September 23, 2019

Dear Members and Friends of the Association for Creatine Deficiencies,

On June 20, we shared with you important news in a joint letter with our colleagues at Lumos Pharma. In it, we announced to the Cerebral Creatine Deficiencies Syndrome (CCDS) community that Ultragenyx was pleased to assume the sponsorship of Vigilan and ensure the continuation of this critically important study. Now three months later, our enthusiasm continues and we are pleased to update you once again.

In the past several weeks, we have worked closely with Lumos Pharma to ensure an efficient, organized, and - most importantly - seamless transition, such that patients, families, and sites will continue their participation in a smooth and fully supported fashion. The Ultragenyx team has connected with all of the physicians, sites, and professionals that support Vigilan on a daily basis. Study transfer activities are continuing well and as planned. In working with our Lumos colleagues, we have found like-minded, passionate, and committed individuals. It has been and continues to be a privilege to work with them, the investigators, site coordinators, and the broader Vigilan community.

On a separate note, we will from time to time update you on important milestones related to Ultragenyx's creatine transporter deficiency (CTD) clinical development program (UX068). This program is in its late preclinical stage, and if activities progress as we hope, we expect to file an Investigational New Drug application with the FDA in 2020. This application is a necessary step before patients can be dosed with an investigational drug treatment as part of a clinical trial.

It is important to note that Vigilan is not an interventional study, meaning that enrolled patients will not receive a study drug as part of their participation, and is therefore separate from the UX068 program. While the two efforts have different objectives, they complement each other; we are confident that what we learn from Vigilan will help to inform future interventional clinical trials.

To complement both the UX068 and Vigilan efforts, Ultragenyx is conducting an online survey to better understand CTD from the perspective of the parent/caregiver. This survey asks questions about the path to diagnosis, symptoms and conditions associated with CTD and how they affect areas such as communication, cognition, behavior, and movement, as well as the impact of the disease on daily life. Since the survey launched in May 2019, 27 participants have completed the survey. The survey will remain open through December 31, 2019 and families from the US, Canada, France, UK, the Netherlands, Germany and Australia are eligible to participate. Please note the survey is available in English only. If you are interested in learning more about the survey, please visit ACD's website.

While this may not have been widely known, Ultragenyx has been committed to discovering and developing a new treatment for CTD for almost a decade. Advancing our program closer toward possible clinical trials and assuming the sponsorship over Vigilan are in line with our commitment to CTD.

We look forward to updating you periodically and thank you for everything you do to support the CCDS community, advance the awareness and knowledge of these conditions, and advocate for increased diagnoses and research. These efforts will be key for future success in developing treatments.

Sincerely,

Camille L. Bedrosian, MD

Chief Medical Officer and Executive Vice President