U-M Institutional Biosafety Committee
Minutes
Approved at the May 15, 2026, IBC Meeting

Meeting Information:

Date: Friday, April 17, 2026
Time: 1:15-2:30 p.m.
Location: Via conference call (Zoom)

Voting Members Present: Matt Chapman, Wanlu Du, Chris Fenno, Sheya Jones, Huira Kopera, Joyce Lai, Tom Lanigan, Patrick Lester, Akira Ono, Jon Oscherwitz, Stephen Rapundalo, Alex Rickard, Jackie Shields, John Thomas, Fei Wen, Christiane Wobus (Chair)

Voting Members Absent: Pamela Bennett-Baker, Daniel Lawrence, Andrew Tai

Alternate Members Present: Ingrid Bergin (alt. for Lester), Jessica Bunn (alt. for Jones), Dalis Collins (alt. for Lester), Crystal O'Donnell (alt. for Jones), Janet Follo (alt. for Jones), Krisna Rao (alt. for Tai)

IBC Staff Members Present: Jen Harley, Alicia Trombley

Guests Present: Carolyn Kuenz, Eric Robertson, Nicoleen Boyle, Andrew Kennedy, Kathy Ignatoski, Jonah Lee, Pat Ward, Sarah Lawson, Ingrid Walstad-Terpak

The meeting was called to order at: 1:15 p.m.
The meeting was adjourned at: 2:00 p.m.

Agenda Items:

1. Updates from the Chair – Christiane Wobus

Dr. Wobus stated there were no updates.

2. Consideration of minutes from the previous meeting

The committee reviewed the minutes from the March 20, 2026, meeting. There were no changes recommended.

> **Motion:** Stephen Rapundalo moved to approve the minutes.
> **Second:** Matt Chapman seconded the motion.
> **Vote:** All in favor.

3. Consideration of revised minutes from an earlier meeting

The committee reviewed revisions made to the minutes from the February 20, 2026, meeting. There were no further changes recommended.

> **Motion:** Sheya Jones moved to approve the minutes.
> **Second:** Matt Chapman seconded the motion.
> **Vote:** All in favor.

4. Biosafety Officer Report – Sheya Jones

Nicoleen Boyle reported on a needle stick injury that does not require reporting to NIH OSP.

Jessica Bunn reported follow up information on an incident that does not require reporting to NIH OSP.

5. Conflict of interest disclosure opportunity

Dr. Wobus asked committee members whether they or their labs were involved with, or were in conflict with, financially or otherwise, any items on today's agenda.

1. Christiane Wobus indicated a conflict with application IBCA0000011_AR11 for Dr. Emmer.
2. Tom Lanigan indicated a conflict with application IBCA0000046_AR15 for Dr. Soleimanpour.

6. Applications for committee action

BSL2 Applications

The following BSL2 application was considered and voted upon separately by the committee due to a conflict of interest. Tom Lanigan chaired this portion of the meeting.

19. IBCA00001782_AR05
Emmer, Brian – Amendment
Current approval: BSL1 (plasmid vectors and SARS-CoV-2 GFP reporter virus vector); BSL2 (lentivirus vectors and AAV vectors); BSL2 (SARS-CoV-2); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL2 for 3 days (mice administered AAV vectors). No work involving biological toxins, animal-derived substances, or plants.
Changes: Added work with LPS (BSL2) with administration to mice (ABSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate.

> **Motion:** Stephen Rapundalo moved to approve the (1) applications listed above, at the containment levels agreed upon, contingent on satisfactory completion of a laboratory inspection in the past year and upon any other contingencies noted above.
> **Second:** Chris Fenno seconded the motion.
> **Vote:** All in favor, with Christiane Wobus recused.

Christiane Wobus resumed chairing the meeting.

8. IBCA00000479_AR06
Soleimanpour, Scott – Renewal
Current approval: BSL1 (vectorless systems, plasmid and AAV vectors); BSL2 (adenoviral and lentiviral vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered AAV vectors). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: Added new gene elements in lentiviral vectors (BSL2) and vectorless systems (BSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate.

> **Motion:** Sheya Jones moved to approve the (1) applications listed above, at the containment

levels agreed upon, contingent on satisfactory completion of a laboratory inspection in the past year and upon any other contingencies noted above.
Second: Joyce Lai seconded the motion.
Vote: All in favor, with Tom Lanigan recused.

