



June 20, 2019

Dear Association for Creatine Deficiencies,

We promised to update your CCDS/CTD community on the months-long work that the Lumos team has undertaken to secure a new sponsor to continue the advancement of the important Vigilant Study that Lumos Pharma started in 2016. The wonderful news is that this study will carry on under the sponsorship of Ultragenyx, a biopharmaceutical company committed to bringing to patients novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. CTD is one of the diseases that Ultragenyx is pursuing.

"It has been a privilege to work with the wonderful group of CTD caregivers and the exceptional Vigilant study teams who clearly see the important findings that are starting to emerge from this study. To know that it will be continued by our fellow colleagues at Ultragenyx gives us reassurance that this important study will be supported and maintained in the years to come," commented Alex Bruchey, Ph.D., Director Clinical Development at Lumos.

In the next weeks, our two companies will be working to ensure a smooth transition regarding all aspects related of sponsorship of the Vigilant Study sponsor change. Camille L. Bedrosian, M.D., Chief Medical Officer at Ultragenyx, remarked, "Vigilan is an extensive and in-depth study designed to learn about many facets of CTD. The results from this study represent a critical step for the success of any current or future effort to develop a treatment for patients living with CTD. We are enthusiastic about assuming sponsorship of Vigilant, and extend our gratitude to the families and sites who have been involved in the study. We thank these participants for their contributions and time, as well as for their long-term commitment. We look forward to partnering with the patients and sites to continue these efforts and ensure the success of Vigilant."

We know that change and transition can bring up questions and concerns and we have addressed some common topics in a FAQ document that provides helpful information relating to the current and future participation of Vigilant.

In closing, when the Lumos team decided on the Vigilant name for this CTD observational study, we hoped to convey that this was a study that needed to be "vigilant," "watchful" and open to see and capture the many physical and mental changes that occur to an individual living with CTD. By keeping this ambitious course, the scientific and medical community could amass and analyze precious data that can be translated into helping develop future therapies for CTD. Now we pass our knowledge and determinative spirit to the new Vigilant team at Ultragenyx to continue supporting this journey.

Everyone at Lumos would like to thank you all for your commitment to this community that will always have a special place in our hearts. Ultragenyx is excited to take on responsibility for the Vigilant study and looks forward to further deepening its partnership with the CTD community.

Always in hope and admiration,

A handwritten signature in blue ink, appearing to read "Richard Hawkins".

Richard Hawkins
CEO, Lumos Pharma

A handwritten signature in blue ink, appearing to read "Camille L. Bedrosian".

Camille Bedrosian, M.D.
CMO, Ultragenyx

