POLICY SUMMIT

American Society of Gene + Cell Therapy

SEPTEMBER 22-24, 2021
A HYBRID EVENT

JW Marriott Washington, D.C.
1331 Pennsylvania Avenue NW
Washington, D.C. 20004

POLICY SUMMIT
American Society of Gene + Cell Therapy

Wednesday, Sept. 22

Program Chairs

FRANCESCA COOK
Vice President, Market Access, REGENXBIO

ADORA NDU, PHARMD, JD
Group Vice President, Head of WWRD Strategy, Scientific Collaborations and Policy, BioMarin Pharmaceutical

9 – 9:50 A.M.

Moving the Field to the Next Level: Looking to the Future

DAVID BARRETT, JD

Chief Executive Officer
American Society of Gene and Cell Therapy

9 – 9:20 a.m.
Introduction and Overview of the State of the Science: Preclinical and Clinical Pipeline of Molecular Therapeutics

PJ BROOKS, PHD

Working Group Coordinator, Somatic Cell Genome Editing Program; Program Director, Office of Rare Diseases Research, National Center for Advancing Translational Sciences (NCATS)

9:20 – 9:40 a.m.
The Role of Government in the Development of New Gene Editing Tools

Q&A

Moderated by David Barrett, JD, Chief Executive Officer, American Society of Gene and Cell Therapy

9:40 – 9:50 a.m.

POLICY SUMMIT
American Society of Gene + Cell Therapy

Wednesday, Sept. 22

9:50 – 10:10 A.M. **BREAK**

10:10 – 11:35 A.M. **Moving the Field to the Next Level: New Developments in Technology**

JACOB RUBENS, PHD

Chief Scientific Officer, Tessera Therapeutics

10:10 – 10:30 a.m.
RNA-Based Gene Writing: New Approaches to Genome Editing

DEBORA BARTON, MD

Chief Medical Officer, CARISMA Therapeutics

10:30 – 10:50 a.m.
CAR-Macrophages (CAR-M): A Novel Approach to Solid Tumor Immunotherapy

GIUSEPPE CIARAMELLA, PHD

President and Chief Scientific Officer, Beam Therapeutics

10:50 – 11:10 a.m.
Prime and Base Editing: What it Means for the Field

PANEL DISCUSSION

Moderated by Hans-Peter Kiem, MD, PhD, Director, Stem Cell and Gene Therapy Program, Fred Hutchinson Cancer Research Center

11:10 – 11:35 a.m.

POLICY SUMMIT
American Society of Gene + Cell Therapy

Wednesday, Sept. 22

11:35 A.M. – 12:45 P.M. LUNCH

12:45 – 1:15 P.M.

High-Cost, High-Value Therapies: Lessons Learned from Previous Payment Debates

DEMETRIOS KOUZOUKAS, JD

Board Member, Clover Health; Former Director, Center for Medicare, and Principal Deputy Administrator, Centers for Medicare & Medicaid Services (CMS)

Fireside chat moderated by Anna Griffin, Therapeutic Lead for Oncology, Government Affairs + Policy Team, Gilead Sciences

POLICY SUMMIT
American Society of Gene + Cell Therapy

Wednesday, Sept. 22

1:15 – 2:45 P.M.

The Status of Medicaid Coverage and Access for Gene and Cell Therapies

MARK TRUSHEIM

Strategic Director, NEWDIGS, and Visiting Scientist, Sloan School of Management, Massachusetts Institute of Technology

1:15 – 1:35 p.m.

The Medicaid Pipeline: Gene Therapy Impacts & Precision Financing Options

DIANE BERRY, PHD

Senior Vice President, Global Health Policy, Government and Patient Affairs, Sarepta Therapeutics

1:35 – 1:55 p.m.

Advocating for Medicaid Coverage

JOSH TRENT

Principal, Leavitt Partners

1:55 – 2:15 p.m.

The State Innovation Landscape

PANEL DISCUSSION

Moderated by Catherine Kelly, Senior Writer, Pink Sheet, Informa Pharma Intelligence

2:15 – 2:45 p.m.

2:45 – 3:15 P.M.

BREAK

POLICY SUMMIT
American Society of Gene + Cell Therapy

Wednesday, Sept. 22

3:15 – 3:45 P.M.

Policy on the Ground: Medicaid Coverage and Reimbursement

ANNE SCHWARTZ, PHD

Executive Director, Medicaid and CHIP Payment and Access Commission (MACPAC)

3:15 – 3:35 p.m.

MACPAC Recommendations to Congress: Gene and Cell Therapy Payment Systems Financing Options

Q&A

Moderated by Dan Farmer, Vice President of Policy Analysis and Strategy, BGR Group

3:35 – 3:45 p.m.

POLICY SUMMIT
American Society of Gene + Cell Therapy

Thursday, Sept. 23

9 – 10:15 A.M.

Prioritizing Patients in Gene Therapy Development

ERIN WARD

President, MTM-CNM Family Connection

9 – 9:15 a.m.
Centering the Patient Voice

PAT FURLONG

Founding President and Chief Executive Officer, Parent Project Muscular Dystrophy

9:15 – 9:30 a.m.
Patient Opportunities and Challenges in Gene Therapy

BENJAMIN FORRED

Director of Clinical Research, Coordination of Rare Diseases at Sanford (CoRDS) Project, Sanford Research

9:30 – 9:45 a.m.
Patient Registries for Rare Disease

PANEL DISCUSSION

Moderated by Lesha Shah, MD, Assistant Professor of Psychiatry and Medical Director of Child, Adolescent and Family Services, Icahn School of Medicine at Mount Sinai

9:45 – 10:15 a.m.

10:15 – 10:45 A.M.

