

NIST Workshop: Launch of the Rapid Microbial Testing Methods Consortium

Thursday, September 17, 2020, 1 PM to 5 PM

SPEAKER AND PANELIST LIST – Abstracts and Bios

SPEAKERS

SHENG LIN-GIBSON, Chief of the Biosystems and Biomaterials Division, NIST

Presentation Title: Overview of NIST Advanced Therapy Program

Abstract: Advanced therapies, including cell therapy, gene therapy, and tissue engineered products, have shown promising clinical efficacy and are changing the paradigm for treating diseases and injuries. Clinical translation and patient access to this broad class of therapeutics requires better defined and characterized products and more robust, reliable, and cost-effective manufacturing processes. Our Program supports the growing industry by addressing manufacturing, characterization, and testing challenges. Our efforts include 1) developing measurement assurance strategies and innovative measurement solutions, 2) convening and working stakeholders to identify industry-wide challenges and pre-competitive solutions, and 3) leading and contributing to the development of global documentary standards and reference materials.

Bio: Dr. Sheng Lin-Gibson is the Chief of the NIST Biosystems and Biomaterials Division. She oversees a multidisciplinary research portfolio that includes advanced therapies, precision medicine, synthetic biology, and complex microbial systems. She leads or contributes to the development of several international standards particularly relevant to emerging biotechnology.

SPENCER HOOVER, Independent Consultant

Presentation Title: Key Characteristics of Rapid Microbial Test Methods for Cell and Gene Therapies

Abstract: This talk will identify some of the reasons why RMTM are needed for Cell and Gene Therapies and why developing those methods are challenging. Ideas on where to look for relevant technologies and how to speed their uptake using a risk-based approach will also be discussed.

Bio: Building on his PhD in Microbiology, Spencer led the development of high multiplex in vitro diagnostics for pathogen detection for several years at Luminex. He has spent the past five years in the cell and gene therapy industry, most recently as the Director of Process and Analytical Development at CCRM in Toronto as part of the collaboration with Cytiva. In 2020,

he has been working as an independent consultant to help various cell and gene therapy companies with facility design, automation, and analytical method development. He co-chaired the RMTM workshop organized by the Standard Coordinating body in 2017 and is looking to neighboring industries for approaches and technologies to address the needs for RMTM in CGT.

JUDITH ARCIDIACONO, International Regulatory Expert, Standards Liaison, FDA

Presentation Title: FDA Requirements and Recommendations for Sterility Testing of Regenerative Medicine Therapies

Abstract: When developing rapid microbial testing methods, it is important to understand the local or regional regulatory requirements for testing Regenerative Medicine Advanced Therapy (RMT) products. This presentation will cover the U.S. regulations for sterility testing described in the US Code of Federal Regulations (CFR), 21 CFR 610.12, and expectations for RMT products outlined in FDA Guidance Documents. Working towards the development of rapid microbial testing methods will require a community effort where developers of testing methods, end users and regulatory agencies collaborate on the development of tests appropriate for RMT products.

Bio: Judith has been with the FDA for 30 years, the first 17 years as a researcher/reviewer and currently is leading international regulatory activities and policy on standards for Regenerative Medicine Therapies. She represents FDA in ISO Technical Committee 276, Biotechnology, the American Society for Testing Materials (ASTM) F04 Committee on Medical and Surgical Materials and Devices, Tissue Engineering Medical Products, and the Parenteral Drug Association Standards Development Organization. She serves as the secretariat for the International Pharmaceutical Regulators Programme (IPRP), Cell Therapy Working Group and Gene Therapy Working Group. Judith represents FDA as a Subject Matter Expert in regenerative medicine policy for the Asia Pacific Economic Cooperation Regulatory Harmonization Steering Committee Priority Work Area for Advanced Therapies. She is also a faculty member for the Northeastern University Center of Excellence for Advanced Therapies and Duke Medical School at the National University of Singapore Center of Regulatory Excellence.

