

CHILDREN'S HOSPITAL BOSTON
Meeting of Boston Children's Hospital Institutional Biosafety Committee
AGENDA
1/15/2026 11:30 am to 1:00 pm
Karp 4 Conference Room or Teams

Members Present: MM, DF, IJ, SG, KK, EG, SD, CH, DC, HDL, SVH, BS, TW, SL, JM, AR

Members Absent: PW

Guests: None

RLSO: DH, FJ

1 IBC Meeting Minutes

[Boston Children's Hospital Institutional Biosafety Committee meeting \(12/18/2025\)](#)

IBC Meeting Minutes were unanimously approved by the committee.

Committee Decision:

- Motion: Approved
- Majority (Approved): 16
- Minority (Against): 0

2 Administrative Updates

- IBC Feedback Survey
 - The committee reviewed and discussed the survey results.

3 Laboratory Events

- **Summary:** A researcher experienced a potential exposure to chemical when recapping a needle.
- **Root Cause:**
 - Recapping needle
- **Corrective Actions:**
 - Never recap needles.

- Dispose of needles immediately after use.
- Use safer sharps alternatives, such as safety-engineered needles.
- Pay attention to details and give adequate time to finish procedures.

4 New submission – Clinical

IBC-P00002148 A Phase I/II Study of MVX-220 Gene Therapy in Participants with Angelman Syndrome

PI: **NB**

Motion: Modifications Required for Approval – Return to IBC Analyst

Discussion: **IBC Discussion:** This is a first-in-human, phase 1/2, open-label, single dose, multicenter trial to evaluate the safety, tolerability, and preliminary efficacy of MVX-220 delivered via intra-cisterna magna injection in adult and pediatric participants with Angelman Syndrome (AS). AS is a rare genetic neurodevelopmental disorder caused by loss of function of the UBE3A gene in the brain, leading to severe developmental delay, absent or minimal speech, movement and balance problems, seizures, and a characteristically happy demeanor. MVX-220 is a non-replicating recombinant AAVhu68 vector expressing a codon optimized version of the human UBE3A gene. The gene therapy product will be prepared and stored in BCH pharmacy and administered in the operating room.

Regulations Applicable to this Protocol: The NIH Guidelines Section III-D and the OSHA Bloodborne Pathogens Standard.

Motion: Modifications Required for Approval

- Majority (Approved): 16
- Minority (Against): 0
- Abstention: 0

Modifications Requested:

- Revise the scientific description summary to include a description of the syndrome and anticipated clinical improvements.
- Clarify whether pediatric participants will be recruited at BCH.

- Provide a description of the study drug, including its mechanism of action.
- State that any potential exposures will be reported to Occupational Health Services (OHS).
- Clarify whether intravenous (IV) administration of AAV presents concerns related to the instruction to avoid PICA. If this refers only to avoiding arterial injury, this should be explicitly stated.

5 New submission – Laboratory

IBC-P00002116 Innate Host Determinants of COVID-19 Severity

PI: **DF**

Motion: Modifications Required for Approval – Return to IBC Analyst

Discussion:

IBC Discussion:

- The committee reviewed the training protocol and advised that the document be simplified to improve clarity and accessibility for training purposes.

IBC-P00002145 Biomarker Core IBC Protocol

PI: **ES**

Motion: Modifications Required for Approval – Return to IBC Analyst

Discussion: **IBC Discussion:** The Biomarker Core aims to validate assays that measure novel biomarkers related to psychiatric disorders and transdiagnostic risk. These biomarkers include indices of stress, trauma, aging, growth, and maturation in children and adolescents. Biospecimens such as hair, saliva, and blood will be used to assess hormones (e.g., cortisol) and molecular markers using ELISA and multiplex quantitative PCR. Work with human blood will be performed at BSL-2 work practices and procedures.

Regulations Applicable to this Protocol: The OSHA Bloodborne Pathogens Standard.

