An Open-Label Phase 1/2 Study to Assess the Safety, Efficacy and Dose of Study Drug UX003 Recombinant Human Beta-glucuronidase (rhGUS) Enzyme Replacement Therapy in Patients With Mucopolysaccharidosis Type 7 (MPS 7)

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UX003-CL201 is an open-label Phase 1/2 study to assess the safety, efficacy, and dose of UX003 in MPS 7 patients via intravenous (IV) administration every other week (QOW) for 36 weeks with up to an additional 36 weeks from the optional continuation period. Up to 5 participants, who are between 5 and 30 years of age inclusive, will be enrolled and treated with UX003.

The initial 12-week treatment period will be followed by a 24-week forced dose titration period to assess the optimal dose. Participants who complete both the initial treatment and forced dose titration periods will continue treatment in a 36- week continuation period.