Institutional Biosafety Committee Minutes

The Institutional Biosafety Committee (IBC) met on Monday, December 15, 2025 at 1:01 p.m. via videoconference. Upon reaching a quorum, the meeting was called to order by the Chairperson.

Meeting Attendance:

Ron Javier, PhD, Chair
Robert Atmar, MD, IBC Vice Chai
Connor Cordray, MPH, CPH, CHMM, CBSP
Julia Goldman, DVM
Richard Hamill, MD
Vance Hobbs, MBA, Alternate
Nandan Mondal, PhD
Paul Nakata, PhD
Kevin, Pope
Lisa Rollins, MS
Poonam Sarkar, PhD

Shalaka Kotkar, PhD, MPH, CPH, CBSP, Alternate
Leticia McGuffey, Alternate
Brooke Mitchell, Alternate Member
Holly Robinson, Alternate
Shubhashish Sarkar, PhD, Alternate
Rebecca, Schwiebert, Ph., DVM, Alternate

CONFLICTS OF INTEREST
The Chairperson reminded the committee members about the conflict of interest (COI) policy and process. Any conflicts of interest recognized or declared during the meeting will be documented below. The affected member(s) will be excused from the meeting during the relevant discussion and vote and will not participate in either.

MEETING CONDUCT
The Chairperson reminded the committee members that all protocols that are discussed at the meeting are to be considered confidential due to potential privacy or proprietary concerns and are not to be discussed outside of the meeting room with non-IBC members. For this reason, this meeting is considered closed.

REVIEW OF November 2025 MINUTES
The minutes for November 17, 2025, IBC meeting were reviewed and a motion was made to approve the minutes as written. With the majority of the members present voting for the motion, the vote count for approval of the minutes was as follows:

For:	11
Abstain:	0
Against:	0

RECOMBINANT OR SYNTHETIC NUCLEIC ACID MOLECULES RESEARCH APPLICATIONS REVIEW

During the review the committee assessed the appropriate biocontainment levels as well as the facilities, procedures, practices, and training of the PI and laboratory personnel involved in the research including appropriate and relevant training, safe conduct of the research, and knowledge of recombinant or synthetic nucleic acids molecules research. The committee also reviewed agent characteristics, types of manipulations planned, sources of the inserted nucleic acid sequences, nature of the inserted nucleic acid sequences, and whether an attempt will be made to obtain expression of a foreign gene, and if so, the protein that will be produced. Furthermore, the committee determined the applicable section(s) of the NIH Guidelines.

It was determined that the chair or IBC member assigned by the chair must review the modifications to assure that all required changes have been made and all required training is complete before an approval letter may be sent and the PI may begin the research. Further questions, or changes requiring more than simple concurrence by the PI and the chair/designee will be brought to the next convened meeting for full committee review.

A. <u>Recombinant or synthetic nucleic acid molecules research -- Full Board New/Renewals</u>

Protocol number: D986
PI: Barrozo, Enrico
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-E
Title: Development of a Gene Therapy Platform for Early-Life Treatment of Hemoglobin Disorders

HEARTH aims to develop an ex vivo genome-editing workflow for autologous $CD34^+$ hematopoietic stem and progenitor cells to enable early-life treatment of severe hemoglobin disorders using CRISPR–Cas9 RNPs, LNPs, and rAAV6 donor templates, with work limited to bench-only studies on coded human specimens. The project involves phased optimization of editing and delivery, translation to fetal/neonatal sources, and rigorous evaluation of durability, genomic integrity, and safety against predefined criteria..

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D987
PI: Singh, Shubham
Containment Level: BSL-2
NIH Guidelines Section: III-D
Title: Generation Of Plasmids And Stable Mammalian Cell Lines To Study Lipid Metabolism In Mammalian Physiology

The lab investigates how altered lipid metabolism supports cancer proliferation and metastasis and contributes to neurodegenerative diseases such as ALS and dementia. The approach includes CRISPR-Cas9 gene editing, overexpression of lipid metabolism genes, lentiviral transduction, and mass spectrometry-based lipid analysis in mammalian cell models.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D29
PI: Stevens, Alexandra
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-E
Title: Genetic Manipulation of Pediatric Leukemia Cells

