PTC Therapeutics and AADC Family Network Announce First-Ever AADC Deficiency Awareness Day in Massachusetts

October 22, 2020

- Massachusetts signs proclamation declaring Oct.23 as AADC Deficiency Awareness Day -

- PTC hosts Facebook Live event featuring leaders from AADC Family Network, MassBio, and Boston Children's Hospital -

SOUTH PLAINFIELD, N.J., Oct. 22, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) and the AADC Family Network today announced that Oct. 23, 2020, has been officially designated as the first Aromatic L-amino Acid Decarboxylase (AADC) Deficiency Awareness Day in the State of Massachusetts, helping enable parents, caregivers, patient advocacy groups, local legislators, and experts raise awareness, share resources, and work together towards a treatment for this devastating disease.

To commemorate the day, PTC will be hosting a Facebook Live event on Friday, Oct. 23, at 10 a.m. EDT featuring commentary from advocacy partners, local ambassadors and other industry experts, including:

- Bob Coughlin, President and CEO of MassBio
- Kelly Heger, Founder of AADC Family Network & Foundation
- Dr. Irina Anselm, Director, Mitochondrial Program & Co-Director, Neurometabolic Program at Boston Children's Hospital
- Dr. Matthew Klein, Chief Development Officer, PTC Therapeutics

The discussion will focus on how to raise awareness among parents and caregivers, patient advocacy groups, legislators and experts to work toward a treatment to address the high unmet medical need caused by AADC deficiency. Interested members of the public are invited to join the event via Facebook Live stream and can RSVP for the event on Facebook.

The initiative was spearheaded by Kelly Heger, AADC deficiency caregiver and founder of the AADC Family Network & Foundation, along with PTC Therapeutics, MassBio and Boston Children's Hospital. Ms. Heger's daughter Jillian was diagnosed with the rare disease at just 5 months old. Since then, Jillian has required 24-hour care from family members, visiting health care professionals, and inpatient hospital teams. Ms. Heger has been a pioneering advocate for AADC awareness and founded the AADC Family Network & Foundation to help the AADC deficiency community access resources and to unite in the campaign for a cure.

"When Jillian was diagnosed with AADC deficiency, she was one of the first children to receive the diagnosis in the United States," said Kelly Heger, Founder AADC Family Network & Foundation. "As Jillian has progressed on her journey, and as we have learned more about the devastating disease in tandem, raising awareness and finding a cure for the AADC deficiency community has become increasingly important. Declaring Oct. 23 as AADC Deficiency Awareness Day in Massachusetts in partnership with PTC Therapeutics and the State is a significant milestone for the rare disease community, and we are hopeful that it will help propel other advocacy organizations, researchers and policymakers towards our shared goal of finding a cure."

Representative Angelo D'Emilia introduced Resolution HD 5268 which passed the Massachusetts House of Representatives on Oct.1, declaring Oct. 23 as AADC Deficiency Awareness Day.

"I am happy to be supporting Oct. 23 as Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency Day here in Massachusetts," said Representative D'Emilia. "The Heger family is an inspiration and our community is proud to support Kelly as the Founder of the AADC Family Network and all of her dedicated work and advocacy."

"We are honored to partner with other industry leaders and advocates to declare an important awareness day for the AADC deficiency community," said Mary Frances Harmon, SVP, Corporate Relations of PTC Therapeutics. "It is particularly meaningful that Massachusetts is the first state to declare this proclamation, with its vibrant network of top-tier life science companies, talent, and resources constantly working on the next generation of therapeutics for patients. At PTC, we remain committed to developing a gene therapy for this patient population, in hopes of delivering meaningful clinical benefits to individuals with this devastating disease."

AADC deficiency is a rare genetic disorder that affects the brain, causes weak muscle tone[1], and results in delays or failure to reach developmental milestones.[2] Patients with severe AADC deficiency have a high risk of death during childhood and there are currently no approved therapies that address the underlying cause.2 AADC deficiency presents with signs and symptoms similar to more common diseases, and is often under and misdiagnosed by physicians, resulting in a long and challenging diagnosis journey for patients and families.

For additional information about aromatic l-amino acid decarboxylase (AADC) deficiency, please visit:

- For patients/caregivers: https://aboutaadc.com/
- For HCPs: https://aadcinsights.com/

About the AADC Family Network & Foundation

The AADC Family Network & Foundation was originally founded as the ALADD Foundation, Inc. in 1998 and was the first foundation for AADC

(Aromatic L Amino Acid Decarboxylase Deficiency). The foundation was established for the charitable and educational purposes of providing research, medical intervention, support and awareness, and to participate in all services and activities designed to carry out and find a CURE for AADC deficiency.

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.

For More Information:

Investors:
Lisa Hayes
+1 (732) 354-8687
lhayes@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167
jbaj@ptcbio.com

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; PTC's strategy, future operations, future financial position, future revenues and 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 eligible patient base and commercial potential of PTC's products and product candidates; expectations with respect to PTC's gene therapy platform; 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; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; 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.

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.

[1] What is AADC Deficiency? Retrieved from https://aboutaadc.com/what-is-aadc/

[2] PTC Therapeutics. (2020, September 9). *PTC Therapeutics Launches PTC PINPOINT - A Genetic Testing Program for Individuals Suspected of Having Neurotransmitter Disorders* [Press Release] Retrieved from https://ir.ptcbio.com/news-releases/news-release-details/ptc-therapeutics-launches-ptc-pinpoint-genetic-testing-program

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