NATIONAL ACADEMIES Sciences Engineering Medicine

Board on Health Sciences Policy

Forum on Regenerative Medicine

Emerging Technologies and Innovation in Manufacturing Regenerative Medicine Therapies: A Workshop

Forum on Regenerative Medicine Workshop

October 17th, 2023 (8:30 AM – 5:00 PM EST)

In-Person Location
Keck 100, The Keck Center
500 Fifth St. NW
Washington, DC 20001

Remote Log On Information
https://www.nationalacademies.org/event/40391_10-2023_emerging-technologies-and-innovation-in-manufacturing-regenerative-medicine-therapies-a-workshop

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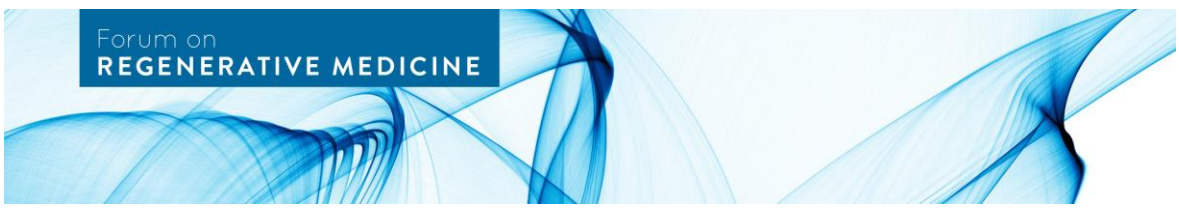
Fall 2023 Workshop
October 17th, 2023

Table of Contents



AGENDA

Emerging Technologies and Innovation in Manufacturing Regenerative Medicine Therapies: A Workshop

Tuesday, October 17, 2023

STATEMENT OF TASK

A planning committee of the National Academies of Sciences, Engineering, and Medicine will organize and hold a one-day public workshop to examine challenges and opportunities in manufacturing regenerative medicine therapeutics as the field evolves to accommodate higher volume production and increased capacity for delivering regenerative medicine treatments to patients. The workshop will explore how new scientific advancements and technological developments in manufacturing cell- and gene-based therapies are shaping the maturing industry of regenerative medicine.

Workshop presentations and discussions may address some of the following topics:

- New developments in closed systems for bioprocessing and biomanufacturing
- Decentralized manufacturing as a strategy to address production and supply chain challenges for regenerative medicine treatments
- Automation, data analysis, computational modeling, and artificial intelligence/machine learning algorithms for advanced biomanufacturing
- Open-source manufacturing to facilitate knowledge sharing and support technology transfer among organizations
- Quality control techniques to improve manufacturing reliability and regulatory strategies to sustainably increase biomanufacturing capacity
- Building multidisciplinary teams equipped to implement new manufacturing approaches and capitalize on the potential of recent developments

The planning committee will organize the workshop, develop the agenda, select and invite speakers, and moderate discussions. Proceedings of the workshop will be prepared by a designated rapporteur in accordance with institutional guidelines.

SESSION I: Opening Remarks & Keynote

8:30 – 8:35 AM ET

Welcoming Remarks

Katherine Tsokas, *Forum Co-Chair*
Vice President
Regulatory, Quality, Risk Management and Drug Safety
Janssen Inc., Canada

Krishnendu Roy, *Forum Co-Chair*
Bruce and Bridgitt Evans Dean of Engineering and University Distinguished Professor
Vanderbilt University

8:35 – 8:40 AM

Graphic Illustrator Introduction

Wade Forbes
Co-founder and Illustrator
RedTale Communications

8:40 – 8:50 AM **Introduction and Charge to the Workshop Speakers and Participants**
Scott Steele, *Workshop Planning Committee Co-Chair*
Senior Advisor, Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

Claudia Zylberberg, *Workshop Planning Committee Co-Chair*
Founder and former CEO
Akron Bio

8:50 – 9:10 AM **Keynote**
Peter Marks
Director of the Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration

9:10 – 9:20 AM **Reactants**
Karin Hoelzer
Director of Policy and Regulatory Affairs
National Organization for Rare Disorders

Sarah Nikiforow
Medical Director, Cell Manipulation Core Facility
Dana-Farber Cancer Institute

9:20 – 9:45 AM **Panel Discussion**
Moderator: Rachel Salzman, American Society for Gene and Cell Therapy

SESSION II: Decentralized Manufacturing as a Strategy to Address Production and Supply Chain Challenges

Moderator: Phil Vanek, Gamma Biosciences

Session Objectives:

- Examine available models of decentralized manufacturing (e.g., distributed manufacturing, point-of-care); explore advantages and disadvantages of different models to scale up and scale out
- Discuss benefits, tradeoffs, and potential strategies to implement different models of decentralized manufacturing
- Discuss scientific/technical, regulatory, and supply chain challenges to implementing these models

9:45 – 10:00 AM **Fabian Gerlinghaus**
Co-founder and CEO
Cellares

10:00 – 10:15 AM **Rahul Singhvi**
Co-founder and CEO
Resilience

10:15 – 10:30 AM **Sarah Nikiforow**
Medical Director, Cell Manipulation Core Facility
Dana-Farber Cancer Institute

10:30 – 10:55 AM **Panel Discussion**

10:55 – 11:15 AM **Break**

SESSION III: Automation and Algorithms in Regenerative Medicine Manufacturing

Moderator: Sohel Talib, California Institute for Regenerative Medicine

Session Objectives:

- Explore recent advancements in and applications for automation technologies in biomanufacturing
- Discuss the role of automation and AI/ML in the future of manufacturing regenerative medicine therapies

11:15 – 11:30 AM ET **Nabiha Saklayen**
Co-founder and CEO
Cellino

11:30 – 11:45 AM **Jan Jensen**
Founder, CEO and CSO
Trailhead Biosystems

11:45 AM – 12:00 PM **Jane Lebkowski**
President
Regenerative Patch Technologies

12:00 – 12:25 PM **Panel Discussion**

12:25 – 1:30 PM **Break**

SESSION IV: Quality Control and Regulatory Considerations

Moderator: Anne Plant, National Institute of Standards and Technology

Session Objectives:

- Discuss quality control and analytical techniques to improve manufacturing reliability
- Discuss what is needed to ensure characterization sufficient to supply confidence for regulatory decision-making (e.g., comparability, harmonization)
- Explore regulatory challenges and potential solutions to support a modern regulatory framework
- Consider possible strategies to address comparability challenges under decentralized models of manufacturing

1:30 – 1:45 PM **Sadik Kassim**
Chief Technology Officer (Genomic Medicines)
Danaher Corporation

1:45 – 2:00 PM

Matthew Hewitt
Vice President and Technical Officer of Cell and Gene Therapies and Biologics
Charles River Laboratories

2:00 – 2:15 PM

Heather Lombardi
Director, Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration

2:15 – 2:40 PM **Panel Discussion**

2:40 – 3:00 PM **Break**

SESSION V: Implementing New Manufacturing Strategies through Partnerships and Innovative Technology Transfer

Moderator: Krishanu Saha, University of Wisconsin-Madison

Session Objectives:

- Discuss how multidisciplinary teams and partnerships can leverage new developments and implement new manufacturing approaches
- Discuss what is needed to build and support effective partnerships
- Discuss benefits of open-source manufacturing and associated challenges
- Explore strategies to promote efficient technology transfer

3:00 – 4:00 PM

Jens Vogel
Senior Vice President & Global Head of Biotech
Bayer Pharmaceuticals

Fyodor Urnov
Professor of Molecular and Cell Biology
Director of Technology & Translation, Innovative Genomics Institute
University of California, Berkeley

Courtney Silverthorn
Associate Vice President, Science Partnerships
Foundation for the National Institutes of Health

Ravi Basavappa
Senior Advisor
Advanced Research Projects Agency for Health (ARPA-H)

Boro Dropulic
Executive Director
Caring Cross

4:00 – 4:40 PM **Panel Discussion**

4:40 – 4:45 PM **Graphic Illustrator Summary**
Wade Forbes
Co-founder and Illustrator
RedTale Communications

4:45 – 5:00 PM **Closing Remarks**
Scott Steele, *Workshop Planning Committee Co-Chair*
Senior Advisor, Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

Claudia Zylberberg, *Workshop Planning Committee Co-Chair*
Founder and former CEO
Akron Bio

FORUM INFORMATION

NATIONAL ACADEMIES Sciences Engineering Medicine

Forum on Regenerative Medicine

The National Academies of Sciences, Engineering, and Medicine's Forum on Regenerative Medicine provides a convening mechanism for interested parties from academia, industry, government, patient and provider organizations, regulators, foundations, and others to 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; identifies and 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; examines 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.

