

*The National
Academies of*

SCIENCES
ENGINEERING
MEDICINE

Forum on
**REGENERATIVE
MEDICINE**

2018 and 2019
Program Activities



ABOUT THE FORUM

The National Academies of Sciences, Engineering, and Medicine offers a variety of activities to address the science policy needs of federal agencies and others who are in need of credible, independent advice, or are seeking a neutral setting for consideration of challenging issues. Forums are convening activities that provide both a mechanism and a venue for interested parties from academia, industry, government, and other stakeholder groups to meet and discuss issues of mutual interest and concern in a neutral setting. The primary purpose of Forums is to foster dialogue across sectors and institutions, and to illuminate issues, but not necessarily to resolve them. The goal of these activities is to develop a mutual understanding of the relevant issues, and to provide a mechanism that fosters collaboration among stakeholders in addressing these issues. Forums are self-governing, i.e., the membership identifies the topics that it wishes to address, and with assistance from staff, develops meeting agendas and identifies workshop topics. As a result, the topics are likely to span a broad range of issues in research, policy and practice.

The National Academies of Sciences, Engineering, and Medicine's Forum on Regenerative Medicine ("the Forum") was established in 2016 and provides a convening mechanism for interested parties from academia, industry, government, patient and provider organizations, regulatory bodies, foundations, societies, associations, and others. Members meet and discuss sensitive and difficult issues in a neutral setting in order to engage in dialogue and discussions that address the challenges facing the application of, and the opportunities for, regenerative medicine to improve health through the development of effective new therapies. The Forum identifies existing and potential barriers to scientific and therapeutic advances; discusses opportunities to assist in facilitating more effective partnerships among key stakeholders; examines the impact that current policies have on the discovery, development, and translation of regenerative medicine therapies; considers the unique challenges of identifying, validating, and bringing regenerative medicine applications to market; and explores the ethical, legal, and social issues posed by regenerative medicine advances.

Over the past three years, the Forum has identified important questions and challenges in the field of regenerative medicine that have led the group to explore a broad array of issues ranging from the unique challenges of manufacturing regenerative medicine products, to the complex needs of people who are exploring regenerative medicine as a treatment, to the difficulties with regulating a rapidly evolving field. By bringing together experts and leveraging a wide range of perspectives and knowledge, Forum members have collaborated to identify strategic opportunities to advance the field of regenerative medicine through hosting public workshops, publishing perspective papers and workshop proceedings, and developing resources for the broader regenerative medicine community to support the advancement of high quality science, efficient and effective therapies, and the safety and health of patients.

Working Groups and Areas of Interest

During 2018-2019, Forum members organized into two working groups and continued to explore issues in a number of other areas.

WORKING GROUPS

Regenerative Engineering and Gene Therapy

The Forum's working group on regenerative engineering and gene therapy is exploring barriers impeding the successful translation of regenerative and gene-based therapies and forward thinking approaches aimed at improving clinical outcomes. The working group hosted a workshop in October 2018 exploring sources of variability related to regenerative engineering products and in November 2019, the group hosted a workshop focused on designing gene-based clinical trials. The working group is currently interested in exploring solutions to challenges related to the immune response to regenerative therapies. The group intends to disseminate information about the value of deep integration of knowledge between materials science, stem cell science, engineering, developmental biology, immunology, computational, and clinical communities.

Systems Thinking

The Forum's working group on systems thinking is interested in advancing the field of regenerative medicine by exploring new technologies and theoretical models that may improve product characterization, manufacturing processes, and patient stratification, resulting in greater clinical success of new therapies. The group is interested in examining opportunities for developing systems-based analytical methods that can advance the mechanistic understanding of regenerative medicine products and help to overcome challenges with manufacturing and patient outcomes.

ADDITIONAL AREAS OF INTEREST

Workforce Issues

There is interest among Forum members in understanding what skillsets industry currently needs and what will be needed in the future to create and manufacture regenerative medicines.

Regulatory and Legislative Affairs

Forum members are interested in exploring regulatory challenges including the development of standards and potential roles for various regulatory bodies involved in enforcing regulations related to unproven therapies.

