

**U-M Institutional Biosafety Committee
Minutes**

Approved at the June 19, 2026, IBC Meeting

Meeting Information:

Date: Friday, May 15, 2026

Time: 1:15-2:30 p.m.

Location: Via conference call (Zoom)

Voting Members Present: Matt Chapman, Wanlu Du, Chris Fenno, Sheya Jones, Huiira Kopera, Joyce Lai, Daniel Lawrence, 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, Tom Lanigan, Andrew Tai

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

IBC Staff Members Present: Jen Harley, Michael Santiago-Castro, Alicia Trombley

Guests Present: Carolyn Kuenz, Eric Robertson, Nicoleen Boyle, 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: 2:07 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 April 17, 2026, meeting. There were no changes recommended.

Motion: Sheya Jones moved to approve the minutes.

Second: Huiira Kopera seconded the motion.

Vote: All in favor.

3. Biosafety Officer Report – Sheya Jones

There were no reportable incidents this month.

4. 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_AR17 for Dr. Wobus.
2. Huiira Kopera indicated a conflict with application IBCA00000700_AR09 for Dr. D'Silva.

3. Chris Fenno indicated a conflict with application IBCA00000700_AR09 for Dr. D'Silva.
4. Wanlu Du indicated a conflict with application IBCA00001174_AR06 for Dr. Du and IBCA00000322_AR08 for Dr. Duan.
5. Dan Lawrence indicated a conflict with application IBCA00000452_AR09 for Dr. Gudjonsson and IBCA00000016_AR12 for Dr. Wang.
6. Alex Rickard indicated a conflict with application IBCA00000654_AR07 for Dr. Fabiilli.

5. Applications for committee action

BSL2 Applications

The following BSL2 applications were considered and voted upon separately by the committee due to a conflict of interest. Chris Fenno chaired this portion of the meeting.

1. IBCA00000011_AR17

Wobus, Christiane – Amendment

Current approval: BSL1 (vectorless systems, plasmid, and baculoviral vectors); BSL2 (lentiviral vectors); BSL2 (RG2 viruses and bacteria); BSL2 (LPS); BSL2 (human- and animal-derived substances from non-human primates); ABSL1 (transgenic mice); ABSL2 for the duration (mice administered human-derived substances or RG2 infectious agents). No work involving plants.

Changes: Added administration of Norovirus to zebrafish embryos (ABSL2 for the duration) and updated risk mitigation practices.

Consensus: The committee agreed with the reviewers that BSL2 risk mitigation practices are 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: Matt Chapman seconded the motion.

Vote: All in favor, with Christiane Wobus recused.

Christiane Wobus resumed chairing the meeting.

14. IBCA00000700_AR09

D'Silva, Nisha – Renewal

Current approval: BSL1 (plasmid, phage, AAV vectors, and AAVrg vectors; vectorless systems); BSL2 (adenovirus and lentivirus vectors); BSL1 (E. coli (genetically crippled strains)); BSL2 (RG2 bacteria); BSL1 (cholera toxin subunit B); BSL2 (LPS); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered cholera toxin subunit B, AAV vectors, AAVrg vectors, or rDNA modified animal cells); ABSL2 for the duration (mice administered human-derived substances or rDNA modified human-derived substances). No work involving 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.

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: Matt Chapman seconded the motion.

Vote: All in favor, with Huira Kopera and Chris Fenno recused.

2. IBCA00000016_AR12

Wang, Xueding – Amendment

Current approval: BSL1 (AAV vectors); BSL2 (human- and animal-derived substances from chickens and swine); ABSL1 (transgenic mice); ABSL1 (mice administered rDNA-modified animal cells; rabbits administered AAV vectors); ABSL2 for the duration (rodents or rabbits administered human-derived substances and mice administered rDNA-modified human-derived substances). No work involving infectious agents, biological toxins, or plants.

Changes: Added work with transgenic rabbits (ABSL1).

Consensus: The committee agreed with the reviewers that the proposed animal housing containment level is considered appropriate.

7. IBCA00000322_AR08

Duan, Bo – Amendment

Current approval: BSL1 (AAV vectors and rabies vectors); BSL2 (lentivirus vectors); BSL1 (Cholera toxin subunit B); BSL2 (Diphtheria toxin, LPS, and tetrodotoxin); ABSL1 (transgenic mice); ABSL1 (mice administered rabies vectors, Cholera toxin subunit B, LPS, Diphtheria toxin, AAV vectors or retrovirus vectors). No work involving infectious agents, human- or animal-derived substances, or plants.