Regenerative medicine holds the potential to create living, functional tissues which can be used to repair or replace those that have suffered irreparable damage due to disease, age, traumatic injury, or congenital defects. Whether through tissue-engineering, synthetic constructs, or cellular therapies, the field holds the promise of providing relief to those suffering from traumatic injuries to neurodegenerative diseases. However, the enormous potential health and economic benefits this relatively new field could potentiate upon society must be balanced by the enactment of the proper policies and procedures to assure these therapies are safe and effective for use.

There are a number of key issues that must be explored and illuminated in order to realize the full potential of regenerative medicine. Ethical, legal, and social issues pose potential challenges with much debate still taking place around the use of adult, embryonic, and induced pluripotent stem cells for research and therapy. Additionally, many prospective advances, while developed for disease treatment, have the potential to be used for enhancement of physical attributes or anti-aging therapy. There is also a concern about possible unanticipated consequences of these treatments and products and the potential for stockpiling of and unequal access to organs. Ensuring the ethical application of regenerative medicine advances will be critical to not only progress the field but also to improve the health of individuals and the public.

Scientific and technical hurdles also exist for which a better fundamental understanding of the underlying cell biology is necessary. This knowledge will allow for more specific engineering of tissues and organs and will diminish the chance of transplant rejection by ensuring biocompatibility with the host tissue. Similarly, it is necessary to understand the cellular response to biomaterials and scaffolds to ensure that the desired biological function is developed and retained. While great advances have been realized to date, to take full advantage of regenerative medicine, the barriers to scientific advance will need to be delineated and potential solutions discussed.

Guidelines for the safe and proper use of regenerative medicine advances will need to be developed, translational barriers identified, and the regulatory environment clearly defined. Commercial aspects will need to be addressed including: the development of cost-effectiveness strategies for growing cells and organs at an industrial capacity; assessments of effectiveness, quality, and biosafety developed; and products certified. Greater dialogue and coordination of efforts between the public and private sectors will enable regenerative medicine products to be brought to market in a safe, effective, and swift manner.

Forum sponsors include federal agencies, medical and scientific associations, foundations, research organizations, patient groups, and industry representatives. For more information about the Forum on Regenerative Medicine, please visit our website at nas.edu/RegenMedForum or contact Sarah Beachy at 202-334-2217, or by email at sbeachy@nas.edu.

NATIONAL ACADEMIES Sciences Engineering Medicine

Forum on Regenerative Medicine

Membership

Co-Chairs: Krishnendu Roy, PhD, Vanderbilt University

Katherine Tsokas, JD, Janssen Inc. Canada

Robert Carter, MD, National Institute of Arthritis and Musculoskeletal and Skin Diseases

Timothy Coetzee, PhD, National MS Society

Tammy Collins, PhD, Burroughs Wellcome Fund

Larry Goldstein, PhD, Sanford Consortium for Regenerative Medicine; UCSD School of Medicine

Thomas Greenwell, PhD, National Eye Institute

Albert Hwa, PhD, National Institute of Diabetes and Digestive and Kidney Diseases

Cato T. Laurencin, MD, PhD, The Connecticut Convergence Institute for Translation in Regenerative Engineering; The University of Connecticut

Michael May, PhD, Centre for Commercialization of Regenerative Medicine

Richard McFarland, PhD, MD, Advanced Regenerative Manufacturing Institute

Jack Mosher, PhD, International Society for Stem Cell Research

Amy Patterson, MD, National Heart, Lung, and Blood Institute

Duanqing Pei, PhD, Chinese Academy of Sciences

Thomas Petersen, MD, PhD, United Therapeutics

Anne Plant, PhD, National Institute of Standards and Technology

Kimberlee Potter, PhD, Department of Veterans Affairs

David Rampulla, PhD, National Institute of Biomedical Imaging and Bioengineering

Derek Robertson, MBA, JD, CHC, Maryland Sickle Cell Disease Association

Derrick Rossi, PhD, New York Stem Cell Foundation

Krishanu Saha, PhD, University of Wisconsin-Madison

Rachel Salzman, DVM, American Society of Gene & Cell Therapy

Eric Sid, MD, National Center for Advancing Translational Sciences

Jay Siegel, MD, retired, Johnson & Johnson

Sohel Talib, PhD, California Institute for Regenerative Medicine

Daniel Weiss, MD, PhD, International Society for Cellular Therapy

Michael Werner, JD, Alliance for Regenerative Medicine

Celia M. Witten, PhD, MD, Food and Drug Administration

Ezequiel Zylberberg, PhD, Akron Biotech

Claudia Zylberberg, PhD, *formerly,* Akron Biotech

Project Staff

Sarah Beachy, PhD, *Forum Director*
Samantha Schumm, PhD, *Program Officer*
Kathryn Asalone, PhD, *Associate Program Officer*
Lydia Teferra, *Research Associate*
Ashley Pitt, *Senior Program Assistant*
Email: regenmed@nas.edu

NATIONAL ACADEMIES Sciences Engineering Medicine

FORUM ON REGENERATIVE MEDICINE

Our **purpose** is to spark exchange and inspire action among diverse interested parties to advance regenerative medicine for the benefit of all.

Our actions are guided by the following principles:

Innovation	Intention	Collaboration	Integrity

We effect change using these strategies:

- Foster a diverse and inclusive future generation of thinkers and doers in the field
- Proactively discern critical scientific, medical, and social issues and provide fit-for-purpose venues for reflection and response
- Engage and collaborate with those whose work aligns with ours
- Translate and disseminate what we learn to catalyze action in the field

The priorities of our work:

Diversity, Equity & Inclusion	Workforce Development	Manufacturing & Supply Chain	Emerging Issues
Advance the Forum's commitment to diversity, equity, and inclusion	Examine incentives and disincentives for expanding the regenerative medicine workforce	Explore obstacles to the delivery of regenerative medicine to patients	Highlight emerging scientific, policy-related, or other issues in the field

We **commit** to upholding the Diversity and Inclusion Statement of the National Academies of Sciences, Engineering, and Medicine. Furthermore, we commit to developing a Diversity, Equity & Inclusion Statement that is more specific to our Forum's work.

The National Academies of Sciences, Engineering, and Medicine value diversity in our members, volunteers, and staff and strive for a culture of inclusion in our workplace and activities. Convening a diverse community to exchange ideas and perspectives enhances the quality of our work and increases our relevance as advisers to the nation about the most complex issues facing the nation and the world.

WORKSHOP INFORMATION

Emerging Technologies and Innovation in Manufacturing Regenerative Medicine Therapies: A Workshop

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Planning Committee Member Roster

Co-Chairs

Claudia Zylberberg, PhD
Formerly, Akron Biotech

Scott Steele, PhD
Senior Advisor
Center for Biologics Evaluation and Research
Food and Drug Administration (FDA)

Members

Thomas Greenwell, PhD
Program Director, Retinal Neuroscience
National Eye Institute (NEI)
National Institutes of Health (NIH)

Rachel Salzman, DVM
Chair, Government Relations Committee
American Society for Gene and Cell Therapy

Kelvin Lee, PhD
Director, Manufacturing USA National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL)
Professor of Chemical and Biomolecular Engineering, University of Delaware

Sohel Talib, PhD
Senior Science Officer and Director of Therapeutics
California Institute for Regenerative Medicine

Anne Plant, PhD
NIST Fellow
National Institute of Standards and Technology

Phil Vanek, PhD
Chief Technology Officer
Gamma Biosciences

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Planning Committee Member Biographies

Scott Steele, PhD *(Co-chair)*, is the Senior Advisor for Translational Science in the Center for Biologics Evaluation and Research (CBER) at the U.S. Food and Drug Administration (FDA). He is responsible for advising on CBER's horizon scanning initiatives and identifying areas of emerging science, technology, and novel methods/tools that are of significant impact to CBER. In this role, Dr. Steele also provides strategic leadership in the development of partnerships and outreach in the area of translational science. He received his BS in Biology from Union College and then performed research at the General Electric Center for Research and Development and at the University of Geneva, followed by a fellowship at the National Institutes of Health. Dr. Steele completed his MA and PhD in Molecular Biology at Princeton University. He later served in the White House Office of Science and Technology Policy, initially as a policy analyst and then as the Executive Director of the President's Council of Advisors on Science and Technology. Prior to Dr. Steele's current appointment, he was at the University of Rochester where he served as the Director of Regulatory Science and Personalized Medicine Programs at the Clinical and Translational Science Institute, Founding Director of the Data Science Center of Excellence, and Associate Professor of Public Health Sciences.

Claudia Zylberberg, PhD *(Co-chair),* is the Founder and former CEO of Akron Bio. She has more than 25 years' experience in the biomedical research and biotechnology industries and first-hand knowledge of what it takes to bring products through R&D and on to approval and commercialization. She is highly knowledgeable about current FDA regulations, qualification of raw materials, process design and validation, and bioassay development. Dr. Zylberberg also has expertise in recombinant proteins, media development, combinational (devices), and platform technologies. She is the inventor of numerous patented proprietary technologies and has an extensive peer-reviewed publication record. Dr. Zylberberg is proud to act as an advisor and consultant to organizations worldwide regarding the regulatory roadmap and commercialization of cell therapies and stem cell banking. She is affiliated with the International Society for Cell Therapy (ISCT) and holds many nonexecutive positions: Board Member and Scientific Advisor, Alliance for Regenerative Medicine (ARM); Board Member, Standard Coordinating Body for Regenerative Medicine; Board Member, Canadian Consortia for Regenerative Medicine; Chair of the Board, ARScience Biotherapeutics; Board Member and Advisor, Jurata Thin Film; Board Member and Senior Advisor, Octomera; Advisor, Phacilitate; and Mentor, Creative Destruction Lab. Previous roles include the following: Board Member, BioFlorida; Board Member, Banner Center of Life Sciences, Palm Beach State College; Chair of Industry Advisory Board, West Palm Beach, Florida.

Thomas Greenwell, PhD, is the Acting Associate Director at the Office of Regenerative Medicine at the National Eye Institute (NEI) of the National Institutes of Health (NIH). He has previously served as the Program Director of Retinal Neuroscience and is a health scientist administrator with over 16 years of experience in science administration and portfolio management at the NIH. He specializes in knowledge and administration of grants in cellular neuroscience and technology applications, especially related to vision. Dr. Greenwell has experience in behavioral models of addiction and stress, fetal alcohol syndrome, and opioid peptides. His interests remain in furthering the public health of the nation, new health science technologies, and government collaborations with pharma and medical device companies.

Kelvin H. Lee, PhD, is Institute Director of the Manufacturing USA National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL), a public-private partnership, and the Gore Professor of Chemical and Biomolecular Engineering at the University of Delaware. He is the Principal Investigator and Site Lead for the Advanced Mammalian Biomanufacturing Innovation Center (AMBIC) and is

affiliated with the United States Manufacturing Innovation Council, a new non-profit for U.S. competitiveness in advanced manufacturing across various sectors. He previously served as Director of the Delaware Biotechnology Institute. Dr. Lee received a B.S.E. in Chemical Engineering from Princeton and Ph.D. in Chemical Engineering from Caltech. He spent several years in the Biotechnology Institute at the ETH in Zurich, Switzerland and completed a postdoc in Caltech's Biology Division. Prior to Dr. Lee's current appointment, he was on the faculty at Cornell University where he held the titles of Samuel C. and Nancy M. Fleming Chair Professor, Professor in the School of Chemical and Biomolecular Engineering, Director of the Cornell Institute for Biotechnology, and Director of the New York State Center for Life Science Enterprise.

Anne Plant, PhD, is a Fellow at the National Institute of Standards and Technology (NIST), where she was previously Chief of the Biosystems and Biomaterials Division. She served for a year in the White House Office of Science and Technology Policy. Dr. Plant has also served in a number of other capacities, including as the NIST Representative to the National Science and Technology Council Life Science Sub-Committee, a member of the National Advisory Council for Biomedical Imaging and Bioengineering, co-chair of the ASTM International Committee F04.46 on Standards for Cell Signaling, and a member of the Editorial Advisory Board of the journal, *Biointerphases*. Dr. Plant has also served on a review panel for the California Institute of Regenerative Medicine, and as a government liaison to the Standards Coordinating Body for Regenerative Medicine. She is a Fellow of the American Institute for Medical and Biological Engineering (AIMBE) and a Fellow of the American Association for the Advancement of Science. Dr. Plant's research has focused on robust quantification of cell response through quantitative cell imaging and theoretical approaches for prediction of complex biological response. She received her Ph.D. from Baylor College of Medicine in Houston, TX in Biochemistry.

Rachel Salzman, DVM, is the Global Head of Corporate Strategy at Armatus Bio, which uses AAV technology to develop novel therapies for neuromuscular diseases. Prior to joining Armatus, Rachel was EVP of Portfolio, External Affairs & Development at Alcyone Therapeutics, a precision medicines company advancing therapies in serious neurological disorders by integrating novel delivery technologies with innovative genetic platforms. In 2017 Dr. Salzman co-founded SwanBio Therapeutics, and served as Chief Executive Officer (CEO) and Director. Prior to her time at Swan, she was the Chief Science Officer (CSO) of The Stop ALD Foundation, a position she held since 2001. The Stop ALD Foundation is a non-profit Medical Research Organization dedicated to employing entrepreneurial approaches and innovative methodology towards effective therapies, cures, and prevention of X-linked adrenoleukodystrophy (ALD), an often-fatal neurodegenerative disease. As CSO she made critical contributions in driving forward the world's first ex vivo lentiviral gene therapy clinical trial conducted in non-HIV infected patients. Prior to these roles, Dr. Salzman worked in private veterinary practice in both large and small animal medicine. Dr. Salzman has been an active member of ASGCT (American Society of Gene and Cell Therapy) for over 20 years and has served in a leadership capacity including elected membership to the Board of Directors, along with multiple committees and task forces designed to build and enhance the state of gene and cell therapy in both the US and EU. Her many contributions were formally recognized by being designated the Sonia Skarlatos Public Service Award recipient in 2015. She

currently represents ASGCT at the National Academy of Sciences Forum on Regenerative Medicine. She has a D.V.M. from Oklahoma State University and a B.S. from Rutgers University.

Sohel Talib, PhD, is a Senior Science Officer and the Director of Therapeutics at California Institute for Regenerative Medicine (CIRM). He is responsible for developing and implementing CIRM's scientific programs and managing a portfolio of clinical stage grants utilizing hematopoietic stem cell gene therapy approaches for the treatment of hemoglobinopathies, Primary Immune Deficiency diseases (PID), HIV AIDS, and cancer. His scientific background is in stem cell and gene therapy research, and he has spent 20 years in the biotech industry. Before joining CIRM, Dr. Talib was the Director of Product Development at Geron Corporation, where he directed immune-oncology program on the development of an autologous dendritic cell vaccine for cancer (Geron VAC-1). Prior to Geron Corporation, he served as the Director of Immunology at Cerus Corporation, a biotech company developing novel allogeneic stem cell therapy for the hematological malignancies. Dr. Talib was a cofounder of Applied Immune Sciences (AIS), which was acquired by Rhone Poulenc Rorer (RPR, currently Sanofi). At RPR Gen Cell, he directed the development and execution of adoptive immunotherapy programs. Dr. Talib received his post-doctoral training at Stanford University, University of California, Berkeley and Roche Institute of Molecular Biology, Nutley. He obtained his Ph.D. in Biochemistry from Aligarh University, India and International DANIDA fellowship from Danish Institute of Protein Chemistry, Copenhagen.

Phil Vanek, PhD, is the Chief Technology Officer at Gamma Biosciences. An entrepreneurial and strategic international business leader, he joined Gamma Biosciences in 2020 from Cytiva's Cell and Gene Therapy business. At Gamma, Dr. Vanek is responsible for technical diligence of potential investments, as well as guiding operational, R&D, and strategic initiatives carried out at portfolio companies. Dr. Vanek received his Ph.D. in Biochemistry and Molecular Biology at Georgetown University Medical Center, with research focused on molecular oncology. He was an instructor at Johns Hopkins University Advanced Academic Programs teaching Biotechnology Marketing in the Master of Biotechnology/MBA program and has held strategy and innovation leadership positions in a number of life sciences companies including Life Technologies, Becton Dickinson, and Lonza. Dr. Vanek is a Board Member of Centre for Commercialization of Regenerative Medicine (CCRM) and CCRM Enterprises in Toronto, Canada and a Board Member of the Foundation for Cell and Gene Medicine (formerly the Alliance for Regenerative Medicine (ARM) Foundation). Dr. Vanek also serves on the advisory boards for Rooster Bio, Biobridge Global, Culture Biosciences, and Curate Biosciences.

NATIONAL ACADEMIES Sciences Engineering Medicine

Board on Health Sciences Policy | *Forum on Regenerative Medicine*

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Speaker Biographies

Ravi Basavappa, Ph.D., is a Senior Advisor at the Advanced Research Projects Agency for Health (ARPA-H). Prior to joining ARPA-H, Dr. Basavappa served in the Office of Strategic Coordination (the "Common Fund" Office) in the Office of the Director, National Institutes of Health (NIH). There, he oversaw the NIH High-Risk, High-Reward Program (comprising the NIH Director's Pioneer Awards, New Innovator Awards, Early Independence Awards and the Transformative R01s) and helped launch a number of other programs including in Single-Cell Analysis, Glycoscience, CryoElectron Microscopy, and Metabolomics. He previously was a Program Director in the Division of Cell Biology and Biophysics at the National Institute of General Medical Sciences (NIGMS). Prior to joining NIH, Dr. Basavappa was Associate Professor of Biophysics and Biochemistry at the University of Rochester School of Medicine. He received his PhD in Biophysics and Theoretical Biology from the University of Chicago and completed a post-doctoral fellowship at Harvard Medical School.

Boro Dropulić, Ph.D., received his PhD from the University of Western Australia and his MBA from the Johns Hopkins University (JHU). He has been in the gene therapy field since the late 1980s. As Chief Executive Officer, Dr. Dropulić brings 30+ years of leadership and experience in the design, development, manufacturing, clinical translation, regulatory, clinical implementation, and commercialization of Lentiviral vector technology. After a Fogarty Fellowship at the NIH, he joined the faculty at JHU where he worked on developing Lentiviral vectors as delivery systems for gene therapy. After 4 years in academia, he founded his first company ViRxSys and led the team that first demonstrated the safety of Lentiviral vectors in humans with his UPenn colleagues. Later he founded Lentigen, which first developed the Lentiviral vector used to produce Kymriah™, the first FDA-approved gene therapy product. Dr. Dropulić implemented and directed the company's CDMO business model and therapeutic pipeline of gene therapy products. Boro led Lentigen until 2021, and then left to co-found and launch Caring Cross, a 501 (c)(3) non-profit, and serves as the Executive Director. Lentiviral vectors are critical to produce many gene therapy products such as CAR-T cells and gene-modified Hematopoietic Stem Cells (HSCs) for the treatment of an increasing number of important diseases. Presently there are huge bottlenecks in obtaining high quality Lentiviral vectors in a reasonable time that motivates investigators and investors alike. This delays and puts at risk the development and commercialization of innovative and potentially curative gene therapies that are desperately needed. Seeing a need to help investigators improve and accelerate their medicinal concepts needing Lentiviral vectors into the clinic, Vector BioMed was launched and Boro serves as the Chief Executive Officer.

Fabian Gerlinghaus, is the Co-Founder and Chief Executive Officer of Cellares. He is driven by a strong sense of purpose and is passionate about building the future of cell therapy manufacturing. With 10+ years of experience as an innovator and a leader, Fabian has established a track record of assembling top-performing teams to successfully drive novel bioprocessing technologies from ideation to commercial readiness. Prior to co-founding Cellares, Fabian served as Chief Innovation Officer at Synthego, where he co-invented the company's proprietary RNA synthesizer technology and helped grow the company from five to more than 230 employees. He successfully led the interdisciplinary team that took synthesizer technology from whiteboard sketch to production-ready instruments within two years, enabling the company to be the first to market with its CRISPR/Cas9 product portfolio. He earned a master's degree in aerospace engineering from the Technical University of Munich, and an honours degree in technology management from the Center for Digital Technology and Management, Munich.

Matthew Hewitt, Ph.D., currently serves as Vice President, Technical Officer for CGT and Biologics at Charles River Laboratories (CRL) playing a critical role in driving CGT strategic vision as well as leading multiple operational initiatives across CRL's CGT sites. Before joining CRL, he was Head of R&D and Clinical Development for Lonza's Personalized Medicine Business Unit leading Cocoon platform development, a closed, automated, scalable cell therapy manufacturing solution. In addition, he executed numerous collaborations across academia and industry leveraging the Cocoon. Prior to Lonza, Matt led the Tumor Immunology and Microenvironment program at Bellicum Pharmaceuticals, focusing on improving cell therapy efficacy in solid tumors. Dr. Hewitt also led the Immunology group at the University of Pennsylvania's Gene Therapy Program, leading and contributing to numerous AAV gene therapy programs. Matt received his PhD in Biophysics and Physiology from the University of Alabama at Birmingham and completed his postdoctoral fellowship at Johns Hopkins University.

Karin Hoelzer, Ph.D., directs Policy and Regulatory Affairs for the National Organization for Rare Disorders (NORD®). In this role, Dr. Hoelzer provides strategic direction to advance NORDs federal policy and regulatory priorities. She works closely with key rare disease partners across the pharmaceutical and biological space to ensure the policy landscape supports innovative approaches and new treatments to help rare disease patients, and adequately incorporates patient preferences and perspectives in therapy development. Her role also involves extensive legislative and communication engagements to advance rare disease policy priorities. Dr. Hoelzer is a health policy, risk analysis, and biomedical research expert, with extensive intellectual property and regulatory expertise across most FDA-regulated products. Most recently, she worked at Maximus, Inc. where she established and led a new health data analytics division to provide more timely data and better insights to government clients in support of the public health response to the COVID-19 pandemic. Prior to working at Maximus, Inc., Karin served as Senior Officer for Health Programs at The Pew Charitable Trusts where she led policy and regulatory efforts to improve the federal oversight of a variety of FDA-regulated products. Dr. Hoelzer joined Pew from the Food and Drug Administration (FDA), where she served as Risk Analyst. In this role, she assessed and quantified the expected impact of changes to FDA policy and regulatory practice.

Jan Jensen, Ph.D., is the founder and CEO/CSO of Trailhead Biosystems Inc. Trailhead Biosystems is the pioneer of HD-DoE methodology in the control of cell fate. Using high dimensional testing as a basis to extract critical process parameters from biological systems, the company is rapidly creating reproducible and scalable methods for the production of specialized human cells. The foundation of HD-DoE is machine-driven experimental designs, executed robotically, combined with deep measurement. Resulting effector/response matrices creates a mathematically-driven basis of process understanding not attainable using hypothesis-driven research. The company currently produces and sells several cell types from the endoderm, ectoderm, and mesoderm germ layers, and rapidly develops novel induction protocols for many distinct cell types.

Sadik Kassim, Ph.D., is a scientist and executive with extensive experience in the biotechnology industry with a specific focus on cell and gene therapy bioprocessing and translational research. Currently, he serves as Chief Technology Officer of Genomic Medicines for the Life Sciences companies at Danaher Corporation, which includes a portfolio of companies such as Aldevron, IDT, Cytiva, and Sciex. Most recently, he was Chief Technology Officer at Vor Bio where he built the technical operations team responsible for process development, analytical development, supply chain and manufacturing support of a CRISPR gene-edited HSPC product and oversaw the company's CAR-T efforts. Prior to Vor, Dr. Kassim served as Executive Director at Kite Pharma and led the development of manufacturing processes for autologous CAR-T and TCR-based cell therapies. As the Chief Scientific Officer at Mustang Bio, Sadik managed the foundational build-out of the company's preclinical and manufacturing activities. Earlier in his career, he was Head of Early Analytical Development for Novartis' Cell and Gene Therapies Unit and worked on research teams at the National Cancer Institute with Dr. Steven Rosenberg, the University of Pennsylvania Gene Therapy Program with Dr. Jim Wilson, and Johnson and Johnson's Immunology Discovery group. Dr. Kassim and his teams have contributed to the successful BLA and MAA applications for three of the commercially available CAR-T therapies: Kymriah, Yescarta, and Tecartus. Sadik holds a Bachelor of Science degree in Cell and Molecular Biology from Tulane University and earned his PhD in Microbiology and Immunology from Louisiana State University.

Jane Lebkowski, Ph.D., is an internationally recognized leader in the development of cell and gene-based therapies with direct Management experience in the multi-disciplinary functions required to translate research discoveries into thera. Dr. Lebkowski has been actively involved in the development of cell and gene therapies since 1986 and is President of Regenerative Patch Technologies (RPT), a biotechnology firm developing composite stem cell-based implants targeting restoration of retinal architecture and function in patients with age related macular degeneration. In this role, Dr. Lebkowski oversees all of RPT's operations.

Heather Lombardi, Ph.D., was recently appointed Director of the Office of Cellular Therapy and Human Tissue within CBER's new Office of Therapeutic Products. Previously, Dr. Lombardi served as the Director, Division of Cellular and Gene Therapies within CBER's Office of Tissues and Advanced Therapies. Prior to her tenure at CBER, Heather was Director, Division of Animal Bioengineering and

Cellular Therapies at the FDA's Center for Veterinary Medicine (CVM). She has expertise in the review of CMC information for innovative products such as proteins, peptides, and cell and gene therapy products for both animals and humans. Dr. Lombardi holds a PhD in biological chemistry from the University of Pennsylvania and a BS in chemistry (biochemistry track) from UNC Chapel Hill.

Peter Marks, M.D., received his graduate degree in cell and molecular biology and his medical degree at New York University and completed Internal Medicine residency and Hematology/Medical Oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings teaching and caring for patients and in industry on drug development and is an author or co-author of over 100 publications. Dr. Marks joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in 2016. Over the past several years he has been integrally involved in the response to various public health emergencies, and in 2022 he was elected a member of the National Academy of Medicine.

Sarah Nikiforow, M.D., is currently an Assistant Professor at Harvard Medical School with the Stem Cell Transplant Program at Dana-Farber Cancer Institute, Medical Director of the Cell Manipulation Core Facility (CMCF), and Technical Director of DFCI's Immune Effector Cell Program. Dr. Nikiforow earned her MD and a PhD in Immunobiology at Yale University working on the differential roles of CD4 and CD8 T cells in immune control over Epstein-Barr virus-induced B-cell transformation. Dr. Nikiforow has pursued a translational research career focusing on immune reconstitution after stem cell transplant and therapeutic use of adoptive cellular products. Through the CMCF and as Principal Investigator of Phase I and II clinical trials, she is working to bring cellular therapies such as chimeric antigen-receptor T cells, genetically-modified stem cells, and regulatory T-cell infusions into the clinic at Dana-Farber. Sarah's work with the Foundation for the Accreditation of Cellular Therapy and the Center for International Blood & Marrow Transplant Research, International Society of Cellular Therapy, and most recently the American Society of Transplant and Cellular Therapy's 80/20 committee have promoted education and set standards for safe implementation of new approaches within the broader and ever-growing cellular therapy field.

Nabiha Saklayen, Ph.D., is the CEO and co-founder of Cellino, where her team is spearheading the ultra-scalable biomanufacturing of personalized regenerative medicines to make "Your Cells, Your Cure" a reality for all patients in an aging and increasingly diverse world. As a purpose-driven physicist, she focused her Ph.D. research on inventing bio/nanophotonics technologies to unlock precision cellular engineering for human health applications. Dr. Saklayen's biophotonics expertise and vision for patient impact inspired Cellino's proprietary approach to cell culture: an autonomous, all-optical cell culture method that unlocks the ultra-scalable manufacturing of high-quality, personalized regenerative medicines. Nabiha is a TED speaker and the inaugural Tory Burch Foundation Fellow in Genomics at the Innovative Genomics Institute, led by Nobel Laureate Dr. Jennifer Doudna. She received her Ph.D. in Physics from Harvard University as a Howard Hughes Medical Institute (HHMI) International Fellow. Dr. Saklayen also co-created I Am A Scientist, an educational program in 50 states that inspires children

to explore science. She is a global citizen who grew up in Saudi Arabia, Bangladesh, Germany, and Sri Lanka.

Courtney Silverthorn, Ph.D., is an Associate Vice President for Science Partnerships at the Foundation for the National Institutes of Health (FNIH). With extensive experience in public-private partnerships and federal technology transfer policy, she serves as the Director of the Accelerating Medicines Partnership® (AMP®) program and is responsible for new business development in platform approaches to therapeutics and the program's Bespoke Gene Therapy Consortium, a multi-year public-private partnership to advance manufacturing and regulatory frameworks for gene therapy treatments for rare diseases. Prior to joining the FNIH, Dr. Silverthorn held several positions in the Technology Partnerships Office at the National Institute of Standards and Technology (NIST), including serving as the Acting Director of the office from 2020 to 2021. During her time at NIST, she led technology transfer activities at the agency and was central to the interagency Lab-to-Market initiative. Courtney's interagency policy coordination efforts included serving as a Co-Chair of the National Science and Technology Council's Lab-to-Market subcommittee and developing and implementing findings from NIST's Return on Investment Initiative. She also served as a Senior Policy Advisor to the Office of Science and Technology Policy, supporting both Lab-to-Market and Citizen Science, and executed hundreds of technology transfer partnerships at the National Cancer Institute and the Frederick National Laboratory for Cancer Research. Dr. Silverthorn earned a Ph.D. in Pharmacology from The Johns Hopkins University School of Medicine, a M.S. in Leadership from Washington University in St. Louis, and a B.S. in Biochemistry and Molecular Biology from Sweet Briar College. Courtney has also earned certificates in Biotechnology Enterprise from Johns Hopkins and in Policy Strategy from the Brookings Institution.

Rahul Singhvi, Sc.D., is a global leader in the Life Sciences industry and serves as the Chief Executive Officer for RESILIENCE. Most recently, he was an Operating Partner at Flagship Pioneering, a Boston-based life sciences innovation firm where he was responsible for founding and operating companies launched from Flagship's innovation foundry, Flagship labs. Before joining Flagship, Dr. Singhvi was the Chief Operating Officer of Takeda's Vaccine Business Unit where he was responsible for worldwide vaccine CMC and manufacturing operations. During his six-year tenure at Takeda, the vaccine business grew to over 500 employees and created an industry leading late-stage pipeline of vaccine candidates against dengue, norovirus, and zika. Before Takeda, Rahul was President and CEO of Novavax, Inc. (Nasdaq: NVAX) where he transformed the company from a specialty pharmaceutical business to a vaccine development company with vaccine candidates against influenza (funded by BARDA) and respiratory syncytial virus (RSV). Dr. Singhvi's professional career began at Merck & Co in 1994, where he held several positions in R&D and manufacturing. Rahul serves on the Executive Advisory Board of the Leonard Davis Institute (LDI) of Health Economics at the University of Pennsylvania and on the Scientific Advisory Board of the anti-microbial resistance research group at the Singapore MIT Advance Research and Technology program. He is a mentor instructor in the Undergraduate Projects Opportunity Program (UPOP) at MIT and is a visiting lecturer at the University College London (UCL). Dr. Singhvi graduated as the top ranked chemical engineer from the Indian Institute of Technology, Kanpur, India and

obtained both his M.S. and Sc.D. chemical engineering degrees from MIT. Rahul received an MBA from the Wharton School of the University of Pennsylvania, where he graduated as a Palmer Scholar.

Fyodor Urnov, Ph.D., is a Professor of Molecular Therapeutics at UC Berkeley and a Scientific Director at its Innovative Genomics Institute. He co-developed the toolbox of human genome and epigenome editing and led the team that developed a strategy for genome editing in the hemoglobinopathies, sickle cell disease and beta-thalassemia, that has yielded sustained clinical benefit for subjects in several ongoing clinical trials. At the IGI Fyodor directs efforts to develop scalable CRISPR-based approaches to treat diseases of the immune system, sickle cell disease, neurodegeneration, and neuroinflammation. His recent op-ed in the New York Times describes a major goal for the field of genome editing, and a key focus of Fyodor's work at the IGI - expanding access to CRISPR therapies for N=1 genetic disease.

Philip Vanek, Ph.D., is an entrepreneurial and strategic international business leader. He joined Gamma Biosciences in 2020 from Cytiva's Cell and Gene Therapy business. At Gamma, Phil is responsible for technical diligence of potential investments, as well as guiding operational, R&D and strategic initiatives carried out at portfolio companies. Dr. Vanek received his Ph.D. in Biochemistry and Molecular Biology at Georgetown University Medical Center, with research focused in molecular oncology. Phil was an instructor at Johns Hopkins University's Biotechnology Marketing in the Masters of Biotechnology / MBA program, and has held strategy and innovation leadership positions in a number of life sciences companies including Life Technologies, Becton Dickinson, and Lonza. Dr. Vanek is a Board Member of CCRM in Toronto Canada and a Board Member of the Foundation for Cell and Gene Medicine. Additionally, Phil is a mentor for the Creative Destruction Lab entrepreneurship program, and an advisor to a number of life sciences companies.

Jens Vogel, Ph.D., is SVP & Global Head of Biotech, Bayer Pharmaceuticals Product Supply, leading biologics operations, development, and strategy across five sites, including cell & gene therapy industrialization. The company is pursuing completely new therapeutic approaches to diseases, strengthening its position in the highly promising area of cell and gene therapy discovery, development, and manufacturing. Dr. Vogel has more than 20 years of professional and leadership experience in biologics development, operations, program management and regulatory affairs. Before rejoining Bayer in March 2020, he was President and CEO of Boehringer Ingelheim Fremont and member of BI's Biopharma Executive Committee. Prior to his years at BI, Jens held various roles of increasing responsibility in Biologics Development at Bayer in Berkeley. He holds a PhD in Biochemical Engineering.

Emerging Technologies and Innovation in Manufacturing Regenerative Medicine Therapies: A Workshop

October 17, 2023

LOCATION
The Keck Center, Room 100
500 5th St. NW
Washington, DC 20001

Speaker Guidance

CONTEXT

In 2022, the Forum on Regenerative Medicine developed a new strategic plan along with three working groups. The Manufacturing & Supply Chain working group considers obstacles to the delivery of regenerative medicine to patients. The Forum is hosting this workshop to explore challenges and opportunities in manufacturing regenerative medicine therapeutics as the field evolves. The workshop will address how new scientific advancements and technological developments in manufacturing cell- and gene-based therapies are shaping the maturing industry of regenerative medicine. Thank you for joining us for this workshop!

SESSION I. OPENING REMARKS & KEYNOTE

Questions for Speakers

1. What are some of the major scientific and technological developments currently shaping the manufacturing of regenerative medicine therapeutics and how have they been impacting this evolving field?
2. What factors limit accessibility to novel cell- and gene-based therapies and how can they be addressed?
3. What are the challenges and opportunities related to regulatory, geographic, cost of goods, and the economics of biomanufacturing?
4. What are some of the lessons learned from the history of biomanufacturing?

SESSION II. DECENTRALIZED MANUFACTURING AS A STRATEGY TO ADDRESS PRODUCTION AND SUPPLY CHAIN CHALLENGES

Session Objectives

- Examine available models of decentralized manufacturing (e.g., distributed manufacturing, point-of-care); explore advantages and disadvantages of different models to scale up and scale out
- Discuss benefits, tradeoffs, and potential strategies to implement different models of decentralized manufacturing available models, strategies
- Discuss scientific/technical and supply chain challenges to implementing these models

Questions for Speakers

1. How do you define decentralized manufacturing? What level of decentralization constitutes "decentralized" manufacturing?
2. How can decentralized manufacturing be leveraged to tackle some of the most pressing supply chain and production issues?
3. What are advantages and disadvantages of different models of decentralized manufacturing? How do the benefits of each balance with their tradeoffs?
4. What are some important considerations and challenges related to the implementation of these models? What systems need to be in place so that their maximum benefits can be realized?
5. What do you think will be the first use case for distributed manufacturing? Why? What makes it unique?

SESSION III. AUTOMATION AND ALGORITHMS IN REGENERATIVE MEDICINE MANUFACTURING

Session Objectives

- Explore recent advancements in and applications for automation technologies in biomanufacturing
- Discuss the role of automation and AI/ML in the future of manufacturing regenerative medicine therapies

Questions for Speakers

1. Describe ways in which automated technologies and artificial intelligence/machine learning algorithms have evolved in recent years and the resulting impact on the field of regenerative medicine manufacturing.
2. What new developments or platforms can contribute to more efficient product development?
3. What are the most critical automation-related challenges that need to be addressed in regenerative medicine?
4. What is the future of these technologies and how will their use, including their benefits and challenges, continue to change?
5. What are key considerations around data acquisition, management, validation, sharing in the context of a decentralized manufacturing model?

SESSION IV. QUALITY CONTROL AND REGULATORY CHALLENGES

Session Objectives

- Discuss quality control and analytical techniques to improve manufacturing reliability
- Discuss what is needed to ensure characterization sufficient to supply confidence for regulatory decision-making (e.g., comparability, harmonization)
- Explore regulatory challenges and potential solutions to support a modern regulatory framework
- Consider possible strategies to address comparability challenges under decentralized models of manufacturing

Questions for Speakers

1. In what ways can manufacturing reliability be improved by employing various quality control and analytical strategies?
2. What are the most pertinent regulatory challenges currently facing the regenerative medicine manufacturing space? What are the necessary components of a modern regulatory framework in which these challenges are addressed?
3. What are strategies that will help establish comparability within decentralized models of manufacturing?
4. Under decentralized models, what does release look like? Is release by exception (i.e., process as the product) plausible as a model?

SESSION V. IMPLEMENTING NEW MANUFACTURING STRATEGIES THROUGH PARTNERSHIPS AND INNOVATIVE TECHNOLOGY TRANSFER

Session Objectives

- Discuss how multidisciplinary teams and partnerships can leverage new developments and implement new manufacturing approaches
- Discuss what is needed to build and support effective partnerships
- Discuss benefits of open-source manufacturing and associated challenges
- Explore strategies to promote efficient technology transfer

Questions for Speakers

1. Describe an example or a case study in which effective partnership was utilized to implement a new manufacturing approach or to enhance manufacturing capacity. What kinds of strategies can be utilized to build more of these partnerships in the future?
2. How can a multidisciplinary team be composed so that it is best equipped to tackle current manufacturing challenges and capitalize on recent advancements and technologies? What does the ideal team look like?
3. What are current barriers to technology transfer? How can technology transfer among various actors, sectors, or teams be improved?
4. What are the advantages and bottlenecks of open-source manufacturing?

NATIONAL ACADEMIES Sciences Engineering Medicine

PREVENTING DISCRIMINATION, HARASSMENT, AND BULLYING:
POLICY FOR PARTICIPANTS IN NASEM ACTIVITIES

The National Academies of Sciences, Engineering, and Medicine (NASEM) are committed to the principles of diversity, inclusion, integrity, civility, and respect in all of our activities. We look to you to be a partner in this commitment by helping us to maintain a professional and cordial environment. **All forms of discrimination, harassment, and bullying are prohibited in any NASEM activity.** This policy applies to all participants in all settings and locations in which NASEM work and activities are conducted, including committee meetings, workshops, conferences, and other work and social functions where employees, volunteers, sponsors, vendors, or guests are present.

Discrimination is prejudicial treatment of individuals or groups of people based on their race, ethnicity, color, national origin, sex, sexual orientation, gender identity, age, religion, disability, veteran status, or any other characteristic protected by applicable laws.

Sexual harassment is unwelcome sexual advances, requests for sexual favors, and other verbal or physical conduct of a sexual nature that creates an intimidating, hostile, or offensive environment.

Other types of harassment include any verbal or physical conduct directed at individuals or groups of people because of their race, ethnicity, color, national origin, sex, sexual orientation, gender identity, age, religion, disability, veteran status, or any other characteristic protected by applicable laws, that creates an intimidating, hostile, or offensive environment.

Bullying is unwelcome, aggressive behavior involving the use of influence, threat, intimidation, or coercion to dominate others in the professional environment.

REPORTING AND RESOLUTION

Any violation of this policy should be reported. If you experience or witness discrimination, harassment, or bullying, you are encouraged to make your unease or disapproval known to the individual at the time the incident occurs, if you are comfortable doing so. You are also urged to report any incident by:

- Filing a complaint with the Office of Human Resources at 202-334-3400 or hrservicecenter@nas.edu, or
- Reporting the incident to an employee involved in the activity in which the member or volunteer is participating, who will then file a complaint with the Office of Human Resources.

Complaints should be filed as soon as possible after an incident. To ensure the prompt and thorough investigation of the complaint, the complainant should provide as much information as is possible, such as names, dates, locations, and steps taken. The Office of Human Resources will investigate the alleged violation in consultation with the Office of the General Counsel.

If an investigation results in a finding that an individual has committed a violation, NASEM will take the actions necessary to protect those involved in its activities from any future discrimination, harassment, or bullying, including in appropriate circumstances **the removal of an individual from current NASEM activities and a ban on participation in future activities**.

CONFIDENTIALITY

Information contained in a complaint is kept confidential, and information is revealed only on a need-to-know basis. NASEM will not retaliate or tolerate retaliation against anyone who makes a good faith report of discrimination, harassment, or bullying.

Updated December 2, 2021

We have a graphic illustrator joining us for the workshop today!

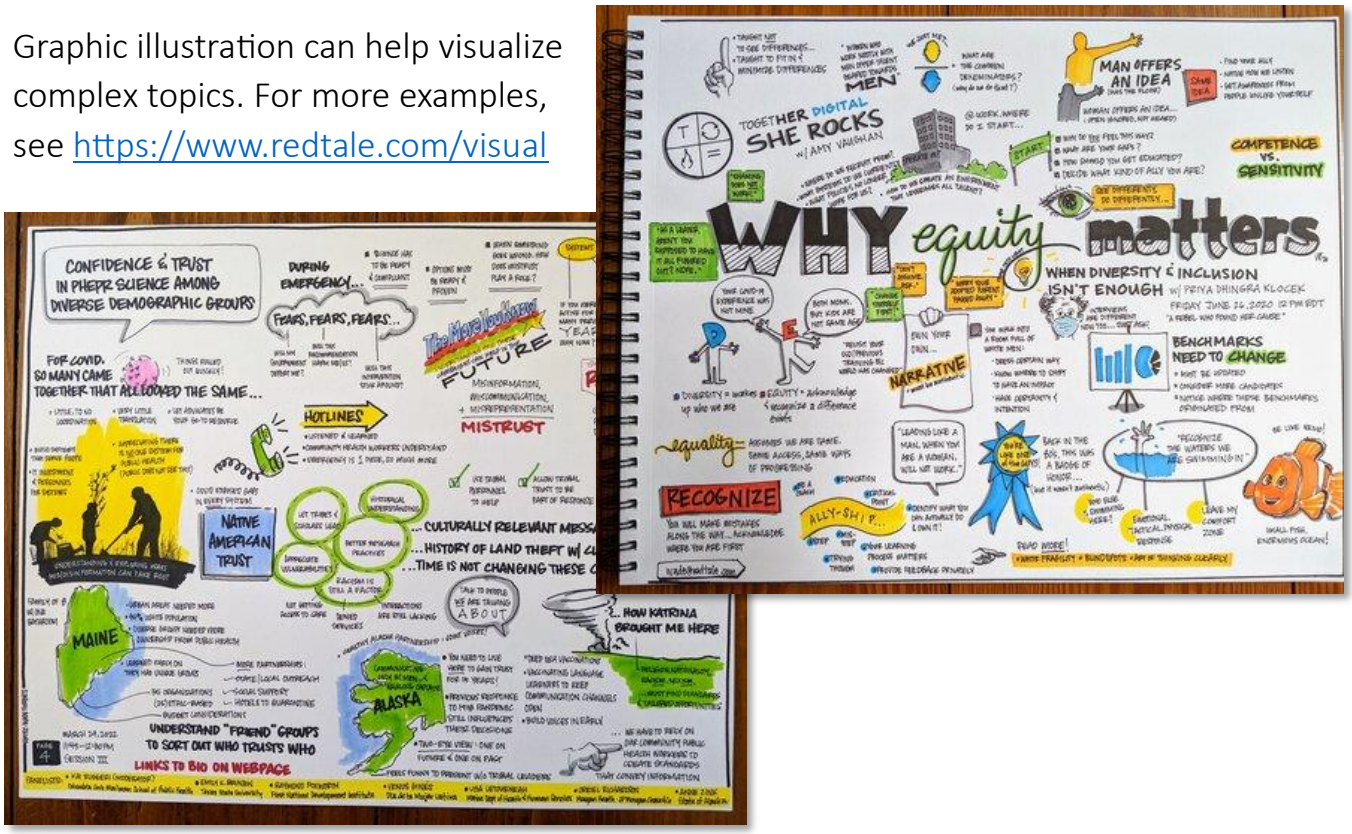
Learn more about Wade Forbes and his work below. Illustrations will be posted to the event webpage as a resource after the event.

WADE FORBES

Co-Founder | Illustrator | Consultant

Wade began illustrating and sketch noting to pursue his true passion of creating art every day. His love of drawing began as a child and was put on hold because he believed he could not make a living as an artist. After spending the last 18 years working in cyber security and consulting with the Department of Defense and the Intelligence community, he's now bringing conversations to life and creating art for his government clients, non-profits, commercial businesses, his church, and many more.

Graphic illustration can help visualize complex topics. For more examples, see https://www.redtale.com/visual



LOGISTICS INFORMATION

Directions to the Keck Center of the Academies
500 Fifth Street, NW, Washington, DC

The Keck Center is located in downtown Washington, DC at 500 Fifth Street, NW, diagonally opposite the Verizon Center and the National Building Museum. It is on the block bounded by Fifth and Sixth Streets and E and F Streets; the only other building on the block is a fire station on the corner of Fifth and F Streets.

Building Entrances, Security, and Directions:

If you are arriving by cab or by Metro, the *pedestrian* entrance is on the Fifth Street (east) side of the building, just past the fire station.

If you are *driving* to the meeting, the *garage* entrance is located on the Sixth Street side, near the north end of the building. Before entering the garage, stop at the security check point. You will be asked the nature of your business and to show ID before entering. If you are planning to drive, please let a staff member know in advance, so your name can be provided to security personnel. Limited guest parking is available on the first level; take the visitors' elevator up to the lobby level and sign-in. Please be aware that parking is allocated on a first come basis and staff cannot reserve spaces for guests.

Arriving by Metro:

Take **Metro's Red Line** to the **Judiciary Square** station. Exit the station by following signs to the Building Museum/Arena/Police Memorial (F St.) exit, between Fourth and Fifth Sts. NW. Turn LEFT and walk WEST on F St. NW. Cross Fifth St. NW and turn LEFT. Walk past the fire station parking lot. The next building on your RIGHT will be 500 Fifth St. NW. (Note: Union Station is on the Red Line).

Take **Metro's Green or Yellow Line** to the **Gallery Place-Chinatown** station. Exit the station by following signs to Seventh and F Streets/Arena. Turn LEFT and walk EAST on F St. NW, two blocks past the Verizon Center. Turn RIGHT on to Fifth St. NW. Walk past the fire station parking lot. The next building on your RIGHT will be 500 Fifth St. NW. [Note: Ronald Reagan Washington National Airport station is on the Yellow Line.)

Additional Parking - IF Keck Center parking lot is full - See map below
Prices are subject to change.

(P-1) Parking Garage on 600 E St NW, Washington, DC - $15.00, 8AM to 6PM.
Laz Parking Mid-Atlantic Inc. - (202) 393-1966.

