Johns Hopkins Institutional Biosafety Committee
JHU IBC Minutes for July 21, 2025
Zoom Meeting

Members Present:	Gary S. Hayward, Ph.D. (IBC Chair, Virology and Gene Therapy); Weiying Pan, Ph.D., RBP (Associate BSO, Molecular Aspect of Drug Design and Biology); Stephen C. Dahl, Ph.D., RBP (BSO, Biology); Alan F. Scott, Ph.D. (IBC member, Molecular Biology and Genetics); Nadia Desir, Ph.D., RBP (Associate BSO, High Containment); Elizabeth A. Laffan, Ph.D. (Non-affiliated Member, Biology); Ms. Claudia MacAuley, L.A.T. (Non-affiliated Member, Biosafety and High Containment); Djikolngar Maouyo, Ph.D. (Non-affiliated Member, Biology); Viji Sitther, Ph.D. (Non-affiliated Member, Plant Biology); Prashant Desai, Ph.D. (IBC member, Virology); Joseph B. Margolick, MD, Ph.D. (IBC member, Medicine, Microbiology and Immunology)
Members Absent:	Douglas Norris, Ph.D. (IBC member, Vector Biology and Entomology); Jason Villano, D.V.M. (IBC member, Animal Science); Brigitte Gaume, Ph.D. (Non-affiliated Member, Biology)
IBC Coordinator:	Ms. Tylicia McRae

The meeting was called to order at 3:19 pm.

Announcements:

No conflicts of interest were reported by IBC members.

Review and Approval of Meeting Minutes

The minutes of the June 16, 2025, meeting were approved as submitted.

Clinical Protocols and Amendments:

Campochiaro Protocol, GT2012210101 (NIH Cit.: III-C-1), "A Randomized, Partially Masked, Controlled, Phase 2b/3 Clinical Study to Evaluate the Efficacy and Safety of RGX-314 Gene Therapy in Participants with nAMD (ATMOSPHERE)"

The IBC received Protocol Version 10.0, dated February 28, 2025, for the above referenced protocol. The amendment adds a bilateral treatment substudy. Minor clarifications and corrections were made throughout the protocol. None of the changes were expected to affect the biosafety of the study.

The IBC voted to approve the amendment.

For Approval: 11
Disapproval: 0
Abstain: 0

Hoke Protocol, GT2507210101 (NIH Cit.: III-C-1), "A Multicenter Phase 1 Double-Blind, Randomized, Sham-Controlled Dose Escalation Study to Determine Safety and Tolerability of Single Dose Intrathecal ST-503 Gene Therapy for Refractory Pain Due to Idiopathic Small Fiber Neuropathy (iSFN)"

The primary objective of this protocol is to assess the safety and tolerability of ST-503 over a 52-week post-dosing observation period. ST-503 is a recombinant AAV9-delivered zinc finger protein-transcription factor for repression of the human NAV1.7 gene.

Female subjects ≥18 years of age who are post-menopausal or who have undergone a hysterectomy will be included in this study. Subjects will be randomized 2:1 ratio to receive ST-503 or sham treatment. Up to approximately 18 subjects in the ST-503-treated group and up to approximately 9 subjects in the sham-treated control group. Three dose levels will be evaluated during dose escalation ($1x10^{14}$ vg, $2x10^{14}$ vg and $4x10^{14}$ vg).

Subjects will be admitted to an in-patient care facility for three days with pre-dose assessments on Day -1, lumbar puncture with ST-503 administration or sham procedure on Day 1, and clinical monitoring until Day 2. ST-503 will be administered via lumbar puncture under general anesthesia or conscious sedation. Subjects in each dose level will be staggered so that each of the first three subjects undergoes the procedure at ≥ 4 weeks after the preceding subject. Assuming no dose limiting toxicities are observed, the interval between dosing will be ≥ 2 weeks after the first three subjects at each dose level.

