Institutional Biosafety Committee

Meeting Minutes

Tuesday, June 24, 2025 3pm Abigail Wexner Research Institute or Virtual via Webex

National Institutes of Health Office of Science Policy has provided guidance on Institutional Biosafety Committee (IBC) meetings and minutes to document and capture that the IBC has adequately fulfilled their responsibilities as defined in Section IV-B-2 of the NIH Guidelines. As described in the March 28, 2025, Guide Notice, NCH AWRI IBC is committed to complying with the transparency aims of the NIH Guidelines and IBC minutes are accessible to the public. Meetings and minutes will include application reviews with particular focus on the following items:

1. *Agent characteristics (e.g. virulence, pathogenicity, environmental stability)*
2. *Types of manipulations planned*
3. *Source of the nucleic acid sequences (e.g., species)*
4. *Nature of the nucleic acid sequences (e.g., structural gene, oncogene)*
5. *Host(s) and vector(s) to be used*
6. *Whether an attempt will be made to obtain expression of a transgene, and if so, the function of the protein that will be produced*
7. *Containment conditions to be implemented (biosafety level and any special provisions)*
8. *Applicable section of the NIH Guidelines the research falls under (e.g. Section III-D-1, Section III-E-3, etc.)*
9. *Verification that the PI and laboratory staff performing the research have been appropriately trained in the safe conduct of the research*

Call to Order:	Meeting called to order by chair at 3:02 PM. Meeting adjourn 3:49 PM.
Committee members in attendance:	Carmen Arsuaga, Allison Bradbury, Alex Brown, Tara Chinn, Dakota Esterline, Sumit Ghosh, Amit Kapoor, Paul Martin, Christopher Montgomery, Addie Moore, Mark Peeples, and Chack-Yung Yu
Members excused:	Katie Campbell, Kevin Cassady, and Mary Walker
Guests in attendance:	Kelly Fallon
Approval of Minutes:	May 27 2025 meeting minutes approved
Action Register:	The Action Register was reviewed and the following approved: Amendments Approved:

Protocol # MS18_IBS00000530 - **Allison Bradbury "AAV delivery to the central nervous system "**

Protocol # MS4_IBS00000501 - **Scott Harper "AAV-mediated gene therapy for dominant myopathies and neurodegenerative diseases"**

Protocol # MS15_IBS00000554 - **Rachid Drissi "Targeting Telomeres and Telomerase, and Epigenetic Alterations in Pediatric Brain Tumors"**

Protocol # MS17_IBS00000530 - **Allison Bradbury "AAV delivery to the central nervous system "**

Protocol # MS12_IBS00000672 - **Karen McCoy "A Phase 1/2 Dose-escalation Study Evaluating the Safety, Tolerability, and Efficacy of VX-522 in Subjects 18 Years of Age and Older With Cystic Fibrosis and a CFTR Genotype Not Responsive to CFTR Modulator Therapy"**

Protocol # MS8_IBS00000559 - **Katherine Miller "Research handling of human tissues in the Institute for Genomic Medicine"**

Protocol # MS2_IBS00000783 - **Liubov Gushchina "In-vitro and In-vivo Gene Therapies for Neuromuscular Disorders"**

Protocol # MS4_IBS00000552 - **Paul Martin "Role of Glycosylation in Neuromuscular Development"**

Protocol # MS2_IBS00000907 - **Beth Kozel "Kozel Lab IBC Protocol"**

Protocol # MS2_IBS00000921 - **Jenny Barker "Local management of MRSA"**

Protocol # MS1_IBS00000659 - **Dean Lee "Sample processing and analysis in the Immune Monitoring Core"**

Contingencies Approved:

Protocol # IBS00000971 - **Anne Connolly "A Seamless Phase 1/3, Multicenter, Single Dose Systemic Gene Transfer Study to Evaluate the Safety, Tolerability, and Efficacy of SRP-9005 in Limb Girdle Muscular Dystrophy Type 2C/R5 Subjects (COMPASS) "**

Protocol # IBS00000986 - **Christopher Beatty "NGN-401 in Rett Synd"**

Protocol # MS4_IBS00000546 - **Yusen Liu "Immune Response to Microbial Infections"**

New Business: The IBC meeting was held as a closed session to ensure that only authorized individuals were present on the NCH campus, in order to uphold patient privacy and maintain the highest standards of safety and security. IBSO update no incidents to report.