BREAK

POLICY SUMMIT
American Society of Gene + Cell Therapy

Thursday, Sept. 23

10:45 A.M. – 12:15 P.M. Issues in Diagnostic Testing

PETER BROSS, MD

Acting Chief, Oncology Branch, Office of Tissues and Advanced Therapies (OTAT)

10:45 – 11 a.m.
Current US Regulatory Requirements for Development of Companion Diagnostics for Oncology Therapies

NINA HUNTER, PHD

Vice President, Regulatory Affairs and Science Policy, REGENXBIO

11 – 11:15 a.m.
Developing Companion Diagnostics

JEFF ALLEN, PHD

President and Chief Executive Officer, Friends of Cancer Research

11:15 – 11:30 a.m.
Companion Diagnostics and IVDs: Policy Issues and Potential for Reforms

CHRISTINA HARTMAN

Vice President, External Affairs, The Assistance Fund

11:30 – 11:45 a.m.
Access to Specialized Testing: Medicaid Coverage of Genetic Testing

PANEL DISCUSSION

Moderated by Deepa Chand, MD, Executive Medical Director, Medical Safety, Novartis Gene Therapies; Division of Pediatric Nephrology, St. Louis Children's Hospital

11:45 a.m. – 12:15 p.m.

POLICY SUMMIT
American Society of Gene + Cell Therapy

Thursday, Sept. 23

12:15 – 1:15 P.M. LUNCH

1:15 – 2:45 P.M. AAV Vector Integration

DOUGLAS MCCARTY, PHD

Co-Chair, AAV Integration Roundtable

1:15 – 1:35 p.m.
Readout from the ASCGT AAV Integration Roundtable

ROSA SHERAFAT-KAZEMZADEH, MD

Medical Officer, Office of Tissues and Advanced Therapies (OTAT)

1:35 – 1:55 p.m.
How FDA's Thinking is Evolving: Integration, Risk/Benefit, and Long-Term Follow-Up Considerations

GRAHAM FOSTER, FRCP, PHD

Professor of Hepatology, Queen Mary University of London

1:55 – 2:15 p.m.
Risk and Benefit: Supporting Informed Decision-Making by Clinicians and Patients

PANEL DISCUSSION

Moderated by John Gray, PhD, Senior Vice President, Genetic Therapies Research, Vertex Pharmaceuticals

2:15 – 2:45 p.m.

POLICY SUMMIT
American Society of Gene + Cell Therapy

Thursday, Sept. 23

2:45 – 3 P.M. — **BREAK**

3 – 4:30 P.M. — **Enhancing Gene Therapy Development Through FDA User Fee Support**

CHRIS JONECKIS, PHD

Associate Director for Review Management, Center for Biologics Evaluation and Research (CBER)

3 – 3:20 p.m.
Efforts at CBER to Support Gene Therapy Development

KAREN MIDTHUN, MD

Principal, Drug and Biological Products, Greenleaf Health

3:20 – 3:40 p.m.
Industry Perspective: PDUFA Impacts on Innovative Product Development

CARTIER ESHAM, PHD

Chief Scientific Officer, Biotechnology Innovation Organization (BIO)

3:40 – 4 p.m.
Future PDUFA Proposals: Impact on Gene Therapy Development

PANEL DISCUSSION

Moderated by Scott McGoohan, JD, Senior Director, Policy and Alliance Development, Vertex Pharmaceuticals

4 – 4:30 p.m.

POLICY SUMMIT
American Society of Gene + Cell Therapy

Friday, Sept. 24

9 – 9:30 A.M.

Keynote Address

ANDI LIPSTEIN FRISTEDT

Deputy Commissioner, Policy, Legislation, and Internal Affairs, Food and Drug Administration

9 – 9:20 a.m.

Key Issues in Regulatory Policy

Q&A

Moderated by Remy Brim, PhD, Practice Co-Head, Health and Life Sciences, BGR Group

9:20 – 9:30 a.m.

POLICY SUMMIT
American Society of Gene + Cell Therapy

Friday, Sept. 24

9:30 – 10:20 A.M.

Workforce Development Challenges in Gene Therapy

VIJAY SURAPANENI

Executive Vice President, Global Operations and Supply Chain, Aldevron

9:30 – 9:45 a.m.

Recruiting and Retaining the Manufacturing Workforce

DONIA SLIMANI

Associate Director of Business Development, Cell Therapy, MilliporeSigma

9:45 – 10 a.m.

Attracting and Retaining Diversity in Biotech

PANEL DISCUSSION

Moderated by S. Kaye Spratt, PhD, Chief Regulatory Officer, BridgeBio Gene Therapy

10 – 10:20 a.m.

10:20 – 10:35 A.M.

BREAK

POLICY SUMMIT
American Society of Gene + Cell Therapy

Friday, Sept. 24

10:35 A.M. – 12 P.M.

From Accelerated Approval to Reimbursement

NANCY BRADISH-MYERS, JD

President, Catalyst Healthcare Consulting, Inc.

10:35 – 10:50 a.m.
Accelerated Approval Standards: Application in Cell and Gene Therapy

RENU VAISH

Vice President, Regulatory Affairs, Kite-Gilead

10:50 – 11:05 a.m.
Achieving Success: Advancing a Product Through the Accelerated Approval Process

ANI KHACHATOURIAN, PHARMD

Strategic Business Consultant, Highmark Inc.

11:05 – 11:20 a.m.
A Payer Perspective on Coverage and Reimbursement of Treatments with Accelerated Approval

RICHARD WHITE

Policy Analyst, National Organization for Rare Disorders (NORD)

11:20 – 11:35 a.m.
The Merits of Separating Accelerated Approval from Coverage and Reimbursement

PANEL DISCUSSION

Jocelyn Ulrich, Deputy Vice President, Medical Innovation Policy, Pharmaceutical Research and Manufacturers of America (PhRMA)

11:35 a.m. – 12 p.m.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

JEFF ALLEN, PHD

Jeff Allen, PhD serves as the President and CEO of Friends of Cancer Research (Friends). During the past 20 years, Friends has been instrumental in the creation and implementation of policies ensuring patients receive the best treatments in the fastest and safest way possible. As a thought leader on many issues related to Food and Drug Administration, regulatory strategy and healthcare policy, he is regularly published in prestigious medical journals and policy publications, and has contributed his expertise to the legislative process on multiple occasions. Recent Friends initiatives include the establishment of the Breakthrough Therapies designation and the development of the Lung Cancer Master Protocol, a unique partnership that will accelerate and optimize clinical trial conduct for new drugs. Dr. Allen received his PhD in cell and molecular biology from Georgetown University, and holds a Bachelors of Science in Biology from Bowling Green State University.