Communication

The Forum members are exploring how patients can become more involved in the research and development process of new therapies and how experts in the field can better communicate about regenerative medicine with the public.

Cost and Access to Therapies

Forum members are exploring barriers for patients in accessing regenerative therapies and trying to understand the various factors that may contribute to cost and access issues.

2018 and 2019 Meetings

FEBRUARY 26-27, 2018

Forum members convened leaders in the field of sports medicine to explore how stem and cell therapies are being used in this area and how information about therapeutic options is being communicated to professional athletes. The group also highlighted case studies in the field of regenerative engineering as well as cell and gene therapies to learn about challenges and successes in moving products through the translational pathway.

OCTOBER 18, 2018 *workshop*

Exploring Sources of Variability Related to the Clinical Translation of Regenerative Engineering Products

FEBRUARY 26-27, 2019

The meeting featured information-gathering sessions on several topics including the role of the immune system in the response to regenerative therapies, workforce development challenges, and advertising and marketing of stem cell therapies.

NOVEMBER 13, 2019 *workshop*

Exploring Novel Clinical Trial Designs for Gene-Based Therapies

JULY 23-24, 2018

Forum members met with stakeholders to discuss issues of cost and reimbursement related to gene therapies and explored the potential role of the Forum in the space. The group also learned about current gene therapy clinical trials and discussed lessons learned in due diligence in product development efforts and how these can be translated to the field of regenerative medicine.

OCTOBER 19, 2018

Forum members met with experts in the field to discuss advancing stem cell therapies for cardiac failure and a novel academic program in regenerative biology and tissue engineering.

JULY 17-18, 2019

Forum members convened stakeholders from the National Library of Medicine, Research!America, and the California State Medical Board Stem Cell Task Force to better understand what types of stem cell therapies are currently available for patients. The meeting also included a strategic planning exercise to help the Forum define priorities for the next three years.

NOVEMBER 14, 2019

Forum members discussed challenges and opportunities for cell manufacturing technologies, potential opportunities for improving gene-based clinical trials, and advertising policies around stem cells.

Public Workshops

OCTOBER 18, 2018

Exploring Sources of Variability Related to the Clinical Translation of Regenerative Engineering Products

[Learn more about this workshop here](#)

To further explore the various factors that contribute to successful regenerative engineering products, an ad hoc committee planned a one-day public workshop in Washington, DC. Invited speakers and participants discussed factors and sources of variability in the development and clinical application of regenerative engineering products, characteristics of high-quality products, and how different clinical needs, models, and contexts can inform the development of a product.

NOVEMBER 13, 2019

Exploring Novel Clinical Trial Designs for Gene-Based Therapies

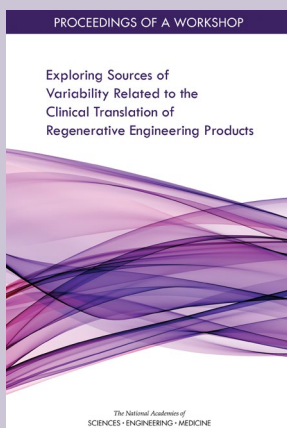
[Learn more about this workshop here](#)

This workshop explored the design complexities and ethical issues associated with clinical trials for gene therapies. Workshop speakers highlighted the importance of natural history datasets, developing clinical endpoints, opportunities for effectively communicating with patients about gene-based clinical trials, and issues with long-term clinical management for patients who participate in gene-based clinical trials. Workshop participants and speakers discussed strategies for supporting the clinical development of safe and effective gene-based therapies and potential next steps to move the field forward.

Influencing Policy

In 2019, Google released a new advertising policy to describe a category of “speculative and experimental medical treatments.” Google’s decision to explore their advertising policy was informed by their participation in the Forum’s February 2019 meeting and subsequently through individual follow up discussions with several members.

