

# INSTITUTIONAL BIOSAFETY COMMITTEE REVIEW

## MEETING MINUTES

**Meeting Date:** Wednesday, July 2, 2025  
**Time:** 8:00 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 8:01 am Central Time.

### 2. Introductions and orientation:

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

### 3. Declaration of quorum:

Six voting members were present, including two local members unaffiliated with the institution and the Institution's Biosafety Officer. 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: 6                      NO: 0                      ABSTAIN: 0

### 7. Review of proposed research:

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

The Chair provided an overview of 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: 6                      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 they have previously discussed a recommendation about relocating a phone that is next to a sink and the Biosafety officer confirmed staff members are aware making sure areas around sinks are clear of excess items.
2. The Committee noted that both serial numbers on the Bioquell Compounding Aseptic Containment Isolator (CACI) Certification Report apply to this piece of equipment.
3. The Committee noted that they have previously discussed the ability of the Bioquell CACI to be easily switched from positive to negative pressure and the Biosafety Officer confirmed that [REDACTED] staff members have policies and SOPs that they follow when preparing agents in the Bioquell.
4. The Committee confirmed that the most recent Biological Safety Cabinet Certifications demonstrate testing of the supply and exhaust interlock since this testing is part of the "Site Installation Assessment Test."
5. The Committee noted they have previously discussed the sticky mat in the [REDACTED].
6. The Committee recommended that items not be stored on the top of biohazardous waste containers.
7. The Biosafety Officer confirmed that the [REDACTED] replaced the small biohazard sticker on the transport container with a larger, larger more visible one. The Committee recommended that the photo of this transport container be updated in the Photos document to reflect this information.

### **11. Site requirements:**

The Chair reviewed training and communication requirements for maintaining IBC approval with the Biosafety Officer.

### **12. Vote on the Site:**

The Committee voted for the following determination on the Site:

X	APPROVED
	CONDITIONALLY APPROVED
	TABLED
	DISAPPROVED

DETERMINATION VOTE - YES: 6                      NO: 0                      ABSTAIN: 0

### **13. Advice to the Institution:** None.

### **14. Meeting adjourned:** The meeting was adjourned at 8:20 am Central Time.