The following BSL2 applications were considered by the committee and voted upon:

1. IBCA00000030_AR05
Seasholtz, Audrey – Renewal
Current approval: BSL1 (plasmid and AAV vectors); BSL2 (LPS); BSL2 (human- and animal-derived substances from NHP); ABSL1 (transgenic mice); ABSL1 (mice administered AAV vectors or LPS). No work involving infectious agents or plants.
Changes: Removed work with animals and LPS.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate.

2. IBCA00000064_AR06
Ye, Bing – Amendment
Current approval: BSL1 (plasmid and AAV vectors); BSL2 (lentivirus, MoLV, and AAV vectors with growth control genes); BSL2 (TTX); BSL2 (human-derived substances); ABSL1 (transgenic mice and fruit flies); ABSL1 (mice and flies administered plasmid vectors; mice administered AAV or retroviral vectors). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: Added work with Pseudomonas aeruginosa (BSL2) with administration to C. elegans (ABSL1), work with Diphtheria toxin (BSL2) with administration to mice (ABSL1), work with transgenic C. elegans, and new gene elements in plasmid vectors (BSL1), retroviral vectors (BSL2), and AAV vectors (BSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon favorable re-review from one reviewer.

3. IBCA00000116_AR04
Cierpicki, Tomasz – Amendment
Current approval: BSL1 (plasmid vectors, MSCV vectors, and vectorless systems); BSL2 (lentivirus vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL2 for the duration (mice administered human-derived substances or rDNA modified human-derived substances). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: Added new gene elements in plasmid vectors (BSL1) and work with additional plasmid vectors (BSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment. Approval is contingent upon minor edits being made to the application.

4. IBCA00000144_AR06
Traynor, John – Amendment
Current approval: BSL1 (plasmid vectors); BSL2 (lentiviral vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered lentiviral vectors). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: Added work with AAV vectors (BSL1) with administration to mice (ABSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1

containment. The proposed animal housing containment level is considered appropriate. Approval is contingent upon favorable re-review from one reviewer.

5. IBCA00000260_AR05
Martin, Donna – Amendment
Current approval: BSL1 (plasmid vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: Added work with lentiviral vectors (BSL2).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. Approval is contingent upon minor edits being made to the application.

6. IBCA00000268_AR04
Fingar, Diane – Renewal
Current approval: BSL1 (plasmid and AAV vectors); BSL2 (lentivirus vectors); BSL2 (LPS); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered AAV vectors or LPS). No work involving infectious agents, animal-derived substances, or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application and favorable re-review from one reviewer

7. IBCA00000429_AR04
Fort, Patrice – Renewal
Current approval: BSL1 (plasmid vectors, AAV vectors, and vectorless systems); BSL2 (AAV vectors with growth control genes); BSL2 (human-derived substances); BSL2 (animal-derived substances: swine and non-human primates); ABSL1 (transgenic mice); ABSL1 (mice administered AAV vectors). No work involving infectious agents, biological toxins, or plants.
Changes: Added work with additional plasmid vectors (BSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

8. *This application was handled separately due to a conflict of interest*.

9. IBCA00000481_AR06
Keshamouni, Venkateshwar – Renewal
Current approval: BSL1 (plasmid vectors and vectorless systems); BSL2 (adenovirus and lentivirus vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered rDNA modified animal cells); ABSL2 for 3 days (mice administered adenovirus vectors); ABSL2 for the duration (mice administered human-derived substances or rDNA modified human-derived substances). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: Added work with additional plasmid vectors (BSL1) and additional retrovirus vectors (BSL2).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

10. IBCA00000601_AR05
Chun, Tae-Hwa – Renewal
Current approval: BSL1 (plasmid vectors and vectorless systems); BSL2 (adenovirus, MoLV, and lentivirus vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL2 for 3 days (mice administered retrovirus vectors). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

11. IBCA00000646_AR03
Liu, Fei – Renewal
Current approval: BSL2 (adenovirus vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: Updated work with adenovirus vectors.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate.

12. IBCA00000719_AR04
Wilson, Thomas – Renewal
Current approval: BSL1 (plasmid vectors and vectorless systems); BSL2 (human-derived substances). No work involving infectious agents, biological toxins, animal-derived substances, animals or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the work described is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. Approval is contingent upon minor edits being made to the application.

