

PTC Therapeutics Establishes Strategic Collaboration with Aldevron to Support GMP Plasmid Manufacturing

October 7, 2019

-Agreement Represents Ongoing Dedication to Deliver Best-In-Class Gene Therapies to Patients in Need-

SOUTH PLAINFIELD, N.J., Oct. 7, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a collaboration with Aldevron, LLC to secure GMP manufacturing capacity for PTC's gene therapy portfolio, including the Friedreich ataxia and Angelman syndrome programs in PTC's growing gene therapy pipeline.

"Our strategic collaboration with Aldevron represents our continued commitment to produce and provide the highest quality product to patients," said Neil Almstead, Ph.D., Chief Technical Operations Officer of PTC Therapeutics. "Our gene therapy pipeline is addressing the unmet needs of multiple patient populations, and we feel an urgent need to develop safe products with the utmost speed. The development of relationships with top-tier companies like Aldevron aligns with our goal of partnering with the best collaborators as we drive meaningful improvements in the lives of patients."

"It is truly an honor to work with PTC's motivated team of experts. They are making enormous contributions to the future of genetic medicine," said Michael Chambers, Founder and CEO of Aldevron. "This is Aldevron's mission – to serve scientists and researchers who are relentlessly pursuing cures for people who need them."

The central nervous system (CNS) gene therapy platform at PTC uses targeted micro-dosing applied directly to areas involved in the disorder. This allows for greater efficacy, durability, lower risk of immunogenicity or other off-target effect, and efficient and scalable manufacturing. PTC has an advancing gene therapy pipeline for CNS disorders, including programs for AADC deficiency, Friedreich Ataxia, and Angelman Syndrome. PTC is preparing a BLA for the treatment of AADC deficiency for submission to the FDA in the fourth quarter of 2019.

About Aldevron

Aldevron is a leading producer of plasmid DNA for the biotechnology and life science industries. It serves the biotechnology industry with custom production of nucleic acids, proteins, and antibodies. Thousands of clients use Aldevron-produced plasmids, RNA and gene editing enzymes for projects ranging from discovery research to clinical trials to commercial applications. These products are critical raw materials and key components in commercially available drugs and medical devices. Aldevron specializes in GMP manufacturing and is known for inventing the GMP-Source™ quality system. It currently operates a 70,000 square foot GMP facility with an additional 189,000 square foot under construction. Aldevron has a staff of more than 400 across its three locations including its headquarters in Fargo, N.D., Madison, W.I., and Freiburg, Germany.

About PTC Therapeutics, Inc.

PTC is a science-led, 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 pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more about PTC, please visit us on 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, other than those of historical fact, contained in this release, are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; timing, results and conduct of PTC's collaboration with Aldevron, LLC; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions; PTC's strategy, future operations, future financial position, future revenues or projected costs; and 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," "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 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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