ultragenyx
pharmaceutical

February 18, 2020

Dear Members and Friends of the Association for Creatine Deficiencies (ACD),

As our first update in 2020, we hope this finds you and your families doing well and enjoying the year.

First, a sincere congratulations to ACD for being one of 30 patient-led organizations awarded a grant by the Chan Zuckerberg Initiative (CZI) in your quest to accelerate research and drive progress in the fight against rare diseases through the newly established Rare As One Network. It is incredibly exciting to see your focus, drive and tenacity be recognized, and we are very pleased that your important work will be further elevated through the significant support and training provided by CZI.

Since our last letter in September 2019, we are pleased to share with you two updates related to our research on creatine transporter deficiency (CTD).

Creating a clinical development plan is a complex and critical part of the drug development process. As is the case with many other aspects of life, there can be various ways to reach a goal, and in drug development, different paths can be taken on the quest to provide patients with a potential therapeutic. We know that time is invaluable in the lives of rare disease patients and families, and our goal is to be as efficient as possible to help address the unmet needs of patients and families impacted by CTD.

Our goal is to develop a safe and effective treatment for patients with CTD that, if successful, could be made available to the broad patient community as promptly as possible. To that end, the Ultragenyx CTD team has been hard at work evaluating the paths we can take and designing a strategy that we believe will allow us to reach our goal faster.

We have formulated a plan that puts emphasis on two important aspects: safety and speed. Our plan involves expanding pre-clinical activities that will be important and necessary for a streamlined clinical development approach. We are increasing the number of pre-clinical activities and expect to file our Investigational New Drug application in 2021. The UX068 program is a priority for Ultragenyx and we are confident the clinical development path enabled by this strategy ultimately will bring this therapy to the broader CTD community more quickly.

In addition, the Ultragenyx Patient Advocacy team will host an advisory board meeting on Thursday, July 30 in Park City, Utah to meet parents and caregivers of children diagnosed with CTD and better understand CTD from their perspective. We believe the experiences of patients and families are essential to successful rare disease drug development, and advisory boards provide patients and caregivers an opportunity to share knowledge and experiences, and communicate their needs and the needs of the CTD community. This meeting will take place before the CCDS Scientific + Patient Symposium registration activities begin on Thursday. If you are interested in learning more about this advisory board, please contact ACD.

Thank you for your tireless efforts to improve the lives of those impacted by CCDS. We are excited about all that 2020 has to offer and look forward to sharing future updates with you, including progress with the Vigilan Study.

Sincerely,

Camille

Camille L. Bedrosian, MD
Chief Medical Officer and Executive Vice President