DAVID BARRETT, JD

David Barrett serves as Chief Executive Officer for the American Society of Gene and Cell Therapy (ASGCT), the preeminent scientific society representing gene therapy and gene-modified cell therapy and the leading source of information for policy makers, patients, and the public. David has experience in leading and growing non-profit organizations, specifically medical and scientific membership associations. Prior to joining ASGCT, Mr. Barrett was Senior Counsel in the Compliance Department at Career Education Corporation. He earned his BS in political science from the University of Wisconsin Madison before completing his Juris Doctorate at DePaul University.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

DEBORA BARTON, MD

Debora Barton, MD is a medical oncologist and the Chief Medical Officer of Carisma Therapeutics. She brings over 20 years of oncology experience, both in academia as a practicing physician and investigator in clinical trials, and in the biotechnology/pharmaceutical industry supporting the development of new drugs for the treatment of cancer. At Carisma she is leading the clinical development of CT-0508, a HER2 targeted, first of its kind CAR-macrophage, as well as the planning of upcoming pipeline clinical trials including CAR-macrophages against new targets and combinations. Most recently she served in key senior executive positions in adoptive cellular therapy and radiopharmaceutical biotech companies including Iovance Biotherapeutics and Advanced Accelerator Applications (the latter being acquired by Novartis during Debora's tenure). In her leadership roles, Debora built Clinical Development, Medical Affairs and Drug Safety teams setting up the infrastructure for the conduct of registrational clinical studies.

Disclosures: CARISMA Therapeutics, employee

DIANE BERRY, PHD

Diane joined Sarepta Therapeutics in December 2011 and serves as Senior Vice President, Global Policy, Government & Patient Affairs. She engages policymakers at the federal, state, and local levels, as well as patient advocacy organizations, to advance critical policies related to newborn screening, regulatory policy, and reimbursement and access with a goal of expediting development and patient access to genetic-based therapies for rare diseases. Previously, Dr. Berry served in leadership roles for the federal government across the legislative and executive branches, overseeing and implementing science and technology and public health activities. She served as a Subcommittee Staff Director and Senior Professional Staff Member for the U.S. House of Representatives Committee on Homeland Security and as Chief Scientist and Senior Biodefense Advisor at the Department of Homeland Security in the Office of Health Affairs. Dr. Berry was also a Senior Science Advisor at McKenna, Long, and Aldridge and a Science and Technology Policy Advisor and Fellow within the Department of Defense through the American Association for the Advancement of Science.

Disclosures: Sarepta, employee, salary and stock

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

REMY BRIM, PHD

Remy L. Brim, PhD is a scientist and health care policy expert focused on strategic policy and advocacy support for clients with FDA-regulated products and activities. She is a Principal at BGR and on the firm's Executive Board. As co-head of BGR's Health Care Practice, she helps clients effectively navigate the complex regulatory and political landscapes required to advance the discovery, development, and delivery of innovative products to patients and consumers. Prior to joining BGR Group, Remy served as Senior FDA Policy Advisor to the Senate HELP Committee's Ranking Member, Patty Murray (D-Wash.). In this role, she was lead negotiator and advisor for U.S. Senate Democrats on FDA medical device, prescription drug, biologic, food safety and cosmetic policy initiatives, including the 21st Century Cures Act and the FDA Reauthorization Act of 2017. She managed FDA-related policy development and relationships for the Ranking Member, both internally with Senate leadership, other Senate offices, and their House counterparts, and externally with the FDA and other governmental organizations, regulated industry companies and trade associations, patient advocacy organizations and other key stakeholders.

PJ BROOKS, PHD

Philip John (PJ) Brooks joined the NCATS Office of Rare Diseases Research as a program director in August 2018. Prior to that time, he was in the NCATS Division of Clinical Innovation, where he was the lead program director for the Clinical and Translational Science Awards (CTSA) Program Collaborative Innovation Awards, designed to fund projects that will result in novel and creative approaches to overcoming roadblocks in translational science. Brooks represents NCATS on the Trans-NIH Gene Therapy Working Group and the Regenerative Medicine Innovation Project, and he also is the Working Group Coordinator for the NIH Common Fund program on Somatic Cell Genome Editing. Brooks recently was elected as the chair of the Interdisciplinary Scientific Committee of the International Rare Diseases Research Consortium. Brooks received his doctorate in neurobiology from the University of North Carolina at Chapel Hill. After completing a postdoctoral fellowship at the Rockefeller University, Brooks became an investigator in the intramural program of the National Institute on Alcohol Abuse and Alcoholism.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

PETER BROSS, MD

Peter Bross is Chief of Oncology Branch and clinical team leader in the FDA Center for Biological Evaluation and Research (CBER) Office of Tissue and Advanced Therapies (OTAT) and previously worked as a clinical reviewer in the Division of Oncology Drug Products in the Center for Drug Evaluation and Research (CDER). Over 20 years at FDA, Dr. Bross has gained expertise in the design and analysis of clinical oncology trials of cellular, tissue and gene therapies, especially cancer vaccines, combination therapies, and companion diagnostics. As a regulatory reviewer, he has reviewed new molecular entities for marketing approvals in solid tumors and hematological malignancies, including oncolytic viruses, cellular immunotherapies, targeted kinase inhibitors, proteasome inhibitors and an antibody-drug conjugate. He has presented FDA perspectives at professional meetings and review findings at FDA advisory committee meetings and has authored several manuscripts. Dr. Bross is a graduate of University of Virginia Medical School and trained in Hematology and Oncology at The George Washington University and has been at FDA since 1999.