Motion: Modifications Required for Approval

- Majority (Approved): 16
- Minority (Against): 0
- Abstention: 0

Modifications Requested:

- Revise the scientific description summary in layman's terms for non-scientific committee members; define all scientific terms.
- Update the PPE language and replace it with standard BCH PPE attire.
- Define PBMC and clarify who is responsible for obtaining samples.
- Clarify where blood draws will occur.
- For Telomere length assays with blood and PBMCs: confirm the lab has a cell sorter in a BSC for cell sorting and PBMC isolation.
- Reconcile the following statements about BSC vs. benchtop handling of blood and stool; specify processing location and describe exposure prevention if done on benchtop. IBC recommends all potential bloodborne pathogen work be performed inside a BSC.
- Saliva should be handled under BSL-2 work practices due to potential bacterial and/or viral content.
- Move hazard and safety control references:
 - Hazards: bloodborne pathogens and chemical exposure.
 - Safety Controls: use BSC, disposable absorbent mats, disinfect with 10% bleach followed by 70% ethanol, enforce PPE.
- Clarify whether samples are from physically healthy BCH patients; specify blood draw location and validation procedures for service-for-fee samples.
- Include CRISPR-telomere project from pending grants; update relevant sections for CRISPR-related work.
- Describe the use of oligos/primers listed in reagents.
- Clarify procedures involving sharp and associated hazards.

- Uncheck the item regarding the use of other tissues/specimens containing pathogens; check the item for recombinant/synthetic nucleic acid work.
- Update BSL-2 entry to include buccal swab and dried blood spot DNA extraction.
- Include PBMC isolation from blood.
- Clarify urine analysis and processing (benchtop vs BSC) and downstream analyses.
- Designate room numbers for work with human materials.
- Include blood and fecal samples as potential occupational exposure risk.
- For shipping, specify domestic/international locations for saliva, hair, PBMCs, genomic DNA, whole blood, and dried blood spot shipments.

6 Human Study Annual Reconfirmation

IBC-P00002039 Cabaletta 201-001

PI: **SP**

Motion: Approved

Discussion: The study is active and open to enrollment.

IBC-P00001814 Neurogene RTT-200

PI: **DL**

Motion: Approved

Discussion: The study is open to enrollment.

7 Administrative Reviews

IBC-RN00001772-1 Renewal 1 : Mechanisms of host immunity

PI: **JC**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no changes to the risk assessment.

IBC-RN04-253-5 Renewal 5 : Characterization of Mutant Mouse Strains and Human Genetic Diseases

PI: **MF**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no updates or changes to the risk assessment.

IBC-RN00001784-1 Renewal 1 : Genovese Lab

PI: **PG**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no updates or changes to the risk assessment.

IBC-RN00000089-4 Renewal 4 : Role of ABCB5 in stem cells

PI: **MF**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no updates or changes to the risk assessment.

IBC-RN00000113-5 Renewal 5 : Effect of Omega-3 Fatty Acids and GPR40 on Human Melanoma

PI: **MP**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no changes or updates to the risk assessment.

IBC-A00002160-1 Amendment 1 : CC-97540 (BMS-986353) for Active SLE

PI: **SP**

Motion: Administrative Approval

Discussion: This lab is updating its room locations, which does not change the current risk assessment

IBC-RN00000698-3 Renewal 3 : Functional validation and developmental mechanisms of novel CCDD candidate genes

PI: **EE**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no changes to the risk assessment.

IBC-RN08-152-4 Renewal 4 : Prolonged duration sciatic nerve blockade

PI: **DK**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no changes to the risk assessment.

IBC-RN05-123-4 Renewal 4 : Mouse and Human Pluripotent Stem Cells

PI: **TS**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no changes or updates to the risk assessment.

IBC-RN00001785-1 Renewal 1 : Investigating Mechanisms of Attention and Attention Dysfunction

PI: **BF**

Motion: Administrative Approval

Discussion: This is a three-year renewal with no changes to the risk assessment.