Participants will be followed for 52 weeks post-dose. Duration of the study is approximately 1.5 to 2 years. Subjects will be encouraged to participate in a separate long-term follow-up study and followed for up to an additional five years.

The medical and pharmacy staff involved in the study will be trained to follow the pharmacy manual for the proper handling of ST-503, as well as the standard operating procedures (SOPs) for incident management, including spill response.

The submission complies with the requirements of the Johns Hopkins IBC, institutional policies, and the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules.

The IBC voted to approve the protocol.

For Approval: 11
Disapproval: 0
Abstain: 0

Konig Protocol, GT2401090301 (NIH Cit.: III-D-3-a; III-C-1), "A Phase 1 Study Evaluating SC291, A Hypoimmune, Allogeneic CD19-Directed CAR T Cell Therapy, in Subjects with Severe Relapsed or Refractory Autoimmune Diseases (GLEAM)"

The IBC received Protocol Version 5.0 dated March 2, 2025, for the above referenced protocol. A retreatment phase was added to the study. The duration of hospitalization was reduced from 7 to 3 days. In accordance with FDA guidance, dose level 3 was revised from 360 x 10^6 to 550 x 10^6 CAR+T cells. The upper age limit was removed, and a weight requirement was added to the study. Subjects with antiphospholipid syndrome and subjects with early-stage prostate cancer can enroll in the study. HHV-6 and HHV-7 were added to laboratory tests. Revisions for clarity and/or consistency were made throughout the protocol. None of the changes were expected to affect the biosafety of the study.

The IBC voted to approve the amendment.

For Approval: 11
Disapproval: 0
Abstain: 0

Konig Protocol, GT2401090301 (NIH Cit.: III-D-3-a; III-C-1), "A Phase 1 Study Evaluating SC291, A Hypoimmune, Allogeneic CD19-Directed CAR T Cell Therapy, in Subjects with Severe Relapsed or Refractory Autoimmune Diseases (GLEAM)"

The IBC received Protocol Version 6.0 dated May 12, 2025, for the above referenced protocol. In accordance with FDA feedback, the staggered dosing interval for Dose Level 3 was increased to 28 days. Outpatient dosing was removed for systemic lupus erythematosus subjects with antiphospholipid syndrome. Revisions for clarity and/or consistency were made throughout the protocol. None of the changes were expected to affect the biosafety of the study.

The IBC voted to approve the amendment.

For Approval: 11
Disapproval: 0
Abstain: 0

Konig Protocol, GT2401090301 (NIH Cit.: III-D-3-a; III-C-1), "A Phase 1 Study Evaluating SC291, A Hypoimmune, Allogeneic CD19-Directed CAR T Cell Therapy, in Subjects with Severe Relapsed or Refractory Autoimmune Diseases (GLEAM)"

The IBC received Protocol Version 7.0 dated May 12, 2025, for the above referenced protocol. Dose-escalation/de-escalation rules were revised for subjects with systemic lupus erythematosus in dose level 2.

The IBC voted to approve the amendment.

For Approval: 11

Disapproval: 0
Abstain: 0

Mukherjee-Clavin Protocol, GT2507210201 (NIH Cit.: III-F), "A First-in-Human, Multi-Center, Randomized, Participant-and Investigator-Blinded, Placebo-Controlled, Single Ascending Dose Study to Assess the Safety, Tolerability, and Pharmacokinetics of EDK060 in Adults with Charcot-Marie Tooth Type 1A (CMT1A)"

The primary objective of the study is to assess the safety, tolerability, and pharmacokinetics of EDK060. EDK060 is a chemically synthesized duplex oligonucleotide formed by a 23-nucleotide long antisense strand paired with a 21-nucleotide long conjugated sense strand. EDK060 targets hPMP22 mRNA with a fatty acid motif that promotes biodistribution and cellular uptake in peripheral nerves.

This protocol is consistent with the exemption provided for clinical trials involving the deliberate transfer of synthetic nucleic acid molecules, or DNA or RNA derived from synthetic nucleic acid molecules into human subjects.

