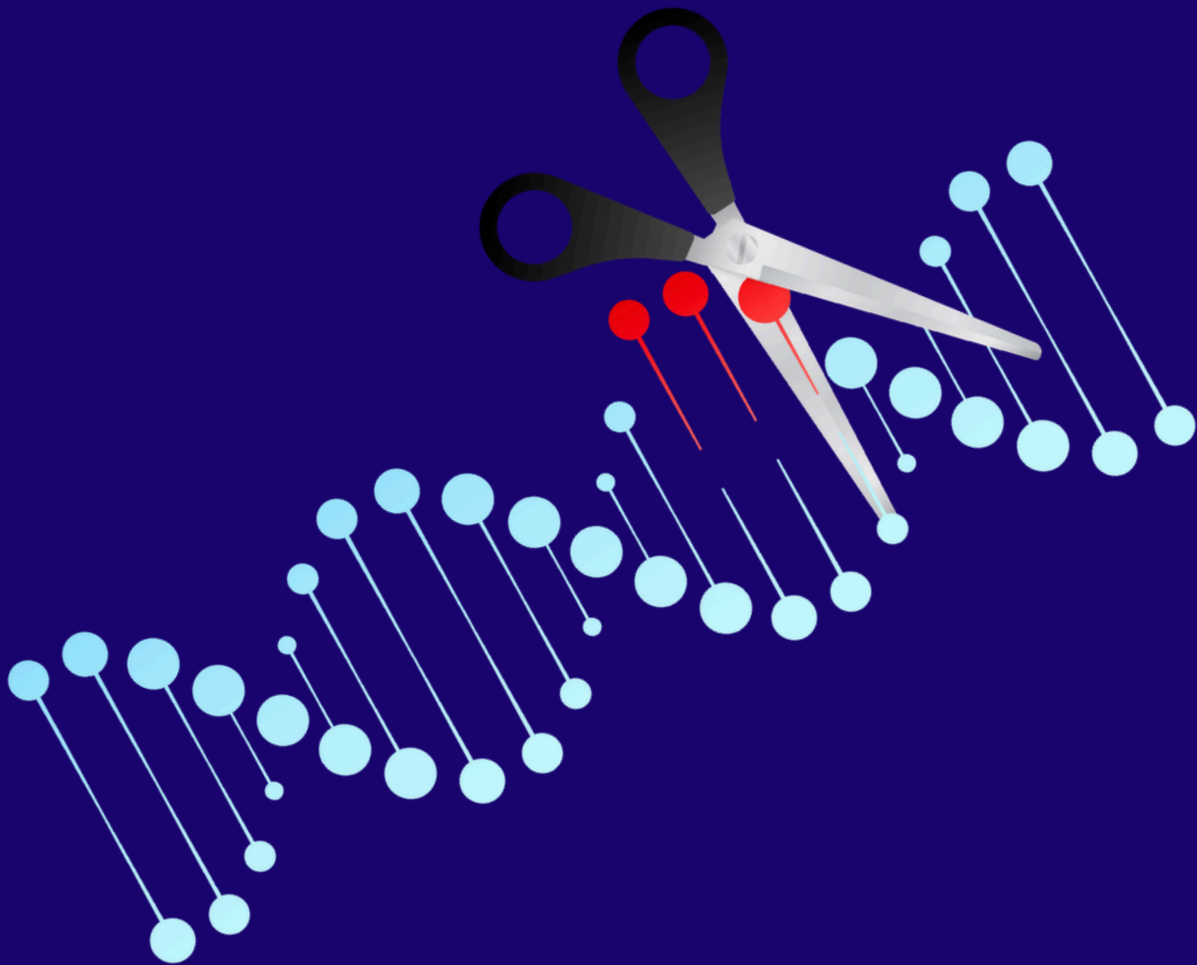


Rethinking Clinical Delivery, Regulation, and Payment Models for Individualized Platform-Based Genome Editing Therapies

September 29th, 2026



Welcome from the Organizers

On behalf of the NIH Somatic Cell Genome Editing (SCGE) Consortium, we are happy to welcome you to this workshop, “Rethinking Clinical Delivery, Regulation, and Payment Models for Individualized Platform-Based Genome Editing Therapies.”

