Johns Hopkins Medicine – Kennedy Krieger Institutional Biosafety Committee
JHM – KKI IBC Minutes for April 20, 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

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The meeting was called to order at 3:04 pm.

**Announcements:**

No conflicts of interest were reported by IBC members.

**Review and Approval of Meeting Minutes**

The minutes of the February 16, 2026 meeting were approved with a minor change to the affiliations of two committee members.

**Clinical protocols and Amendments:**

**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 IBC received notification of the study withdrawal. No further action is considered warranted.

**Smith-Hicks Protocol, GT2506160201 (NIH Cit.: III-C-1),** "A Baseline-Controlled, Open-

Label, Multicenter, Single-Arm, Pivotal Study to Evaluate the Efficacy, Safety, and Tolerability of NGN-401 in Subjects with Rett Syndrome (EMBOLDEN)"

The IBC received Protocol Amendment 12, Version 13 dated November 07, 2025, for the above referenced study. The required hospital confinement period following NGN-401 administration was reduced from five days to two days based on safety data obtained in the Phase 1/2 portion of the study. Subjects and caregivers will remain local to the hospital and will continue to undergo daily in-clinic safety evaluations on Study Days 3, 4, and 5 after discharge. All safety assessments during this post-discharge period remain unchanged, except that the Study Day 5 ECG has been removed because it has not yielded any clinically meaningful findings to date. Text was also added regarding tokenization and data privacy. None of the changes are 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, GT2506160201 (NIH Cit.: III-C-1),** "A Baseline-Controlled, Open-Label, Multicenter, Single-Arm, Pivotal Study to Evaluate the Efficacy, Safety, and Tolerability of NGN-401 in Subjects with Rett Syndrome (EMBOLDEN)"

The IBC received Protocol Amendment 13, Version 14, dated February 12, 2026, for the above referenced study. Clinical efficacy and safety data from ongoing trial participation were updated. Safety reporting details for a new delivery device used for IP administration were added. Minor formatting changes and clarifications were made throughout the protocol. None of the changes are expected to affect the biosafety of the study.

The IBC voted to approve the amendment.

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 3:11 pm.