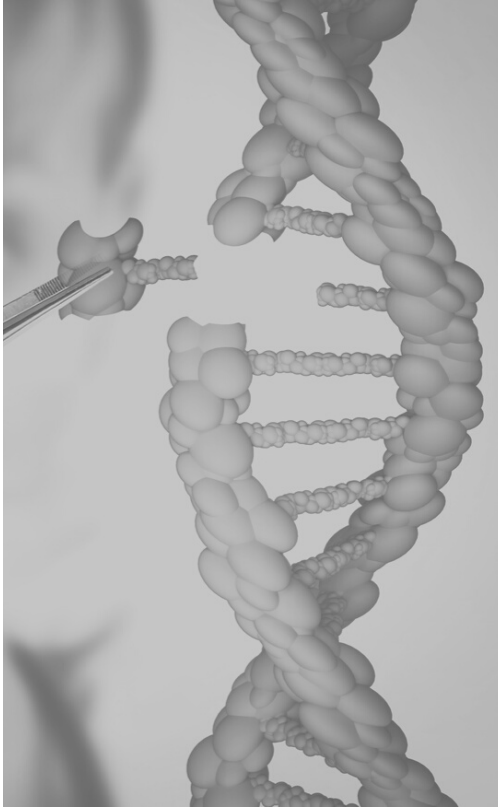

ASGCT 2021

Monday, May 10

3 - 7 p.m. ET

Standards in Gene Therapy and Genome Editing

Pre-Meeting Workshop

In collaboration with National Institute of Standards and Technology (NIST), Standards Coordinating Body (SCB), and United States Pharmacopeia (USP)

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The American Society of Gene & Cell Therapy is honored to acknowledge the following organizations for their support of this Pre-Meeting Workshop:

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Standards in Gene Therapy and Genome Editing

Co-Chairs: Fouad Atouf, Ph.D., Dawn Henke, Ph.D., and Samantha Maragh, Ph.D.

Session 1

State of Standards in the Field

3-3:30 p.m.

The Role of Standards and Recent Efforts on Genome Editing Standards

Samantha Maragh, Ph.D., NIST

3:30-4 p.m.

The Standards Landscape and Workflow of Physical and Documentary Standards

Dawn Henke, Ph.D., SCB

Session 2

Filling the Gaps: Collaborative Standards Advancement Projects

4-4:15 p.m.

Kelvin Lee, Ph.D., National Institute for Innovation in Manufacturing Biopharmaceuticals

4:15-4:30 p.m.

Wyatt Vreeland, Ph.D., NIST

4:30-4:45 p.m.

Fouad Atouf, Ph.D., USP

4:45-5:15 p.m.

Panel Discussion Moderated by Dawn Henke, Ph.D., SCB

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Session 3

Regulatory and Developer Perspectives

5:30-6:05 p.m.

FDA Perspective on the Use of Standards in Gene Therapy

Judy Arcidiacono, FDA CBER, Office of Tissues and Advanced Therapies

6:05-6:25 p.m.

Regulatory and Developer Perspectives Analytic, Raw Materials, Manufacturing Standards, Quantification for AAV, and Lentiviral Vectors

Mehrshid Alai-Safar, Ph.D., Kite Pharma

6:25-6:45 p.m.

Off Target Approaches for CRISPR

Tony Ho, M.D., CRISPR Therapeutics

Session 4

Summary of Most Pressing Standards Needs and Next Steps

Fouad Atouf, Ph.D., USP

Dawn Henke, Ph.D., SCB

Samantha Maragh, Ph.D., NIST

Speakers

Mehrshid Alai-Safar, Ph.D.
Kite Pharma

Mehrshid Alai is VP of Global Regulatory CMC for Kite, a Gilead Company, where she is responsible for all aspects of Regulatory CMC, including development and life cycle management. Mehrshid has her Ph.D. in Biochemistry, Cell, and Molecular Biology from The Johns Hopkins University School of Medicine. Prior to Kite, Mehrshid was Interim Head of Global Regulatory CMC for Baxalta/Shire. She had the responsibility to oversee CMC aspects of all products including small molecules, biologics, complex biologics and devices. Previous to that she held various roles in R&D, Quality and Regulatory in Baxter. She is a member of Gene Therapy Working party and USP Complex Biologics Expert Committee.

Judith Arcidiacono

FDA CBER Office of Tissues and Advanced Therapies

Judith is an international regulatory expert at the FDA, CBER Office of Tissues and Advanced Therapies or OTAT. Her primary role in OTAT is to facilitate the international harmonization of regulatory requirements for cell, tissue and gene therapies, and to lead standards development activities for products regulated by OTAT. For standards, she works closely with the National Institute of Standards and Technology and the Standards Coordinating Body to facilitate standards development for advanced therapies. She represents FDA in ISO Technical Committee 276, Biotechnology, ASTM F04 Committee on Tissue Engineered Products, and the Parenteral Drug Association Standards Committee. Her work on international harmonization of regulatory requirements includes serving as secretariat for the International Pharmaceutical Regulators Programme Cell Therapy Working Group and Gene Therapy Working Group. She is the co-chair of the Priority Work Area for Advanced Therapies in APEC Regulatory Harmonization Subcommittee and serves as faculty at the Northeastern University Center for Regulatory Excellence and Duke University Medical School- National University of Singapore program for regulatory excellence.