The SCGE Consortium is funded as an NIH Common Fund program that aims to accelerate the clinical implementation of somatic genome editing. Through collaborations within the SCGE, with the broader community, and with the FDA, SCGE-funded projects are working towards IND-enabling applications and first-in-human clinical trials, including a team of researchers—co-led by Dr. Kiran Musunuru and Dr. Rebecca Ahrens-Nicklas—that created a personalized gene editing therapy to treat a rare metabolic disease, CPS1 deficiency, in Baby KJ. In addition, these collaborations have led to the development of a first of its kind gene editing umbrella trial of multiple genome editing drug products for multiple related rare diseases within the same trial, including some for diseases that are so rare that they would not be of commercial interest on their own.

This breakthrough represents a fundamentally different approach to gene editing clinical trials that embraces the inherent modular capacity of genome editing technology as a therapeutic platform to treat genetic disease. This raises important questions that extend beyond the scientific and technical challenges of developing an individual therapy to rethinking traditional models for clinical development, regulatory review, and reimbursement, which were largely designed around therapies developed and evaluated as one product and for one disease at a time. Applying those models to platform-based therapies, particularly for individualized treatments, creates challenges around how evidence is generated, how safety and efficacy are assessed across closely related products, how therapies are delivered at scale, and how the costs of development and treatment are covered.

We have designed this workshop to begin thinking long-term about the implications of this novel approach for marketing approval, implications for reimbursement, and how to make these therapies accessible and affordable to all patients who might benefit from them, regardless of the prevalence of their disease. With the advances in the science from SCGE and the broader field, we are truly able to develop more treatments for all patients, more quickly, but access and reimbursement challenges remain. Today, we are bringing together a broad group of stakeholders with different perspectives to examine how clinical trial design, clinical delivery models, and regulatory frameworks can be aligned with existing or novel reimbursement pathways to support sustainable and scalable delivery of genome editing therapies.

Thank you to the organizers and speakers for convening this workshop. We hope that this workshop will be insightful and leave you feeling optimistic about the potential of our collaborative efforts to bring genome editing therapies to all patients who can benefit from them.

Sincerely,

PJ Brooks
NIH NCATS

Timothy LaVaute
NIH NINDS

Felicia Qashu
NIH OD

Agenda

**All times are in Eastern Daylight Time*

Welcome and Opening Remarks

9:00 AM - 9:10 AM

Welcome

Joni Rutter

National Center for Advancing Translational Science (NCATS) at the National Institute of Health (NIH)

Welcome Video

Jay Bhattacharya

NIH

Opening Remarks

Kiran Musunuru

University of Pennsylvania

Session 1: Clinical Trial Design and Evidence Generation for Aligning Early Clinical Development with Regulatory and Reimbursement Expectations

9:10 AM - 9:15 AM

Session 1 Chair Introduction

Janet Woodcock

Retired U.S. Food and Drug Administration (FDA)

9:15 AM - 9:35 AM

"Scale up, or scale down? Thoughts on developing and deploying individualized gene editing therapies"

Kiran Musunuru

University of Pennsylvania

9:35 AM - 9:55 AM

Title Pending

Timothy Yu

Boston Children's Hospital/Harvard Medical School

9:55 AM - 10:25 AM

Panel Discussion 1

Janet Woodcock, Kiran Musunuru, Tim Yu, Sonia Vallabh (*The Broad Institute*)

10:25 AM - 10:40 AM

Break and Networking

Session 2: Evolving Regulatory Frameworks for Evaluating Platform Based Therapies for Individualized Genome Editing Therapies

