Ultragenyx, Inc.
60 Leveroni Ct
Novato, CA 94949

October 24, 2024

Wilson Disease Association
Attn: Rhonda Rowland, President
224W 35th Street Ste 500 #676
New York, NY 10001

Dear Wilson Disease Association,

Re: UX701 Clinical Trial Update

Thank you so much for your continued interest in the Ultragenyx Cyprus2+ study of UX701. We recently announced an update on our Phase 1/2/3 Cyprus2+ study of UX701, an investigational gene therapy for the treatment of Wilson disease.

Before we select a final dose to take into the next phase of the study, we are planning to add another cohort (group of participants) at an increased dose with an optimized immunomodulation regimen. Our intent is that this will make gene therapy delivery more efficient and more effective. These types of changes are a normal part of the dose finding process in drug development, particularly for gene therapy programs where patients are only given one dose. UX701 has not been approved by any regulatory authority, and additional research and information are needed to fully evaluate its safety and efficacy.

Ultragenyx is grateful to the Wilson disease community and to the individuals who are participating in the trial as we work toward the goal of bringing new therapies to patients and families. We will be submitting a protocol amendment to regulatory agencies for the additional cohort. We remain committed to keeping the Wilson disease community updated on our progress as we continue with the clinical trial. In the meantime, please reach out to the Patient Advocacy team at patientadvocacy@ultragenyx.com with any questions.

Sincerely,

Kristin Smith
Director, Patient Advocacy