

INSTITUTIONAL BIOSAFETY COMMITTEE REVIEW

MEETING MINUTES

Meeting Date: Monday, July 20, 2026
Time: 9:00 am Central Time
Location: Zoom Teleconference
Institution: Northwestern University, Chicago, IL
Principal Investigator: Danny Bega, MD, MSCI
Protocol: Ultragenyx Pharmaceutical, Inc., UX701-CL301
NCT Number: NCT04884815
Meeting Type: Continuing Review of Protocol and Site
Title: A Randomized, Double-Blind, Placebo-Controlled, Multicenter, Seamless, Adaptive, Safety, Dose-Finding, and Phase 3 Clinical Study of UX701 AAV-Mediated Gene Transfer for the Treatment of Wilson Disease

1. Call to order:

The Meeting was called to order at 9:25 am Central Time.

2. Introductions and orientation:

Introductions were made and the Chair oriented members to the meeting procedures.

3. Declaration of quorum:

Five voting members were present, including two local members unaffiliated with the institution. Also present were three Institutional Representatives and IBC Services staff. The Chair declared that a quorum was present.

4. Conflict of Interest:

The Chair requested that voting members report any conflict of interest regarding this meeting. No conflicts of interest were reported.

5. Public posting:

An Institutional Representative confirmed that notice of the meeting was publicly posted. No public comments were received by the site or the Committee regarding this review.

6. Approval of previous meeting minutes:

Minutes Approved - YES: 5 NO: 0 ABSTAIN: 0

7. Review of proposed research:

The Chair provided an overview of the protocol and status of the study.

The Chair noted changes since the last review.

8. Determination for biosafety level and period of IBC oversight:

The Committee previously determined that **BSL-1 containment facilities and practices plus Standard Precautions** are required for UX701, since it consists of a recombinant adeno-associated virus administered in a clinical setting. The Committee reaffirmed this determination.

The Committee previously determined that IBC oversight will continue for **3 months after the last subject's last dose of UX701 locally**, provided that other biosafety criteria for study closure are also met. The Committee reaffirmed this determination.

9. Vote on the Protocol:

The Committee voted for the following determination on the Protocol:

X	APPROVED
	CONDITIONALLY APPROVED
	TABLED
	DISAPPROVED

DETERMINATION VOTE - YES: 5 NO: 0 ABSTAIN: 0

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10. Review of proposed facilities and practices:

The Chair provided an overview of the arrangement for the facilities and practices.

Points of Discussion:

1. The Committee noted that the Bioquell Compounding Aseptic Containment Isolator was due for recertification at the end of June 2026, and acknowledged that IBC Services had previously asked Institutional staff members to submit the newer certification to IBC Services, with no response to this request.
2. The Committee determined the following as a Condition of Approval:
 - a. The Institution must submit a current Bioquell CACI Certification (including the HEPA filter certification results) to IBC Services, and this new Certification must indicate that the Bioquell CACI and HEPA filter passed all required tests.
3. The Committee determined that the Bioquell CACI must not be used for study agent preparation until the designated member of the IBC (IBC Chair) confirms that this piece of equipment is working properly.
4. The Committee noted that the Biological Safety Cabinets in the IDS Pharmacy, which were most recently certified in November 2025 are acceptable to use for study agent preparation.
5. The Committee noted that the Biohazard Sign for the Galtier location and recommended that the Institution confirm how this sign is posted, and that if possible a photo of it posted be submitted to IBC Services.
6. The Principal Investigator confirmed that the Galter and Arkes buildings just across the street from each other and are connected by a walkway bridge.
7. The Committee recommended that the Institution confirm that the Institution's Environmental Health and Services staff move biohazardous waste from the Arkes dosing floor to the Feinberg and Olson Loading Docks where it is ultimately picked up by the commercial biohazardous waste hauler.

11. Site requirements:

The Chair reviewed training and communication requirements for maintaining IBC approval with the Institutional Representatives.

12. Vote on the Site:

The Committee voted for the following determination on the Site:

	APPROVED
X	CONDITIONALLY APPROVED
	TABLED
	DISAPPROVED

For a vote of CONDITIONALLY APPROVED, the following condition must be met no later than August 20, 2026, in order to maintain IBC approval:

- a. A current Bioquell CACI Certification (including the HEPA filter certification results) must be submitted to IBC Services, and this new Certification must indicate that the Bioquell CACI and HEPA filter passed all required tests.

DETERMINATION VOTE - YES: 5

NO: 0

ABSTAIN: 0

13. Advice to the Institution: None.

14. Meeting adjourned: The meeting was adjourned at 9:31 am Central Time.

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15. Post-meeting notes:

1. The Committee noted that the BSCs in the Galter IDS Pharmacy, most recently certified in November 2025, were due for recertification in May 2026 and recommended that updated Certifications be submitted to IBC Services.
2. The condition of approval was met on the following date:

Resolution of condition for Conditional Approval:

Condition No.	Resolution	Date of Resolution	Approved by Chair
a			

Documents reviewed:

Agenda
Protocol, Amendment 9, dated 01-29-2025
Investigator's Brochure, Edition 7.0, dated 01-29-2025
Pharmacy Manual, Version 10.0, dated 03-18-2025
Biological Risk Assessment and Summary, updated 04-08-2025
Research Modification Evaluation, Change in Dosing Location, dated 06-15-2026
Site Map, Arkes Building, 14th Floor, dated 06-11-2026
Site Map, Galter, 17th Floor, IDS Pharmacy, updated 11-20-2025
Site Map, Feinberg and Olson Loading Docks, received 10-16-2018
Site Inspection Checklist, Non-Cell Therapies, expires 03-05-2027, updated 06-12-2026
Photos, Arkes 14th Floor, dated 05-29-2026
Photos, Galter, 17th Floor, IDS Pharmacy, dated 04-07-2026
Photos, Feinberg and Olson Loading Dock, dated 03-25-2026
Biohazard Sign, UX701, dated 06-12-2026
Biological Safety Cabinet Certifications, Galter 17th Floor, expire 05-2026
Bioquell CACI Certification, Galter 17th Floor, dated 06-17-2025
Bioquell CACI Certification, HEPA, Galter 17th Floor, expires 05-2026
SOP, Biosafety for UX701, dated 06-12-2026
Training, Shipping Certification, expires 06-05-2027
CRRF, dated 04-02-2026
Prior Meeting Minutes, Continuing, dated 07-02-2025