Johns Hopkins Medicine – Kennedy Krieger Institutional Biosafety Committee
JHM – KKI IBC Minutes for May 18, 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); Elizabeth A. Laffan, Ph.D. (Non-affiliated Member, Biology); 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); 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 3:03 pm.

Announcements:

No conflicts of interest were reported by IBC members.

Review and Approval of Meeting Minutes

The minutes of the April 20, 2026, meeting were approved as submitted.

Clinical protocols and Amendments:

Lindsey Protocol, GT2605180101 (NIH Cit.: III-F), "A Phase 2, Open-Label, Basket Study Investigating the Safety and Efficacy of GTX-102 in Adult and Pediatric Subjects with Deletion- or Nondeletion-type Angelman Syndrome; A Phase 2, Open-label, Randomized Study Investigating the Safety and Efficacy of GTX-102 in Mutation-type Angelman Syndrome ≥ 4 to < 18 Years of Age"

The primary objective of this study is to evaluate the safety and efficacy of GTX-102. GTX-102 is an 18-mer antisense oligonucleotide (ASO) that targets the human UBE3A-AS transcript when administered directly into the cerebrospinal fluid via intrathecal lumbar bolus injection. GTX-

102 is designed to inhibit transcription of UBE3A-AS on the paternal allele, thereby unsilencing the paternal copy of UBE3A and enabling expression of UBE3A protein in neurons. Given that patients with Angelman syndrome (AS) have absent or reduced functional UBE3A expression in the brain, GTX-102 has the potential to benefit all AS subpopulations included in this study.

Since GTX-102 contains fewer than 100 nucleotides, lacks the biological properties necessary for genome integration, cannot replicate within cells, and cannot be transcribed or translated, this study is exempt from the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules.

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

There were 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 3:14 pm.