Meeting Minutes

Institution:	UC - Irvine
Meeting Date:	Wednesday, September 3, 2025
Meeting Time	9:00 AM Pacific Time
Meeting Type:	Virtual Platform Teleconference (Remote) Open to the Public

Members in Attendance:

Member	Voting	Member Type
Noriea, Nicholas	Yes	Chair: Biosafety Expert/HGT Expert
Campbell, Mark	Yes	Core Member: Biosafety Expert/HGT Expert
Rastein, Daniel	Yes	Core Member: Biosafety Expert/HGT Expert
Makmura, Linna	Yes	Local Unaffiliated Member
Zhou, Jennifer	Yes	Local Unaffiliated Member
Abegania, Judi	Yes	Biological Safety Officer

Invited Members Not in Attendance:

Member	Voting	Member Type
Tafoya, Christine	Yes	Biological Safety Officer

Guests:	Azadbadi, Zahra
Staff:	Stark, Casey

Call to Order: The IBC Chair called the meeting to order at 9:00 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

Previous Meeting Minutes: Minutes from 8-1-25 were approved with no changes.

New Business:

PI:	Kedhar, Sanjay MD
Sponsor:	Krystal Biotech, Inc.
Protocol:	KB801-01 A Phase 1/2, Multicenter, Double-Masked, Placebo-Controlled Study of KB801 in Subjects with Stage 2 or 3 Neurotrophic Keratitis
Review Type:	Initial Review
NIH Guidelines Section:	III-C-1

Trial Summary: KB801-01 is a randomized, double-masked, placebo-controlled Phase I/II trial sponsored by Krystal Biotech, Inc. and designed to assess the safety, tolerability, and preliminary efficacy of KB801 in adult participants with Stage 2 or 3 neurotrophic keratitis (NK). KB801 is a replication-defective, non-integrating herpes simplex virus type 1 (HSV-1)-based vector engineered to deliver functional, full-length human nerve growth factor (NGF). The investigational product (IP) is administered topically into the eye

Biosafety Containment Level (BSL): Because the study agent KB801 is based on a recombinant Risk-Group 2 herpesvirus containing more than two-thirds of the native genome, BSL2 containment is considered the default biocontainment level under the *NIH Guidelines*.

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. These potential risks are mitigated through a combination of relevant staff training, safe clinical practices (including Standard Precautions and sharps safety) and 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: While the Sponsor does not provide specific occupational health recommendations, given the theoretical risk for ICP4

complementation leading to replication-competent KB801, study staff who are immunocompromised or have active HSV-1 infections (e.g., cold sores) should consider consulting Occupational Health or their primary care physician prior to handling KB801 or materials that have come into contact with KB801.
    - 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 PI's credentials 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 discussed the occupational health guidelines and special precautions related to the study agent and noted that the biohazard sign does not contain the study agent name or special precautions statement. The Pharmacy Site Representative confirmed that BSL2 study agents and special precautions can be added to the pharmacy biohazard sign. The biohazard sign will be administratively updated following institutional guidelines.
    - The Committee stipulated that the Site confirm that study participants and/or at-home administrators will be provided with the occupational health recommendations that should be followed when handling the study agent.
    - The Site Checklist will be administratively updated to include that biohazard signage is only posted in pharmacy areas per institutional policy.
    - The Sabai AP indicated that the Site confirmed that study agent will be prepared on the clean benchtop next to the sink. The Committee had no concerns.

Motion: A motion of Approval with Stipulations 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:
    - Site confirm that occupational health recommendations will be communicated to study participants and/or at-home dosing administrators by 10/3/2025. The Committee agreed that resolution of this stipulation can be approved following review by the AP

PI:	Goyal, Namita MD
Sponsor:	uniQure biopharma B.V.
Protocol:	AMT-162-001 A Phase 1/2, Multi-Center, Single Ascending Dose Study to Evaluate the Safety, Tolerability, and Exploratory Efficacy of Intrathecally Administered Gene Therapy AMT-162 in Adult Participants with SOD1 Amyotrophic Lateral Sclerosis (SOD1-ALS)
Review Type:	Annual Review
NIH Guidelines Section:	III-C-1

Trial Summary: AMT-162-001 (EPISOD1) is a Phase 1/2 clinical trial sponsored by uniQure biopharma B.V. and designed to assess the safety, tolerability, and efficacy of AMT-162, a recombinant adeno-associated virus (AAVrh10) genetically modified to express a silencing artificial micro-RNA targeting superoxide dismutase 1 (SOD1), in participants with amyotrophic lateral sclerosis (ALS) with SOD1 pathogenic variations. The investigational product (IP) is administered by intrathecal administration.