Changes: Added work with human-derived substances (BSL2) and administration of rDNA modified animal cells to mice (ABSL2 for 3 days).

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.

8. IBCA00000452_AR09

Gudjonsson, Johann – Renewal

Current approval: BSL1 (plasmid vectors); BSL2 (lentivirus vectors); BSL2 (Papillomaviruses); BSL2 (LPS); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered LPS); ABSL2 for the duration (mice administered human-derived substances or Papillomaviruses). No work involving animal-derived substances or plants.

Changes: Updated genetic elements and risk mitigation practices (BSL2). Removed work with LPS and papillomaviruses.

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 by one reviewer.

12. IBCA00000654_AR07

Fabiilli, Mario – Renewal

Current approval: BSL1 (plasmid vectors); BSL2 (adenovirus vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered plasmid vectors); ABSL2 for 3 days (mice administered adenovirus vectors); ABSL2 for the duration (mice administered human-derived substances). 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.

19. IBCA00001174_AR06

Du, Wanlu – Amendment

Current approval: BSL1 (plasmid vectors and AAV vectors); BSL2 (lentivirus vectors); BSL2 (Salmonella enterica (Typhimurium)); BSL2 (Iberitoxin); BSL2 (human-derived substances); BSL2 (animal-derived substances: non-human primates); ABSL1 (transgenic rodents); ABSL1 (mice administered AAV vectors); ABSL2 for 3 days (mice administered rDNA modified animal cells); ABSL2 for the duration (mice administered rDNA modified human-derived substances or Salmonella enterica (Typhimurium)). No work involving plants.

Changes: Added work with additional human-derived substances (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.

Motion: Sheya Jones moved to approve the (5) 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: Matt Chapman seconded the motion.

Vote: All in favor, with Dan Lawrence, Wanlu Du, and Alex Rickard recused.

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

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

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

3. IBCA00000049_AR11

Ferrario, Carrie – Amendment

Current approval: BSL1 (plasmid and AAV vectors); BSL2 (lentivirus vectors); BSL2 (LPS and TTX); BSL2 (human-derived substances); ABSL1 (transgenic rodents); ABSL1 (rodents administered AAV vectors or retrovirus vectors; rats administered LPS). No work involving infectious agents, animal-derived substances, or plants.

Changes: Updated risk mitigation practices (BSL2).

Consensus: The committee agreed with the reviewers that the described BSL2 risk mitigation practices are appropriate.

4. IBCA00000114_AR06

Vanini, Giancarlo – Amendment

Current approval: BSL1 (AAV vectors); BSL2 (plasmid and AAV vectors with toxin genes); ABSL1 (transgenic mice); ABSL1 (mice administered AAV vectors). No work involving infectious agents, biological toxins, human- or animal-derived substances, or plants.

Changes: Added new gene elements in AAV vectors (BSL1) and updated risk mitigation practices.

Consensus: The committee agreed with the reviewers that the described work is appropriate for BSL1 containment. The committee agreed with the reviewers that the described BSL2 risk mitigation practices are appropriate.

5. IBCA00000138_AR08

Shavit, Jordan – Amendment

Current approval: BSL1 (plasmid vectors and vectorless systems); BSL2 (Klebsiella pneumoniae);

BSL2 (LPS); BSL2 (human-derived substances); ABSL1 (transgenic mice and zebrafish); ABSL1 (zebrafish administered LPS); ABSL2 for the duration (zebrafish administered *Klebsiella pneumoniae*). No work involving animal-derived substances or plants.

Changes: Added work with pathogenic *E. coli* (BSL2) with administration to zebrafish (ABSL2 for the duration).

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.

6. IBCA00000312_AR05

Cadigan, Kenneth – Renewal

Current approval: BSL1 (plasmid vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice, worms, and fruit flies). No work involving infectious agents, biological toxins, animal-derived substances, or plants.

Changes: Added new gene elements in plasmid vectors (BSL1) and updated research goals. Removed work with rodents.

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.

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

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

9. IBCA00000516_AR03

Shayman, James – Renewal

Current approval: BSL1 (plasmid vectors and vectorless systems); BSL2 (LPS); BSL2 (human- and animal-derived substances from NHP); ABSL1 (transgenic mice); ABSL1 (mice administered LPS). No work involving infectious agents or plants.

Changes: Added new gene elements in plasmid vectors (BSL1). Removed work with animal-derived substances and human plasma.

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.

