PTC Therapeutics Provides Update on EMA Regulatory Review Timeline For Its AADC Deficiency Gene Therapy Program

April 14, 2022

- CHMP Opinion Now Expected May 2022 -

SOUTH PLAINFIELD, N.J., April 14, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ:PTCT) announced today that it has completed the Scientific Advisory Group and Oral Explanation meetings for its gene therapy treatment for AADC deficiency (PTC-AADC) with the Committee for Advanced Therapies (CAT). The CAT is responsible for the scientific assessment of advanced therapies based on its expert member's assessment of the quality, safety and efficacy of the product. The CAT is scheduled to provide a draft opinion for adoption to the Committee for Medicinal Products for Human Use (CHMP) in May. If approved, PTC's AADC gene therapy would be the first marketed gene therapy directly infused into the brain.

"We are looking forward to treating patients with our gene therapy treatment for AADC deficiency," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "AADC deficiency is such a terrible disease and we believe that this treatment has the potential to be transformational for patients with this disorder."

The AADC gene therapy Marketing Authorization Application is supported by the findings of three clinical studies conducted in Taiwan. In addition, data from the compassionate use treatment of two patients in Europe were included in the application. PTC-AADC administration occurs through a stereotactic surgical procedure, a minimally invasive neurosurgical procedure used for the treatment of a number of pediatric and adult neurological disorders.

About PTC-AADC
PTC-AADC, is a one-time gene replacement therapy currently under regulatory review in Europe for the treatment of AADC deficiency. It is a recombinant adeno-associated virus serotype 2 (AAV2)-based gene therapy, containing the human DDC gene.[i] It is designed to correct the underlying genetic defect, by delivering a functioning DDC gene directly into the putamen, increasing the AADC enzyme and restoring dopamine production.[ii,iii]

About aromatic L-amino acid decarboxylase (AADC) deficiency
AADC deficiency is a fatal, rare genetic disorder that typically causes severe disability and suffering from the first months of life, affecting every aspect of life – physical, mental and behavioral. The suffering of children with AADC deficiency may be exacerbated by: episodes of distressing seizure-like oculogyric crises, which can happen daily and last for hours, causing the eyes to roll up in the head, frequent vomiting, behavioral problems, difficulty sleeping, and life-threatening complications such as respiratory infections and gastrointestinal problems.

Currently, there is no disease-modifying treatment approved for AADC deficiency and the lives of affected children are highly medicalized, sometimes involving many different medications to help manage symptoms, ongoing physical, occupational and speech therapy, and interventions, including surgery, to manage potentially life-threatening complications such as infections, severe feeding and breathing problems.

While several diagnostic tests for AADC deficiency are available, the condition remains largely undiagnosed.

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 innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and Twitter at @PTCBio.

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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 expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; expectations with respect to PTC's gene therapy platform, including any regulatory submissions and manufacturing capabilities; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of its other 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; expectations with respect to PTC's gene therapy platform, including any regulatory submissions and potential approvals, manufacturing capabilities and the potential financial impact and benefits of its leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; 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 PTC-AADC.

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.

[i] PTC Therapeutics. Data on File.

[ii] Chien YH, *et al.* Efficacy and safety of AAV2 gene therapy in children with aromatic L-amino acid decarboxylase deficiency: an open-label, phase 1/2 trial. *Lancet Child Adolesc Health.* 2017;1(4):265-273.

[iii] Chien *et al.* AGIL-AADC gene therapy results in sustained improvements in motor and developmental milestones through 5 years in children with AADC deficiency. Poster presented at the 48th Annual Meeting of the Child Neurology Society, Charlotte, NC, USA, Oct 23-26, 2019.

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