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 II: HEMATOLOGY AND IMMUNITY

Objectives: To examine the state of the science in research and novel applications of new technology to repair, regenerate, or renew tissue and function in the blood and the immune system; 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: Cynthia Dunbar, President, American Society of Gene and Cell Therapy; Senior Investigator, Molecular Hematopoiesis Section, National Heart, Lung, and Blood Institute

10:45 A.M. Fyodor Urnov
Associate Director
Altius Institute for Biomedical Sciences

Harry Malech
Chief, Laboratory of Host Defenses
Chief, Genetic Immunotherapy Section
National Institute of Allergy and Infectious Diseases

Michel Sadelain
Director, Center for Cell Engineering and Gene Transfer and Gene Expression Laboratory
Memorial Sloan Kettering Cancer Center

11:30 A.M. Patient Perspective:
Jennifer Fields
Patient Advocate

11:35 A.M. Discussion with Speakers and Attendees

12:00 P.M. Working Lunch
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
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  
National Heart, Lung, and Blood Institute

5:45 P.M.  
ADJOURN