Biosafety Containment Level (BSL): The study agent is based on a replication-defective, recombinant Risk Group 1 AAV virus, requiring the use of BSL-1 containment under the *NIH Guidelines* for research.

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 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 biosafety cabinet (BSC) certification for one of the preparation areas is expired. The Committee stipulated that the Site provide an updated BSC certification report.
  - The Site Checklist will be administratively updated to include that biohazard signage is only posted in pharmacy areas per institutional policy.
  - The Committee discussed the biosafety containment level for this study and agreed that BSL-1 (plus Standard Precautions) would be appropriate. At the specific request of the Site, the Committee agreed to approve the study at BSL-2 to allow for this study to be conducted in a manner that was consistent with other clinical studies approved at the Site.

Motion: A motion of Approval with Stipulations 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:
  - Site provide updated biosafety cabinet certification report by 10/3/2025. The Committee agreed that resolution of this stipulation can be approved following review by the AP

PI:	Habib, Ali MD
Sponsor:	Arcellx, Inc.
Protocol:	ARC-311 A Phase 1 Study of Anitocabtagene Autoleucel for the Treatment of Subjects with Non-Oncology Plasma Cell-Related Diseases
Review Type:	Annual Review
NIH Guidelines Section:	III-C-1

Trial Summary: ARC-311 is an open-label Phase I dose-escalation study sponsored by Arcellx, Inc. designed to assess the safety, tolerability, and preliminary efficacy of anitocabtagene autoleucel

(anito-cel) in subjects with generalized myasthenia gravis (GMG) in whom immunosuppressive therapy is clinically indicated. Anito-cel (formerly CART-ddBCMA) consists of autologous T cells that have been genetically modified ex vivo with a replication-deficient lentiviral vector to express a chimeric antigen receptor (CAR) against B-cell maturation antigen (BCMA). The investigational product (IP) is administered by intravenous infusion

Biosafety Containment Level (BSL): The study agent anitocabtagene autoleucel consists of primary human cells that have been transduced with a recombinant derivative of a Risk Group 3 lentiviral vector. BSL2 containment is the recommended minimum biocontainment level under the *NIH Guidelines*.

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. These potential risks are mitigated through a combination of relevant staff training, safe clinical practices (including Standard Precautions and sharps safety) and 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 Chair noted that the Site is proposing to add the Cell Therapy Lab (CTL) as a storage and preparation location. The Committee had no concerns.

- The Chair noted the form describes that if manipulations outside of standard preparation procedures are performed, they will be done within a biosafety cabinet (BSC). The Chair noted the current Facility Details Report form limitations that prevent BSC certification reports from being uploaded when benchtop preparation is selected. The Chair recommended for future reviews where benchtop preparation takes place, the BSC certification reports be uploaded as an event attachment within the portal system. The Sabai AP additionally confirmed that both preparation locations have current BSC reports available.
- The Site Checklist will be administratively updated to include that biohazard signage is only posted in pharmacy areas per institutional policy.

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

PI:	Habib, Ali MD
Sponsor:	Novarits
Protocol:	CYTB323O12101 An open-label, multi-center, phase I/II study to assess safety, efficacy, and cellular kinetics of YTB323 in participants with treatment-resistant generalized myasthenia gravis
Review Type:	Annual Review
NIH Guidelines Section:	III-C-1

Trial Summary: CYTB323O12101 is an open-label, multi-center, non-confirmatory Phase I/II study sponsored by Novartis Research and Development designed to assess the safety and efficacy of YTB323 (rapcabtagene autoleucel) in adults with treatment-resistant generalized myasthenia gravis (gMG). YTB323 is an autologous T cell product engineered to express a chimeric antigen receptor (CAR) targeting CD19. The investigational product (IP) is administered by intravenous infusion.

Biosafety Containment Level (BSL): Because the study agent YTB323 consists of primary human cells transduced with a lentiviral vector, BSL2 containment is the recommended minimum biocontainment level under the *NIH Guidelines*.

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. These potential risks are mitigated through a combination of relevant staff training, safe clinical practices (including Standard Precautions and sharps safety) and 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 Chair noted that the Site is proposing to add the Cell Therapy Lab (CTL) as a storage and preparation location. The Committee had no concerns.
  - The Committee discussed that if study agent is prepared in the pharmacy it would occur within the biosafety cabinet (BSC) as the Sponsor indicates that all preparation will require infusion bag penetration. The Facility Details report will be administratively updated to indicate BSC preparation in the pharmacy areas.
  - The Site Checklist will be administratively updated to include that biohazard signage is only posted in pharmacy areas per institutional policy.

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 10:34 AM

Post-Meeting Pre-Approval Note: None