



**USP Workshop on CMC for Gene Therapy:
Regulations, Standards and Quality
February 18-19, 2020
USP Headquarters, Rockville, Maryland, USA
FINAL AGENDA~ February 17, 2020**

Day One: Tuesday, February 18, 2020

- 8:00 a.m. Registration & Coffee**
- 8:30 a.m. USP Welcome**
Fouad Atouf
Vice President, Global Biologics, USP
- 8:40 a.m. Workshop Overview**
Mo Heidarani
Chair, USP Gene Therapy Workshop Steering Committee, and Member, USP BIO3 – Complex Biologics Expert Committee
- 8:50 a.m. –10:30 a.m. Session I – Regulatory and Raw Material Considerations**
Session Chair: Maritza McIntyre
USP Gene Therapy Workshop Steering Committee member; Advanced Therapies Partners
- 8:55 a.m. Suitable Raw Material Qualification for Gene Therapy Products**
Angela Whatley
CBER, FDA
- 9:20 a.m. Current Good Manufacturing Practices (CGMP) Compliance Issues for Gene Therapy Products**
Heath Coats
Parexel
- 9:45 a.m. Considerations for Nucleic Acids and Enzymes in Gene Therapy: Evaluating the Impact of How Materials are Used on Design, Characterization and Specifications**
James Brown
Aldevron
- 10:10 a.m. Panel Discussion/Q&A**
- 10:30 a.m. Networking Break**
- 10:50 a.m. –2:20 p.m. Session II – Analytical and Control Strategies**
Session Chair: Mo Heidarani
Chair, USP Gene Therapy Workshop Steering Committee; Member, USP BIO3 – Complex Biologics Expert Committee; Parexel
- 10:55 a.m. Phase-Appropriate Analytical Control Strategies for Recombinant AAV-Based Gene Therapy Products: Case Studies in the Characterization of a Process-Related Impurity and Development of an In-Vitro Potency Assay**
Eric Yearley
BridgeBio

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- 11:20 a.m. **Viral Vector Manufacturing and Control Strategies**
Richard Snyder
ThermoFisher
- 11:45 a.m. **Developing a Potency Assay for GeneRide Products: an AAV-based Genome Editing Platform**
Lauren Drouin
LogicBio Therapeutics
- 12:10 p.m. Networking Lunch**
- 1:00 p.m. **Potency Assessment for Novel AAV-based drugs: Exploration of Different Biological Activities and Stability Indicating Properties of the Assays**
Marina Feschenko
Biogen
- 1:25 p.m. **Analytical Control Strategy for mRNA-based Vaccines**
Frank Zhang
GlaxoSmithKline
- 1:50 p.m. Session II Panel Discussion/Q&A**
- 2:10 p.m. –3:30 p.m. Session III – Novel Approaches for Characterization**
Session Chair: Nate Weinstock
USP Gene Therapy Workshop Steering Committee member; Biogen
- 2:15 p.m. **Charge Detection Mass Spectrometry for Characterization of AAV Capsids**
Benjamin Draper
Megadalton
- 2:40 p.m. **Encapsidated rAAV Genome Characterization by Next Generation Sequencing (NGS)**
Hui-wen Liu
Biogen
- 3:05 p.m. **Analytical Methods for the Quantification of Residual DNA and Empty Capsids in Gene Therapy Vectors**
Sushma Ogram
ThermoFisher
- 3:30 p.m. **Panel Discussion/Q&A**
- 3:50 p.m. –5:00 p.m. Networking Reception**



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Day Two: Wednesday, February 19, 2020

- 8:00 a.m.** **Registration & Coffee**
- 8:30 a.m. – 10:30 a.m.** **Session IV – Novel Approaches for Characterization, continued**
Session Chair: James Brown
USP Gene Therapy Workshop Steering Committee member; Aldevron
- 8:35 a.m.** **Characterize and Quantify AAV by DLS and SEC-MALS**
Sophia Kenrick
Wyatt Technology Corporation
- 9:00 a.m.** **Holistic Characterization of AAV Capsid Particles Using Advanced Analytical Tools**
Qin Zou
Pfizer
- 9:25 a.m.** **Analytical Methodologies for Analysis of AAV Viral Vector and Nucleic Acids for Gene Therapy**
Sahana Mollah
SCIEX
- 9:50 a.m.** **Panel Discussion / Q&A**
- 10:30 a.m.** **Networking Break**
- 11:00 a.m. –2:00 p.m.** **Session V – Standards to Facilitate Gene Therapy Product Development and Quality**
Chair: Maura Kibbey
USP
- 11:05 a.m.** **Update from the Standards Coordinating Body in Support of Gene Therapy**
Peter Reczek and Catherine Zander
Standards Coordinating Body
- 11:30 a.m.** **An International Collaboration on the Establishment of the 1st WHO International Standard for Gene Therapy**
Yuan Zhao
NIBSC
- 12:00 p.m.** **Lunch**
- 1:00 p.m.** **Global Harmonization and Standards for Regenerative Medicine Therapies**
Judith Arcidiacono
CBER, FDA
- 1:25 p.m.** **USP Standards and Best Practices for Gene Therapy**
Jim Richardson
USP



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- 1:50 p.m.** **Q&A and Extended Panel Discussion on Standards to Support Gene Therapy**
- 2:50 p.m.** **Workshop Wrap-up and Next Steps**
Mo Heidarani
Chair, USP Gene Therapy Workshop Steering Committee
- 3:00 p.m.** **Workshop Conclusion**