



## **Spinal Muscular Atrophy (SMA) Foundation and PTC Therapeutics Expand Partnership to Advance Drug Discovery and Development Research in Regenerative Medicine**

March 9, 2021

**-SMA Foundation and PTC Therapeutics to commit to an initial investment of up to \$60 million in research to discover and develop promising regenerative treatments in neuromuscular diseases-**

NEW YORK and SOUTH PLAINFIELD, N.J., March 9, 2021 /PRNewswire/ -- The Spinal Muscular Atrophy (SMA) Foundation and PTC Therapeutics, Inc. (NASDAQ: PTCT) have entered into a new collaboration focused on regenerative medicine to further advance scientific research in SMA and other neuromuscular disorders with the goal of developing new treatments. SMA is a severe, inherited, progressive neuromuscular disease that causes devastating muscle atrophy and disease-related complications. The availability of several disease-modifying therapies for SMA has made regenerative interventions the next frontier in drug discovery and development. The SMA Foundation–PTC partnership will provide funding, managed by the SMA Foundation, to academic institutions and other collaborators to advance foundational research in the area of regenerative medicine.

"In neuromuscular disease, correcting the underlying cause is just the first step. Patients need therapies to help restore lost function. We are excited to expand our partnership with PTC Therapeutics to empower researchers to address this therapeutic gap in neuromuscular disorders with an emphasis on SMA," said Loren Eng, President, The SMA Foundation. "Our longstanding partnership with PTC lets us harness our research capabilities and PTC's drug discovery and development expertise and access to core technology platforms, all of which supported the recent approval of Evrysdi™ (risdiplam) in SMA. We look forward to our initial funding of new projects under the collaboration with PTC."

"We have had an extremely productive partnership with the SMA Foundation for well over a decade and are excited to continue this collaboration to investigate regenerative medicine to find new therapies for patients with SMA and other neuromuscular disorders," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "The SMA Foundation has been the leader in supporting SMA research and we look forward to doing the critical work to accelerate the next generation of therapeutics for these patients."

### **About Spinal Muscular Atrophy (SMA)**

Spinal muscular atrophy (SMA) is a severe, inherited, progressive neuromuscular disease that causes devastating muscle atrophy and disease-related complications. It is the most common genetic cause of infant mortality and one of the most common rare diseases, affecting approximately 1 in 11,000 babies. SMA leads to the progressive loss of nerve cells in the spinal cord that control muscle movement. 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.

SMA is caused by mutations or deletions in the survival motor neuron 1 (SMN1) gene that results in reduced levels of SMN protein. SMN protein is produced throughout the body and increasing evidence suggests SMA is a multi-system disorder and the loss of SMN protein may affect many tissues and cells, which can stop the body from functioning.

### **About PTC Therapeutics, Inc.**

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](http://www.ptcbio.com) and follow us on Facebook, Instagram, and LinkedIn and on Twitter at @PTCBio.

### **About the SMA Foundation**

The Spinal Muscular Atrophy (SMA) Foundation is a nonprofit organization whose mission is to develop treatments for SMA, the leading genetic cause of death in young children. Since its inception, the Foundation has funded over \$150 million in research and therapeutics development. The Foundation collaborates with leading academic medical centers, the federal government (DoD, NIH, CDC, FDA), and nearly every pharmaceutical company in the world. When the SMA Foundation was established in 2003,

there were no treatments for SMA. Due to the Foundation's investments and drug discovery efforts, today there are three FDA-approved drugs.

**For More Information:**

**PTC Therapeutics, Inc.**

**Investors:**

Kylie O'Keefe

+1 (908) 300-0691

[kokeefe@ptcbio.com](mailto:kokeefe@ptcbio.com)

**Media:**

Jane Baj

+1 (908) 912-9167

[jbaj@ptcbio.com](mailto:jbaj@ptcbio.com)

**Contact for SMA Foundation**

Kelly Howell

+1 (646) 253-7100

[khowell@smafoundation.org](mailto:khowell@smafoundation.org)


**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; advancement of PTC's joint collaboration program in SMA; 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; 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.

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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