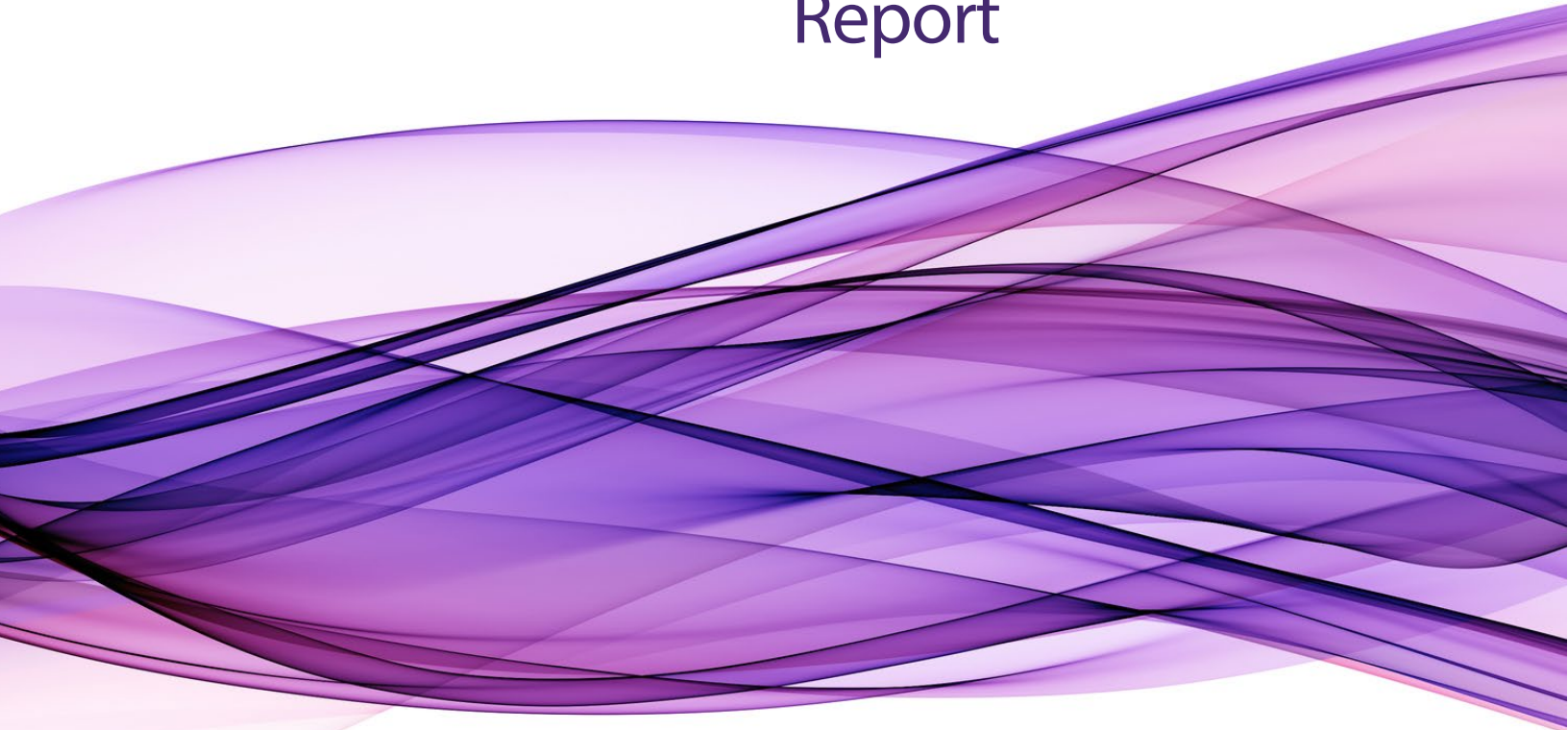


*The National
Academies of*

SCIENCES
ENGINEERING
MEDICINE

Forum on REGENERATIVE MEDICINE

2020 Annual
Report



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 facilitation 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 four 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 diversity 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 serving as a resource for the community to foster the advancement of high quality science and work towards safe and effective therapies for patients.

Working Groups and Areas of Interest

During 2020, the Forum members continued to focus on two broad areas through their working groups – Systems Thinking and Regenerative Engineering & Gene Therapy. The members and staff also continued to explore challenges and discuss possible solutions in a number of other areas relevant to the development of regenerative medicines including communication, workforce development, cost of and access to therapies, and regulatory and legislative affairs.

A more detailed description of the working groups and areas of interest is below.

WORKING GROUPS

Regenerative Engineering and Gene Therapy

The Forum's working group on regenerative engineering and gene therapy explores barriers impeding the successful translation of regenerative and gene-based therapies and forward thinking approaches aimed at improving clinical outcomes. The working group is currently interested in exploring the relationship between the immune system and improving clinical outcomes associated with a regenerative medicine therapy to examine what is known about preparing patients to receive cell and gene-based therapies and how materials are designed with the immune response in mind. To that end, the group is developing plans for a public workshop on this topic for late 2021.

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 held a public workshop in October 2020 focused on examining opportunities to implement systems-based analytical methods that can help advance the mechanistic understanding of regenerative medicine products, and help to overcome challenges with manufacturing and patient outcomes. In 2021, the group plans to explore the challenges of successfully training (or retraining) the up and coming workforce in regenerative medicine to be skilled in data sciences and other cross-disciplinary areas.