DEEPA CHAND, MD

Deepa has over 20 years of experience as a subspecialty Pediatric physician: Dual Board Certification (Pediatric Nephrology and General Pediatrics) and over 5 years of early and late stage development and safety experience in the pharmaceutical industry. Deepa is the safety lead for the gene therapy program at Novartis and has been intimately involved with various aspects of the program including registration and definition of the safety profile for systemic gene therapy.

Disclosures: Employee and shareholder at Novartis

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

GIUSEPPE CIARAMELLA, PHD

Dr. Giuseppe Ciaramella is the President and Chief Scientific Officer of Beam Therapeutics. Dr. Ciaramella has more than 20 years of drug discovery experience at Moderna, AstraZeneca, Boehringer Ingelheim, Pfizer and Merck. Throughout his career he has held several leadership roles, with a particular focus in the fields of antivirals, immunology and biotherapeutics. Prior to joining Beam Therapeutics, Dr. Ciaramella was the CSO of the Infectious Diseases division of Moderna Therapeutics, where he was instrumental in generating some of the first mRNA vaccines to be dosed in humans, several of which are progressing through clinical studies. He has contributed to several clinical candidates, both small molecule and biologics and to the anti-HIV drug Maraviroc (Selzentry TM), which won the USA Prix Galien for Best Pharmaceutical in 2008. He is a member of the Infectious Diseases Society of America (IDSA) and of the American Society of Gene and Cell Therapy (ASGCT). Dr. Ciaramella holds a PhD in Biochemistry and Molecular Biology from University College London.

Disclosures: Employee and shareholder of Beam Therapeutics

E. CARTIER ESHAM, PHD

Cartier Esham has over 20 years of policy and advocacy experience and serves as the Chief Scientific Officer at the Biotechnology Innovation Organization (BIO). In this role, Dr. Esham manages and directs BIO's policy development, research and educational initiatives on emerging science, regulatory issues, industry analysis and emerging companies issues. Prior to joining BIO, Dr. Esham was a Vice President and Director of Research at Dutko Worldwide, a private consulting firm in Washington D.C., where she worked on a variety of environmental, education, science, technology, and health carerelated issues on the federal, state, and local levels. Dr. Esham has a PhD in Microbiology from the University of Georgia, a master's degree in Marine Biology from the University of North Carolina at Wilmington and a bachelor's degree from the University of Kentucky.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

DAN FARMER

Dan Farmer, Vice President of Policy Analysis and Strategy within the Health and Life Sciences Practice at BGR Government Affairs, is a veteran of payment policy work in both the public and private sector. He has experience navigating the federal regulatory process, as well as moving legislation through the Hill. Dan comes to the firm from Cleveland Clinic, one of the nation's premier healthcare systems, where he led federal advocacy efforts on the Hill and in the Administration. Dan's career in health policy began on Capitol Hill, where he worked in the U.S. House of Representatives. He advised Rep. Zack Space (D-OH), a Member of the House Energy and Commerce Committee, through the drafting and passage of the Affordable Care Act. Dan received his undergraduate degree from Carleton College in Minnesota. He and his wife have three young daughters.

BENJAMIN FORRED

Ben has worked in the field of biomedical research since 2009. He spent nearly a decade working in a research laboratory with a background in cell and molecular biology. He also holds an MBA from the University of South Dakota. Currently, Ben serves as a director of clinical research at Sanford Research and is responsible for the project management and study start up teams. He is also the director of the Experimental Therapeutics Screening Facility at Sanford Research, where they seek to bridge academic research relationships with biotech and pharma groups to develop treatments for rare conditions. Lastly, as Director of the Coordination of Rare Diseases at Sanford (CoRDS) Registry, Ben helps facilitate new research relationships between non-profit advocacy organizations, researchers, pharma, and the CoRDS team.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

GRAHAM FOSTER, FRCP, PHD

Professor Foster is the Professor of Hepatology at Queen Mary University of London and a consultant at Barts Health Trust. He trained in Medicine at Oxford and London Universities in the 1980s and completed a PhD in Molecular Biology in 1992. Professor Foster has a long-standing interest in the management of chronic viral hepatitis and runs a clinical research program studying the natural history of viral hepatitis, its impact upon patients and their communities and novel therapies for this disease. He supervises a laboratory research program investigating the basic virology of hepatotropic viruses' novel replication models for hepatitis C. He is the editor of The Journal of Viral Hepatitis and has published widely in the field of viral liver disease. He is a past President of BASL, NHSE Clinical Lead for Hepatitis C, Chairman the NHSE Hepatolbiliary Specialised Commissioning Clinical Reference Group

Disclosures: Consulting and speaker fees from Abbvie, Biomarin, GSK, Gilead, UniQure, MSD

ANDI LIPSTEIN FRISTEDT

Andi Lipstein Fristedt is the Deputy Commissioner for Policy, Legislation, and International Affairs. In this role, she provides strategic policy direction to advance the FDA's mission and vision of protecting and promoting public health and oversees the agency's engagement with Congress and global partners. Ms. Fristedt worked for nearly a decade in various capacities with the U.S. Senate Committee on Health, Education, Labor, and Pensions (HELP), first as a senior advisor to Chairman Tom Harkin and later to Ranking Member and Chair Patty Murray. In addition to serving as the Senate's top Democratic public health staffer beginning in 2012, she was the HELP Committee's Deputy Health Policy Director from 2017 until she joined the FDA in 2021. Ms. Fristedt spearheaded the Senate Democratic response to COVID-19, developing extensive policy proposals with respect to COVID-19 therapeutics, diagnostics, and vaccines. During her time in the Senate, Ms. Fristedt also led the drafting or negotiation of a wide range of public health legislation that was signed into law, including two reauthorizations of the Pandemic and All-Hazards Preparedness Act, legislation increasing the legal age of tobacco sale to 21, and key provisions of the 21st Century Cures Act, the Lower Health Care Costs Act, and the SUPPORT for Patients Act.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

