

The National Academies of
SCIENCES • ENGINEERING • MEDICINE

The Forum on Regenerative Medicine

**Navigating the Manufacturing Process and Assuring the Quality
of Regenerative Medicine Therapies – A Workshop**

June 26, 2017

National Academy of Sciences Building

Lecture Room

2101 Constitution Ave. NW

Washington, DC 20418

Statement of Task:

An ad hoc planning committee will plan and conduct a one day public workshop to examine and discuss challenges, opportunities, and best practices associated with defining and measuring the quality of cell and tissue products and raw materials in the research and manufacturing of regenerative medicine therapies. The goal of the workshop will be to learn from existing examples of manufacturing of early generation regenerative medicine products and to address how progress could be made in identifying and measuring critical quality attributes as well as designing and adhering to standards to help in the navigation of the scale-up process from a research laboratory to the manufacturing environment. Gathering this information will help inform and facilitate future Forum discussions around the issues of implementing regenerative medicine therapies and technologies, such as designing and implementing effective manufacturing processes, examining regulatory pathways, and considering bioethical matters. Discussions during this workshop will be held with a broad array of stakeholders which may include research scientists, clinicians, patients, regulators, and representatives from pharmaceutical and biotech companies. The planning committee will develop the workshop agenda, select and invite speakers, and moderate the discussions. Proceedings from the workshop will be prepared by a designated rapporteur in accordance with institutional policies and procedures.

Objectives:

- To explore how unique cell populations are defined and evaluated for quality and purity.
- To highlight the importance of establishing a strong scientific understanding of source cells, as well as the characteristics of regenerative cellular and tissue products.
- To learn about the logistical challenges and successes associated with identifying and measuring critical quality attributes and developing effective regulatory and manufacturing standards.
- To examine possible mechanisms and technologies to improve the ability of researchers, manufacturers, and regulators to ensure that new therapies are safe, effective, and can be produced efficiently.

8:30 AM

Opening Remarks

R. ALTA CHARO, *Forum Co-Chair*
Warren P. Knowles Professor of Law
University of Wisconsin-Madison

JAY P. SIEGEL, *Forum Co-Chair*
Chief Biotechnology Officer
Head, Scientific Strategy and Policy
Johnson & Johnson

8:35 AM

Charge to Workshop Speakers and Participants

CLAUDIA ZYLBERBERG, *Workshop Co-Chair*
Founder and CEO
Akron Biotech

STEVEN OH, *Workshop Co-Chair*
Acting Deputy Director, Division of Cellular and Gene Therapies
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research
United States Food and Drug Administration

8:45 AM

Opening Keynote

ADRIAN GEE
Professor of Cell and Gene Therapy
Baylor College of Medicine

**SESSION I: TRANSITIONING FROM DISCOVERY AND DEVELOPMENT TO
MANUFACTURING FOR REGENERATIVE THERAPIES**

Session Objectives:

- To discuss challenges and opportunities associated with bringing new discoveries from the lab to manufacturing and navigating the process of scaling up the manufacturing of new therapies.
- To learn about methods and capabilities for manufacturing and quality control data collection for the purpose of informing the transition from R&D to the implementation of good manufacturing practices (GMP).
- To illuminate potential opportunities and models to reach scale and commercialization with current infrastructure and to assess probable future needs.

Moderator:

Krishanu Saha, Assistant Professor, University of Wisconsin – Madison

9:10-9:55 AM

Speakers:

BRUCE LEVINE
Barbara and Edward Netter Professor in Cancer Gene Therapy
University of Pennsylvania Perelman School of Medicine

LAURA NIKLASON
Professor of Anesthesiology & Biomedical Engineering
Yale University

BOB PRETI
President and CEO
PCT Cell Therapy Services, LLC

9:55-10:30 AM Discussion with Workshop Participants

10:30-10:45 AM BREAK

SESSION II: IDENTIFYING AND MEASURING CRITICAL QUALITY ATTRIBUTES OF REGENERATIVE MEDICINE PRODUCTS AND SOURCE CELLS

Session Objectives:

- To examine methods and processes used to identify and measure critical quality attributes for raw materials and regenerative medicine products.
- To discuss measurement methodology and how to ensure that measurements are accurate and reproducible.
- To survey technologies and future needs in the measurement of critical quality attributes.