13. IBCA00000843_AR04
Parent, Carole – Amendment
Current approval: BSL1 (plasmid and AAV vectors); BSL2 (MSCV vectors); BSL2 (Pertussis toxin, Cholera toxin, LPS, Phalloidin); BSL2 (human-derived substances). No work involving infectious agents, animal-derived substances, animals or plants.
Changes: Added work with RG2 fungi (BSL2).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment. The proposed animal housing containment level is considered appropriate.

14. IBCA00001009_AR10
Hammer, Gary – Amendment
Current approval: BSL1 (plasmid and AAV vectors); BSL2 (lentivirus vectors and AAV vectors with tetanus toxin gene); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered AAV vectors or rDNA modified animal cells); ABSL2 for the duration (mice administered human-derived substances or rDNA modified human-derived substances). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: Added work with vectorless systems (BSL1) with administration to mice (ABSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1

containment. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

15. IBCA00001399_AR06
Beard, Daniel – Amendment
Current approval: BSL1 (plasmid vectors); BSL2 (human-derived substances); BSL1 (animal-derived substances: swine); ABSL1 (transgenic rats). No work involving infectious agents, biological toxins, or plants.
Changes: Updated research goals and updated risk mitigation practices.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate.

16. IBCA00001658_AR03
Moon, Stephanie – Renewal
Current approval: BSL1 (vectorless systems and plasmid vectors); BSL2 (lentiviral and MoLV vectors); BSL2 (human-derived substances). No work involving infectious agents, biological toxins, animal-derived substances, animals or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate.

17. IBCA00001721_AR03
Kennedy, Lucy - ULAM Germ-Free Mouse Facility – Renewal
Current approval: BSL2 (RG2 bacteria); BSL2 (human-derived substances);BSL1 (animal-derived substances: wild mice); ABSL1 (transgenic mice); ABSL1 (mice administered animal-derived substances); ABSL2 for the duration (mice administered human-derived substances or RG2 bacteria). No work involving rDNA, biological toxins or plants.
Changes: Updated risk mitigation practices and removed work with animal-derived substances.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

18. IBCA00001764_AR05
Cras-Meneur, Corentin - Islet Core – Renewal
Current approval: BSL2 (adenovirus vectors); BSL2 (human-derived substances); ABSL1 (transgenic rodents). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

19. *This application was handled separately due to a conflict of interest*.

20. IBCA00002295_AR02
Piotrowski-Daspit, Alexandra – Renewal
Current approval: BSL1 (plasmid vectors and vectorless systems); BSL2 (lentiviral vectors); BSL2 (human-derived substances and animal-derived substances from NHP, sheep, swine); ABSL1 (transgenic

mice); ABSL1 (mice administered vectorless systems or plasmid vectors). No work involving infectious agents, biological toxins, or plants.
Changes: Removed work with lentivirus vectors.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

21. IBCA00002366_AR03
Jensen, Paul – Renewal
Current approval: BSL1 (plasmid vectors and vectorless systems); BSL2 (RG2 bacteria). No work involving biological toxins, human- or animal-derived substances, animals or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate.

22. IBCA00002381_AR05
Thompson, Cody – Renewal
Current approval: BSL2 (animal-derived substances from wild animals). No work involving rDNA, infectious agents, biological toxins, human-derived substances, animals or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate.

23. IBCA00002500_AR08
Kozik, Ariangela – Amendment
Current approval: BSL1 (plasmid vectors and vectorless systems); BSL1 (RG1 bacteria); BSL2 (RG2 bacteria); BSL2 (LPS); BSL2 (human-derived substances). No work involving animal-derived substances, animals or plants.
Changes: Added work with additional risk group 2 bacteria (BSL2).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate.

24. IBCA00002702_AR05
Ruas, Jorge – Amendment
Current approval: BSL1 (vectorless systems, plasmid, and baculoviral vectors); BSL2 (AAV, MSCV, and adenoviral vectors); BSL2 (Notexin, LPS); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered Notexin, LPS, plasmid vectors or vectorless systems); ABSL2 for 3 days (mice administered adenoviral, baculoviral, or MSCV vectors). No work involving infectious agents, animal-derived substances, or plants.
Changes: Added new gene elements in plasmid vectors (BSL1) and in adenoviral vectors (BSL2) and work with lentiviral vectors (BSL2).
Consensus: The committee agreed with the reviewers that the work described is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. Approval is contingent upon minor edits being made to the application.

