Institutional Biosafety Committee

Meeting Minutes

Tuesday, July 29, 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 3:01pm. Meeting adjourn 4:05pm.
Committee members in attendance:	Carmen Arsuaga, Allison Bradbury, Kevin Cassady, Tara Chinn, Dakota Esterline, Sumit Ghosh, Amit Kapoor, Addie Moore, Mark Peeples, and Chack-Yung Yu
Members excused:	Alex Brown, Katie Campbell, Paul Martin, Christopher Montgomery, and Mary Walker
Guests in attendance:	Kelly Fallon
Approval of Minutes:	June 24, 2025, meeting minutes approved.
Action Register:	The Action Register was reviewed and the following approved:

Amendments Approved:

Protocol # MS4_IBS00000611 -Nicole Skinner "Investigation of B cell responses to hepatitis C virus"

Protocol # MS14_IBS00000507 -Genevieve Kendall "Therapeutic Strategies for Pediatric Sarcomas"

Protocol # MS14_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 # MS2_IBS00000621 -Kevin Cassady "Cancer Immunotherapy "

Protocol # MS5_IBS00000473 -Yusen Liu "Regulation of host defense against bacterial infection"

Protocol # MS3_IBS00000620 -Amit Kapoor "Study of rodent hepaciviruses, pegiviruses, torque tenoviruses and hepeviruses"

Protocol # MS13_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"

Contingencies Approved:

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

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

New Business:

Meeting Purpose: The IBC meeting was held as a closed session to ensure that only authorized individuals were present on the NCH campus, to uphold patient privacy and maintain the highest standards of safety and security.

Details:

Amendment # IBA2_IBS00000848 - Stacy, Mitchel - "Amendment 2 for IBCSC Protocol #IBS00000848"

1. **Agent characteristics (e.g. virulence, pathogenicity, environmental stability):**
   Use of human source material at biosafety level 2.
2. **Types of manipulations planned:**
   Study involves using human source material and isotopes
3. **Source of the nucleic acid sequences (e.g., species):**
   N/A
4. **Nature of the nucleic acid sequences (e.g., structural gene, oncogene):**
   N/A
5. **Host(s) and vector(s) to be used:**
   N/A

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:**
   N/A
7. **Containment conditions to be implemented (biosafety level and any special provisions):**
   Biosafety Level 2
8. **Applicable section of the NIH Guidelines the research falls under (e.g. Section III-D-1, Section III-E-3, etc.):**
   Appendix G-II-B
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 major contingencies including updates to location of work, transportation, storage, mitigation strategies, and disposal procedures to be addressed prior to approval.

The Institutional Biosafety Committee has determined the status of the protocol to be: **Withheld with Contingencies - Major**

Protocol # IBS00001006 - Mann, Nina - "Genetic landscape of childhood kidney disease"

1. **Agent characteristics (e.g. virulence, pathogenicity, environmental stability):**
   Use of risk group 2 agent at biosafety level 2.
2. **Types of manipulations planned:**
   Study involves in vitro delivery of non-replicating, recombinant RG2 vector
3. **Source of the nucleic acid sequences (e.g., species):**
   WT1; KMT2C, KMT2D, EP300, RBBP5, WDR5; NPHS1, NPHS2, NEPH1;U2AF65; Renilla, luciferase
4. **Nature of the nucleic acid sequences (e.g., structural gene, oncogene):**
   Study of known and novel genes that can cause childhood chronic kidney disease.
5. **Host(s) and vector(s) to be used:**
   Human cell lines
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:**
   Gene of interest transgene expression to study chronic kidney diseases
7. **Containment conditions to be implemented (biosafety level and any special provisions):**
   Biosafety level 2

8. **Applicable section of the NIH Guidelines the research falls under (e.g. Section III-D-1, Section III-E-3, etc.):**
   Appendix G-II-B; section III-D-3
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 abstract, disposal practices, documentation of regulatory elements, training requirements, and mitigation strategies to be addressed prior to approval.

