Johns Hopkins Medicine – Kennedy Krieger Institutional Biosafety
Committee JHM-KKI IBC Minutes for November 17, 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); 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); Ms. Claudia MacAuley, L.A.T. (Non-affiliated Member, Biosafety and High Containment); Alan F. Scott, Ph.D. (IBC member, Molecular Biology and Genetics); 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)

Members Absent: Brigitte Gaume, Ph.D. (Non-affiliated Member, Biology); Djikolngar Maouyo, Ph.D. (Non-affiliated Member, Biology); Douglas Norris, Ph.D. (IBC member, Vector Biology and Entomology); Jason Villano, D.V.M. (IBC member, Animal Science)

IBC Coordinator: Ms. Tylicia McRae

The meeting was called to order at 3:00 pm.

Announcements:

No conflicts of interest were reported by IBC members.

Review and Approval of Meeting Minutes

The minutes from the October 20, 2025, meeting were approved as submitted.

Clinical protocols and Amendments:

Leung Protocol, GT2205160101 (NIH Cit.: III-C-1), "A Two-Part Multicenter Study: A Randomized, Double-Blind, Placebo-Controlled Dose Escalation Safety Phase (Part 1) followed by Double-Blind, Placebo-Controlled, Adaptive Phase (Part 2) Study to Evaluate the Safety and Efficacy of AB-1003 in Adult Subjects with LGMD2I/R9 Mutations in the Gene Encoding Fukutin Related Protein (FKRP"

The IBC received Protocol Amendment 4, Version 5 dated October 11, 2023 for the above referenced study. The product code was revised from LION-101 to AB-1003. No changes in the manufacturing process, active substance or final formation has occurred. Findings from previous protocol clarification letters have been incorporated. Administrative changes and clarifications 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

Leung Protocol, GT2205160101 (NIH Cit.: III-C-1), "A Two-Part Multicenter Study: A Randomized, Double-Blind, Placebo-Controlled Dose Escalation Safety Phase (Part 1) followed by Double-Blind, Placebo-Controlled, Adaptive Phase (Part 2) Study to Evaluate the Safety and Efficacy of AB-1003 in Adult Subjects with LGMD2I/R9 Mutations in the Gene Encoding Fukutin Related Protein (FKRP"

The IBC received Protocol Amendment 5, Version 6 dated January 11, 2024 for the above referenced study. The schedule of activities was updated. A footnote was added to allow the sites to extend the screening period for some screening procedures. 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

Leung Protocol, GT2205160101 (NIH Cit.: III-C-1), "A Two-Part Multicenter Study: A Randomized, Double-Blind, Placebo-Controlled Dose Escalation Safety Phase (Part 1) followed by Double-Blind, Placebo-Controlled, Adaptive Phase (Part 2) Study to Evaluate the Safety and Efficacy of AB-1003 in Adult Subjects with LGMD2I/R9 Mutations in the Gene Encoding Fukutin Related Protein (FKRP"

The IBC received Protocol Amendment 6, Version 7 dated September 14, 2024 for the above referenced study. The amendment reflects a change in the visit schedule. Minor clarifications 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

Leung Protocol, GT2205160101 (NIH Cit.: III-C-1), "A Two-Part Multicenter Study: A Randomized, Double-Blind, Placebo-Controlled Dose Escalation Safety Phase (Part 1) followed by Double-Blind, Placebo-Controlled, Adaptive Phase (Part 2) Study to Evaluate the Safety and Efficacy of AB-1003 in Adult Subjects with LGMD2I/R9 Mutations in the Gene Encoding Fukutin Related Protein (FKRP"

The IBC received Protocol Amendment 7, Version 8 dated September 16, 2025 for the above

referenced study. The exclusion criteria were updated to define the cutoff value for anti-AAV9 neutralizing antibodies as >1:5. Clarifications were made throughout the schedule of activities. Formatting and grammatical changes 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

Smith-Hicks Protocol, GT2502170401 (NIH Cit.: III-C-1), "A Phase 1-2, Double Blind, Sham-Controlled Multiple Ascending Dose Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Intrathecally-Administered ION440 in Patients with MECP2 Duplication Syndrome"

The IBC received Protocol Amendment 3 dated March 7, 2025 for the above referenced protocol. None of the changes were expected to affect the biosafety of the study. ION440 is a 20-nucleotide (20-mer) antisense oligonucleotide (ASO). This protocol is consistent with the exemption 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.

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 11 dated July 28, 2025 for the above referenced study. The amendment combines country-specific RTT-200 protocols into a single global protocol and converts the Phase 1/2 study to a baseline-controlled, multicenter, single-arm, pivotal study (Embolden) to allow for the collection of registrational data for the evaluation of efficacy, safety, and tolerability of NGN-401. 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

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 dated September 29, 2025 for the above referenced study. Secondary objectives, secondary endpoints and exploratory endpoints were added to the protocol. Preliminary efficacy data were updated for a 12-month timepoint. Laboratory assessments and vaccine requirements were updated. Text was updated to clarify that EEGs could be performed anytime during the Screening and Baseline periods. Clarifications, updates to formatting and abbreviations were made throughout the protocol. None of the changes were expected to affect the biosafety of the study.

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 3:25 pm.