Evrysdi® Distinguished by Two Prestigious Awards for Outstanding Innovation in Drug Discovery

December 6, 2021

SOUTH PLAINFIELD, N.J., Dec. 6, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Evrysdi® (risdiplam) has received multiple awards that recognize it as one of the most innovative therapies of 2021 and for its life-saving impact. The British Pharmacological Society has chosen Evrysdi as the 2022 Drug Discovery of the Year award. In addition, Evrysdi was awarded the 2021 Drug Discovery Prize by the Society for Medicines Research.

"Our journey of modulating splicing by small molecules to treat diseases began more than 15 years ago," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "At the time, the common dogma espoused that it was not possible to identify oral small molecule drugs that modified splicing. We are excited to see our vision come to fruition, and we are now able to leverage our splicing technology to address multiple other diseases where new treatments are desperately needed. We are honored to have Evrysdi recognized by these awards. More importantly, we are most thrilled by the transformative impact this oral therapy has had on people living with SMA."

Evrysdi was selected from a list of 150 innovative medicines for the British Pharmacological Society's Drug Discovery of the Year award. The Drug Discovery of the Year Award was established by the British Pharmacological Society to celebrate the role pharmacology plays in the development of new medicines and recognize the achievements of groups of medical scientists who research and develop new drugs. The Society for Medicines Research Drug Discovery prize is awarded every three years to a single, novel approved medicine. It recognizes outstanding contributions, achievements, and inventions in the world of drug discovery and development.

Evrysdi was discovered through PTC's innovative alternative splicing platform. Roche leads the clinical development as part of a collaboration with the SMA Foundation and PTC Therapeutics. Evrysdi is commercialized by Roche and in the United States by Genentech, a member of the Roche Group.

About Spinal Muscular Atrophy (SMA)
Spinal muscular atrophy (SMA) is a severe, progressive neuromuscular disease that can be fatal. It affects approximately 1 in 10,000 babies and when untreated is the leading genetic cause of infant mortality. SMA is caused by a mutation of the survival motor neuron 1 (SMN1) gene, which leads to a deficiency of SMN protein. This protein is found throughout the body and is essential to the function of nerves that control muscles and movement. Without it, nerve cells cannot function correctly, leading to progressive muscle weakness over time. Depending on the type of SMA, an individual's physical strength and their ability to walk, eat or breathe can be significantly diminished or lost.

About Evrysdi® (risdiplam)
Evrysdi is a survival motor neuron 2 (SMN2)-directed RNA splicing modifier designed to treat SMA caused by mutations in chromosome 5q that lead to SMN protein deficiency. Evrysdi is designed to treat SMA by increasing and sustaining the production of SMN protein levels in the central nervous system and peripheral tissues and is administered daily at home in liquid form by mouth or feeding tube. Evrysdi has been approved in more than 60 countries including the United States, the European Union and Japan. Evrysdi is marketed by Roche and in the United States by Genentech, a member of the Roche Group.

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, Instagram, LinkedIn and Twitter at @PTCBio.

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 Statement
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: advancement of PTC's joint collaboration program in SMA, including the commercialization of any products therein or royalty or milestone payments; the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses, licensing or commercialization of its products and products candidates and other matters; 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; the enrollment, conduct, and results of ongoing studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development under the program, including any regulatory submissions and commercialization with respect to Evrysdi; 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 Evrysdi.

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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