Dear AADC deficiency Community,

For more than 20 years it has been our mission to bring life-changing medicines to patients with rare disorders. In these challenging times, we are even more committed to ensuring that patients have access to safe and effective treatments.

PTC-AADC is an investigational, one-time gene replacement therapy that, if approved, will be the first and only therapy to treat AADC deficiency to go through the rigorous process for approval through regulatory agencies such as the European Medicines Agency (EMA) and United States (US) Food and Drug Administration (FDA). The efficacy and safety of PTC-AADC has been studied across three separate clinical trials, with the first patient dosed over 10 years ago (2010). Notably, the trials for PTC-AADC represent the largest cohort of AADC deficiency patients ever studied. The results of the trials have been published and presented in peer-reviewed forums, describing the clinical profile of the treatment.

We know families are desperate for a safe and effective treatment that has the potential to change the course of this devastating disease and we are working as quickly as we can to make PTC-AADC available to patients around the world. For a therapy to be approved and available for patient access, development and regulatory review takes time. Research on PTC-AADC has been ongoing for over 10 years, and we are thrilled to now be in the regulatory review stage. Regulatory review is essential to ensure a treatment is rigorously studied, and the safety profile is well understood. Furthermore, this review will ensure that the manufacturing process is reproducible meeting quality standards, and the packaging maintains stability of the treatment

In Europe, the PTC-AADC application for approval has been submitted to the European Medicines Agency (EMA) and is currently under review by the Committee for Medicinal Products for Human Use (CHMP). We expect a decision in the fourth quarter of 2021. In addition, PTC plans to submit a Biologics License Application (BLA) to the US FDA by the end of 2021 to begin the approval process for the US.

We are proud of the progress that we have made toward bringing a potential therapy to AADC deficiency patients worldwide. While we all wish the process could move faster, please know that safety is of upmost importance to us, and we are committed to keeping children with AADC deficiency safe. We will continue to provide updates on our progress. In the meantime, please register on www.aboutaadc.com so that we can keep you informed. Our patient engagement team is available to speak with you and answer questions. They can be reached at 1-833-PTC-HOPE (+1-833-782-4673), or AADCpatientengagement@ptcbio.com.

I am touched by the overwhelming bravery and determination of the families I have met on this journey. We understand what this means for you, and this drives us every day in our work.

Sincerely,

Stuart Peltz, PhD
CEO, PTC Therapeutics