This project aims to develop RNA interference and CRISPR/Cas9-based vectors to suppress or disrupt genes involved in signal transduction, DNA repair, and histone methylation, in order to study their roles in chemotherapy resistance in leukemia cells. Using lentiviral transduction, electroporation, and molecular biology techniques, we will track cells with fluorescent and luciferase markers and evaluate gene function through in vitro assays and in vivo xenograft models under chemotherapy conditions.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D61
PI: Bellen, Hugo
Containment Level: BSL-1
NIH Guidelines Section: III-D and III-E
Title: Sequencing Of Drosophila and Vertebrate Cdnas

This project focuses on functionally testing human genes conserved in Drosophila by integrating full-length human cDNAs into fly genomes using phiC31-mediated transgenesis and assessing their ability to rescue fly mutants. Candidate genes, including those with disease associations or variants of unknown significance, will undergo site-directed mutagenesis and in vivo functional assays such as viability, behavior, and stress sensitivity to evaluate pathogenicity.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D175
PI: Sreekumar, Arun
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-E
Title: Metabolomic Profiling of Cancer Progression

The laboratory focuses on identifying biomarkers and pathways involved in cancer progression through genomic, proteomic, and metabolomic profiling of patient-derived specimens, cell lines, and mouse models, using techniques such as RNA sequencing, mass spectrometry, and protein arrays. Functional studies include gene manipulation via viral transduction or CRISPR/Cas9, in

vivo xenograft experiments to assess tumor growth and metastasis, and evaluation of therapeutic targets using inhibitors or combination therapies, with strict adherence to IRB protocols and tumor size limits.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D253
PI: Schiff, Rachel
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-E
Title: Oncogenic Pathways, Mechanisms of Resistance and New Treatment Strategies in Breast Cancer

This project investigates endocrine and growth factor receptor pathways in breast cancer, focusing on mechanisms of tumor growth, metastasis, and resistance to ER+ and HER2+ therapies through gene perturbation using cDNAs, shRNA, siRNA, and dTAG systems delivered via lentiviral and eukaryotic vectors. Functional assays in vitro and xenograft models in vivo will assess the impact of pathway modulation, while all procedures—including live-cell imaging—adhere to institutional biosafety standards and utilize non-infectious reagents for gene editing and degradation studies.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D341
PI: Horrigan, Frank
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-E
Title: Ion Channel Mutagenesis and Expression in Cultured Cells

This research focuses on understanding ion channel structure-function relationships and pharmacology by mutating cloned channel genes and expressing them in cultured cells or Xenopus oocytes for electrophysiological analysis. Studies include mapping allosteric coupling in BK channels, identifying drug binding sites, and investigating TRP and XPR1 channel regulation, using site-directed mutagenesis, plasmid-based expression systems, BacMam transduction, and high-throughput screening assays.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D467
PI: Lee, Hyun-Sung
Containment Level: BSL-2
NIH Guidelines Section: III-D
Title: Characterization and Immunomodulation of Tumor Macrophages in Malignant Pleural Mesothelioma and Non-Small Cell Lung Cancer

This study evaluates the immunologic effects and efficacy of MTG-201 (Ad5-SGE-REIC/Dkk-3), an FDA-approved agent, in combination with checkpoint inhibitors (anti-PD-1 or anti-CTLA-4) using a syngeneic mouse model of malignant mesothelioma. The protocol involves intraperitoneal tumor cell inoculation, treatment with adenoviral and antibody-based therapies, bioluminescence imaging for tumor burden assessment, and strict adherence to euthanasia and biosafety guidelines.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the

approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D492
PI: Yallampalli, Chandrasekhar
Containment Level: BSL-2
NIH Guidelines Section: III-D, III-E and III-F
Title: Complement in Preeclampsia

This project investigates the role of dysregulated complement activation in preeclampsia by conditionally knocking down the placental complement inhibitor Crry in mice using doxycycline-inducible lentiviral shRNA vectors. The study will assess whether complement-mediated placental injury induces hypertension and antiangiogenic factors and will explore therapeutic potential of CGRP family peptides in mitigating these effects through in vivo infusion and gene delivery approaches.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D633
PI: WILLIAMS, MARGOT
Containment Level: BSL-1
NIH Guidelines Section: III-D and III-F
Title: Molecular Regulation of Embryonic Axis Extension

This research uses zebrafish embryos as a model to study how patterning and morphogenesis are coordinated during development, focusing on the role of Nodal signaling in anteroposterior axis extension. Experimental approaches include microinjection of RNA, DNA, and transgenes into single-cell embryos, combined with live imaging and RNA in situ hybridization to analyze cell behaviors and gene expression changes.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D656
PI: Patras, Katy
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-F
Title: Contribution of Host Metabolism, Immunity, and Microbiota to Bacterial Colonization And Infection of The Female Urogenital Tract.

