# PTC Therapeutics Announces FDA Acceptance and Priority Review of the BLA for Upstaza™

May 14, 2024

*- PDUFA target action date of November 13, 2024 -*

WARREN, N.J., May 14, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that the FDA has accepted for filing the Biologics License Application (BLA) for Upstaza™ (eladocagene exuparvovec), a gene therapy for the treatment of AADC deficiency. The application has been granted Priority Review with a target regulatory action date of November 13, 2024.

"We are excited to be one step closer to bringing an approved therapy to patients with AADC deficiency in the United States," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "The data collected to date continue to support the transformative benefit of Upstaza, this highly innovative gene therapy directly infused into the brain."

**About Upstaza ™(eladocagene exuparvovec)**
Upstaza is a one-time gene replacement therapy indicated for the treatment of patients aged 18 months and older with a clinical, molecular, and genetically confirmed diagnosis of aromatic L–amino acid decarboxylase (AADC) deficiency with a severe phenotype. It is a recombinant adeno-associated virus serotype 2 (AAV2)-based gene therapy, containing the human DDC gene.[1] 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.[2,3]

The efficacy and safety profile of Upstaza has been demonstrated across clinical trials and compassionate use programs.[1] The first patient was dosed in 2010. In clinical trials, Upstaza demonstrated transformational neurological improvements. The most common side effects were initial insomnia, irritability and dyskinesia.

Administration of Upstaza occurs through a stereotactic surgical procedure, a minimally invasive neurosurgical procedure used for the treatment of a number of pediatric and adult neurological disorders. The Upstaza administration procedure is performed by a qualified neurosurgeon in centers specialized in stereotactic neurosurgery.

**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 causing the eyes to roll up in the head, frequent vomiting, behavioral problems, and difficulty sleeping.

The lives of affected children are severely impacted and shortened. Ongoing physical, occupational and speech therapy, and interventions, including surgery, also are often required to manage potentially life-threatening complications such as infections, severe feeding and breathing problems.

**About PTC Therapeutics, Inc.**
PTC is a 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 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: the future expectations, plans and prospects for PTC, including with respect to the expected timing of regulatory review, commercialization and other matters with respect to its products and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; 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 Upstaza, including any regulatory review and potential approvals, commercialization, manufacturing capabilities, 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 Quarterly Report on Form 10-Q and 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 Upstaza.

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.

**Acronyms:**
AADC: Aromatic l-Amino Acid Decarboxylase
BLA: Biologics License Application
FDA: U.S. Food and Drug Administration

References:


1. Tai CH, *et al.* Long-term efficacy and safety of eladocagene exuparvovec in patients with AADC deficiency. *Mol Ther.* 2022;30(2):509-518.
2. 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.
3. 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.