PAT FURLONG

When doctors diagnosed her two sons, Christopher and Patrick, with Duchenne in 1984, Pat didn't accept "there's no hope and little help" as an answer. She immersed herself in Duchenne, working to understand the pathology of the disorder, the extent of research investment and the mechanisms for optimal care. Her sons lost their battle with Duchenne in their teenage years, but she continues to fight—in their honor and for all families affected by Duchenne. In 1994, she, together with other parents of young men with Duchenne, founded Parent Project Muscular Dystrophy to change the course of Duchenne and, ultimately, to find a cure. Today, she continues to lead the organization and is considered one of the foremost authorities on Duchenne in the world. Along with leading PPMD, she speaks regularly about Duchenne and related topics at both national and international conferences. Pat has served on the Board of Genetic Alliance, the Muscular Dystrophy Coordinating Committee and the U.S. Department of Health & Human Services.

JOHN GRAY, PHD

Dr. John Gray, PhD is currently Senior Vice President of Genetic Therapies Process Research at Vertex Cell & Genetic Therapies. Prior to this role, Dr. Gray served as Senior Vice President and Chief Scientific Officer at Audentes Therapeutics, as well as playing key roles at St. Jude Children's Hospital, Harvard Medical School, and Pfizer. Dr. Gray obtained his Bachelors in Biochemistry at UC Berkeley before completing his PhD in Chemistry and Biochemistry at University of Colorado Boulder.

Disclosures: Holds equity in Vertex Pharmaceuticals.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

ANNA GRIFFIN

Anna Griffin is currently the Therapeutic Lead for Oncology for Gilead's Government Affairs + Policy team. Prior to her current role, Anna was the Head of Government Affairs and Policy for Kite, which was acquired by Gilead in 2017 and currently has two FDA-approved CAR T therapies on the market. In both positions, Anna has worked to develop and implement strategies to ensure patient access to innovative therapies, like CAR T. She engages key contacts at both the federal and state level to educate policy makers on the benefits of these transformative therapies and advocates for policies to improve patients' access to care. As with too many of us, Anna has been personally touched by cancer and is motivated to improve the paradigm of cancer treatment by the faces of real cancer patients she knows and loves. She has worked in the biopharmaceutical industry for more than 15 years and began her career in Washington DC as a Hill staffer for two former Members of Congress from North Carolina, her hometown state. Anna is a graduate of the University of North Carolina – Chapel Hill.

CHRISTINA HARTMAN

As Vice President, External Affairs, Christina leads The Assistance Fund's Washington, DC, office and its efforts to pave the way for patient-focused policy and advocacy to lower patients' out-of-pocket costs and ensure their access to critical treatment. Christina came to TAF from the EveryLife Foundation, where she led policy and advocacy efforts to advance treatment and diagnostic opportunities for rare disease patients. At the American College of Cardiology she worked with staff and member leadership to improve cardiovascular health outcomes. At the Pew Charitable Trusts Christina worked with a range of partners to advance legislative goals that incentivize the development of new antibiotic drugs. As an analyst at the Centers for Disease Control and Prevention and in the Office of the Secretary for the U.S. Department of Health and Human Services (HHS), she served as Project Officer for a cooperative agreement between HHS and the World Health Organization. Christina's engagement in the rare disease space is a direct result of her own experience with her youngest daughter Charlotte, who has a rare genetic disorder. She has a bachelor's degree from The Catholic University of America and a Master of Public Health from The George Washington University.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

NINA HUNTER, PHD

Nina Hunter joined REGENXBIO in May 2020 as Vice President, Regulatory Affairs and Science Policy. In this role Nina leads regulatory policy and collaborates with internal and external stakeholders to develop and execute strategies for development of AAV gene therapy products. Prior to REGENXBIO, she was Director of Office of Clinical Policy and Programs at the Food and Drug Administration where she provided leadership across programs including combination products, orphan products, pediatric therapeutics, and patient engagement. Prior to this role she worked on molecular-based assays, including companion diagnostics in the Center for Devices and Radiologic Health. Nina holds a BA from Bowdoin College and a PhD in Genetics from Harvard Medical School.

CHRIS JONECKIS, PHD

Chris Joneckis currently serves as the Associate Director for Review Management in the Center for Biologics Evaluation and Research (CBER) at FDA. In this capacity he Is the Center's authoritative expert on review management, directing the review management staff and providing leadership for review program activities for biologics, devices and combination products executed throughout the offices of CBER. He is responsible for the development, implementation and oversight of several CBER programs including policies, procedures and standards for review, data standards, information technology, regulatory affairs, document control, regulatory database and regulatory business operations and Chemistry Manufacturing and Controls policy. He is the CBER lead for user fee negotiations. He is involved in numerous Agency groups drafting policy and legislation in multiple areas. Before his current position, Dr. Joneckis served as the Senior Advisor for Chemistry, Manufacturing and Control (CMC) Issues. Dr. Joneckis holds a PhD in Pharmacology from the University of North Carolina at Chapel Hill. He joined CBER in 1994 as a regulatory and CMC reviewer in the former Office of Therapeutics Research and Review (OTRR).

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

CATHY KELLY

Cathy Kelly has been covering reimbursement and market access news for more than a decade for "The Pink Sheet" and other publications that are now part of Informa's pharmaceutical intelligence resources. Her expertise spans legislative, regulatory and public policy topics as well as coverage trends in public and private insurance markets. Cathy is especially interested in following the ongoing transformation of the US health care system and its effect upon major stakeholders in the biopharma industry. She is based in Washington D.C.