Protocol # IBS00000996 - Flanigan, Kevin - "INS1201-101 (ASCEND)"

1. **Agent characteristics (e.g. virulence, pathogenicity, environmental stability):** Use of risk group 1 agent at biosafety level 1 without animals.
2. **Types of manipulations planned:** Study involves in vivo delivery of non-replicating, recombinant RG1 vector
3. **Source of the nucleic acid sequences (e.g., species):** Muscle specific MHCK7 promoter, SV40 Intron, microdystrophin transgene, SV40 polyadenylation signal, AAV2 ITRs.
4. **Nature of the nucleic acid sequences (e.g., structural gene, oncogene):** MHCK7 promoter and micryodystrophin transgene
5. **Host(s) and vector(s) to be used:** Human research participants
6. **Whether an attempt will be made to obtain expression of a transgene, and if so, the function of the protein that will be produced:** Expression of microdystrophin.
7. **Containment conditions to be implemented (biosafety level and any special provisions):** Biosafety level 1
8. **Applicable section of the NIH Guidelines the research falls under (e.g. Section III-D-1, Section III-E-3, etc.):** III-C-1, Appendix G-II-A.
9. **Verification that the PI and laboratory staff performing the research have been appropriately trained in the safe conduct of the research:** Verified the PI and laboratory staff performing the research have been appropriately trained in the safe conduct of the research

Major Points of Discussion: Withheld with minor contingencies including updates to the technical abstract, preparation and transportation of the study agent, replication defective verification procedures, waste management considerations, and associated training needs be addressed prior to approval of protocol.

The Institutional Biosafety Committee has determined the status of the protocol to be: **Withheld with Contingencies - Minor - (At time of meeting) : Approved (current)**

Protocol # IBS00001003 - Dr. De Los Reyes, Emily - "Phase I/II Intrathecal Gene Delivery Clinical Trial of scAAV9.P546.SLC6A1 for SLC6A1 neurodevelopmental disorder"

1. **Agent characteristics (e.g. virulence, pathogenicity, environmental stability):**
   Use of risk group 1 agent at biosafety level 1 without animals.
2. **Types of manipulations planned:**
   Study involves in vivo delivery of non-replicating, recombinant RG1 vector
3. **Source of the nucleic acid sequences (e.g., species):**
   A P546 promoter, SV40 Intron, SLC6A1 transgene, bGH polyadenylation signal, AAV2 ITRs.
4. **Nature of the nucleic acid sequences (e.g., structural gene, oncogene):**
   A P546 promoter and SLC6A1 transgene
5. **Host(s) and vector(s) to be used:**
   Human research participant
6. **Whether an attempt will be made to obtain expression of a transgene, and if so, the function of the protein that will be produced:**
   SLC6A1 transgene expression to replace the mutation
7. **Containment conditions to be implemented (biosafety level and any special provisions):**
   Biosafety level 1
8. **Applicable section of the NIH Guidelines the research falls under (e.g. Section III-D-1, Section III-E-3, etc.):**
   III-C-1, Appendix G-II-A.
9. **Verification that the PI and laboratory staff performing the research have been appropriately trained in the safe conduct of the research:**
   Verified the PI and laboratory staff performing the research have been appropriately trained in the safe conduct of the research

Major Points of Discussion: Withheld with minor contingencies including updates to the technical abstract, preparation and transportation of the study agent, replication defective verification procedures, planned experiment information, equipment and facilities utilized, waste management considerations, and associated training needs be addressed prior to approval of protocol.

The Institutional Biosafety Committee has determined the status of the protocol to be: **Withheld with Contingencies - Minor - (At time of meeting) : Approved (current)**