This research explores how commensal and pathogenic bacteria interact with the female urogenital tract to identify pathways that maintain health and inform therapeutic development. Using molecular cloning and targeted mutagenesis of bacterial genes, we study host-pathogen interactions through in vitro assays with human epithelial cell lines and in vivo mouse models of urinary tract infection and vaginal colonization, analyzing samples for genomic, transcriptomic, proteomic, and microscopic changes.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D823
PI: Kanca, Oguz
Containment Level: BSL-1
NIH Guidelines Section: III-D and III-F
Title: CRISPR Mediated Genome Engineering to Generate Genetic Reagents to Study Drosophila Genes

This project uses CRISPR/Cas9-mediated homologous recombination in Drosophila to generate GAL4 knock-in alleles of genes conserved with humans, prioritizing those linked to diseases in OMIM, to enable functional annotation and phenotypic characterization. Verified transgenic lines will be shared publicly, and selected alleles will undergo detailed behavioral and metabolic assays, as well as rescue experiments with human or variant cDNAs to assess structure-function relationships and the impact of disease-associated variants.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D825
PI: Zhou, Zheng
Containment Level: BSL-1
NIH Guidelines Section: III-D
Title: Expression of Transgenic Reporter Genes In C. Elegans for The Study of Engulfment

This research investigates how programmed cell death is managed in C. elegans by cloning and expressing genes involved in signal transduction pathways in worm and E. coli systems. Transgenic worms are generated via microinjection and analyzed using fluorescence microscopy, with all experiments conducted under safe containment and proper sterilization protocols.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D984
PI: Perin, Emerson
Containment Level: BSL-2

NIH Guidelines Section: III-C
Title: Endocardial Delivery Of XC001 Gene Therapy For Refractory Angina Coronary Treatment: A 26-Week (With 26 Week Extension) Phase 2b Randomized, Multi-Center, Double-Blind, Sham Controlled Study To Evaluate Efficacy and Safety (The EXACT-2 Trial)

The EXACT-2 clinical trial evaluates the safety, feasibility, and efficacy of catheter-based intramyocardial delivery of XC001 (AdVEGFXC1), a replication-deficient adenovirus expressing VEGF-A isoforms, in patients with refractory angina due to obstructive coronary artery disease. The study includes an initial open-label safety run-in followed by a randomized, double-blind, sham-controlled Phase 2b trial, with XC001 administered via 22 transendomyocardial injections using the Extroducer® catheter and follow-up assessments at multiple time points up to 52 weeks.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D985
PI: Postalian Yrausquin, Alexander
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: A Phase I Study of Safety and Preliminary Efficacy of YAP101 in Subjects with Ischemic Heart Failure and Reduced Ejection Fraction

This study is an open-label, dose-escalation trial evaluating the safety, tolerability, and pharmacodynamics of YAP101, a gene therapy designed to transiently suppress Hippo signaling and promote cardiomyocyte regeneration in patients with ischemic heart failure following myocardial infarction. YAP101 will be administered via 15 transendocardial injections during a single catheterization procedure, with dose escalation guided by a 3+3 design and safety monitoring including ECG, echocardiography, and laboratory assessments.

After the presentation by the assigned reviewer and discussion, the committee requested the following modification: 1). Please ensure all personnel complete training

Next, a motion was made and seconded to approve the protocol with modifications required to secure approval. The motion passed with a majority of the members present voting for the motion. The vote count for the approval of the protocol with modifications required to secure approval was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

B. Recombinant or synthetic nucleic acid molecules research -- Full Board Amendments

Protocol number: D456
PI: St-Pierre, Francois
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-E
Title: Development of Molecular Sensors and Actuators

This project focuses on developing and improving sensors that report brain activity and actuators that modulate neuronal function, such as voltage-sensitive fluorescent indicators and optogenetic tools. To achieve this, lentiviruses, AAVs, and gamma retroviruses carrying genes for these tools will be used to infect mammalian cell lines and dissociated neurons, followed by expression analysis through live-cell microscopy and FACS.

