Cyprus^{2+} Gene Therapy for the Treatment of Wilson Disease

ultragenyx pharmaceutical

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Dear Members and Friends of WDA,

We hope the New Year finds you and your families in good health. On behalf of the Ultragenyx Wilson disease study team, I would like to share a brief update on our CYPRUS^{2+} study.

CYPRUS^{2+} is a study to assess safety, tolerability and efficacy of UX701, an investigational gene therapy for the treatment of Wilson disease. UX701 is delivered with a one-time intravenous (IV) infusion. CYPRUS^{2+} uses an innovative seamless Phase 1/2/3 clinical trial design that allows us to progress through the phases of drug development more efficiently. UX701 has been granted Orphan Drug Designation in the United States and the European Union, as well as Fast Track Designation in the United States. This type of research takes time, so it will be a while before we know whether or when UX701 will receive regulatory approval.

The CYPRUS^{2+} study continues to enroll adults living with Wilson disease, currently well-managed on standard of care. We will be enrolling across multiple sites in the United States, Canada, Latin America, Europe and Japan. Please find a full summary of the study at https://clinicaltrials.gov/ct2/show/NCT04884815

I would also invite you to email me at PatientAdvocacy@Ultragenyx.com if you are interested in learning about any of the WD research programs sponsored by Ultragenyx. In addition, if you are interested in learning more about gene therapy, I would be happy share our brochure called Understanding Gene Therapy. You may also find our brochure by searching for “gene therapy” on our Patient Advocacy website at www.UltraRareAdvocacy.com.

We sincerely appreciate that the WD community has been a strong ally as we advance the science. We are committed to providing updates on our research as information becomes available to share.

Warm regards,

Kristin Smith
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Ultragenyx Pharmaceutical Inc.