25. IBCA00002812_AR02
Fung, Herman – Amendment
Current approval: BSL1 (plasmid and baculovirus vectors; vectorless systems); BSL2 (human-derived

substances). No work involving infectious agents, biological toxins, animal-derived substances, animals or plants.
Changes: Added work with MoLV and lentivirus vectors (BSL2) and updated research goals with human-derived substances.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. Approval is contingent upon favorable review from one reviewer.

26. IBCA00003150
Kropp, Erin - Initial Application
Proposed: BSL1 (plasmid vectors); BSL2 (lentiviral vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered rDNA-modified animal cells); ABSL2 for the duration (mice administered rDNA-modified human-derived substances). No work involving infectious agents, biological toxins, animal-derived substances, or plants.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 and BSL2 containment and that BSL2 risk mitigation practices are likewise appropriate. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application and favorable review from one reviewer.

> **Motion:** Tom Lanigan motioned to approve the (24) IBC applications listed above at the containment levels agreed upon, contingent on satisfactory completion of a laboratory inspection in the past year and upon any other contingencies noted above.
> **Second:** Stephen Rapundalo seconded the motion.
> **Vote:** All in favor.

BSL1 Applications

The following BSL1 applications were considered by the committee and voted upon.

27. IBCA00000639_AR05
Miller, Ann – Renewal
Current approval: BSL1 (plasmid vectors, vectorless systems, and lentivirus-modified animal cells); ABSL1 (transgenic frogs); ABSL1 (frogs administered vectorless systems or plasmid vectors). No work involving infectious agents, biological toxins, human- or animal-derived substances, or plants.
Changes: Added new gene elements in plasmid vectors and vectorless systems (BSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

28. IBCA00000791_AR03
Watson, Brendon – Renewal
Current approval: BSL1 (AAV vectors); ABSL1 (transgenic rodents); ABSL1 (rodents administered AAV vectors). No work involving infectious agents, biological toxins, human- or animal-derived substances, or plants.
Changes: Added work with transgenic Nile grass rats (ABSL1) and additional transgenic mice (ABSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment. The proposed animal housing containment level is considered appropriate.

29. IBCA00001741_AR03
Dantzer, Ben – Renewal

Current approval: BSL1 (animal-derived substances: voles); ABSL1 (transgenic voles). No work involving rDNA, infectious agents, biological toxins, human-derived substances or plants.
Changes: Updated research goals. Removed work with transgenic voles.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment. The proposed animal housing containment level is considered appropriate.

30. IBCA00001925_AR01
Demirci, Hakan – Renewal
Current approval: ABSL1 (transgenic mice). No work involving rDNA, infectious agents, biological toxins, human- or animal-derived substances, or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the proposed animal housing containment level is considered appropriate.

31. IBCA00002262_AR01
Farkash, Evan – Renewal
Current approval: BSL1 (animal-derived substances: swine). No work involving rDNA, infectious agents, biological toxins, human-derived substances, animals or plants.
Changes: No major changes.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment.

32. IBCA00002417_AR01
Hsu, Cindy – Renewal
Current approval: BSL1 (animal-derived substances: swine). No work involving rDNA, infectious agents, biological toxins, human-derived substances, animals or plants.
Changes: Updated research goals.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment.

33. IBCA00002572_AR01
Sztain, Terra – Amendment
Current approval: BSL1 (plasmid vectors). No work involving infectious agents, biological toxins, human- or animal-derived substances, animals or plants.
Changes: Added new gene elements in plasmid vectors (BSL1).
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment.

34. IBCA00003158
Zhu, Yunlu - Initial Application
Proposed: BSL1 (plasmid vectors and vectorless systems); ABSL1 (transgenic zebrafish); ABSL1 (zebrafish administered plasmid vectors). No work involving infectious agents, biological toxins, human- or animal-derived substances, or plants.
Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment. The proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application.

> **Motion:** Matt Chapman motioned to approve the (8) IBC applications listed above at the containment levels agreed upon.
> **Second:** Joyce Lai seconded the motion.

Vote: All in favor

7. Discussion Items

Item 1. Human Gene Transfer Application – Krishna Rao

HUM00289353
PI: Dinesh Khanna
Title: Breakfree-SSc BMS (CA0611005)
Sponsor: Juno Therapeutics, Inc.