ANI KHACHATOURIAN, PHARMD

Ani Khachatourian joined Highmark Blue Cross Blue Shield in 2014 as a Managed Care Pharmacy Resident, later transitioning to a Senior Pharmacist role, and currently serves as a Strategic Business Consultant in Specialty Pharmacy. Throughout her time at Highmark she has served on various interdisciplinary teams, focused on providing holistic solutions that ensure safe, appropriate, and cost-effective use of specialty drugs at every stage of the patient's journey. In addition to her role on the Specialty Pharmacy team, Ani also serves on the Clinical Policy Management Committee in providing oversight of medical policy for Highmark Health, led by evidence-based methodologies and accepted practice standards within the medical community. Ani received her BS in Human Biology, with a concentration in Health Care and Social Issues from the University of California, San Diego, before receiving her Doctor of Pharmacy degree from Western University of Health Sciences.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

HANS-PETER KIEM, MD, PHD

Dr. Hans-Peter Kiem is a world-renowned pioneer in stem-cell and gene therapy and in the development of new gene-editing technologies. His focus has been the development of improved treatment and curative approaches for patients with genetic and infectious diseases or cancer. For gene editing, his lab works on the design and selection of enzymes, known as nucleases, which include CRISPR/Cas. By combining gene therapy's ability to repair problem-causing genes and stem cells' regenerative capabilities, he hopes to achieve cures of diseases as diverse as HIV, leukemia and brain cancer. With preclinical models of HIV, Dr. Kiem and his colleagues have demonstrated that they can modify a key viral entry gene and prevent it from working in transplanted blood stem cells. He also hopes to apply these technologies to cure genetic blood disorders such as Fanconi anemia and sickle cell disease. He is also pioneering in vivo gene therapy approaches to make gene therapy and gene editing more broadly available and accessible to patients and those living with HIV, especially in resource-limited settings.

DEMETRIOS KOUZOUKAS, JD

Demetrios L. Kouzoukas brings a unique perspective afforded by his deep experience, spanning business, law and government, in every health care sector. He is currently a member of the Board of Directors for Clover Health and serves as a member of the American Medical Association's Digital Medicine Payment Advisory Group. At different times, Mr. Kouzoukas has served as Chief Executive of the Medicare program at the Centers for Medicare & Medicaid Services, Principal Associate Deputy Secretary of the U.S. Department of Health and Human Services, as HHS Deputy General Counsel, and as a Public Member of the Administrative Conference of the United States. Mr. Kouzoukas was also a senior executive in the Medicare business at United Healthcare. He has also built innovative practices at two leading health care law firms.

Disclosures: Director, Clover Health

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

DOUGLAS MCCARTY, PHD

Dr. Douglas McCarty has more than 30 years of experience in AAV research encompassing the biology of the parent virus, vector design, and vector delivery. Most recently he worked at Pfizer as Senior Director of Vector Design, Biomedical Research & Design. Prior to Pfizer he was Associate Professor at The Research Institute at Nationwide Children's Hospital and in the Department of Pediatrics at The Ohio State University. He has been a member of the American Society of Gene & Cell Therapy since 1999 and has served on a number of NIH peer review committees in the gene therapy space. His major research interests have been in the development and application of recombinant adeno-associated virus (rAAV) gene therapy vectors. Dr. McCarty completed his PhD in Immunology and Medical Microbiology at the University of Florida, Gainesville and his post-doctoral study on AAV Virology at SUNY Stony Brook.

Disclosures: Licensing fees, employee/co-inventor, Nationwide Children's Hospital; ownership interest, co-inventor, Askbio.

SCOTT MCGOOHAN, JD

Scott V. McGoohan, JD currently serves as Senior Director, Policy and Alliance Development for Vertex Pharmaceuticals and is responsible for gene editing and gene therapy policy. Prior to joining Vertex Scott served as Director of Scientific and Regulatory Affairs at the Biotechnology Innovation Organization, where he led a broad array of activities related to the innovative biopharmaceutical industry, and served as an industry negotiator on the FDA User Fee Agreement reauthorizations. He previously held roles with the American Clinical Laboratory Association, Harvard Medical School, and Genzyme, and worked as a contract lobbyist and scientific advisor, representing pharmaceutical and device companies and their interests on Capitol Hill. Scott received his Bachelors of Science from Boston College, specializing in molecular biology, and his Juris Doctorate from the University of Wisconsin Law School.

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American Society of Gene + Cell Therapy

SPEAKERS

KAREN MIDTHUN, MD

Dr. Karen Midthun serves as a Principal, Drug and Biological Products at Greenleaf Health, where she provides specialized insight to the strategic and technical guidance that Greenleaf provides to FDA-regulated entities. Prior to joining Greenleaf she had a long career in public service, mostly dedicated to FDA's Center for Biologics Evaluation and Research (CBER), where she served as Director before her retirement. During her FDA tenure, she played a critical role in facilitating policy and technology development in the areas of blood products, vaccines, and cell, tissue, and gene therapies. Prior to her role as Center Director, she served as the Deputy Director of CBER and the Director of the Office of Vaccines Research and Review within CBER. Before joining the FDA in 1993, Dr. Midthun was on the faculty of the Department of International Health at the Johns Hopkins Bloomberg School of Public Health, where she was involved in the clinical development of investigational vaccines and was an attending physician at the Johns Hopkins Hospital.

Disclosures: Employee; salary, provides consulting services to a variety of entities producing health care goods and services; Greenleaf Health

NANCY BRADISH MYERS, JD

Nancy Bradish Myers, JD, CEO and Founder of Catalyst Healthcare Consulting, Inc., is a Washington-based attorney with deep expertise in health care law and regulation, policy development and government relations. Ms. Myers advises clients on regulatory and health policy matters, ranging from crisis management to long-term strategy development. Her leadership positions have spanned the federal government, drug and biotechnology industry associations, health insurance and the equity research/investment world. At FDA she served in a number of roles, most recently as special assistant and senior strategic advisor in FDA's Office of the Commissioner. Within the private sector, she served as special counsel for science policy for the Pharmaceutical Research and Manufacturers of America (PhRMA), vice presidential-level political healthcare analyst for Lehman Brothers, reimbursement counsel and director of government affairs for the Biotechnology Industry Organization (BIO), senior lobbyist for the Blue Cross Blue Shield Association/BCBS of NJ, and a stint on Capitol Hill.