Talaat Protocol, GT2307170201 (NIH Cit.: III-C-1), "A Phase I Study of the Safety and Immunogenicity of the Recombinant, Live-Attenuated, Bovine/Human Parainfluenza Virus Vector Vaccine Expressing the 6P-Prefusion-Stabilized Version of the SARS-CoV-2 Spike Protein, Administered in Two Sequential Doses as Nasal Spray to Adults 18 to 50 Years of Age."

The IBC acknowledged receipt of the IRB continuing review approval documents for the above referenced protocol. No further action is considered warranted.

Recombinant DNA/Pathogen Research Registrations and Amendments

13 research registrations and 2 amendments were presented for IBC consideration.

Review of Incidents:

No incidents were reported at this meeting.

IBC Training:

Dr. Pan prepared a PowerPoint file summarizing recent developments and applications of gene editing and shared it with the IBC members.

Public Comments:

There were no public comments.

The meeting was adjourned at 4:47 pm.

July, 2025, R-DNA Research Registrations and Amendments

Name	Synopsis	IBC#	BSL	ABSL	NIH Cit.	Agent	Select Agent
New Registrations							
Cao, Xu	Cre-dependent pseudorabies viruses (PRVs) obtained from commercial vendors will be injected into mouse brains for retrograde neuronal tracing. All experiments involving PRVs will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of PRVs, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. Pathogen/Infectious agent registration for Cre-dependent pseudorabies virus (PRV) Ba2017 RegSupport: R-DNA: DN1806280107; AAVs: P2402210102	P2410230601	2	2	III-D-1-a; III-D-4-b	Cre-dependent pseudorabies virus (PRV) Ba2017	No
Discussion/Review							
Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.						
Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.						
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.						
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.						
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**						
Cao, Xu	Replication-incompetent lentiviral vectors obtained from commercial vendors will be used to transduce wild-type angiogenin (ANG), mutant ANG, GCaMP6, hM4Di, and Vgat shRNAs into cultured cells and mice, in order to investigate the role of ANG in osteoclast differentiation and the regulation of bone metabolism. All experiments involving viral vectors and human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of viral vectors and human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents.	P2410230701	2	2		Replication-incompetent lentiviral vectors	No

Pathogen/Infectious agent registration for Replication-incompetent lentiviral vectors
RegSupport: R-DNA: DN1806280107; Human tissue registration:B0907290216

Discussion/Review

Protocol Narrative Assessment: Acceptable. The proposal adequately describes the experiments to be performed.

Hazard Assessment and Precautions: Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.

Training: Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.

Decontamination and Waste Disposal: Acceptable. The proposal adequately describes proper decontamination and disposal procedures.

Committee Vote: **For: 11 Opposed: 0 Abstentions: 0**

Graham,Thomas	Plasmid-based CRISPR/Cas9 systems, PiggyBac transposon vectors, and other plasmids will be used to express various human and Drosophila genes involved in transcriptional regulation. These include transcription factors (e.g., human Sp1 and Drosophila ecdysone receptor), histone acetyltransferases (e.g., human p300), RNA polymerase II (Pol II), Pol II-interacting proteins (e.g., DSIF, NELF, Spt6, TFIIS, the PAF1 complex, CycT1, and Cdk9), transcriptional coactivators (e.g., BRD4), and histone H3.3. These constructs will be introduced into cultured cells and Drosophila melanogaster to investigate mechanisms of transcriptional regulation by probing molecular interactions in live cells. All experiments involving human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. DN--Uses recombinant DNA in cultured cells and animals RegSupport: Human tissue registration: B2506240101	DN2506240101	2	1	III-D-4-a; III-F-8	No

Discussion/Review

Protocol Narrative Assessment: Acceptable. The proposal adequately describes the experiments to be performed.

Hazard Assessment and Precautions: Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.

Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Lian, Xizhen	Lipid nanoparticles and polymeric materials will be used to deliver mRNA encoding reporter genes, Cas9, and gRNAs targeting DNMT1, HBB, and the BCL11A enhancer into cultured cells and mice. Recombinant human cells will also be implanted into mice. All experiments involving human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. DN--Uses recombinant DNA in cultured cells and animals RegSupport: Human tissue registration:B2506300201	DN2506300201	2	2	III-D-4-a, III- F-8	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Queen, Jessica	Replication-incompetent lentiviral vectors obtained from commercial vendors will be used to transduce Cre recombinase into organoid cell cultures, activating recombination that leads to mutation or deletion of selected alleles. Transgenic mice carrying mutations in oncogenes, tumor suppressor genes, and receptors for bacterial virulence factors will be obtained from commercial vendors and other investigators, and used to study the role of the microbiome in disease. All experiments involving viral vectors and human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of viral vectors and human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. DN--Uses recombinant DNA in cultured cells and animals RegSupport: Lentiviral vectors:P2506300401; Human tissue registration: B2506300101	DN2506300101	2	1	III-D-3-a; III-D-4-c-(2)		No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Queen, Jessica	Replication-incompetent lentiviral vectors obtained from commercial vendors will be used to transduce Cre recombinase into organoid cell cultures, activating recombination that leads to mutation or deletion of selected alleles. All experiments involving viral vectors and human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of viral vectors and human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. Pathogen/Infectious agent registration for Replication-incompetent lentiviral vectors RegSupport: R-DNA: DN2506300101; Human tissue registration: B2506300101	P2506300401	2	III-D-3-a	Replication-incompetent lentiviral vectors	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and	Acceptable. The proposal adequately describes the potential biohazards inherent
Precautions:	in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Yarchoan, Mark	Live-attenuated, genetically modified Listeria monocytogenes expressing KrasG12D will be obtained from another investigator, propagated in vitro, and injected into mice as a vaccine against hepatocellular carcinoma. All experiments involving live-attenuated, genetically modified Listeria monocytogenes will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of live-attenuated, genetically modified Listeria monocytogenes, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. Pathogen/Infectious agent registration for Live-attenuated genetically modified Listeria monocytogenes RegSupport: R-DNA: DN2207070203	P2505230301	2	2	III-D-1-a; III-D-4-b	Live-attenuated genetically modified Listeria monocytogenes	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and	Acceptable. The proposal adequately describes the potential biohazards inherent
Precautions:	in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Yarmus, Lonny	A replication-incompetent adenoviral vector, Ad5CMVCre-eGFP, will be obtained from a commercial vendor and injected into Oncopigs carrying transgenic Cre-inducible mutations in KRAS and P53 to induce tumor formation. This model will be used to evaluate the efficacy and safety of a novel transbronchial cryoablation technology for the treatment of lung cancer. All experiments involving viral vectors will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of viral vectors, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. DN--Uses recombinant DNA in animals RegSupport: Adenoviral vector: P2506250101	DN2506200101	2	2	III-D-4-b; III-D-4-c (2)	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Yarmus_Lonny	A replication-incompetent adenoviral vector, Ad5CMVCre-eGFP, will be obtained from a commercial vendor and injected into Oncopigs carrying transgenic Cre-inducible mutations in KRAS and P53 to induce tumor formation. This model will be used to evaluate the efficacy and safety of a novel transbronchial cryoablation technology for the treatment of lung cancer. All experiments involving viral vectors will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of viral vectors, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. Pathogen/Infectious agent registration for Replication-incompetent adenoviral vector RegSupport: R-DNA: DN2506200101	P2506250101	2	2	III-D-4-b	Replication-incompetent adenoviral vector	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Umapathi, Priya	Replication-incompetent adeno-associated viral vectors obtained from a commercial vendor will be used to perform CRISPR/Cas9-mediated gene editing of the Lamin A/C (LMNA) gene in cultured cells and mice to study LMNA-related cardiomyopathy. All experiments involving viral vectors and human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of viral vectors and human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. Pathogen/Infectious agent registration for Replication-incompetent adeno-associated viral vectors RegSupport: R-DNA:DN2302090103; Human tissue registration:B2303230103	P2506270101	2	2	III-D-3-a; III-D-1-a; III-D-4-b	Replication-incompetent adeno-associated viral vectors	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.

