



Dear Members of the AACDC Community,

I'd like to update you on PTC's efforts to make our developing AACDC gene therapy treatment available to patients worldwide. We know how important it is to receive information about our progress in the development of a potential treatment.

I founded PTC in 1998 to develop treatments for rare disorders. More than 20 years later, our mission to bring life-changing medicines to patients hasn't changed. We have grown, expanded and invested in treatments so that we can address even more rare disorders including rapidly advancing our gene therapy program in AACDC Deficiency.

We are currently finalizing the documentation that will be reviewed by regulatory authorities, and we plan to begin regulatory submissions by the end of 2019. Our targeted treatment has been tested in patients in multiple regulated clinical trials- to date we have treated 26 patients. The first patients that were treated have been followed for more than 8 years, making our gene therapy program the most extensively studied gene therapy for AACDC Deficiency. The results of the trials have been published and presented in peer-reviewed forums describing the clinical benefits and safety profile. These robust clinical data will be included in the comprehensive data package that will be submitted to the US (FDA), European (EMA) and Brazilian (ANVISA) regulatory authorities.

Regulatory review is one of the final steps in the process of getting treatments to patients, however once the medicine is approved, sufficient quantities need to be produced with regulated, consistent, quality-controlled methods to meet the demands of the patients who need it. We are expanding our manufacturing process to ensure that patients have access to medicine that meets the high-quality standards established by regulatory authorities worldwide. We are also actively engaging with physicians who will be interested in providing this treatment for their patients.

We understand how important it is for you to have the most accurate, up to date information and to have your voice heard and understood, so please register on www.aboutaadcd.com so that we can continue to keep you informed. Karen Galambos, Global Patient Advocacy is also available to speak with you and answer any questions that you have – you can reach her at +1 732-413-5056 or kgalambos@ptcbio.com.

I can assure you the dedicated employees at PTC truly care. We know the drug development process is a long road, but every day we are motivated by the patients and caregivers like you.

Sincerely,

A handwritten signature in cursive script that reads "Stuart Peltz". The signature is written in dark ink and is positioned above the printed name and title.

Stuart Peltz, PhD
CEO
PTC Therapeutics