PTC Therapeutics Announces Results from Long-Term AADC Deficiency Gene Therapy Treatment Demonstrating Sustained Improvements

October 24, 2019

- Results represent 5 year follow up -

SOUTH PLAINFIELD, N.J., Oct. 24, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced new results from its investigational gene therapy, PTC-AADC, in patients living with aromatic L-amino acid decarboxylase (AADC) deficiency. The data demonstrated clinically meaningful and sustained improvements in motor, cognitive and language milestones. These included the ability to sit, walk, and talk and represents up to five years of follow up post-treatment.[1,2] PTC-AADC is a one-time gene therapy treatment of the human dopa decarboxylase (*DDC)* gene administered into the putamen, which supports production of key neurotransmitters. The data were presented at the Child Neurology Society 48th Annual Meeting.

"We are excited to see the transformational effects in AADC deficiency patients in this long-term study as patients with severe AADC deficiency never achieve the ability to sit, walk or talk," said Stuart Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "We are on track to submit a BLA to the FDA by the end of the year and are proud to be on the verge of bringing the first commercial treatment for AADC deficiency patients which is in line with our mission of bringing clinically differentiated treatments to patients with rare disorders."

New analysis evaluated outcomes of 26 patients with AADC deficiency across three separate clinical trials,[2] making it the most comprehensive analysis of patients treated with PTC-AADC to date. Specifically, these results showed that 12 months post-treatment with PTC-AADC, patients' mean body weight had increased from 12.0 kg to 15.2 kg, and the frequency of oculogyric crises (involuntary upward eye movement) was reduced.[2] Dyskinesia (involuntary movements) was the most frequently recorded adverse event, however most events were mild or moderate and all cases resolved by 10 months post-treatment.[2]

"In addition to failing to reach key developmental milestones, such as walking and talking, children with AADC deficiency can experience severe symptoms that affect their everyday lives. These symptoms can include episodes of oculogyric crises, which can last for minutes or hours and involve sustained upward movement of the eyes, involuntary movements of the neck, tongue protrusions and jaw spasms, which can be very distressing for patients and their families," said Claudio Santos, M.D., Senior Vice President, Global Medical Affairs, PTC Therapeutics. "The post-treatment data presented at CNS confirm reductions in the number of patients experiencing oculogyric crises, suggesting that this gene therapy treatment has the potential to make a real difference in the lives of patients with AADC deficiency."

A separate analysis of a long-term study demonstrated the sustained efficacy of PTC-AADC up to five years.[1] These are the longest data available for any investigational treatment for AADC deficiency. These results showed clinically meaningful and sustained improvements in motor, cognitive and language milestones up to five years post-treatment with PTC-AADC.[1]

An additional abstract building on the existing understanding of AADC deficiency was also presented, giving a disease state overview that highlights the potential importance of a gene therapy to treat this condition.[3]

About aromatic L-amino acid decarboxylase (AADC) deficiency
Aromatic L-amino acid decarboxylase (AADC) deficiency is a rare genetic condition caused by a mutation in the dopa decarboxylase (*DDC*) gene, resulting in a lack of functioning AADC enzyme, which is responsible for the final step in the synthesis of key neurotransmitters dopamine and serotonin.[4]

AADC deficiency results in delays or failure to reach developmental milestones such as head control, sitting, standing, walking, or talking, low muscle tone (also known as muscular hypotonia), severe, seizure-like episodes involving involuntary eye movement (also known as oculogyric crises), autonomic abnormalities, and the need for life-long care.[4] Given this neurologically devastating illness, patients with severe AADC deficiency have a high risk of death during childhood. There are currently no approved therapies that address the underlying cause.

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 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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References:

1. 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, Oct 23-26, 2019 Charlotte, NC, USA. Poster P207.
2. Hwu et al. Safety and Improved Efficacy Outcomes in Children With AADC Deficiency Treated with AGIL-AADC Gene Therapy: Results From Three Clinical Trials.. Poster presented at the 48th Annual Meeting of the Child Neurology Society, Oct 23-26, 2019 Charlotte, NC, USA. Poster P231.
3. Himmelreich et al. Epidemiology, molecular genetics, and new treatment options for aromatic amino acid decarboxylase deficiency.. Poster presented at the 48th Annual Meeting of the Child Neurology Society, Oct 23-26, 2019 Charlotte, NC, USA, Poster P207
4. Wassenberg et al. Consensus guideline for the diagnosis and treatment of aromatic l-amino acid decarboxylase (AADC) deficiency. *Orphanet J Rare Dis*. 2017; 12:12.

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; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions; 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: expectations with respect to the potential financial impact or PTC's ability to realize the anticipated benefits of the acquisition of Agilis and its gene therapy platform, including with respect to the business of Agilis and expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the former Agilis equityholders with respect thereto and PTC's ability to obtain marketing approval of PTC-AADC and other product candidates acquired from Agilis, will not be realized or will not be realized within the expected time period; significant transaction costs, unknown liabilities, the risk of litigation and/or regulatory actions related to the acquisition of its gene therapy pipeline, as well as other business effects, including the effects of industry, market, economic, political or regulatory conditions; the eligible patient base and commercial potential of PTC-AADC; 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.

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