An Open-Label Treatment Protocol With UX003 rhGUS Enzyme Replacement Therapy for an Advanced Stage MPS 7 Patient

Cohen Children's Medical Center, New Hyde Park, New York, United States
Emergency access granted to treat a single patient with advanced Mucopolysaccharidosis Type 7
with UX003 Recombinant Human Beta-glucuronidase (rhGUS) Enzyme Replacement Therapy via IV
administration every other week (QOW) for up to 144 weeks.