After the presentation by the assigned reviewer and discussion, the committee requested the following modification: 1). Section C: Please clarify when imagining is done. 2) Section E: Please clarify if retrovirus should be included in Section E2. 3) Please clarify where the rat tissue comes from

Next, a motion was made and seconded to approve the protocol with modifications required to secure approval. The motion passed with a majority of the members present voting for the motion. The vote count for the approval of the protocol with modifications required to secure approval was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D624
PI: Lagor, William
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-F
Title: Genome Editing Testing Center

The Genome Editing Testing Center (GETC), a collaboration between Baylor College of Medicine and Rice University, provides a centralized resource for evaluating genome editing reagents and delivery systems in mouse models, focusing on efficiency, specificity, and safety. The center offers two tracks—reporter models for biodistribution and editing efficiency, and disease models for therapeutic correction—using delivery methods such as

AAV, adenovirus, lipid nanoparticles, and virus-like particles, with analyses tailored to experimental needs.

After the presentation by the assigned reviewer and discussion, the committee requested the following modification: 1). Section C: Please perform all viral injections in a Biosafety Cabinet and add this information to this section. 2) Section C: Please describe if mouse imaging for phenotypes will be done by research personnel on this protocol or by others.

Next, a motion was made and seconded to approve the protocol with modifications required to secure approval. The motion passed with a majority of the members present voting for the motion. The vote count for the approval of the protocol with modifications required to secure approval was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D771
PI: Shivanna, Binoy
Containment Level: BSL-1
NIH Guidelines Section: III-E
Title: Modulation Of Oxidant Stress-and Inflammation-Induced Lung Injury in Mice In Vivo

This project tests whether STAT1 overexpression exacerbates experimental bronchopulmonary dysplasia and pulmonary hypertension by conditionally activating Stat1 in lymphatic endothelial cells during neonatal hyperoxia or LPS exposure, using Rosa26CAG-LSL-Stat1/Prox1-CreERT2 mice. Complementary studies will boost adrenomedullin signaling via lung-targeted nanoparticles and assess cardiopulmonary outcomes through lung function testing, echocardiography/MRI/PET, histopathology, and inflammatory profiling to identify therapeutic strategies for severe neonatal lung disease,

After the presentation by the assigned reviewer and discussion, the committee requested the following modification: 1). Section C: Please describe the room number and process to safely secure the diphtheria toxin injection for mice.

Next, a motion was made and seconded to approve the protocol with modifications required to secure approval. The motion passed with a majority of the members present voting for the motion. The vote count for the approval of the protocol with modifications required to secure approval was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D781
PI: Sandweiss, Alexander

Containment Level: BSL-2
NIH Guidelines Section: III-F
Title: Transient Transfection for Cell-Based Assay of anti-NMDAR IgG

This study uses a cell-based assay to detect anti-NMDAR antibodies, the hallmark of autoimmune encephalitis, by transiently transfecting HEK293 cells with NMDAR-expressing plasmids and analyzing colocalization of antibodies via immunofluorescence. Samples from mice (IACUC AN-8685) and humans (IRB H052095) will be tested, with cDNA used only for transient transfection of HEK293 cells, which are fixed before antibody detection.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D90
PI: RAMOS, CARLOS
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: (Sagan) Phase I Study of Activated T-Cells Expressing Second Or Third Generation Cd19-Specific Chimeric Antigen Receptors for Advanced B-Cell Non-Hodgkin's Lymphoma

This Phase I trial evaluates the safety and persistence of autologous T cells genetically modified to express CD19-specific CARs with either CD28 alone or CD28 plus CD137 costimulatory domains in patients with relapsed/refractory NHL, ALL, or CLL. The study uses a dose-escalation design followed by an expansion cohort at the maximum tolerated dose, with endpoints focused on safety and comparative persistence of the two CAR-T cell populations, tracked via real-time PCR over a 15-year follow-up.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D728
PI: Salem, Bahey
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: Tricar: Trivalent Autologous T-Lymphocytes Co-Expressing Three Chimeric Antigen Receptors Targeting Cd19, Cd20 and Cd22 in Acute B-Lineage Leukemia

