

The National Academies of
SCIENCES • ENGINEERING • MEDICINE

Board on Health Sciences Policy
Forum on Regenerative Medicine

***The State of the Science in the Field of Regenerative Medicine: Challenges
of and Opportunities for Cellular Therapies - A Workshop***

October 13, 2016

**National Academy of Sciences Building
Room 125
2101 Constitution Avenue NW
Washington, DC 20418**

MEETING OBJECTIVES

- TO EXAMINE THE STATE OF THE SCIENCE FOR THERAPIES THAT GENERATE, REPAIR, OR REPLACE TISSUES BY CONVENING SCIENTISTS, CLINICIANS, INDUSTRY, PATIENT EXPERTS, AND OTHER STAKEHOLDERS.
- TO HIGHLIGHT THE CHALLENGES, SUCCESSES, AND LESSONS LEARNED WITH RESPECT TO TRANSLATION OF REGENERATIVE THERAPIES FROM EARLY DISCOVERY INTO CLINICAL PRACTICE WITH THE GOAL OF REACHING PATIENTS.
- TO ILLUMINATE NEXT STEPS FOR THE FIELD AND WAYS THAT THE FORUM COULD BE A FACILITATOR OF PROGRESS.

AGENDA

8:30 A.M.

Welcoming Remarks

R. Alta Charo, *Forum Co-Chair*
Warren P. Knowles Professor of Law and Bioethics
School of Law and School of Medicine and Public Health
University of Wisconsin-Madison

Jay Siegel, *Forum Co-Chair*
Chief Biotechnology Officer
Head, Scientific Strategy and Policy
Johnson and Johnson

INTRODUCTION: OVERVIEW OF THE FIELD OF REGENERATIVE MEDICINE AND FOCUS OF THE WORKSHOP

Objectives: To describe and explore the broad field of regenerative therapies at a high level and to highlight successful research and clinical applications as well as barriers to scientific and therapeutic advances as the field moves forward.

8:35 A.M. **Charge to Workshop Speakers and Participants: Considering the State of the Science in Regenerative Therapies**

Cynthia Dunbar, *Workshop Co-Chair*
President
American Society of Gene and Cell Therapy
Senior Investigator, Molecular Hematopoiesis Section
National Heart, Lung, and Blood Institute

8:45 A.M. **Areas of Challenge and Success in Regenerative Therapies**

Lorenz Studer
Director, Center for Stem Cell Biology
Memorial Sloan Kettering Cancer Center

9:05 A.M. Clarifying Questions

SESSION I: SKIN AND MUSCULOSKELETAL TISSUES

Objectives: To examine the state of the science in research and novel applications of new technology to repair, regenerate, or renew skin or musculoskeletal tissues; to consider the obstacles that hinder progress in research; and to highlight scientific successes, lessons learned, and opportunities to move the field forward with the goal of bringing new therapies to patients.

Moderator: **Audrey Kusiak**, Scientific Program Manager, Rehabilitation Research and Development Service, Office of Research and Development, Department of Veterans Affairs

9:15 A.M. Anthony Oro
Professor of Dermatology
Member of Program in Epithelial Biology, Institute for Stem Cell
Biology and Regenerative Medicine
Stanford University

Anthony Ratcliffe
President and CEO
Synthasome, Inc.

Patricia Furlong
Chief Executive Officer
Parent Project Muscular Dystrophy

SESSION III: NEUROLOGICAL AND OPHTHALMOLOGICAL TISSUES

Objectives: To examine the state of the science in research and novel applications of new technology to repair, regenerate, or renew neurological and ophthalmological tissues; to consider the obstacles that hinder progress in research; and to highlight scientific successes, lessons learned, and opportunities to move the field forward with the goal of bringing new therapies to patients.

Moderator: **Brian Fiske**, Senior Vice President, Research Programs, The Michael J. Fox Foundation for Parkinson's Research

1:00 P.M. Sally Temple
Principal Investigator and Scientific Director
Neural Stem Cell Institute

Ann Tsukamoto
Former Executive Vice President, Scientific and Strategic Alliances
StemCells, Inc.

Peter Coffey
Professor, Neuroscience Research Institute
University of California, Santa Barbara

1:45 P.M. **Discussion with Speakers and Attendees**

SESSION IV: CARDIOVASCULAR AND LUNG TISSUES

Objectives: To examine the state of the science in research and novel applications of new technology to repair, regenerate, or renew cardiovascular and lung tissues; to consider the obstacles that hinder progress in research; and to highlight scientific successes, lessons learned, and opportunities to move the field forward with the goal of bringing new therapies to patients.

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

2:15 P.M. Deepak Srivastava
The Younger Family Director, Gladstone Institute of Cardiovascular Disease; Director, Rodenberry Center for Stem Cell Biology and Medicine; Professor University of California, San Francisco

Eduardo Marbán
Director, Heart Institute

Professor of Medicine
Cedars-Sinai

Brigitte Gomperts
Associate Professor, Pulmonary and Pediatric Medicine
University of California, Los Angeles

3:00 P.M. **Discussion with Speakers and Attendees**

3:30 PM **Break**

SESSION V: RENAL TISSUES

Objectives: To examine the state of the science in research and novel applications of new technology in repairing or regenerating tissue in the kidney, to consider the obstacles that hinder progress in research; and to highlight scientific successes, lessons learned, and opportunities to move the field forward with the goal of bringing new therapies to patients.

Moderator: **Deborah Hoshizaki**, Program Director, Division of Kidney, Urologic, and Hematologic Diseases, National Institute of Diabetes and Digestive and Kidney Diseases

3:45 PM Ben Humphreys
Chief, Renal Diseases Division, Department of Medicine
Washington University School of Medicine

David Baron
Chief Scientific Officer
PKD Foundation

4:15 P.M. **Discussion with Speakers and Attendees**

SESSION VI: FINAL DISCUSSION AND NEXT STEPS

Objectives: To reflect on the current state of the science of regenerative therapies, explore the existing and potential scientific barriers to advancing the field of regenerative medicine, and to discuss strategies and lessons learned for facilitating efficient, effective, and translatable research.

Moderator: **Alta Charo**, Warren P. Knowles Professor of Law and Bioethics School of Law and School of Medicine and Public Health University of Wisconsin-Madison

4:35 P.M.

Looking Toward the Future: The Promise and Challenges of Regenerative Therapies

George Daley
Director, Stem cell Transplantation Program
Boston Children's Hospital and Dana-Farber Cancer Institute
Dean, Harvard Medical School

4:55 P.M.

Reflections on the Day/Next Steps

Patricia Furlong
Chief Executive Officer
Parent Project Muscular Dystrophy

Deepak Srivastava
The Younger Family Director, Gladstone Institute of Cardiovascular Disease; Director, Rodenberry Center for Stem Cell Biology and Medicine; Professor University of California, San Francisco

Sally Temple
Principal Investigator and Scientific Director
Neural Stem Cell Institute

Ann Tsukamoto
Former Executive Vice President, Scientific and Strategic Alliances
StemCells, Inc.

5:15 P.M.

Discussion with Speakers and Attendees

5:35 P.M.

Concluding Remarks

R. Alta Charo, *Forum Co-Chair*
Warren P. Knowles Professor of Law and Bioethics
School of Law and School of Medicine and Public Health
University of Wisconsin-Madison

Cynthia Dunbar, *Workshop Co-Chair*
President
American Society of Gene and Cell Therapy
Senior Investigator, Molecular Hematopoiesis Section
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5:45 P.M.

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