Visitors can also park on Colonial Parking 6th & Pennsylvania Avenue, N.W., Diplomat 932 F St. & E Streets, N.W. and Carr Park 601 F St.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6th St NW
Rosa Mexicano
Shakespeare Theatre Company
Fuel Pizza and Wings
Denson Liquor Bar
Harman Center for the Arts
Bricklayers & Trowel International Fund
DCFD-Engine 2
Pedestrian Entrance
National Academy of Sciences
Cash Register
The Cop Shop
Venable LLP
Lobby
Crime Lab
Apartment 13 Crime Scene
NASA Federal Credit Union (National...
Marian Koshland Science Museum State-of-the-art...
Institute Of Medicine
District ChopHouse & Brewery
Iron Horse Tap Room
Central Liquor Store
Bakers & Baristas
Legal Counsel for the Elderly
AARP
Bicentennial Building
Keck Center Parking Entrance
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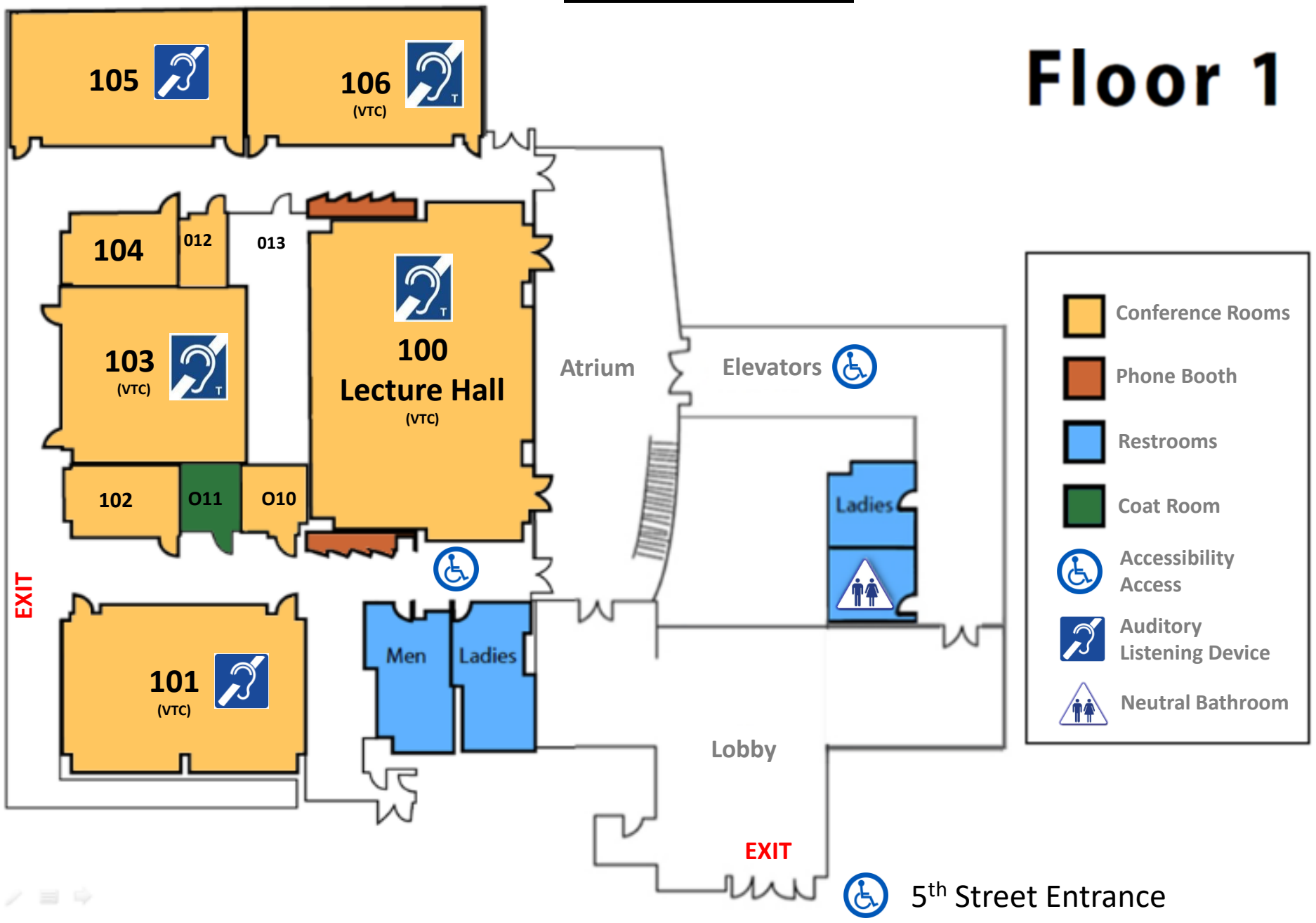
Starbucks
US Justice Department

Keck Building Layout



NATIONAL ACADEMIES Sciences Engineering Medicine

Current Operating Status – National Academies

Effective March 20, 2023

All facilities of the National Academies of Sciences, Engineering, and Medicine are **open**.

Visitors must show a valid government ID (or a digital photo of the card) to the security staff at the Keck Center or the NAS Building, or to the management staff at the Beckman Center, when they enter the facility. View more information about visiting one of our buildings.

COVID-19 Updates and Guidance

As of March 20, 2023, COVID-19 proof of vaccination requirements are no longer in effect. Wearing a mask or respirator is not a requirement, but anyone who chooses to wear one should feel free and comfortable to do so.

For most up to date operating status, click here.

BACKGROUND INFORMATION

Links to Additional Resources

Articles, Papers, and Reports

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Press Releases

ARPA-H announces site selections by launching nationwide health innovation network. 2023. https://arpa-h.gov/news/arpanet-h/

Cellares Raises $255M Series C to Launch First Integrated Development and Manufacturing Organization (IDMO) and Pioneering Smart Factory to Meet Global Demand for Life-Saving Cell Therapies. 2023. https://www.cellares.com/news/cellares-raises-255m-series-c-to-launch-first-integrated-development-and-manufacturing-organization-idmo-and-pioneering-smart-factory-to-meet-global-demand-for-life-saving-cell-therapies/

Cellares and Lyell to Evaluate Automated Manufacturing of Lyell's CAR T-Cell Therapy on Cellares' Cell Shuttle Platform. 2023. https://www.cellares.com/news/cellares-and-lyell-to-evaluate-automated-manufacturing-of-lyells-car-t-cell-therapy-on-cellares-cell-shuttle-platform/

Cellino Named "Scalable Solutions" Winner of Inaugural ARPA-H Dash to Accelerate Health Outcomes. 2023. https://www.businesswire.com/news/home/20230517005356/en

Resilience Approved for $410M Financing from the Department of Defense, in Partnership with the Development Finance Corporation, to Establish Resilient Biomanufacturing Capacity. 2023. https://resilience.com/news/resilience-approved-for-410m-financing-from-the-department-of-defense-in-partnership

Other Links and Resources

Bayer. Cell and Gene Therapy: https://www.bayer.com/en/pharma/cell-and-gene-therapy; https://www.bayer.com/sites/default/files/factsheet-cell-therapy-0.pdf

Bespoke Gene Therapy Consortium: https://fnih.org/our-programs/accelerating-medicines-partnership-amp/bespoke-gene-therapy-consortium-bgtc/

FDA Request for Information on the Development of Cell and Gene Therapies (*comments requested by November 20, 2023I*): https://www.regulations.gov/document/FDA-2023-N-3742-0001

NORD (National Organization for Rare Diseases). Educational materials on gene therapy and gene editing: https://rarediseases.org/nord-expands-gene-therapy-offerings-through-new-online-resources/

NORD (National Organization for Rare Diseases). Patient-focused fact sheet about gene therapy: https://rarediseases.org/wp-content/uploads/2022/03/NRD-2241-Gene-Therapy-Sell-Sheet_FNL.pdf

NORD (National Organization for Rare Diseases). Short educational video answering key questions, based on a patient and caregiver survey, about gene therapy: https://www.youtube.com/watch?v=5ChXI6cSQs0

Forum Publications

Activity/Output	Year	URL/Link
Workshop Proceedings		
Training the Regenerative Medicine Workforce for the Future	2023	https://nap.nationalacademies.org/catalog/27013/training-the-regenerative-medicine-workforce-for-the-future-proceedings-of
Understanding the Role of the Immune System in Improving Tissue Regeneration	2022	https://nap.nationalacademies.org/catalog/26551/understanding-the-role-of-the-immune-system-in-improving-tissue-regeneration
Applying Systems Thinking to Regenerative Medicine	2021	https://www.nap.edu/catalog/26025/applying-systems-thinking-to-regenerative-medicine-proceedings-of-a-workshop
Exploring Novel Clinical Trial Designs for Gene-Based Therapies	2020	https://www.nap.edu/catalog/25712/exploring-novel-clinical-trial-designs-for-gene-based-therapies-proceedings
Exploring Sources of Variability Related to the Clinical Translation of Regenerative Engineering Products	2019	https://www.nap.edu/catalog/25371/exploring-sources-of-variability-related-to-the-clinical-translation-of-regenerative-engineering-products
Navigating the Manufacturing Process and Ensuring the Quality of Regenerative Medicine Therapies	2017	https://www.nap.edu/catalog/24913/navigating-the-manufacturing-process-and-ensuring-the-quality-of-regenerative-medicine-therapies
Exploring the State of the Science in the Field of Regenerative Medicine	2017	https://www.nap.edu/catalog/24671/exploring-the-state-of-the-science-in-the-field-of-regenerative-medicine
Perspectives		
Implementing Systems Thinking and Data Science in the Training of the Regenerative Medicine Workforce	2022	https://www.nature.com/articles/s41536-022-00271-2
Integrating United States Biomanufacturing Infrastructure across Vaccines and Therapeutics	2021	https://nam.edu/integrating-united-states-biomanufacturing-across-vaccines-and-therapeutics/

Reducing Risks and Delays in the Translation of Cell and Gene Therapy Innovations into Regulated Products	2019	https://nam.edu/reducing-risks-and-delays-in-the-translation-of-cell-and-gene-therapy/
Manufacturing Cell Therapies: The Paradigm Shift in Health Care of This Century	2018	https://nam.edu/manufacturing-cell-therapies-the-paradigm-shift-in-health-care-of-this-century/