ADDITIONAL AREAS OF INTEREST

Workforce Issues

The Forum seeks to understand what skillsets are needed by the workforce now and in the future to discover, develop, and manufacture regenerative medicines.

Regulatory and Legislative Affairs

Forum members are continually updated on regulatory developments, including standards and potential roles for various regulatory bodies at the state and national levels involved in enforcing regulations related to unproven offerings. A subset of Forum members from this group also recently published a perspective paper in 2019 focused on how to reduce risks and delays in the translation of cell and gene therapy innovations.

Communication

The Forum members continue to explore 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 continue to track barriers for patients in accessing regenerative therapies with the goal of understanding the various factors that may contribute to cost and access issues.

2020 Meetings

March 11-12, 2020

The meeting agenda included updates on topics related to developments in the field including: a recent report on FDA's regulation of regenerative medicine products, a proposed classification system for stem cell-based products, and a common framework for defining the quality of ancillary materials. Special sessions at the meeting focused on challenges related to workforce development, cost of and access to therapies, and the use of systems thinking approaches to develop and manufacture regenerative medicine therapies plans and areas for exploration.

July 21-22, 2020

The summer meeting of the Forum included relevant updates from members and presentations on international regulatory convergence efforts, the impact of COVID-19 on the development of regenerative medicines, and the use of social media by patients to gather information about experimental therapies. The meeting agenda also included two educational sessions that helped inform the members as they developed two future workshop topics- one session focused on aspects of systems thinking and the other session examined the role of the immune system in regenerative medicine.

October 22-23, 2020

Applying Systems Thinking to Regenerative Medicine – A Virtual Workshop

October 28, 2020

At this meeting, members debriefed following the public workshop, Applying Systems Thinking to Regenerative Medicine, and discussed possible paths forward. The meeting agenda also included time for the two working groups to meet and discuss their projects and next steps.

Public Workshop

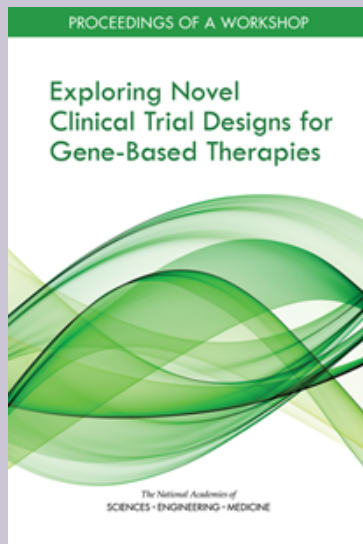
OCTOBER 22-23, 2020

Applying Systems Thinking to Regenerative Medicine – A Virtual Workshop

Learn more about this workshop [here](#).

This workshop explored how cross-disciplinary systems thinking approaches can support the identification of relevant quality attributes and streamline manufacturing and regulatory processes of regenerative medicine products. Workshop speakers discussed new advances in data acquisition, data analysis, and theoretical frameworks that could be applied to aid in the development of regenerative medicine products.

PUBLICATION



Exploring Novel Clinical Trial Designs for Gene-Based Therapies: Proceedings of a Workshop

Released: April 9, 2020

View report [here](#).

Forum Membership

CO-CHAIRS:

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Janssen, Inc. Canada

Sangeeta N. Bhatia, M.D., Ph.D.,
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Research

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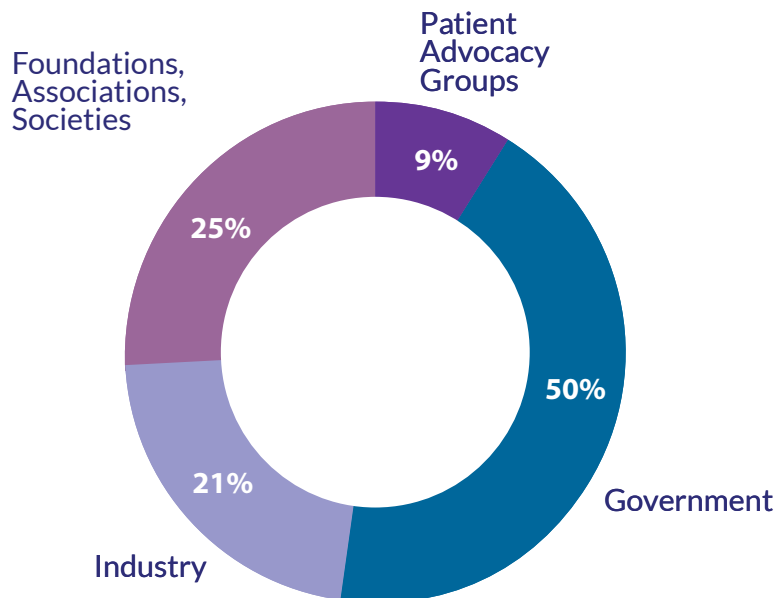
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The New York Stem Cell Foundation

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(AS OF MARCH 2021)

May not equal 100% due to rounding

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Program Officer

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Research Assistant

Meredith Hackmann
Associate Program
Officer

Kelly Choi
Senior Program Assistant (*until July
2020*)

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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.