DAWN HENKE, Senior Scientific Program Manager, Standards Coordinating Body

Presentation Title: Progress Toward Documentary Standards to Support Rapid Microbial Testing

Abstract: This talk will be focused on standards related to rapid microbial testing methods for regenerative medicine manufacturing. Current published standards, standards under development, and needed standards will be discussed. A focus of this talk will be engagement and education about standards.

Bio: Dawn Henke is the senior scientific program manager at the standards coordinating body. She has a Ph.D. in genetics and genomic sciences from University of Alabama at Birmingham and was a postdoctoral fellow at the National Institutes of Health.

KAI NESEMANN, Product Manager, Microbiology, Sartorius Lab Instruments GmbH & Co. KG

Presentation Title: Rapid detection of bacteria and fungi in ATMPs prior treatment – Validation of a real-time PCR-based test

Abstract:

- First rapid sterility test for short-shelf life ATMPs compliant to international guidance including the new USP<1071> as well as EP 5.1.6., EP 2.6.27 and USP<1223>.
- Obtain the QC result PRIOR to treatment and safeguard the patient's health by avoiding exposure to additional risks.
- Full validation results of a qPCR method for sensitivity, specificity, robustness and equivalency of total bacteria and fungi detection.
- KEYWORDS: rapid final release of ATMPs; 16S/18S ribosomal DNA detection; Paul-Ehrlich-Institute; quantitative Polymerase Chain Reaction (qPCR); TaqMan® probe.

Bio: Kai Nesemann joined Sartorius Lab Instruments GmbH in July 2016. He is global product manager microbiology and responsible for the DNA-based rapid detection portfolio as well as for continuous air monitoring in microbiological quality control. Special focus is given on the biopharmaceutical production for in-process and final release testing of Mycoplasma as well as total bacterial and fungal contamination. Validation of robust rapid tests with results prior treatment is of particular importance for patient safety in regenerative medicine. Nesemann graduated at the Georg-August-University in Goettingen, Germany. He has many years of experience in academic research and he worked as a Ph.D. student in the department for molecular microbiology and genetics of Prof. Dr. Gerhard.

PANELISTS

RICHARD HAMMOND, Technology Director, Cambridge Consultants Ltd.

Bio: Richard is Head of Bioinnovation at Cambridge Consultants, leading a multi-disciplinary team in the design and development of novel equipment and processes for ATMP research and manufacture. Prior to joining Cambridge Consultants, Richard worked for Alere (now part of Abbott) leading teams developing in-vitro diagnostics for infectious diseases using molecular techniques such as isothermal DNA amplification. Richard trained as an engineer and holds BA and MEng degrees from the University of Cambridge. See <https://www.linkedin.com/in/richard-hammond-15774a8/>

TOM LEACH, Associate Director, Drug Product Process Engineering and Packaging, AstraZeneca

Bio: I am a biochemist and chemical engineer by training, and have worked in biopharmaceutical drug product development for 17 years. Most of my career has involved formulation and process development for monoclonal antibody drug products. Currently, I am working in cell and gene therapy, and serve on a global AstraZeneca microbiology forum which aims to modernize and advance microbiological controls in manufacturing and in drug products.

RICHARD MCFARLAND, Chief Regulatory Officer, ARMI | BioFabUSA

Bio: Richard McFarland is an immunopathologist and the Chief Regulatory Officer at the Advanced Regenerative Manufacturing Institute (ARMI) where he oversees regulatory affairs for ARMI and its BioFabUSA program. Currently he is also serving as President of the Standards Coordinating Body. Prior to joining ARMI in 2017, Dr. McFarland was Associate Director for Policy of the Office of Tissues and Advanced Therapies (and its predecessor office) at the Food and Drug Administration's Center for Biologics Evaluation and Research (FDA/CBER) for eleven years after six years as a reviewer in FDA/CBER. In addition, he, served on the federal government's interagency committee for tissue engineering and regenerative medicine, the Multi-agency Tissue Engineering Sciences group (MATES) for fifteen years, including five years as its Chair.

