PTC Therapeutics Announces Waylivra™ Approval in Brazil as First Treatment for Familial Chylomicronemia Syndrome

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- Approval Addresses Unmet Needs of Patients with No Other Treatment Options -

SOUTH PLAINFIELD, N.J., Aug. 23, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the Brazilian Health Regulatory Agency, ANVISA (Agência Nacional de Vigilância Sanitária), has approved Waylivra™ (volanesorsen) as the first treatment for familial chylomicronemia syndrome (FCS) in Brazil. 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.

"We are excited to announce Waylivra's approval in Brazil as the first treatment for the underlying cause of FCS," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "This approval is another example of PTC leveraging its strong capabilities in Latin America to bring transformational therapies to patients with high unmet needs."

ANVISA's approval of Waylivra is based on results from the Phase 3 APPROACH study and the APPROACH Open-Label Extension study and is supported by results from the Phase 3 COMPASS study. Results from the Phase 3 APPROACH trial, the largest study ever conducted in patients with FCS, show that treatment with Waylivra reduced triglycerides 94% when compared to placebo. All patients in the trial maintained a low-fat diet.

Waylivra is a self-administered, subcutaneous injection that comes in a single-use, prefilled syringe and is the first approved pharmacotherapy indicated for the treatment of FCS. It is an innovative antisense oligonucleotide (ASO), with a novel mode of action designed to reduce the production of apolipoprotein C-III (Apoc-III), a protein that slows down the breakdown of fats called apolipoprotein C-III. By blocking the production of this protein, the medicine reduces the level of triglycerides in the blood and, as a result, fat accumulation in the body, which is expected to reduce the risk of pancreatitis.

About Waylivra
Waylivra (volanesorsen), a product of Ionis Pharmaceuticals, Inc.'s proprietary antisense technology, has received conditional marketing approval in the E.U. 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 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 globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. The Company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Instagram, Facebook, Twitter, and LinkedIn.

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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.

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