The Institutional Biosafety Committee has determined the status of the protocol to be: **Withheld with Contingencies - Minor**

Protocol # IBS00001016 - Mo, Alisa - "Identification of genes and disease mechanisms involved in neurodevelopmental disorders"

1. **Agent characteristics (e.g. virulence, pathogenicity, environmental stability):**
   Use of risk group 2 agent at biosafety level 2.
2. **Types of manipulations planned:**
   Study involves in vitro delivery of non-replicating, recombinant RG2 vector
3. **Source of the nucleic acid sequences (e.g., species):**
   SHANK3, NRXN1, EHMT1, REST, CHD2
4. **Nature of the nucleic acid sequences (e.g., structural gene, oncogene):**
   Gene associated with genetic mutations
5. **Host(s) and vector(s) to be used:**
   Human cell lines
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:**
   Genetic manipulation to generate mutations of interest in cell lines and correction of known mutations.
7. **Containment conditions to be implemented (biosafety level and any special provisions):**
   Biosafety level 2
8. **Applicable section of the NIH Guidelines the research falls under (e.g. Section III-D-1, Section III-E-3, etc.):**
   Appendix G-II-B; section III-D-3
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 major contingencies including updates to abstract, disposal procedures, mitigation strategies, training requirements, and procedure clarification to be addressed prior to approval.

The Institutional Biosafety Committee has determined the status of the protocol to be: **Withheld with Contingencies - Major**

Protocol # IBS00001008 - Peeples, Mark - "Human respiratory viruses and primary human respiratory cell culture"

1. **Agent characteristics (e.g. virulence, pathogenicity, environmental stability):**
   Use of risk group 2 agents at biosafety level 2.
2. **Types of manipulations planned:**
   Study involves in vitro and in vivo procedures to study proteins, antibodies, and host cell infection.
3. **Source of the nucleic acid sequences (e.g., species):**
   RSV genes, fluorescent protein marker genes, enzymatic marker genes, cystic fibrosis chloride channel, micro RNAs targeting to prevent replication in specific cell types
4. **Nature of the nucleic acid sequences (e.g., structural gene, oncogene):**
   Genes encoding for proteins of interest related to respiratory viruses.
5. **Host(s) and vector(s) to be used:**
   Cell lines and in vivo studies
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 cellular proteins, viral proteins, small hairpin RNAs, CRISPR and guide RNAs in transduced cells. Viral and cellular genes into expression plasmids for stable production of protein in mammalian cells.
7. **Containment conditions to be implemented (biosafety level and any special provisions):**
   Biosafety level 2
8. **Applicable section of the NIH Guidelines the research falls under (e.g. Section III-D-1, Section III-E-3, etc.):**
   Appendix G-II-A; Appendix G-II-B; Section III-D-1, III-D-4
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 abstract, location of work, source of materials, transportation, mitigation strategies, and training requirements to be addressed prior to approval.

The Institutional Biosafety Committee has determined the status of the protocol to be: **Withheld with Contingencies - Minor**

Protocol # IBS00001012 - Patel, Anup - "ETX-DS-002 in Dravet"

1. **Agent characteristics (e.g. virulence, pathogenicity, environmental stability):**
   Use of risk group 1 agent at biosafety level 1.
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):**
   eTFSCN1A (which consists of zinc-finger DNA-binding domain (DBD) fused to a transcriptional activator domain, VP64, via short linker sequence); regulatory element (REGABA) promoter; and SCN1A mRNA
4. **Nature of the nucleic acid sequences (e.g., structural gene, oncogene):**
   Gene associated with genetic mutations including Dravet Syndrome
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:**
   Designed to promote increased production of the SCN1A gene.
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.):**
   Appendix G-II-B; Section III-C
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 abstract, transportation, sample collection, location of work, mitigation strategies, and disposal procedures to be addressed prior to approval.

The Institutional Biosafety Committee has determined the status of the protocol to be: **Withheld with Contingencies - Minor**

Old Business: