

Forum on
**REGENERATIVE
MEDICINE**

2016 and 2017
Program Activities



ABOUT THE FORUM

The National Academies of Sciences, Engineering, and Medicine offer 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 two 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 of 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 information 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.

nationalacademies.org/RegenMedForum

Areas of Interest

The Forum members identified three areas of interest for further exploration and work that evolve to reflect developments in the field.



Communication

The Forum's working group on communications has identified several areas of interest, such as gaining a better understanding of how people weigh and consider decisions related to medical treatments and therapies, gather and collect information from different sources, and use resources to inform their treatment decisions. This group is interested in learning more about what types of resources are in existence for use by those making treatment decisions about their disease/condition and where there are gaps in content and communication of those materials.



Manufacturing, Cell/ Tissue Sourcing, and Tissue Engineering

The Forum's working group on manufacturing and cell/tissue sourcing identified several areas of interest, including defining and measuring critical quality attributes for regenerative medicine products and materials, developing standards for clinical trials and manufacturing, and exploring challenges of manufacturing "living" therapies. The group published a perspective paper on these topics titled *Manufacturing Cell Therapies: The Paradigm Shift in Health Care of This Century* in June 2017 and hosted a public workshop titled *Navigating the Manufacturing Process and Ensuring the Quality of Regenerative Medicine Therapies* on June 26, 2017 (see page 3 for the links to these publications). The working group will continue exploring these topics in 2018, and plans to expand their focus to include challenges in the developing fields of tissue engineering and 3-dimensional scaffolding.



Regulation

The Forum's regulatory working group has considered a wide array of regulatory challenges including developing regulatory standards, navigating the regulatory approval process, and learning about regulation of regenerative medicine in other countries. This group follows current legislation and policy related to regulation of cellular therapies with the goal of keeping members current on recent developments. The Forum also convenes regulatory and legislative experts, patients, and other stakeholders on the topics of legislation and its impact on regulation.

2016 and 2017 Meetings

JUNE 28, 2016 1st meeting

Forum members discussed the function of a forum, defined the Forum's purpose and goals, explored challenges and successes in the field, and outlined plans and areas for exploration.

OCTOBER 14, 2016 2nd meeting

Members explored opportunities for the Forum to engage in activities related to regulatory issues such as setting standards for potency and safety; manufacturing and clinical translation challenges such as the costs and technical barriers related to scaling up production and defining critical quality attributes; and approaches to stakeholder education to improve understanding about and inform realistic expectations for regenerative medicine research.

JUNE 26, 2017 Workshop

Navigating the Manufacturing Process and Ensuring the Quality of Regenerative Medicine Therapies

OCTOBER 26, 2017 5th meeting

Forum members discussed what resources patients need in order to make educated decisions about regenerative medicine therapies and what resources patient advocacy organizations have in place or need to develop to meet those needs. The group also learned about the state of the field in tissue engineering and 3-D scaffolding and explored new legislative and regulatory developments in regenerative medicine, both nationally and internationally.

OCTOBER 13, 2016 Workshop

Exploring the State of the Science in the Field of Regenerative Medicine: Challenges of and Opportunities for Cellular Therapies

MARCH 28–29, 2017 3rd meeting

Forum members discussed regulations and legislation that impact regenerative medicine, as well as the issue of unproven stem cell therapies being offered in U.S. clinics. Members learned about various approaches to communicating science and educating patients, decision makers, and providers about regenerative medicine. Speakers also discussed the 21st Century Cures Act.

JUNE 27, 2017 4th meeting

Forum members discussed current regulations and legislation that impact regenerative medicine, learned about the psychological and sociological factors of patient decision making, and explored ways in which the Forum can continue to move the field of regenerative medicine forward in the areas of research, regulation, and manufacturing.

Public Workshops

OCTOBER 13, 2016

Exploring the State of the Science in the Field of Regenerative Medicine: Challenges of and Opportunities for Cellular Therapies

<http://bit.ly/2cpKsVL>

This workshop highlighted opportunities and challenges associated with developing regenerative medicine cellular therapies and related technologies. Stakeholder groups, including research scientists, clinicians, patients, and representatives from pharmaceutical and biotech companies, presented their perspectives and participated in discussions about new advances in the field and opportunities for using cell-based therapies for various cell and tissue types such as blood and immunological, skin and musculoskeletal, cardiovascular and lung, neurological and ophthalmological, and renal tissues.