This Phase I trial evaluates the safety and efficacy of autologous trivalent CAR-T cells incorporating a 4-1BB co-stimulatory domain and targeting CD19, CD20, and CD22 in patients with relapsed or refractory B-lineage leukemia, including CD19-negative cases. The study uses a Bayesian Optimal Interval design for dose escalation, followed by expansion at the maximum tolerated dose, with comprehensive monitoring of toxicity, disease response, CAR-T persistence, and long-term safety over a 15-year follow-up.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D836
PI: Barto, Tara
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: 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

This first-in-human, open-label, dose-escalation study evaluates the safety, tolerability, and efficacy of VX-522, an mRNA therapy encoding CFTR protein delivered via nebulization, in patients with cystic fibrosis across three treatment arms: VX-522 alone, VX-522 plus ivacaftor, and VX-522 plus ivacaftor and prednisone/prednisolone. The protocol includes detailed dosing schedules, PPE and biosafety precautions for nebulization, pharmacy handling procedures, and cleaning/disinfection requirements for the investigational eFlow nebulizer system.

Following the presentation by the assigned reviewer and discussion of the protocol, the committee IBC concluded that all aspects of review and approval criteria (described above) were met.

Next, a motion was made and seconded to approve the protocol. The motion passed with a majority of the committee members present voting for the motion. The vote count for the approval of the protocol with all applicable approval criteria was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

Protocol number: D881
PI: Nair, Ajith
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: MAGNITUDE: A Phase 3, Multinational,Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate The Efficacy and Safety Of NTLA-2001 in Participants with Transthyretin Amyloidosis with Cardiomyopathy (ATTR-CM)

This Phase III trial (ITL-2001-CL-301) evaluates the efficacy and safety of NTLA-2001, an in vivo CRISPR/Cas9 gene-editing therapy delivered as a single intravenous infusion to disrupt the transthyretin (TTR) gene in adults with ATTR cardiomyopathy. Approximately 765 participants will be randomized 2:1 to NTLA-2001 or placebo, with pre-medication to reduce infusion reactions, clinical monitoring for 18 months, and long-term follow-up for 15 years to assess cardiovascular outcomes, safety, and immunogenicity.

After the presentation by the assigned reviewer and discussion, the committee requested the following modification: 1). Section C: Please mention if the investigative gene therapy LNP will be obtained as ready to go infusion formulation or any manipulation will be required on site. 2) Please ensure all personnel complete training.

Next, a motion was made and seconded to approve the protocol with modifications required to secure approval. The motion passed with a majority of the members present voting for the motion. The vote count for the approval of the protocol with modifications required to secure approval was as follows: For, 11; Against, 0; Abstaining, 0.

There were no members who recused and absented themselves during the discussion and vote on this protocol due to a conflict of interest.

C. Recombinant or synthetic nucleic acid molecule Closure Administrative Report
The Director, Research Compliance reported to the IBC that there were two rDNA IBC protocols closed for the month of December.

D. Recombinant or synthetic nucleic acid molecule Minor Administrative Report
The Director, Research Compliance reported to the IBC that there were five administrative rDNA IBC protocols for the month of December.

E. Recombinant or synthetic nucleic acid molecules research -- Exempt Protocols
The Director, Research Compliance reported to the IBC that there were no exempt protocols submitted in the month of December.

F. IBC Inspection Report
The Biosafety Officer (BSO) informed the committee that there were three inspections performed for the month of December.

G. Research Compliance Services (RCS) Update
The Director, Research Compliance informed the committee that there were two post-approval monitoring sessions completed.

H. Member Discussion
There were no items to report for the month of December.

I. Spills, Incidents, or Exposures
There were no items to report for the month of December

J. RAC Decisions and Updates
There were no items to report for the month of December.

K. Issues from the Floor and Public Comments
There were no issues raised from the floor or public comments.

L. Adjournment
The meeting was adjourned at 1:40 pm

UPCOMING EVENTS:
The next IBC meeting is scheduled for Tuesday, January 21, 2026.