Institutional Biosafety Committee Minutes

The Institutional Biosafety Committee (IBC) met on Tuesday, August 19, 2025 at 1:00p.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 Chair
Manu Banadakoppa, PhD
Connor Cordray, MPH, CPH, CHMM, CBSP
Monica Darden, M.A
Julia Goldman, DVM
Shirley Hutchins, MSN
James Kelaher, MD
Paul Nakata, PhD
Robin Parihar, MD
Lisa Rollins, MS
Shannon Ronca, PhD, MPH
Poonam Sarkar, PhD

Vance Hobbs, MBA, Alternate
Leticia McGuffey, Alternate
Brooke Mitchell, Alternate
Holly Robinson, Alternate
Shubhashish Sarkar, PhD, Alternate
Rebecca, Schwiebert, PhD, 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 July 2025 MINUTES

The minutes for July 15, 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:	13
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: D156
PI: Plon, Sharon
Containment Level: BSL-2
NIH Guidelines Section: III-D III-E and III-F
Title: Protocol for Recombinant DNA used in Expression Cell Lines for Cryoem/et

This research program focuses on identifying and characterizing genes involved in hereditary cancer predisposition and genomic stability. The lab uses genomic sequencing to identify candidate genes, which are then studied through recombinant DNA techniques. These include cloning wild-type and mutant cDNAs into plasmids to assess their effects on cell growth, DNA damage response, and splicing.

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, 13; 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: D620
PI: Jiang, Zheng
Containment Level: BSL-2
NIH Guidelines Section: III-D and III-F
Title: Study Melanopsin Phototransduction Pathway in Mice with Virally Introduced Transduction Components

This project aims to restore vision in photoreceptor-degenerated retinas by enhancing light responses in retinal cells. By co-expressing ion channels and related components, the team seeks to improve melanopsin signaling, with functional outcomes assessed through electrophysiology, behavioral tests, and brain activity mapping in mouse models.

After the presentation by the assigned reviewer and discussion, the committee requested the following modification: 1). Section D8: Please complete point of use.

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, 13; 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: D629
PI: Zuniga-Sanchez, Elizabeth
Containment Level: BSL-1
NIH Guidelines Section: III-D
Title: Synaptic Specificity Determinants in the Mouse Retina

This project investigates how photoreceptors in the retina recognize and connect with their specific synaptic partners, a process critical for restoring vision. By identifying and testing candidate recognition molecules using CRISPR/Cas9, overexpression, and AAV-based labeling in mouse models, the research aims to restore damaged retinal circuits.

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, 13; 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: D957
PI: Bacino, Carlos
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: H-50899 / A Study to Evaluate the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of Intrathecally Administered ion582 in Patients with Angelman Syndromee

The study evaluates the safety, tolerability, pharmacokinetics, and pharmacodynamics of an oligonucleotide designed to treat Angelman Syndrome by treating genes through degradation of RNA.

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, 13; 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: D966
PI: Lerner, Seth
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: A Phase 1/2, Single-arm, Open-Label Trial to Evaluate the Safety and Efficacy of Nadofaragene Firadenovec Instilled to the Renal Pelvis in Adult Subjects with Low-grade Upper Tract Urothelial Carcinoma (LG-UTUC)

This single-arm, multi-center study aims to address the high unmet need for non-surgical treatment options in LG-UTUC, especially for patients unsuitable for endoscopic ablation or radical nephroureterectomy.

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, 13; 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: D970
PI: Chao, Hsiao-Tuan
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: A Phase 1/2a, Open-Label, Multi-Center, Dose-Escalation Trial to Assess Safety, Tolerability, and Efficacy of a Single Dose of CAP-002 Gene Therapy Administered to Pediatric Patients with Syntaxin-Binding Protein 1 (STXBP1) Encephalopathy

The study includes sequential and concurrent dosing across three cohorts, with long-term follow-up over five years and monitoring for vector shedding, immune response, and biomarkers, supported by corticosteroid prophylaxis to manage potential immune reactions.

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, 13; 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: D293
PI:, Wehrens, Xander
Containment Level: BSL-2
NIH Guidelines Section: III-D, III-E and III-F

Title: Molecular Cloning of Antigens to Study Pathogenesis and Vaccine Development

Researchers will use various molecular biology techniques including PCR, gene synthesis, and CRISPR to study genes involved in heart function through both in vitro cell line experiments and in vivo mouse models. Gene delivery will be achieved using viral vectors like AAV9 to assess the impact of genetic manipulation or therapy on cardiac structure, function, and disease progression.

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, 13; 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: D716
PI: Frankfort, Benjamin
Containment Level: BSL-2
NIH Guidelines Section: III-D
Title: Mechanisms of Neurogenesis - from Cells to Animal Models

Researchers aim to develop a system to isolate and study specific retinal ganglion cell subtypes, enabling targeted gene delivery and analysis of their injury susceptibility. Using genetics, viruses, and AAV vectors, they will manipulate gene expression to investigate biology and gene knockdown and overexpression experiments.

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, 13; 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: D570
PI: Makawita, Shalini
Containment Level: BSL-2
NIH Guidelines Section: III-C
Title: Vista (Virus Specific t Cells and Adenovirus): A First in Human Phase I Trial of Binary Oncolytic Adenovirus in Combination with Her2-Specific Car vst Cells in Patients with Advanced Her2 Positive Solid Tumors

This is a single-arm, Phase I clinical trial using a 3+3 dose-escalation design to evaluate the safety, feasibility, and preliminary efficacy of intratumoral CAdVEC injection combined with HER2-specific CAR T-cell therapy in patients with advanced, refractory HER2-positive solid tumors.

After the presentation by the assigned reviewer and discussion, the committee requested the following modifications: 1). Please clarify containment procedures for transport. 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, 13; 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 is an open-label, dose-escalation study evaluating the safety, tolerability, and efficacy of an mRNA therapy for cystic fibrosis. The trial includes a 28-day run-in period, intensive dosing and monitoring over several weeks, and long-term follow-up, with strict protocols for drug preparation, administration, and equipment disinfection to ensure safety and consistency.

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, 13; 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 IBC Laboratory Compliance Assurance Associate reported to the IBC that there were two rDNA IBC protocols closed for the month of August.

D. Recombinant or synthetic nucleic acid molecule Minor Administrative Report
The IBC Laboratory Compliance Assurance Associate reported to the IBC that there were nine administrative rDNA IBC protocols for the month of August.

E. Recombinant or synthetic nucleic acid molecules research -- Exempt Protocols
The IBC Laboratory Compliance Assurance Associate reported to the IBC that there were no exempt protocols submitted in the month of August.

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

G. Research Compliance Services (RCS) Update
The IBC Laboratory Compliance Assurance Associate informed the committee that there were three post-approval monitoring sessions completed.

H. Member Discussion
The Director, Research Compliance informed the committee about the use of a virus in animals that was administered in a BSC but was not in the biohazard area. The animals were moved and there is no risk to personnel or other mice, as the virus is selective and not replication competent. The IACUC is conducting an assessment to determine if there was any non-compliance.

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

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

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 1:28 pm

UPCOMING EVENTS:

The next IBC meeting is scheduled for Tuesday, September 16, 2025.