tract, eyes, kidneys, central nervous system, thyroid and bone marrow. The progressive accumulation of TTR amyloid deposits in these tissues and organs leads to sensory, motor and autonomic dysfunction often having debilitating effects on multiple aspects of a patient's life. Patients with hATTR amyloidosis often present with a mixed phenotype and experience overlapping symptoms of polyneuropathy and cardiomyopathy.

Ultimately, hATTR amyloidosis results in death within three to 15 years of symptom onset. Therapeutic options for the treatment of patients with hATTR amyloidosis are limited and there are currently no disease-modifying drugs approved for the disease. There are an estimated 50,000 patients with hATTR amyloidosis worldwide. Additional information on hATTR amyloidosis, including a full list of organizations supporting the hATTR amyloidosis community worldwide, is available at www.hattrchangethecourse.com.

About PTC Therapeutics, Inc.
PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more about PTC, please visit us on www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

For More Information:

Investors:
Kylie O'Keefe
+1 (908) 300-0691
kokeefe@ptcbio.com

Media:
Jeanine Clemente
+1 (908) 912-9406
jclemente@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 contained in this release, other than statements of historic fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC, including with respect to the commercialization of its products and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "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: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; 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 any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Tegsedi.

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.

References

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[1] Benson MD,et al. Inotersen Treatment for Patients with Hereditary Transthyretin Amyloidosis. N Engl J Med. 2018; 379:22-3.
[2] Schmidt H, Waddington-Cruz M, Botteman MF, Carter JA, Chopra AS, Stewart M et al. Global prevalence estimates of transthyretin familial amyloid polyneuropathy (ATTR-FAP).
[3] Gertz MA. Hereditary ATTR Amyloidosis: Burden of Illness and Diagnostic Challenges. Am J Manag Care. 2017;23(7 suppl):S107-S112.

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