PUBLICATIONS



Exploring Sources of Variability Related to the Clinical Translation of Regenerative Engineering Products: Proceedings of a Workshop (2019)

[View report here](#)

NAM PERSPECTIVE



Reducing Risks and Delays in the Translation of Cell and Gene Therapy Innovations into Regulated Products: Discussion Paper (2019)

[Read paper here](#)

Perspectives, published by the National Academy of Medicine (NAM), are individually authored by Roundtable and Forum members and outside experts in health and health care. The views expressed in these papers are those of the authors and not necessarily of the authors’ organizations or of the NAM. Perspectives are intended to help inform and stimulate discussion. They have not been subjected to the review procedures of and are not reports of the NAM or the National Academies of Sciences, Engineering, and Medicine.

Forum Membership

CO-CHAIRS:

Tim Coetzee, Ph.D.
National Multiple Sclerosis Society

Katherine Tsokas, J.D.
Johnson & Johnson

James C. Beck, Ph.D.
Parkinson's Foundation

Sangeeta N. Bhatia, M.D., Ph.D.
Massachusetts Institute of
Technology

Philip John (P.J.) Brooks, Ph.D.
National Center for Advancing
Translational Sciences (NCATS)

George Q. Daley, M.D., Ph.D.
Harvard Medical School; Boston
Children's Hospital & Dana-Farber
Cancer Institute

Brian Fiske, Ph.D.
The Michael J. Fox Foundation for
Parkinson's Research

Larry Goldstein, Ph.D.
Sanford Consortium for Regenerative
Medicine; UCSD School of Medicine

Candance Kerr, M.S., Ph.D.
National Institute on Aging (NIA)

Robert S. Langer, Sc.D.
Massachusetts Institute of
Technology

Cato T. Laurencin, M.D., Ph.D.
The Connecticut Convergence
Institute for Translation in
Regenerative Engineering; The
University of Connecticut

Terry Magnuson, Ph.D.
University of North Carolina, Chapel
Hill

Michael May, Ph.D.
Centre for Commercialization of
Regenerative Medicine

Richard McFarland, Ph.D., M.D.
Advanced Regenerative
Manufacturing Institute

Jack Mosher, Ph.D.
International Society for Stem
Cell Research

David Owens, Ph.D.
National Institute of Neurological
Disorders and Stroke (NINDS)

Amy Patterson, M.D.
National Heart, Lung, and Blood
Institute (NHLBI)

Duanqing Pei, Ph.D.
Chinese Academy of Sciences

Thomas Petersen, M.D., Ph.D.
United Therapeutics Corporation

Anne Plant, Ph.D.
National Institute of Standards and
Technology

Kimberlee Potter, Ph.D.
Department of Veterans Affairs

David Rampulla, Ph.D.
National Institute of Biomedical
Imaging and Bioengineering

Derek Robertson, M.B.A., J.D.
Maryland Sickle Cell Disease
Association

Kelly Rose, Ph.D.
Burroughs Wellcome Fund

Krishnendu Roy, Ph.D.
Georgia Institute of Technology

Krishanu Saha, Ph.D.
University of Wisconsin-Madison

Rachel Salzman, D.V.M.
American Society of Gene and Cell
Therapy

Ivonne Schulman, M.D.
National Institute of Diabetes and
Digestive and Kidney Diseases
(NIDDK)

Jay Siegel, M.D.
Retired, Johnson & Johnson

Lana Skirboll, Ph.D., M.S.
Sanofi

Martha Somerman, D.D.S., Ph.D.
National Institute of Dental and
Craniofacial Research (NIDCR)

Michael Steinmetz, Ph.D.
National Eye Institute (NEI)