10. IBCA00000563_AR06

Parkos, Charles – Renewal

Current approval: BSL1 (plasmid and baculoviral vectors); BSL2 (adenoviral, MoLV, and lentiviral vectors); BSL2 (RG2 bacteria); BSL2 (LPS); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered LPS); ABSL2 for 3 days (mice administered adenoviral or lentiviral vectors); ABSL2 for the duration (mice administered human-derived substances, rDNA-modified human-derived substances, or RG2 bacteria). No work involving animal-derived substances or plants.

Changes: Added new gene elements in lentivirus 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.

11. IBCA00000634_AR04

Lo, Joe – Renewal

Current approval: BSL2 (human-derived substances). No work involving rDNA, infectious agents, biological toxins, animal-derived substances, animals or plants.

Changes: Updated research goals and risk mitigation practices.

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.

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

13. IBCA00000659_AR06

Martens, Eric – Renewal

Current approval: BSL1 (plasmid vectors); BSL2 (plasmid vectors in RG2 host); BSL1 (RG1 bacteria and viruses); BSL2 (RG2 bacteria); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL2 for the duration (mice administered human-derived substances, RG2 infectious agents, or rDNA-modified bacteria). No work involving biological toxins, animal-derived substances, or plants.

Changes: Added work with additional RG2 bacteria (BSL2) with administration to mice (ABSL2 for the duration).

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.

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

15. IBCA00000765_AR06

Antonellis, Anthony – Renewal

Current approval: BSL1 (plasmid vectors and vectorless systems); BSL2 (lentivirus and MSCV vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice and *C. elegans*); ABSL1 (*C. elegans* administered vectorless systems). 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 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.

16. IBCA00000792_AR08

Pennathur, Subramaniam – Amendment

Current approval: BSL1 (plasmid and AAV vectors, vectorless systems); BSL2 (lentiviral vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered vectorless systems or AAV vectors). No work involving infectious agents, biological toxins, animal-derived substances, or plants.

Changes: Added work with additional human-derived substances (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.

17. IBCA00000874_AR07

Rui, Liangyou – Renewal

Current approval: BSL1 (plasmid and AAV vectors); BSL2 (lentiviral, adenoviral, MoLV, MSCV, pseudorabies, rabies and AAV vectors with growth control genes); BSL2 (LPS, Diphtheria toxin); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered LPS, Diphtheria

toxin, rDNA-modified animal cells, AAV, plasmid, rabies, or pseudorabies vectors); ABSL2 for 3 days (mice administered adenoviral, retroviral, or AAV vectors with growth control genes); ABSL2 for the duration (mice administered human-derived substances or rDNA-modified human-derived substances). 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.

18. IBCA00000985_AR11

Li, Peng – Renewal

Current approval: BSL1 (plasmid and AAV vectors); BSL2 (HSV and rabies vectors; AAV vectors with growth control genes); BSL1 (Cholera toxin subunit B and 192-IgG Saporin); BSL2 (LPS); ABSL1 (transgenic mice); ABSL1 (mice administered Cholera toxin subunit B, Saporin, LPS, AAV vectors, or rabies virus vectors); ABSL2 for 3 days (mice administered HSV vectors). No work involving infectious agents, human- or animal-derived substances, or plants.

Changes: Added work with additional 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 minor edits being made to the application.

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

20. IBCA00001301_AR04

Courtney, Adam – Amendment

Current approval: BSL1 (plasmid vectors); BSL2 (retrovirus vectors); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered 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 new gene elements in lentivirus and MSCV 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.

21. IBCA00001946_AR04

Xu, Guan – Amendment

Current approval: BSL2 (human- and animal-derived substances from swine); ABSL1 (transgenic mice). No work involving rDNA, infectious agents, biological toxins, or plants.

Changes: Updated risk mitigation practices.

Consensus: The committee agreed with the reviewers that the described BSL2 risk mitigation practices are appropriate.

22. IBCA00002339_AR05

Shenoy, Anukul – Renewal

Current approval: BSL1 (vectorless systems and plasmid vectors); BSL2 (AAV vectors); BSL2 (RG2 bacteria, viruses, and fungi); BSL2 (Pertussis toxin, Diphtheria toxin, Pneumolysin, and LPS); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered vectorless systems, Diphtheria toxin, Pneumolysin, Pertussis toxin, LPS, or rDNA-modified animal cells); ABSL2 for 3 days

(mice administered AAV vectors); ABSL2 for the duration (mice administered RG2 infectious agents or rDNA-modified RG2 bacteria). No work involving animal-derived substances or plants.

Changes: Added work with additional AAV vectors (BSL2) 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. The proposed animal housing containment level is considered appropriate.

23. IBCA00002393_AR02

Kim, Minji – Renewal

Current approval: BSL1 (plasmid vectors); BSL2 (human-derived substances). No work involving rDNA, infectious agents, biological toxins, animal-derived substances, animals or plants.