Committee Vote: **For: 11** **Opposed: 0** **Abstentions: 0**

Umapathi, Priya	Replication-incompetent adeno-associated viral vectors obtained from a commercial vendor will be used to perform CRISPR/Cas9-mediated gene editing of the Lamin A/C (LMNA) gene in cultured cells and mice to study LMNA-related cardiomyopathy. All experiments involving viral vectors and human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of viral vectors and human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. Pathogen/Infectious agent registration for Replication-incompetent lentiviral vectors RegSupport: R-DNA:DN2302090103; Human tissue registration:B2303230103	P2506270201	2	III-D-3-a	Replication-incompetent lentiviral vectors	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.

Committee Vote: **For: 11** **Opposed 0:** **Abstentions: 0**

Amendments

Dimopoulos, George	Plasmid vectors obtained from a commercial vendor will be used to generate mutants of Chromobacterium species Panama (Csp_P) to investigate the metabolites responsible for insecticidal activity. These mutations are expected to abolish the virulence of Csp_P toward mosquitoes. All experiments involving Chromobacterium species will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of Chromobacterium species, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. Amendment for R-DNA registration: DN1610140109 RegSupport: Chromobacterium: P1507060111	DN1610140109	2	2	III-D-1-a; III-D-4-b	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.
Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Sawa, Akira	An FMR1 knockout human iPSC line, originally generated using lentiviral vectors and the CRISPR/Cas9 system by Sumitomo Pharma, will be obtained from the company and cultured in vitro to investigate the cellular and molecular mechanisms underlying Fragile X Syndrome (FXS). All experiments involving human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. Amendment for R-DNA registration: DN0705070219 RegSupport: Human tissue registration: B0705010119	DN0705070219	2		III-D-3-a	No

Discussion/Review

Protocol Narrative Assessment:	Acceptable. The proposal adequately describes the experiments to be performed.

Hazard Assessment and Precautions:	Acceptable. The proposal adequately describes the potential biohazards inherent in the research program and identifies proper containment procedures and PPE.
Training:	Acceptable. The proposal adequately describes the training required for personnel to perform the experiments.
Decontamination and Waste Disposal:	Acceptable. The proposal adequately describes proper decontamination and disposal procedures.
Committee Vote:	**For: 11 Opposed: 0 Abstentions: 0**

Exempt Registrations

Sfanos, Karen	Plasmid vectors obtained from a commercial vendor will be used to express CYP17A1 in cultured cells, serving as a positive control for the development of immunohistochemistry and in situ hybridization assays. All experiments involving human cells will be conducted using Biosafety Level 2 (BSL-2) practices. Laboratory personnel will be trained in the safe handling of human cells, and will follow established standard operating procedures (SOPs) for the disposal of contaminated materials and for responding to laboratory incidents. DE--Uses synthetic nucleic acid molecules in cultured cells. RegSupport: Human tissue registration:B1403120112	DE2505200101	2	Exempt: III- F-8		No
Discussion/Review	It falls under the exempt category under the NIH guidelines for research involving recombinant or synthetic nucleic acid molecules, so IBC review is not required					
Thomas, Aline	Transgenic mice obtained from a commercial vendor will be used to develop new molecular imaging biomarkers and probes for the noninvasive detection of mitochondrial dysfunction and its underlying mechanisms. DE---Uses transgenic mice.	DE2506110101	1	1 Exempt: III- D-4-c-(2)		No
Discussion/Review	It falls under the exempt category under the NIH guidelines for research involving recombinant or synthetic nucleic acid molecules, so IBC review is not required					