Johns Hopkins Medicine – Kennedy Krieger Institutional Biosafety Committee
JHM – KKI IBC Minutes for February 16, 2026
Zoom Meeting

Members Present: Gary S. Hayward, Ph.D. (IBC Chair, Virology and Gene Therapy); Weiying Pan, Ph.D., RBP (BSO, Molecular Aspect of Drug Design and Biology); Nadia Desir, Ph.D., RBP (IBC member, Research Lab Safety and High Containment); Viji Sitther, Ph.D. (Non-affiliated Member, Plant Biology); Prashant Desai, Ph.D. (IBC member, Virology and Oncology); Ms. Claudia MacAuley, L.A.T. (Non-affiliated Member, Biosafety and High Containment); Alan F. Scott, Ph.D. (IBC member, Genetic Medicine and Molecular Biology); Jason Villano, D.V.M. (IBC member, Animal Science); Djikolngar Maouyo, Ph.D. (Non-affiliated Member, Biology)

Members Absent: Stephen C. Dahl, Ph.D., RBP (IBC member, Biology); Elizabeth A. Laffan, Ph.D. (Non-affiliated Member, Biology); Brigitte Gaume, Ph.D. (Non-affiliated Member, Biology); Douglas Norris, Ph.D. (IBC member, Vector Biology and Entomology); Joseph B. Margolick, MD, Ph.D. (IBC member, Medicine, Microbiology and Immunology); Mr. Daniel Hendrickson, MS, MA (IBC member, Assistant Vice President, Safety, Security, and Environment of Care)

IBC Coordinator: Ms. Tylicia McRae

The meeting was called to order at 3:25 pm.

Review and Approval of Meeting Minutes

The minutes of the November 17, 2025 meeting were approved as submitted.

Announcements:

No conflicts of interest were reported by IBC members.

Clinical protocols and Amendments:

Leung Protocol, GT2506160301 (NIH Cit.: III-C-1), "A Phase 1/2, Open-Label, Dose-Escalation Study to Evaluate the Safety, Tolerability, and Biological Activity of EPI-321, an AAVrh74-delivered Epigenetic Editing Therapy in Adult FSHD Patients"

The IBC received Protocol Version 7.0 dated November 19, 2025 for the above referenced study. The total number of participants increased from up to 9 to up to 12. Additional timepoints were added for needle muscle biopsy, leukocyte methylation, ELISpot and total antibody binding assays. Administrative changes and modifications 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: 9
Disapproval: 0
Abstain: 0

Smith-Hicks Protocol, GT2602160101 (NIH Cit.: III-C-1), "REVEAL 101 Study: An Open-Label, Dose-Escalation and Dose-Expansion Study of the Safety and Efficacy of a Single Intrathecal Administration of TSHA-102, an AAV9-Delivered Gene Therapy, in the Treatment of Females with Rett Syndrome"

The primary objective of the study is to assess the safety, tolerability and efficacy of TSHA-102. TSHA-102 is a recombinant, non-replicating, self-complementary adeno-associated virus serotype 9-based gene therapy product. TSHA-102 was designed to mediate levels of MECP2 in the CNS on a cell-by-cell basis without risk of overexpression. Up to 32 female participants with Rett Syndrome will be enrolled in the study. The study will take place in two parts. Part A is a first-in-human evaluation of the safety, tolerability, and exploratory efficacy of TSHA-102. The maximum tolerated dose or the maximum administered dose will be established in this part of the study. Part B is a registrational study of efficacy and safety of the selected TSHA-102 dose in a dose expansion cohort. The duration of the study is approximately 5.5 years including a 4-year extended observation period. All participants will be invited to enroll in a 10-year open-label extension study beyond the extended observation period.

The medical and pharmacy staff involved in the study have been trained to follow the pharmacy manual for the proper handling of TSHA-102, as well as the standard operating procedures (SOPs) for incident management, including spill response.

The submission complies with the requirements of the KKI 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: 9
Disapproval: 0
Abstain: 0

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

There was no research registrations 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 3:47 pm.