Fouad Atouf, Ph.D.
United States Pharmacopeia (USP)

Fouad Atouf is Vice President, Global Biologics, for USP. He leads all scientific activities related to the development and maintenance of documentary and reference standards for biologics and antibiotics and oversees the biologics laboratories in USP–U.S. and USP–India. His department supports the work of the associated USP Expert Committees. Dr. Atouf has been at USP for over 10 years and served in a variety of scientific leadership roles including being the regional champion for the Middle East and North Africa Region, where he helped facilitate programs designed to enhance the understanding of the role of regulations and standards in the registration of medicinal products.
Dr. Atouf has strong background and experience in the development and regulation of cellular and tissue-based products. Prior to joining USP in 2006, his research at the U.S. National Institutes of Health focused on developing methods for the in vitro generation of cell-based therapies for diabetes.
Dr. Atouf is the author of numerous publications in peer-reviewed journals and a frequent speaker at national and international scientific conferences.
Dr. Atouf earned his master's degree in Biochemistry and his Ph.D. in Cell Biology from the Pierre & Marie Curie University, Paris, France.

Dawn Henke, Ph.D.
Standards Coordinating Body (SCB)

Dawn holds a Ph.D. in Genetics and Genomic Sciences from University of Alabama at Birmingham. Prior to joining SCB, she worked as a post-doctoral fellow at the National Institutes of Health in the National Eye Institute performing stem cell research developing retinal organoids for testing and therapeutic purposes from stem cells.

Tony Ho, M.D.
CRISPR Therapeutics

Tony is EVP, Head of R&D at CRISPR Therapeutics since 2017. He is a highly accomplished R&D leader with experience throughout his nearly 20-year career across all phases of R&D, including discovery, early and late-stage clinical development and regulatory. He was previously SVP and Head of Oncology Integration and Innovation at AstraZeneca and oversaw the development and commercialization of Lynparza and Imfinizi (anti-PD-L1). 10 Phase 3 trials designed under his leadership have now read out positive. Prior to joining AstraZeneca, Tony was the Neurology and Ophthalmology Clinical Section Head at Merck Research Laboratories, Merck & Co., and led multiple development programs including the approval of Maxalt for pediatric migraine and Zioptan for glaucoma. Prior to joining Merck, Tony was the Co-Founder and Chief Scientific Officer of Neuronyx, a regenerative medicine company. He received his M.D. from the Johns Hopkins University School of Medicine and his B.S. in Electrical Engineering at the University of California, Los Angeles. He completed his residency and neurophysiology fellowship in the Department of Neurology at the Johns Hopkins Hospital. He was an Assistant Professor at Johns Hopkins Hospital and is an adjunct Associate Professor at the University of Pennsylvania and Johns Hopkins University.

Kelvin Lee, Ph.D.
National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL)

Kelvin Lee is Director of the National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL), one of 16 Manufacturing USA institutes. He is also Gore Professor of Chemical and Biomolecular Engineering at the University of Delaware.

Samantha Maragh, Ph.D.
National Institute of Standards and Technology (NIST)

Dr. Samantha Maragh Leads the Genome Editing Program at the National Institute of Standards and Technology (NIST). This program has a primary focus on measurements and assay qualification to support genome editing applications with emphasis on gene therapy applications. Included in this program is the NIST Genome Editing Consortium, with over 35 members across academia, not-for-profit industry and government, focused on addressing pre-competitive technical measurement and standards challenges within the genome editing community. Samantha also participates on representing the U.S. as a technical expert to the International Standards Organizations Technical Committee on Biotechnology (ISO TC 276). She is currently a U.S. liaison representing the interests and expertise of the U.S. on standards relating to nucleic acids measurements and is leading the development of an ISO standard for Genome Editing Vocabulary. Samantha received her B.S. in Biology (Cellular and Molecular Biology) and a minor in Chemistry from Loyola University in Baltimore, M.S. in Biotechnology from Johns Hopkins University, and her Ph.D. in Human Genetics and Molecular Biology from the Johns Hopkins School of Medicine.

Wyatt Vreeland, Ph.D.
National Institute of Standards and Technology (NIST)

Dr. Wyatt N. Vreeland performed his Ph.D. thesis research at Northwestern University in Chemical and Biological Engineering where he developed synthetic organic chemistries for production of large bio-mimetic molecules to be used in various genomic applications. After completing his Ph.D. research, Dr. Vreeland joined NIST as a National Research Council (NRC) postdoctoral fellow under the mentorship of Dr. Laurie Locascio. During this time, he and colleagues invented microfluidic systems for the formation of state-of-the-art lipid nanoparticles which now are immensely important vaccine materials. Dr. Vreeland is now a permanent member of NIST's scientific research staff. In these duties he manages a research lab that develops novel microfluidic systems to create cutting-edge nanomaterials of interest in the biopharmaceutical community. Recently he has become the NIST lead on a collaborative project with NIIMBL, NIST, and USP to conduct a large interlab study on metrology of viral vectors for gene therapy.

Disclosures

T. Ho

Crispr Therapeutics; Stocks and Options; Employee.