IBC-A00001814-7 Amendment 7 : Neurogene RTT-200

PI: DL

Motion: Administrative Approval

Discussion: The surgical and pharmacy manuals were updated. These updates do not change the biosafety risk of the study product.

IBC-A00002033-3 Amendment 3 : Taysha REVEAL 101 Study

PI: DL

Motion: Administrative Approval

Discussion: The pharmacy manual was updated. This update does not change the biosafety risk of the study product.

8 Laboratory Study Annual Reconfirmation

93-016 Zebrafish models of blood development and cancer.

PI: LZ

10-275 Molecular Basis of Cancer

PI: SP

IBC-P00000212 AAV-mediated gene therapy for nerve regeneration

PI: XT

09-218 Transcriptional Regulation of Hematopoiesis

PI: **AC**

IBC-P00001411 Investigate the molecular mechanism underlying brain disorders

PI: **XT**

11-214 Molecular mechanisms of blood diseases

PI: **SA**

IBC-P00000587 Studies on Allergic and Primary Immunodeficiency Diseases

PI: **TC**

05-123 Mouse and Human Pluripotent Stem Cells

PI: **TS**

08-152 Prolonged duration sciatic nerve blockade

PI: **DK**

IBC-P00001618 In vitro use of Pertussis toxin

PI: **DD**

IBC-P00000182 diphtheria toxin administration

PI: **CK**

06-323 Characterization of Stem Cells in Normal Lung and Lung Cancer

PI: **CK**

IBC-P00000979 Cardiovascular Development and Regeneration in Zebrafish

PI: **CB**

IBC-P00000880 Microbiome Ecology and Host Interactions

PI: **SN**

IBC-P00000720 Use of Porcine Cells for in vitro Testing of Adjuvant Candidates

PI: **OL**

IBC-P00001938 Use of Ferret Cells and Tissue for in vitro Testing of Adjuvant Candidates

PI: **DD**

IBC-P00001881 Investigating and manipulating neural circuits in health and disease

PI: **JF**

IBC-P00001491 *Pericytes in pulmonary arterial hypertension

PI: **KY**

IBC-P00000089 Role of ABCB5 in stem cells

PI: **MF**

IBC-P00000120 Screening for small druglike molecules in vitro

PI: **CW**

IBC-P00000127 Response of the mouse nervous system to peripheral nerve injury - Adenovirus Use

PI: **CW**

IBC-P00001505 Direct activation of pain-sensing neurons by bacteria and their ligands

PI: **CW**

IBC-P00001586 Human Neuron Core

PI: **MS**

IBC-P00000092 ANALYSIS OF BACTERIAL GENOTYPIC DIVERSITY DURING ACUTE INFECTIONS

PI: **GP**

IBC-P00001865 Immune evaluation of Clostridium difficile vaccines.

PI: **DD**

10-001 Implantation of murine tumor cell lines in knockout and transgenic mice and elucidation of the mechanism through which NETs are involved in cancer progression

PI: **DW**

IBC-P00000241 Developmental differences in platelets, megakaryocytes, and angiogenesis.

PI: **MSV**

IBC-P00000427 Salmonella paratyphi vaccine

PI: **YL**

IBC-P00000554 Use of human blood for testing and evaluating the efficacy of medical devices

PI: **JK**

IBC-P00000447 Ion Channel Function in Auditory and Vestibular Hair Cells

PI: **JH**

IBC-P00001975 pre-clinical evaluation of purified cell wall lipids from m.tuberculosis

PI: **SVH**

99-075 Intestinal Disease: Enterocyte Toxin Interaction

PI: **WL**

IBC-P00001720 The HEALthy Brain and Child Development Study (HBCD)

PI: **PG**

IBC-P00000100 Bioenhanced Repair

PI: **MM**