Emerging Technologies and Innovation in Manufacturing Regenerative Medicine Therapies: A Workshop

Tuesday, October 17, 2023

STATEMENT OF TASK

A planning committee of the National Academies of Sciences, Engineering, and Medicine will organize and hold a one-day public workshop to examine challenges and opportunities in manufacturing regenerative medicine therapeutics as the field evolves to accommodate higher volume production and increased capacity for delivering regenerative medicine treatments to patients. The workshop will explore how new scientific advancements and technological developments in manufacturing cell- and gene-based therapies are shaping the maturing industry of regenerative medicine.

Workshop presentations and discussions may address some of the following topics:

- New developments in closed systems for bioprocessing and biomanufacturing
- Decentralized manufacturing as a strategy to address production and supply chain challenges for regenerative medicine treatments
- Automation, data analysis, computational modeling, and artificial intelligence/machine learning algorithms for advanced biomanufacturing
- Open-source manufacturing to facilitate knowledge sharing and support technology transfer among organizations
- Quality control techniques to improve manufacturing reliability and regulatory strategies to sustainably increase biomanufacturing capacity
- Building multidisciplinary teams equipped to implement new manufacturing approaches and capitalize on the potential of recent developments

The planning committee will organize the workshop, develop the agenda, select and invite speakers, and moderate discussions. Proceedings of the workshop will be prepared by a designated rapporteur in accordance with institutional guidelines.

SESSION I: Opening Remarks & Keynote

8:30 – 8:35 AM ET — **Welcoming Remarks**

Katherine Tsokas, *Forum Co-Chair*
Vice President
Regulatory, Quality, Risk Management and Drug Safety
Janssen Inc., Canada

Krishnendu Roy, *Forum Co-Chair*
Bruce and Bridgitt Evans Dean of Engineering and University Distinguished Professor
Vanderbilt University

8:35 – 8:40 AM — **Graphic Illustrator Introduction**

Wade Forbes
Co-founder and Illustrator
RedTale Communications

8:40 – 8:50 AM **Introduction and Charge to the Workshop Speakers and Participants**
Scott Steele, *Workshop Planning Committee Co-Chair*
Senior Advisor, Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

Claudia Zylberberg, *Workshop Planning Committee Co-Chair*
Founder and former CEO
Akron Bio

8:50 – 9:10 AM **Keynote**
Peter Marks
Director of the Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration

9:10 – 9:20 AM **Reactants**
Karin Hoelzer
Director of Policy and Regulatory Affairs
National Organization for Rare Disorders

Sarah Nikiforow
Medical Director, Cell Manipulation Core Facility
Dana-Farber Cancer Institute

9:20 – 9:45 AM **Panel Discussion**
Moderator: Rachel Salzman, American Society for Gene and Cell Therapy

SESSION II: Decentralized Manufacturing as a Strategy to Address Production and Supply Chain Challenges

Moderator: Phil Vanek, Gamma Biosciences

Session Objectives:

- Examine available models of decentralized manufacturing (e.g., distributed manufacturing, point-of-care); explore advantages and disadvantages of different models to scale up and scale out
- Discuss benefits, tradeoffs, and potential strategies to implement different models of decentralized manufacturing
- Discuss scientific/technical, regulatory, and supply chain challenges to implementing these models

9:45 – 10:00 AM **Fabian Gerlinghaus**
Co-founder and CEO
Cellares

10:00 – 10:15 AM **Rahul Singhvi**
Co-founder and CEO
Resilience

10:15 – 10:30 AM **Sarah Nikiforow**
Medical Director, Cell Manipulation Core Facility
Dana-Farber Cancer Institute

10:30 – 10:55 AM **Panel Discussion**

10:55 – 11:15 AM **Break**

SESSION III: Automation and Algorithms in Regenerative Medicine Manufacturing

Moderator: Sohel Talib, California Institute for Regenerative Medicine

Session Objectives:

- Explore recent advancements in and applications for automation technologies in biomanufacturing
- Discuss the role of automation and AI/ML in the future of manufacturing regenerative medicine therapies

11:15 – 11:30 AM ET **Nabiha Saklayen**
Co-founder and CEO
Cellino

11:30 – 11:45 AM **Jan Jensen**
Founder, CEO and CSO
Trailhead Biosystems

11:45 AM – 12:00 PM **Jane Lebkowski**
President
Regenerative Patch Technologies

12:00 – 12:25 PM **Panel Discussion**

12:25 – 1:30 PM **Break**

SESSION IV: Quality Control and Regulatory Considerations

Moderator: Anne Plant, National Institute of Standards and Technology

Session Objectives:

- Discuss quality control and analytical techniques to improve manufacturing reliability
- Discuss what is needed to ensure characterization sufficient to supply confidence for regulatory decision-making (e.g., comparability, harmonization)
- Explore regulatory challenges and potential solutions to support a modern regulatory framework
- Consider possible strategies to address comparability challenges under decentralized models of manufacturing

1:30 – 1:45 PM **Sadik Kassim**
Chief Technology Officer (Genomic Medicines)
Danaher Corporation

1:45 – 2:00 PM

Matthew Hewitt
Vice President and Technical Officer of Cell and Gene Therapies and Biologics
Charles River Laboratories

2:00 – 2:15 PM

Heather Lombardi
Director, Office of Cellular Therapy & Human Tissue
Office of Therapeutic Products (OTP)
Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration

2:15 – 2:40 PM **Panel Discussion**

2:40 – 3:00 PM **Break**

SESSION V: Implementing New Manufacturing Strategies through Partnerships and Innovative Technology Transfer

Moderator: Krishanu Saha, University of Wisconsin-Madison

Session Objectives:

- Discuss how multidisciplinary teams and partnerships can leverage new developments and implement new manufacturing approaches
- Discuss what is needed to build and support effective partnerships
- Discuss benefits of open-source manufacturing and associated challenges
- Explore strategies to promote efficient technology transfer

3:00 – 4:00 PM

Jens Vogel
Senior Vice President & Global Head of Biotech
Bayer Pharmaceuticals

Fyodor Urnov
Professor of Molecular and Cell Biology
Director of Technology & Translation, Innovative Genomics Institute
University of California, Berkeley

Courtney Silverthorn
Associate Vice President, Science Partnerships
Foundation for the National Institutes of Health

Ravi Basavappa
Senior Advisor
Advanced Research Projects Agency for Health (ARPA-H)

Boro Dropulic
Executive Director
Caring Cross

4:00 – 4:40 PM **Panel Discussion**

4:40 – 4:45 PM **Graphic Illustrator Summary**
Wade Forbes
Co-founder and Illustrator
RedTale Communications

4:45 – 5:00 PM **Closing Remarks**
Scott Steele, *Workshop Planning Committee Co-Chair*
Senior Advisor, Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

Claudia Zylberberg, *Workshop Planning Committee Co-Chair*
Founder and former CEO
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