Moderator: **Martha Somerman**, Director, National Institute of Dental and Craniofacial Research, National Institutes of Health

10:45-11:30 AM Speakers:
ANNE PLANT
Chief of the Biosystems and Biomaterials Division
National Institute of Standards and Technology

LINDA KELLEY
Director, Cell Therapies Processing Facility
Moffitt Cancer Center

ROBERT DEANS
Chief Technology Officer
BlueRock Therapeutics

11:30-12:00 PM Discussion with Workshop Participants

12:00-1:00 PM LUNCH

SESSION III: DESIGNING TECHNOLOGIES TO MEET THE MANUFACTURING NEEDS OF NEW REGENERATIVE THERAPIES

Session Objectives:

- To explore existing technologies that facilitate the efficient and cost effective development of products that meet manufacturing and regulatory standards.

- To illuminate opportunities for new technologies and manufacturing models to increase efficiency and quality.
- To discuss novel and more precise in-process and final release testing technologies.
- To review existing infrastructure such as GMP facilities in academic centers and the commercial sector.
- To understand the open and closed systems models applicable to the manufacturing setting and IT support.

Moderator: Thomas Petersen, Vice President, Regenerative Medicine, United Therapeutics Corporation

1:00-1:45 PM

Speakers:

ISABELLE RIVIÈRE
Director, Cell Therapy and Cell Engineering Facility
Memorial Sloan Kettering Cancer Center

RODNEY RIETZE
Lead, cGMP Process Automation for Cell and Gene Therapies
Novartis

PHIL VANEK
General Manager, Cell Therapy Technologies
GE Healthcare

1:45-2:15 PM

Discussion with Workshop Participants

**SESSION IV: REGULATORY CHALLENGES AND OPPORTUNITIES FOR
REGENERATIVE MEDICINE THERAPIES**

Session Objectives:

- To consider the regulatory landscape for regenerative medicine including:
 - developing standards
 - enforcing regulation
 - meeting the needs of patients

Moderator: Jiwen Zhang, Senior Director, Regulatory Affairs, Cell Therapy & Regenerative Medicine, GE Healthcare

2:15-3:00 PM

Speakers:

ROBERT MCBURNEY
President and CEO
Accelerated Cures Project for Multiple Sclerosis
Co-Principal Investigator
iConquerMS Patient-Powered Research Network

STEVEN BAUER
Chief, Cellular and Tissue Therapy Branch
Center for Biologics Evaluation and Research
United States Food and Drug Administration

KATHERINE TSOKAS
Senior Director, Global Regulatory Affairs
Johnson & Johnson

3:00-3:35 PM **Discussion with Workshop Participants**

3:35-3:50 PM **BREAK**

SESSION V: CLOSING KEYNOTE AND PANEL

Session Objectives:

- To summarize the lessons learned and topics discussed throughout the workshop day.
- To discuss ways forward to support the development, manufacture, and regulation of safe and effective regenerative medicine therapies.

Moderator: **Krishnendu Roy**, Robert A. Milton Chair and Professor & Technical Lead,
National Cell Manufacturing Consortium, Georgia Institute of Technology

3:50-4:10 PM **Closing Keynote:**
DEAN KAMEN
Advanced Regenerative Manufacturing Institute
DEKA Research & Development Corporation

4:10-4:30 PM **Panelist Reflections**
BOB PRETI
President and CEO
PCT Cell Therapy Services, LLC

ANNE PLANT
Chief of the Biosystems and Biomaterials Division
National Institute of Standards and Technology

PHIL VANEK
General Manager, Cell Therapy Technologies
GE Healthcare

ROBERT MCBURNEY
President and CEO
Accelerated Cures Project for Multiple Sclerosis
Co-Principal Investigator
iConquerMS Patient-Powered Research Network

4:30-5:00 PM **Discussion with Workshop Participants**

5:00 PM **Final Remarks from Workshop Co-Chairs**

CLAUDIA ZYLBERBERG, *Workshop Co-Chair*
Founder and CEO
Akron Biotech

STEVEN OH, *Workshop Co-Chair*
Acting Deputy Director, Division of Cellular and Gene Therapies
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research
United States Food and Drug Administration

5:15 PM

Adjourn