Krishna Rao described the study for the committee. He and Andrew Tai have reviewed the current submission and express support for approval of this trial. Dr. Tai noted in his review that Systemic sclerosis (SSc) is a rare multiorgan autoimmune disease characterized by inflammation, fibrosis, and vasculopathy. Lung and skin involvement are common; about half develop interstitial lung disease (ILD), which accounts for about 35% of all SSC-related deaths. Because B cells play a pathogenic role in SSc, anti-CD19 CAR-T therapy may improve outcomes. This is a phase 3, randomized, open-label clinical trial of an anti-CD19 CAR-T cell therapy in patients age 16 or older with active SSc and ILD. CD4+ and CD8+ T cells are selected from the patient's PBMCs and then transduced by a replication-defective lentiviral vector encoding the CD19 CAR. Importantly, the CD19 CAR is identical to the CAR encoded by the FDA-approved product lisocabtagene maraleucel ("liso-cel"). The CAR-T cell product is made by a newer manufacturing process that shortens manufacturing time and leads to a less-differentiated T cell phenotype with greater expansion potential when compared to the FDA-approved liso-cel product. Observed/potential AEs are consistent with those seen with other CD19 CAR-T cell products.

Motion: Sheya Jones moved to approve the human gene transfer application at BSL2 containment.
Second: Stephen Rapundalo seconded the motion.
Vote: All in favor.

Item 2. Human Gene Transfer Application – Krishna Rao

HUM00289356
PI: Jacqueline Madison
Title: Breakfree-SLE BMS (CA0611011)
Sponsor: Juno Therapeutics, Inc.

Krishna Rao described the study for the committee. He and Andrew Tai have reviewed the current submission and express support for approval of this trial. Dr. Tai noted in his review that Systemic lupus erythematosus (SLE) is an immune-mediated disease with dysfunction of B cells that leads to autoantibody formation. This in turn results in damage to virtually any organ system, most often musculoskeletal and renal. Because SLE pathogenesis is mediated by B cells, deep depletion of B cells may 'reset' the immune system in SLE. This is a phase 2, open-label study of a CD19 CAR-T cell product in participants ≥ 16 years of age with active SLE who have not had an adequate clinical response to corticosteroids and at least two immunosuppressants for at least 3 months each. CD4+ and CD8+ T cells are selected from the patient's PBMCs and then transduced by a replication-defective lentiviral vector encoding the CD19 CAR. Importantly, the CD19 CAR is identical to the CAR encoded by the FDA-approved product lisocabtagene maraleucel ("liso-cel"). The CAR-T cell product is made by a newer manufacturing process that shortens manufacturing time and leads to a less-differentiated T cell

phenotype with greater expansion potential when compared to the FDA-approved liso-cel product. Observed/potential AEs are consistent with those seen with other CD19 CAR-T cell products.

> **Motion:** Tom Lanigan moved to approve the human gene transfer application at BSL2 containment.
> **Second:** Sheya Jones seconded the motion.
> **Vote:** All in favor.

Item 3. Human Gene Transfer Application – Krishna Rao

HUM00289328
PI: Julie Ziobro
Title: ENDEAVOR
Sponsor: Encoded Therapeutics, Inc.

Krishna Rao described the study for the committee. He and Andrew Tai have reviewed the current submission and express support for approval of this trial. Dr. Tai noted in his review that Dravet syndrome is an autosomal dominant genetic disorder manifesting primarily as severe prolonged seizures and global developmental delays, with an increased risk of death related to seizures. Over 85% of cases are caused by loss-of-function mutations in the SCN1A gene encoding the alpha subunit 1 of the voltage-gated sodium channel. It affects about 1:15,000 children. Current standard of care uses antiepileptic medications, but seizure freedom is uncommon, and they do not address the developmental delay and intellectual disability. This is a phase 1/2 clinical trial of a replication-defective recombinant AAV9 vector encoding an engineered transcription factor designed to promote transcription of the SCN1A gene in GABAergic cells. In nonclinical studies, the vector reduced the frequency and severity of seizures and prolonged survival in a Dravet syndrome mouse model. Widespread transgene expression was detected up to at least 470 days in mice and a year in NHPs. No adverse findings were identified.

> **Motion:** Sheya Jones moved to approve the human gene transfer application at BSL1 containment.
> **Second:** Joyce Lai seconded the motion.
> **Vote:** All in favor.

8. Matters Arising

There were no matters arising.