Disclosures: Regulatory consultant for several biopharma and medical device companies.

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American Society of Gene + Cell Therapy

SPEAKERS

JACOB RUBENS, PHD

Jacob (Jake) Rubens is Co-Founder and Chief Scientific Officer of Tessera Therapeutics, as well as a Principal at Flagship Pioneering. Jake joined Flagship Pioneering in 2015 and works as part of a venture-creation team, founding and growing companies based on new biotechnology. At Flagship, Jake launched Kaleido Biosciences and co-founded Sana Biotechnology. Jake was the Head of Innovation at Cobalt Biomedicine, where he invented and developed the company's Fusosome platform prior to its merger with Sana Biotechnology. Before joining Flagship, Jake received his PhD in microbiology from MIT, working in the Synthetic Biology Center with Professor Tim Lu with the support of a National Science Foundation Graduate Research Fellowship. At MIT Jacob invented gene circuits that allow engineered cells to do novel analog, digital, and hybrid computations, enabling the emerging field of "intelligent" cell therapies. Jake's work has resulted in multiple pending patents and publications, including articles in Nature and Nature Communications. Jake was honored in 2017 in Forbes' 30 under 30 list in science.

Disclosures: Tessera Therapeutics, Sana Biotechnology, Flagship Pioneering

ANNE SCHWARTZ, PHD

Anne Schwartz is executive director of the Medicaid and CHIP Payment and Access Commission (MACPAC), a non-partisan legislative branch agency that provides policy and data analysis and makes recommendations to Congress, the Secretary of the U.S. Departmentof Health and Human Services, and the states on a wide array of issues affecting Medicaid and the State Children's Health Insurance Program (CHIP). She has served in this position since 2013. Dr. Schwartz previously served as deputy editor of the journal Health Affairs; vice president at Grantmakers In Health, a national organization providing strategic advice and educational programs for foundations and corporate giving programs working on health issues; and special assistant to the executive director and senior analyst at the Physician Payment Review Commission, a precursor to the Medicare Payment Advisory Commission. Earlier, she held positions on committee and personal staff for the U.S. House of Representatives. Dr. Schwartz holds a doctorate in health policy from the School of Hygiene and Public Health at The Johns Hopkins University.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

LESHA SHAH, MD

Lesha D. Shah, MD, is Assistant Professor of Psychiatry and a member of the Institutional Review Board at the Icahn School of Medicine. Dr. Shah's academic focus is research ethics and physician perspectives around medical decision-making for children and adolescents. She studies issues of consent and capacity as they interface with family complexity and innovative medicine including clinical trial participating and access to investigational agents. As Co-Chair of the Pediatric Gene Therapy and Medical Ethics working group and member of the ASGCT Ethics committee, she is committed to addressing emerging ethical issues surrounding gene therapy in pediatric populations and identifying nascent best practices for patients and families. Dr. Shah has published on pediatric medical ethics in the Journal of the American Academy of Child and Adolescent Psychiatry, Pediatrics, and the American Journal of Bioethics.

ROSA SHERAFAT-KAZEMZADEH, MD

Dr. Rosa Sherafat is a board-certified pediatric endocrinologist and has been a medical officer in the General Medicine Branch II, in the Division of Clinical Evaluation and Pharmacology/Toxicology (DCEPT), Office of Tissue and Advanced Therapies (OTAT), in the Center for Biologics Evaluation and Research at the US FDA, since 2018. Dr. Sherafat received her medical degree from Tehran University of Medical Sciences, Tehran, Iran. She completed residency in pediatrics at University of Illinois Medical Center, Chicago, IL and fellowship in pediatric endocrinology in Cincinnati Children's Hospital, Cincinnati, OH. Prior to joining the FDA, Dr. Sherafat was an associate professor of pediatrics in the Department of Pediatrics, Medstar Georgetown University Hospital, Washington, DC (2007-2018). She is a member of CBER pediatric working group and has participated in the revision of the FDA Guidance for Industry, Long Term Follow Up after Administration of Human Gene Therapy Products (published January 2020) and a member of the planning committee for the FDA Cellular, Tissue and Gene Therapies Advisory Committee (CTGTAC) Meeting, Toxicity Risks of AAV Vectors for Gene Therapy, (September 2021).

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

DONIA SLIMANI

Donia Slimani is responsible for business development in the Eastern region of North America within MilliporeSigma's Cell Therapy Manufacturing franchise. She has previously held multiple roles across three continents focused primarily on process development, scalability, and manufacturing readiness for monoclonal antibodies and viral vector therapeutics. Donia received her MSc in Engineering, Systems and Microsystems for Physics & Biotechnology from the Grenoble Institute of Technology in France. She is also leading the community outreach initiative as part of the DE&I effort of the company.

S. KAYE SPRATT, PHD

Kaye Spratt, PhD joined BridgeBio Gene Therapy in 2019 as Senior Vice President, Regulatory Affairs, bringing 30 years of experience in the pharmaceutical industry, including over 5 years specializing in Regulatory Affairs. Kaye currently serves in the role of Chief Regulatory Officer for BridgeBio Gene Therapy. Her regulatory efforts have focused primarily on the clinical development of rare pediatric and neurodegenerative disease. Prior to joining BridgeBio Gene Therapy she served as Senior Vice President of Regulatory Affairs at the clinical-stage gene therapy company Abeona Therapeutics, Inc where she secured US and Ex-US orphan designations, FastTrack, RMAT and Breakthrough designations for clinical stage products and secured Scientific Advice from local and national regulatory agencies. Earlier in her career, Dr. Spratt served as Nonclinical Director/Assay Development and Quality Control Director, with increasing responsibilities for over 17 years at Sangamo Therapeutics. At Sangamo she was a major contributor to early discovery and development for several IND candidates in multiple therapeutic areas. Dr. Spratt has a BS degree in Biology from Langston University and a PhD in Microbiology/Molecular Biology from Meharry Medical College.

