Brazilian Regulatory Authority Grants Approval for Waylivra™ for Familial Partial Lipodystrophy

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- First global regulatory approval for treatment of this rare genetic disease -

SOUTH PLAINFIELD, N.J., Dec. 1, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that the Brazilian Health Regulatory Agency, ANVISA (Agência Nacional de Vigilância Sanitária), has approved Waylivra™ (volanesorsen) as the first treatment for familial partial lipodystrophy (FPL) in Brazil. This is the first approval globally for Waylivra for the FPL indication. Waylivra is also approved in Brazil for the treatment of Familial Chylomicronemia Syndrome (FCS).

"Waylivra's approval as the first treatment for FPL is an important milestone for FPL patients in Brazil," 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 first of its kind transformational therapies to patients with high unmet needs."

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]. FPL is a highly burdensome disease, leading to significant metabolic complications that are not managed by current therapies, and it is a distressing condition for the patient.

ANVISA's approval for Waylivra for FPL was based on results from the Phase 2/3 BROADEN study in patients with familial partial lipodystrophy. The study met its primary endpoint demonstrating a statistically significant reduction in triglyceride levels in patients with FPL treated with Waylivra compared to placebo-treated patients.

In the study, there was a mean reduction from baseline of 88 percent in triglyceride levels in patients treated with Waylivra compared to 22 percent reduction in placebo-treated patients at three months. Clinically meaningful and statistically significant reductions in triglyceride levels were sustained over 12 months of treatment in FPL patients. In addition to achieving the primary endpoint, the study achieved an important secondary endpoint of a statistically significant reduction in liver fat.

About Waylivra™

Waylivra (volanesorsen) is a product of Ionis Pharmaceuticals, Inc.'s proprietary antisense technology. Waylivra 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. The approval by ANIVSA for FPL is the first global approval for this indication.

Waylivra has been in-licensed for commercialization in Latin America by PTC Therapeutics from Akcea Therapeutics, Inc., a wholly-owned subsidiary of Ionis.

About Familial Partial Lipodystrophy (FPL)

FPL is a rare lipid disorder characterized by abnormal fat distribution across the body and a range of metabolic abnormalities, including severe insulin resistance, dyslipidemia and hypertriglyceridemia, hepatic steatosis and, in affected women, features of hyperandrogenism. People with FPL often present with polycystic ovarian syndrome or unusually insulin-resistant diabetes and are at increased risk of acute pancreatitis in addition to long-term, progressive consequences including premature cardiovascular disease and liver disease, resulting in cirrhosis. They are unable to store fat or triglycerides in normal fat stores, so excess triglycerides are stored in the liver and muscle and accumulate at high levels in the bloodstream.

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 cognition that affects 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 Facebook, on Twitter at @PTCBio, and on 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.

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

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