Waylivra™ Receives Innovative Drug Category Pricing to Treat Familial Chylomicronemia Syndrome in Brazil

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- Additional application submitted to ANVISA for a new indication to treat familial partial lipodystrophy -

SOUTH PLAINFIELD, N.J., Jan. 4, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Waylivra™ (volanesorsen) has successfully received Category 1 classification from Câmara de Regulação do Mercado de Medicamentos - CMED (Drug Market Regulation Chamber) in Brazil. Waylivra is the only treatment for familial chylomicronemia syndrome (FCS) in Brazil. Category 1 classification is given to innovative treatments that provide greater efficacy than current standards of care, and it allows for pricing in line with international markets.

In addition, PTC submitted an application to the Brazilian Health Regulatory Agency, Agência Nacional de Vigilância Sanitária (ANVISA), for approval of Waylivra for the treatment of familial partial lipodystrophy (FPL). If approved, Waylivra will be the first approved treatment for FPL in Brazil, and this will mark the first approval globally for this indication. The application has been submitted under the Rare Disease pathway RDC205/2017. PTC anticipates a decision from the regulatory authorities in the second half of 2022.

"Both FCS and FPL are rare genetic diseases that cause significant issues for patients suffering from these disorders," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "I am pleased with the decision by CMED to grant FCS patients access to Waylivra in Brazil. This innovative treatment will have a positive impact on patients' lives. I am also very enthusiastic about the possibility of bringing Waylivra to patients suffering from FPL who are also in need of a treatment."

FCS is a rare genetic disease which results in significant disease burden to patients, including potentially fatal pancreatitis and chronic complications due to permanent organ damage. FPL is a rare genetic, highly burdensome disease, which is both visually and physically distressing for the patient. It leads to significant metabolic complications that are not managed by current therapies.

About Waylivra™
Waylivra (volanesorsen), a product of Ionis Pharmaceuticals, Inc.'s proprietary antisense technology, has received conditional marketing approval in the European Union as a treatment for FCS. In addition, Waylivra has been granted Orphan Drug Designation by the European Medicines Agency for the treatment of FCS. Waylivra has been in-licensed by PTC from Akcea Therapeutics, Inc., a wholly-owned subsidiary of Ionis, for commercialization in Latin America.

About Familial Chylomicronemia Syndrome (FCS)
FCS is a rare disease caused by impaired function of the enzyme lipoprotein lipase (LPL) and characterized by severe hypertriglyceridemia (>880mg/dL) and a risk of unpredictable and potentially fatal acute pancreatitis. Because of limited LPL function, people with FCS cannot breakdown chylomicrons, lipoprotein particles that are 90% triglycerides. They can experience daily symptoms including abdominal pain, generalized fatigue and impaired cognitions that affect their ability to work. People with FCS also report major emotional and psychosocial effects including anxiety, social withdrawal, depression, and brain fog. The lack of treatments specifically indicated for patients with FCS means that patients must rely on a very restrictive, low-fat diet in combination with lifestyle changes and control of other secondary causes of HTG, which generally do not guarantee the necessary quality of life for those who have FCS. Diet alone does not fully control TG levels and is not sufficient to mitigate the risk of pancreatitis and enzyme replacement therapy would be ineffective in FCS due to half-life limitations.

About Familial Partial Lipodystrophy (FPL)
FPL is a rare genetic metabolic disease characterized by selective, progressive loss of body fat (adipose tissue) from various areas of the body leading to ectopic fat deposition in liver and muscle and development of insulin resistance, diabetes, dyslipidemia and fatty liver disease[1]. Individuals with FPL often have reduced subcutaneous fat in the arms and legs and the head and trunk regions may or may not have loss of fat. Conversely, affected individuals may also have excess subcutaneous fat accumulation in other areas of the body, especially the neck, face and intra-abdominal regions.

About PTC
PTC is a science-driven, 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 innovate to identify new therapies and can globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. Our mission is to provide access to best-in-class treatments for patients who have little to no treatment options. The company's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. We believe this allows us to maximize value for all our stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Instagram, Facebook, Twitter, and 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 Waylivra.

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.

[1] Chan JL, Oral EA. *Endocr Pract* 2010;16:310–323

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