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April 2024

Dear families living with CDKL5 Deficiency Disorder,

We would like to provide you with a program update as a follow-up from our announcement in November 2023 regarding UX055, our investigational gene therapy program for the treatment of CDD.

We remain actively engaged in the program as our team seeks to optimize the dosing strategy and design of a potential first-in-human study. To do this, we are generating additional pre-clinical data from studies in animal models to increase our understanding of how UX055 affects the expression of CDKL5, the enzyme that is deficient in some cells in patients living with CDD. This additional data will help us better understand both the disease and how UX055 may be able to restore CDKL5 expression in the neurons of patients.

While we are not able to share any timeline at this point, the program is certainly not paused or stopped.

We remain committed to the UX055 program and need additional time to conduct this research before advancing to the next stage of development. We will share more information when it becomes available.

We would also like to share with you upcoming plans of engagement with the CDD community:

- May: We are hosting a meeting with the Ultragenyx Global Gene Therapy Advisory Council. The Council, which includes a representative from the CDD community, is helping us identify gene therapy topics and opportunities to educate and support rare disease communities.
- June 13-15: We are sponsoring the International Foundation for CDKL5 Research's 6th International Family Education and Awareness Conference and several of our team members look forward to attending in person.

Sincerely,

Kristin Voorhees

Kristin Voorhees, MA
Senior Director, Patient Advocacy and Patient Engagement