Lisa Strovink, M.B.A.
The New York Stem Cell Foundation

Sohel Talib, Ph.D.
California Institute for Regenerative
Medicine

Philip Vanek, Ph.D.
GE Healthcare

Daniel Weiss, M.D., Ph.D.
International Society for Cellular
Therapy

Michael Werner, J.D.
Alliance for Regenerative Medicine

Celia M. Witten, Ph.D., M.D.
U.S. Food and Drug Administration

Claudia Zylberberg, Ph.D.
Akron Biotech

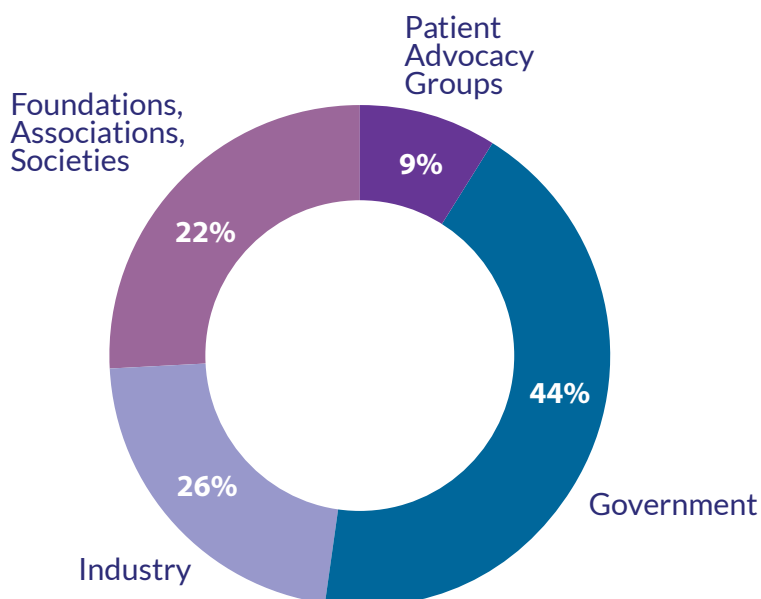
(AS OF MARCH 2020)

Forum Sponsors

Advanced Regenerative Manufacturing Institute
 Akron Biotech
 Alliance for Regenerative Medicine
 American Society of Gene & Cell Therapy
 Burroughs Wellcome Fund
 California Institute for Regenerative Medicine
 Centre for Commercialization of Regenerative Medicine
 U.S. Department of Veterans Affairs
 U.S. Food and Drug Administration
 GE Healthcare
 International Society for Cellular Therapy
 International Society for Stem Cell Research
 Johnson & Johnson
 The Michael J. Fox Foundation for Parkinson's Research
 National Institute of Standards and Technology

National Institutes of Health
 National Center for Advancing Translational Sciences
 National Eye Institute
 National Heart, Lung, and Blood Institute
 National Institute of Biomedical Imaging and Bioengineering
 National Institute of Dental and Craniofacial Research
 National Institute of Diabetes and Digestive and Kidney Diseases
 National Institute of Neurological Disorders and Stroke
 National Institute on Aging
 The New York Stem Cell Foundation
 Parkinson's Foundation
 Sanofi
 United Therapeutics Corporation

(AS OF MARCH 2020)



May not equal 100% due to rounding

FORUM STAFF

Sarah H. Beachy, Ph.D.
Senior Program Officer and
Forum Director

Meredith Hackmann
Associate Program Officer

Siobhan Addie, Ph.D.
Program Officer

Michael Berrios, M.A.
Senior Program Assistant
(until March 2020)

Kelly Choi
Senior Program Assistant
(from March 2020)

BOARD ON HEALTH SCIENCES POLICY STAFF

Andrew Pope, Ph.D.
Senior Board Director

Bridget Borel
Program Coordinator

ABOUT THE NATIONAL ACADEMIES

The National Academy of Sciences, National Academy of Engineering, and National Academy of Medicine work together as the National Academies of Sciences, Engineering, and Medicine ("the Academies") to provide independent, objective analysis and advice to the nation and conduct other activities to solve complex problems and inform public policy decisions. The Academies also encourage education and research, recognize outstanding contributions to knowledge, and increase public understanding in matters of science, engineering, and medicine.

The Health and Medicine Division (HMD), formerly known as the program unit of the Institute of Medicine, is a division of the Academies. HMD's aim is to help those in government and the private sector make informed health decisions by providing evidence upon which they can rely. Each year, more than 3,000 individuals volunteer their time, knowledge, and expertise to advance the nation's health through the work of HMD.

Many of the studies that HMD undertakes are requested by federal agencies and independent organizations; others begin as specific mandates from Congress. While our expert, consensus committees are vital to our advisory role, HMD also convenes a series of forums, roundtables, and standing committees, as well as other activities, to facilitate discussion; discovery; and critical, cross-disciplinary thinking.