STACY SPRINGS, Senior Director of Programs; Executive Director Biomanufacturing Initiatives, Massachusetts Institute of Technology's Center for Biomedical Innovation

Bio: Dr. Stacy Springs serves as the Senior Director of Programs at MIT's Center for Biomedical Innovation and as the Executive Director of Biomanufacturing Initiatives including MIT's Biomanufacturing Program, (BioMAN), it's Consortia on Adventitious Agent Contamination in Biomanufacturing, (CAACB), and the BioACCESS initiative. The objective of BioMAN is to develop knowledge, science, technologies and strategies that advance the global manufacture and delivery of high quality biopharmaceuticals. The CAACB pools biopharmaceutical manufacturing expertise in the area of adventitious agent contamination to better enable a

safe and dependable delivery of life-saving biologics. BioACCESS seeks to better understand the growing need for and barriers to safe, effective, and affordable health services in low- and middle-income countries, especially biologic therapies for chronic and non-communicable diseases. In addition, Dr. Springs serves as the co-captain for the Flagship Project 2 team of the Singapore-MIT Critical Analytics for Manufacturing Personalized-Medicines (SMART CAMP) project where she addresses rapid critical quality attributes (CQA) for safety of cell sources and cell therapy products, informing process analytic technologies and speeding product release.

She holds a PhD in Chemistry from the University of Texas at Austin and gained postdoctoral training in protein and biophysical chemistry at Princeton University.

RADHAKRISHNA TIRUMALAI, Principal Scientific Liaison, US Pharmacopeial Convention (USP)

Bio: Dr. Tirumalai has been at the USP since 2003 and is currently a Principal Scientific Liaison-General Chapters in the Science Division. He is the Liaison to the USP Expert Committee on Microbiology. He works with the industry, regulatory agencies and other external science based organizations in the development and revision of General Chapters. Dr. Tirumalai represents USP on PDA expert task forces and committees related to Microbiology and Sterility Assurance 2005-till date, the organizing committee of PDA Global Microbiology Conference 2006-2018, on AAMI expert working groups related to Microbiology, Sterilization, Sterility Assurance and Biocompatibility 2004-till date, and on the editorial board of FDA's Pharmaceutical Microbiology Manual.

Dr. Tirumalai's prior industry experience encompasses process and product research and development, transfer, and product manufacturing. He has a Ph.D. degree in Biochemistry. He has authored numerous publications, review articles and several book chapters. He has organized numerous workshops and conferences on Pharmaceutical Microbiology topics and is a frequent speaker at conferences and has taught Pharmacopeial Microbiology courses at numerous locations globally.

CLAUDIA ZYLBERBERG, CEO, Akron Biotech

Bio: Claudia Zylberberg, Ph.D. is a leader in regenerative medicine. She is the founder and CEO of Akron Biotechnology, a manufacturer of cGMP-grade ancillary materials for the tissue, cell and gene therapy industry. She also co-founded AssureImmune, an adult stem cell bank. Dr. Zylberberg holds numerous patents and has developed several patent-pending platform technologies in cryopreservation, novel formulations and others. She has authored and co-authored several peer-reviewed publications and has received grants from the NIH and Department of Defense, among others. In her early years, Dr. Zylberberg worked at Nabi Biopharmaceuticals, specializing in human plasma-derived products. Her experience in product development and protein manufacturing has been instrumental for the development of key materials to accelerate the regen med industry. In addition, she co-founded the Standards Coordinating Body (SCB) and is a board member of ISCT, ARM, AABB's NBF, and the NAS

(Regenerative Medicine Forum). Other advisory positions include ISO US TAG, BioFlorida, ISSCR, CBA, and Biomedical Engineering, University of Miami.

Dr. Zylberberg is also the author of a children's book, *You're Full Genes* and recently launched an updated version. Originally introduced in 2001, *You're Full of Genes* renews Dr. Zylberberg's call for an educated public. Sales proceeds will support three foundations working on scientific advancements and education in the field: the CCRM Foundation, the ARM Foundation for Cell & Gene Medicine and Duke University's Center for Autism and Brain Development.