Johns Hopkins Medicine – Kennedy Krieger Institutional Biosafety
Committee JHM-KKI IBC Minutes for October 20, 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); Nadia Desir, Ph.D., RBP (Associate BSO, 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); Elizabeth A. Laffan, Ph.D. (Non-affiliated Member, Biology); Douglas Norris, Ph.D. (IBC member, Vector Biology and Entomology); Ms. Claudia MacAuley, L.A.T. (Non-affiliated Member, Biosafety and High Containment); Jason Villano, D.V.M. (IBC member, Animal Science); Mr. Daniel Hendrickson, MS, MA (IBC member, Assistant Vice President, Safety, Security, and Environment of Care)

Members Absent: Alan F. Scott, Ph.D. (IBC member, Molecular Biology and Genetics); Joseph B. Margolick, MD, Ph.D. (IBC member, Medicine, Microbiology and Immunology); Brigitte Gaume, Ph.D. (Non-affiliated Member, Biology)

IBC Coordinator: Ms. Tylicia McRae

The meeting was called to order at 3:01 pm.

Announcements:

No conflicts of interest were reported by IBC members.

Review and Approval of Meeting Minutes

The minutes from the September 15, 2025, meeting were approved as submitted. Dr. Villano abstained from voting on the minutes.

Clinical protocols and Amendments:

Harris Protocol, GT2510200301 (NIH Cit.: III-F), "Phase 3 Study of the Efficacy and Safety of ION582 in Children and Adults with Angelman Syndrome"

The primary objective of the protocol is to evaluate the efficacy of ION582 in participants with Angelman Syndrome receiving ION582 vs placebo. ION582 is a novel 20-nucleotide, 5-10-5 2′-*O*-methoxyethyl-DNA gapmer, mixed-backbone antisense oligonucleotide (ASO) designed to promote RNase H1–mediated degradation of human noncoding UBE3A-ATS RNA transcript.

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.

Leung Protocol, GT2510200101 (NIH Cit.: III-C-1), "A Phase 1/2/3 Open-label Study to Evaluate the Safety, Tolerability, Efficacy, Pharmacodynamics, and Pharmacokinetics of Intravenous RGX-202 Gene Therapy in Males with Duchenne Muscular Dystrophy (DMD)"

The primary objective of the protocol is to evaluate the safety and tolerability of RGX-202 in ambulant males with Duchenne Muscular Dystrophy. RGX-202 is a recombinant adeno-associated virus serotype 8 (AAV8) that contains a vector genome encoding a miniaturized dystrophin protein, (microdystrophin). This study is being conducted in 3 parts: a phase 1/2 study (Part 1) and a 2-part phase 3 study (Parts 2 and 3). Participants aged ≥ 4 years and < 12 years of age will be enrolled in Cohorts 1. Part 1 will include up to 15 participants (3 participants dosed with 1×10^{14} GC/kg and 12 participants dosed with 2×10^{14} GC/kg RGX-202). Part 2 will include a total of ~ 30 participants, which will include up to 12 participants from Part 1. Part 3 will include ~ 30 participants. The total number of participants is approximately 65. RGX-202 will be administered via a single IV infusion. A prophylactic immunosuppression regimen will be administered prior to RGX-202 administration and over the first 12 weeks following RGX-202 administration to mitigate a potential immune response. Following infusion with RGX-202, participants will be evaluated every other day from Day 2 through Day 14, every 4 days through Day 30, weekly for Weeks 5-8 and every 2 or 4 weeks through Week 20. Quarterly evaluations will be conducted through Month 24. The duration of the study for Part 1 is approximately 60 weeks, while Parts 2 and 3 last approximately 26 months.

The medical and pharmacy staff involved in the study will be trained to follow the pharmacy manual for the proper handling of RGX-202, 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: 12
Disapproval: 0
Abstain: 0

IBC Review and Recommendations of Pathogen, Infectious Agents and Biological Toxin Research Registrations

2 research registrations were presented for IBC consideration.

Review of Incidents:

No incidents were reported at this meeting.

Public Comments:

There were no public comments.

The meeting was adjourned at 3:21 pm.

October, 2025, KKI IBC R-DNA Research Registrations and Amendments

Name	Synopsis	IBC#	BSL	ABSL	NIH Cit.	Agent	Select Agent
New Registrations							
Fatemi, S. Ali	Replication-incompetent adeno-associated viral (AAV) vectors obtained from commercial sources will be used to transduce GFP and the DARS2 gene, which encodes mitochondrial aspartyl-tRNA synthetase, into cultured cells and mice to develop a gene therapy for leukoencephalopathy with brainstem and spinal cord involvement and lactate elevation (LBSL). 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 cultured cells and animals. RegSupport: Replication-incompetent adeno-associated viral (AAV) vectors:P2508270101; Human tissue registration: B0912010116	DN2508270101	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: 12 Opposed: 0 Abstentions: 0**

Fatemi, S. Ali	Replication-incompetent adeno-associated viral (AAV) vectors obtained from commercial sources will be used to transduce GFP and the DARS2 gene, which encodes mitochondrial aspartyl-tRNA synthetase, into cultured cells and mice to develop a gene therapy for leukoencephalopathy with brainstem and spinal cord involvement and lactate elevation (LBSL). 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 adeno-associated viral vectors RegSupport: R-DNA:DN2508270101; Human tissue registration: B0912010116	P2508270101	2	2	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: 12 Opposed: 0 Abstentions: 0**