Changes: Updating 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.

24. IBCA00002698_AR02

Cernak, Timothy – Amendment

Current approval: BSL2 (RG2 bacteria, fungi, and parasites); BSL2 (Saxitoxin); BSL2 (human-derived substances); BSL2 (animal-derived substances: wild vertebrate animals). No work involving rDNA, animals or plants.

Changes: Added work with Gonyautoxin (BSL2) with administration to amphibians (ABSL2 for the duration).

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.

25. IBCA00003095

Lacy, Katelyn - Nucleic Acid Therapeutics Core - Initial Application

Proposed: BSL1 (vectorless systems); BSL2 (human-derived substances); ABSL1 (transgenic mice); ABSL1 (mice administered vectorless systems). 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 of the application is contingent upon favorable review by one reviewer.

Discussion: Jackie Shields explained when this core lab serves as a *provider* of synthetic nucleic acid products, the OSTP Framework for Nucleic Acid Synthesis will apply. Currently, the OVPR research compliance team is working with Dr. Lacey and the core director to establish a governance plan ensuring conditions of the Framework can be met before they initiate work as a provider. The Framework focuses on biosecurity safeguards, not on biosafety. The IBC's review of this application is by virtue of IBC's regular purview over biosafety; the IBC has not been assigned responsibility for reviewing compliance with the Framework.

26. IBCA00003166

Yoder-Nowak, Teresa - Initial Application

Proposed: BSL1 (animal-derived substances from cattle and pigs); ABSL1 (leeches administered animal-derived substances). No work involving rDNA, infectious agents, biological toxins, human-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: Sheya Jones motioned to approve the (19) 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: Chris Fenno seconded the motion.

Vote: All in favor.

BSL1 Applications

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

27. IBCA00000717_AR03

Semrau, Jeremy – Amendment

Current approval: BSL1 (plasmid vectors). No work involving infectious agents, biological toxins, human- or animal-derived substances, animals 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 containment.

28. IBCA00001220_AR03

Tong, Xin – Renewal

Current approval: BSL1 (plasmid and AAV vectors). No work involving infectious agents, 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 containment.

29. IBCA00003170

Su, Yajuan - Initial Application

Proposed: ABSL1 (transgenic mice). No work involving rDNA, infectious agents, biological toxins, human- or animal-derived substances, or plants.

Consensus: The committee agreed with the reviewers that the proposed animal housing containment level is considered appropriate.

30. IBCA00003180

Zhou, Kevin - Initial Application

Proposed: ABSL1 (transgenic zebrafish). No work involving rDNA, infectious agents, biological toxins, human- or animal-derived substances, or plants.

Consensus: The committee agreed with the reviewers that the proposed animal housing containment level is considered appropriate. Approval is contingent upon minor edits being made to the application and favorable review by one reviewer.

Motion: Sheya Jones motioned to approve the (4) IBC applications listed above at the containment levels agreed upon.

Second: John Thomas seconded the motion.

Vote: All in favor

6. Discussion Items

Item 1. Human Gene Transfer Application – Christiane Wobus**HUM00286418****PI: Thomas Wubben****Title:** A Phase 1/2/3 Study of ATSN-201 Gene Therapy in Subjects with RS1-Associated X-linked Retinoschisis**Sponsor:** Atsena Therapeutics, Inc.

Christiane Wobus described the study for the committee. Andrew Tai has reviewed the current submission and expressed support for approval of this trial. Dr. Tai noted in his review that X-linked retinoschisis (XLRS) is one of the most common causes of juvenile macular degeneration in males. Affected males experience reduced visual acuity that cannot be corrected with glasses. There is no specific treatment for XLRS. XLRS is associated with loss-of-function mutations in the RS1 gene, which encodes an extracellular protein expressed primarily by photoreceptors. The therapeutic strategy is to introduce a normal RS1 gene by an AAV vector delivered by subretinal injection. Preclinical studies have demonstrated efficacy in a RS1 KO mouse model of XLRS. Biodistribution studies have shown minimal vector shedding in tears and nasal swabs, and detection of vector sequence in some NHPs in non-ocular tissues (spleen, liver, mandibular and/or mesenteric lymph nodes). Major vector-related risks include intraocular inflammation and progressive perifoveolar atrophy.

Motion: Sheya Jones moved to approve the human gene transfer application at BSL1 containment.

Second: Matt Chapman seconded the motion.

Vote: All in favor.

7. Matters Arising

Jackie Shields reminded committee members to complete the IBC Member Survey which is in Qualtrics.