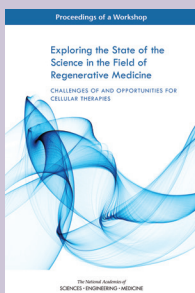
JUNE 26, 2017

Navigating the Manufacturing Process and Ensuring the Quality of Regenerative Medicine Therapies

<http://bit.ly/2r3PhGy>

This workshop focused on exploring what measurements, characteristics, and technologies may be important in the development of new products and therapies. A better understanding of which characteristics define a source cell or tissue can support the development of a quality product. Likewise, a deep understanding of the critical characteristics of the final regenerative medicine product can help ensure its consistency, safety, and potency. Workshop speakers explored the various 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. Workshop participants learned about examples of manufacturing of early generation regenerative medicine products and examined ways to address how progress could be made in identifying and measuring critical quality attributes, designing and adhering to standards, and navigating the scale-up process from a research laboratory to the manufacturing environment.

PUBLICATIONS



Exploring the State of the Science in the Field of Regenerative Medicine: Challenges of and Opportunities for Cellular Therapies: Proceedings of a Workshop (2017)

<http://bit.ly/2xu5bRV>



Navigating the Manufacturing Process and Ensuring the Quality of Regenerative Medicine Therapies: Proceedings of a Workshop (2017)

<http://bit.ly/2j0QOvI>

NAM PERSPECTIVE



Manufacturing Cell Therapies: The Paradigm Shift in Health Care of This Century: Discussion Paper (2017)

<http://bit.ly/2wvT0Um>

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.

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The ALS Association

American Society of Gene & Cell Therapy

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California Institute for Regenerative Medicine

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Christopher & Dana Reeve Foundation

U.S. Department of Veterans Affairs

U.S. Food and Drug Administration

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International Society for Stem Cell Research

Johnson & Johnson

Juno Therapeutics, Inc.

The Michael J. Fox Foundation for Parkinson's Research

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National Institutes of Health

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National Institute of Arthritis and Musculoskeletal and Skin Diseases

National Institute of Biomedical Imaging and Bioengineering

National Institute of Dental and Craniofacial Research

National Institute of Diabetes and Digestive and Kidney Diseases

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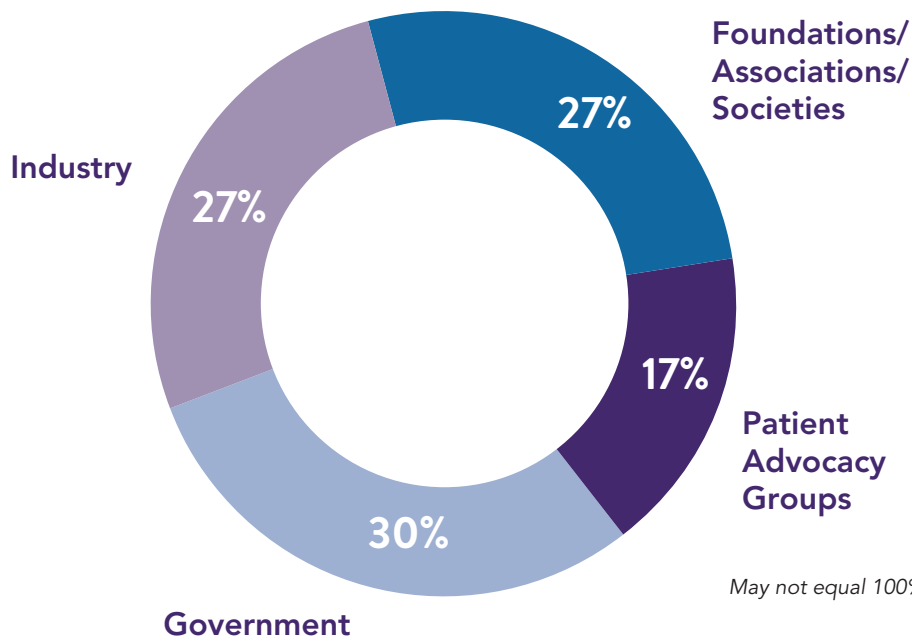
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(AS OF DECEMBER 2017)



May not equal 100% due to rounding

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and inform public policy decisions. The National 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 National 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.
