Johns Hopkins Medicine
All Children's Hospital Institutional Biosafety Committee
JHACH IBC Minutes for June 4, 2025
Zoom Meeting

Members Present: Neil A Goldenberg, MD, Ph.D. (Acting Chair, Pediatrics – Hematology); Stephen C. Dahl, Ph.D., RBP (BSO, Biology); Nasreen Haideri, Ph.D. (Non-affiliated Member); Gary S. Hayward, Ph.D. (IBC Chair, Virology and Gene Therapy); Jason Villano, D.V.M. (IBC member, Animal Science); Jane F. Pfeilsticker, M.S., (Non-affiliated Member); Weiying Pan, Ph.D., RBP (Associate BSO, Molecular Aspect of Drug Design and Biology); Nadia Desir, Ph.D., RBP (Associate BSO, High Containment); Alan F. Scott, Ph.D. (Molecular Biology and Genetics); Rui Zhou, Ph.D. (Medicine – Endocrinology and Metabolism); Milton Griggs, BS (Project Manager, Safety Administration)

Members Absent: Martin Trapecar, Ph.D. (IBC Chair, Medicine – Endocrinology and Metabolism)

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.

Dr. Dahl informed members of NIH's new requirement to publicly post IBC Rosters and meeting minutes. Effective June 1, 2025, the names and roles of all IBC members will be posted on the NIH website. IBC meeting minutes will be posted on the Health, Safety and Environment website.

Review and Approval of Meeting Minutes:

The minutes of the October 10, 2024 meeting were approved with the recusal of Dr. Goldenberg.

Clinical protocols and Amendments:

Chellapandian Protocol, GT2506040101 (NIH CIT.: III-C-1), "A Phase 1/2, Open-Label Study to Evaluate the Safety and Efficacy of Autologous CD19-Specific Chimeric Antigen Receptor T Cells (CABA-201) in Subjects with Active Idiopathic Inflammatory Myopathy or Active Juvenile Idiopathic Inflammatory Myopathy"

The primary objective of the study is to evaluate the safety and tolerability of CABA-201 in participants with active idiopathic inflammatory myopathies. CABA-201 is an autologous,

genetically engineered T cell product that has been edited to express the human anti CD-19 single chain variable fragment fused to tandem 4-1BB and CD3ζ signaling domains.

A minimum of 24 subjects with dermatomyositis (DM), antisynthetase syndrome (ASyS), immune-mediated necrotizing myopathy (IIM) and juvenile idiopathic inflammatory myopathies (JIIM) are expected to be enrolled in the study. At least six participants in each cohort. Cohorts with DS, ASyS and IIM will be treated in parallel. Participants with JIIM will be treated following a 28-day safety evaluation.

Patients will receive a single intravenous infusion of CABA-201 at a dose of 1 x 10^6 CAR T cells/kg of body weight on Day 1. They are then evaluated for dose-limiting toxicities (DLTs). If DLTs are observed in 2 or more subjects in any cohort, dosing will be halted and a lower dose will be evaluated. Up to 24 additional subjects from any cohort may be treated with a selected dose in an optional expansion cohort.

All participants will be followed for approximately 156 weeks for long-term safety.

The IBC voted to approve the protocol.

For Approval: 11
Disapproval: 0
Abstain: 0

Recombinant NDA/Pathogen Research Registrations and Amendments:

No 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 3:36 pm.