Disclosures: Employee, BridgeBio Gene Therapy

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

VIJAY SURAPANENI

Vijay brings over 30 years of global leadership experience in biopharmaceutical and device operations, technology, and engineering. Vijay is currently Aldevron's EVP of Global Operations and Supply Chain across both Fargo, North Dakota and Madison, Wisconsin locations with responsibility for Manufacturing, Technical Operations, Facilities/EHS/Validation, Engineering, Supply Chain and Operational Excellence. Prior to joining Aldevron in 2019, Surapaneni spent six years at Catalent Pharma Solutions, with his most recent role as Vice President of Engineering and Capacity Planning where he managed and delivered significant capital projects across the global network. Surapaneni previously served as Vice President of Operations for Biologics, Specialty and Oral Drug Delivery sites in US and Europe and was instrumental in expanding the service offerings and delivering growth. Surapaneni led the Operations due diligence activities for the purchase of Cook Pharmica, a biologics CDMO and Pharmatek, an oral drug development CDMO and integrated both companies into Catalent.

JOSH TRENT

Josh Trent is a principal based in Leavitt Partners' Washington, D.C. office where he develops and manages multi-sector alliances and advises clients throughout the health care sector on federal health care policy and strategy. Previously, Josh served as Chief Health Counsel for the Committee on Energy and Commerce of the U.S. House of Representatives. He previously led the House's work on Medicaid/CHIP and 340B, and led the Committee's work on Medicare Advantage. Josh was the lead House negotiator in The SUPPORT Act (P.L. 115-271), and led the House's reauthorization of The Pandemic and All-Hazards Preparedness Act (eventually P.L. 116-22). He crafted key Medicaid, Medicare, and CHIP policies in the Bipartisan Budget Act (P.L. 115-123), and led the House's work to enact a historic extension of the CHIP (P.L. 115-120). He also assisted in the development of the Comprehensive Addiction and Recovery Act (P.L. 114-198) and The 21st Century Cures Act (P.L.114-255). Prior to working in the House, Josh served for five years in the Senate. Before coming to Capitol Hill, Josh spent six years in the George W. Bush Administration, including three years at the White House and a year and a half at HHS.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

MARK TRUSHEIM

Mark Trusheim is Strategic Director, MIT NEWDIGS where he also co-leads the Financing and reimbursement of Cures in the US (FoCUS) Project; and a Visiting Scientist at the MIT Sloan School of Management. Through MIT he has also served as a Special Government Employee for the FDA's Office of the Commissioner. Mark's research focuses on the economics of biomedical innovation, especially precision financing for patient access, precision medicine, adaptive pathways, platform trials, biosimilars, and digital health advances. Mark is also President of Co-Bio Consulting, LLC. Prior to MIT, his career spanned big data at Kenan Systems, marketing at Searle Pharmaceuticals, eHealth as Vice President of Monsanto Health Solutions, genomics as President of Cereon Genomics, and policy as the President of the Massachusetts Biotechnology Council. He holds degrees in Chemistry from Stanford University and Management from MIT.

Disclosures: President & owner, Co-Bio Consulting, LLC

JOCELYN ULRICH

Jocelyn Ulrich, MPH, is Deputy Vice President, Medical Innovation Policy in the Policy, Research and Membership Department at the Pharmaceutical Research and Manufacturers of America (PhRMA). At PhRMA she is responsible for developing legislative and policy analysis and research studies on a range of issues impacting innovative biopharmaceutical companies, including intellectual property issues, FDA policy issues, the R&D process, the value of innovation, and other issue areas impacting the environment for innovation. In addition to her experience at PhRMA she has over 20 years of experience in the pharmaceutical industry at Pfizer, Human Genome Sciences, and EMD Serono in roles in clinical research management, investigator-initiated and collaborative research, and global policy and corporate affairs. Jocelyn holds an MPH in global health policy and management from New York University.

POLICY SUMMIT
American Society of Gene + Cell Therapy

SPEAKERS

RENU VAISH

Renu Vaish currently serves as VP of Global Regulatory Affairs and brings nearly 20 years of pharmaceutical and biotechnology experience in regulatory affairs to Kite Pharma Inc. Ms. Vaish has held multiple positions of increasing responsibility at Sanofi-Aventis, Centocor, Genzyme, Millennium Pharmaceuticals, Amgen and Celgene. She is an experienced regulatory professional with diverse experiences in small molecules in oncology and other therapeutic areas, and has a track record of product approvals with global health authorities.

ERIN WARD

Erin is Co-Founder and President of MTM-CNM Family Connection, a non-profit dedicated to connecting individuals and families from the Myotubular and Centronuclear Myopathy community to research, resources, and life-enhancing relationships. Erin serves as Director of biennial, national, patient-professional collaborative conferences for the MTM-CNM community. Erin has also helped to lead the MTM-CNM community's recent patient engagement efforts with the FDA, including both a Patient Listening Session and Externally-Led Patient-Focused Drug Development Meeting. Holding a master's degree in Education and a Certificate of Advanced Study in Counseling, Erin combines personal experiences as a mother to a young adult son living with Myotubular Myopathy with her professional skills to work towards improving patient-professional partnerships across both clinical and therapeutic drug development systems. Erin has served as Associate Faculty for the Institute for Professionalism and Ethical Practice at Boston Children's Hospital and Harvard Medical School for 15 years.

RICHARD WHITE

Mr. Richard White is a Policy Analyst at the National Organization for Rare Disorders (NORD) in Washington DC where he handles a life sciences portfolio with a regulatory focus in order to further NORD's mission of bringing treatments to those with Rare Diseases. Richard also advocates for NORD's policies on Capitol Hill as well as at various federal agencies. Prior, he served as Manager of Science and Regulatory Affairs at Biotechnology Innovation Organization until 2020. Mr. White holds a Bachelor's degree in Political Science from Loyola University Maryland.