

Meeting Minutes



Institution:	Case Western Reserve University		
Meeting Date:	July 06, 2026		
Meeting Time	10:00 AM Eastern Time		
Meeting Type:	Virtual Platform Teleconference (Remote) Open to the Public		
Members in Attendance:	Member	Voting	Member Type
	Hauke, Caitlyn	Yes	Chair: Biosafety Expert/HGT Expert
	Rastein, Daniel	Yes	Core Member: Biosafety Expert/HGT Expert
	Campbell, Mark	Yes	Core Member: Biosafety Expert/HGT Expert
	Wilson, LaKetta	Yes	Local Unaffiliated Member
	Yun, Yang (unable to log in until 10:18 AM EST)	Yes	Local Unaffiliated Member
	Karlo, Colleen	Yes	Site Contact
Invited Members Not in Attendance:	Member	Voting	Member Type
	Tepfenhart, Benjamin	Yes	Site Contact
Guests:	DiFrancesco, Kathryn		
Staff:	Smith, Jennifer		

Call to Order: The IBC Chair called the meeting to order at 10:01 AM. A quorum was present as defined in the Sabai IBC Charter.

Conflicts of Interest: The IBC Chair reminded all members present to identify any conflicts of interest (COI). No COI was declared by any voting member of the IBC for any of the items on the agenda.

Public Comments: No public comments were made prior to or at the meeting.

Review of Prior Business: None

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Previous Meeting Minutes: Minutes from 6/15/26 were approved by the IBC with no changes. There were no votes against and no abstentions.

New Business:

PI:	Sandhu, Naemat
Sponsor:	Ultragenyx Pharmaceutical
Protocol:	UX701-CL301 A Phase 3, Randomized, Double-blind, Placebo-controlled Study of Adeno-associated Virus Serotype 8-mediated Gene Transfer of Glucose-6-phosphatase in Patients with Glycogen Storage Disease Type Ia
Review Type:	Annual Review
NIH Guidelines Section:	III-C-1

Trial Summary: UX701-CL301 is a first-in-human Phase I/II/III clinical trial sponsored by Ultragenyx Pharmaceutical Inc. and designed to assess the safety and efficacy of rivunatpagene miziparvovec (UX701) in adult participants with Wilson Disease. Rivunatpagene miziparvovec is a recombinant, replication-defective adeno-associated virus (AAV) vector designed to express a truncated human ATP7B protein to restore functional ATP7B in the body. The investigational product (IP) is administered by intravenous infusion.

Biosafety Containment Level (BSL): The study agent rivunatpagene miziparvovec consists of an AAV vector which does not contain any hazardous transgenes which can be classified as a Risk Group 1 (RG1) agent for which Biosafety Level 1 (BSL-1) may be considered. However, rivunatpagene miziparvovec is manufactured in the presence of a helper virus, therefore the AAV may be classified as an RG2 agent for which Biosafety Level 2 (BSL-2) is the recommended containment level. The administration of this agent in a clinical setting further requires compliance with the OSHA Bloodborne Pathogen Standard (29 CFR 1910.1030).

Risk Assessment and Discussion:

- The Committee reviewed the clinical trial Sponsor's study documents and the Sabai-generated comprehensive study-specific Risk Assessment which collectively provided a thorough description of the recombinant or synthetic nucleic acid molecules (investigational product/s) and the proposed clinical research activities involving the IP.
 - In summary, the primary risks in this clinical trial include potential occupational exposure from accidental spills or splashes of the IP during preparation and/or administration procedures and needlesticks due to the use of needles during preparation and/or administration. These potential risks are mitigated through a combination of relevant staff training, safe clinical practices (including Standard Precautions and sharps safety) and

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use of appropriate PPE (as prescribed in the Risk Assessment and documented in the IBC submission package).

- The Site confirmed that only study personnel who have been educated on the potential biohazards and the precautions to be taken when working with the IP will handle the IP or any materials contaminated by the IP.
 - The Site confirmed that study personnel are sufficiently trained in the practices and techniques required to safely work with the IP.
 - The Site confirmed that staff members receive Bloodborne Pathogens training.
 - Occupational Health Recommendations: None
 - The Committee had no additional significant comments or recommendations regarding the description of the potential risks and occupational exposure hazards associated with handling the IP in this clinical trial, or the proposed mitigation strategies, as detailed in the Risk Assessment.
- The Committee reviewed the Site's facility details, relevant study-specific procedures and practices, the Annual Review Report, and other applicable information provided by the Site for the purposes of the IBC review.
 - The Site verified that the information provided by the Chair was accurate.
 - The Committee noted that the Exhaust Alarm Verification test had not been completed in the current BSC certification report, though it should be tested in accordance with applicable NSF testing standards. The Committee recommended that the Site ensure that the Exhaust Alarm Verification test is performed during the next BSC certification.

Motion: A motion of Full Approval for the study at BSL-2 was passed by unanimous vote. There were no votes against and no abstentions.

- Contingencies stated by the Committee: None
- Stipulations stated by the Committee: None

Review of Incidents: Nothing to report.

IBC Training: Nothing to report.

Reminder of IBC Approval Requirements.

Adjournment: The IBC Chair adjourned the meeting at 10:29 AM.

Post-Meeting Pre-Approval Note: None