10:40 AM - 10:45 AM

Session 2 Chair Introduction

Brian Stultz

U.S. FDA

Agenda

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- 10:45 AM - 11:15 AM **"CMC Considerations for Individualized Genome Editing Products"**
Jacob Bitterman
U.S. FDA
- "Clinical Considerations for Individualized Therapies"**
Aimee Magnarelli
U.S. FDA
- 11:15 AM - 11:35 AM **"The Rare Therapeutic Launch Pad (RTLP) - Switching from Research to Treatment"**
Dan O'Connor
Association of the British Pharmaceutical Industry
- 11:35 AM - 12:05 PM **Panel Discussion 2**
Brian Stultz, Aimee Magnarelli, Jacob Bitterman, Dan O'Connor

12:05 PM - 1:05 PM **Lunch and Networking**

Session 3: Existing Pathways to Coverage and Reimbursement for Genome Editing Therapies

- 1:05 PM - 1:15 PM **Session 3 Chair Introduction**
Deanna Portero
Orphan Therapeutics Accelerator
- 1:15 PM - 1:45 PM **"Navigating Gene Therapy Access and Reimbursement: Insights from UCSF"**
Jessica Galens, Jack Tyndall, & Mitchell Zack
University of California San Francisco (UCSF) Benioff Children's Hospitals
- 1:45 PM - 2:05 PM **The Cell and Gene Therapy (CGT) Access Model: Testing Outcomes-Based Agreements in Medicaid"**
Caroline Larson
Centers for Medicare and Medicaid Services (CMS)
- 2:05 PM - 2:40 PM **Panel Discussion 3**
Deanna Portero, Caroline Larson, Jessica Galens, Jack Tyndall, Mitchell Zack

2:40 PM - 2:55 PM **Break and Networking**

Session 4: Exploring Innovative Approaches for Reimbursement for Genome Editing Therapies

- 2:55 PM - 3:00 PM **Session 4 Chair Introduction**
Shyam Patel
California Institute for Regenerative Medicine (CIRM)

Agenda

**All times are in Eastern Daylight Time*

3:00 PM - 3:20 PM **“Sustainability for Interventional Genetics: evolving beyond drug development and reimbursement to a procedural model”**

Winston Yan

The Broad Institute

3:20 PM - 3:40 PM **“Title Pending”**

David Tischler

CureTrust

3:40 PM - 4:00 PM **“What Needs to Happen to Make it Happen?”**

Jayson Slotnik

Health Policy Strategies, Inc.

4:00 PM - 4:30 PM **Panel Discussion 4**

Shyam Patel, Winston Yan, Jayson Slotnik, Will Shrank

4:30 PM - 4:45 PM **Break and Networking**

Final Discussion and Closing Remarks

4:45 PM - 5:15 PM **Final Discussion**

PJ Brooks (*NIH*), Rebecca Ahrens-Nicklas (*Children’s Hospital of Philadelphia*), Anna Kwilas (*U.S. FDA*), Janet Woodcock, Jack Tyndall, Winston Yan, Jayson Slotnik

5:15 PM - 5:25 PM **Closing Remarks**

Kiran Musunuru & Rebecca Ahrens-Nicklas

University of Pennsylvania | Children’s Hospital of Philadelphia

Speaker Biographies



Joni Rutter, PhD

Director of National Center for Advancing Translational Science (NCATS) at the National Institutes of Health (NIH)

Dr. Joni Rutter earned her PhD in pharmacology and toxicology from the Geisel School of Medicine at Dartmouth. She has held numerous senior positions at NIH, including director of the Division of Neuroscience and Behavior at the National Institute of Drug Abuse (NIDA) and director of scientific programs in the All of Us Research Program. Dr. Rutter currently serves as the Director of NCATS and as working group co-chair for the NIH Common Fund program on Somatic Cell Genome Editing (SCGE).



Jay Bhattacharya, MD, PhD, MA

Director of the NIH

Dr. Jay Bhattacharya earned his undergraduate degree, an MA, and PhD in economics from Stanford University, as well as his MD from Stanford University School of Medicine. He is a professor of health policy, emeritus, at Stanford University. Dr. Bhattacharya previously served as the Acting Director of the Center for Disease Control and Prevention (CDC) and currently serves as Director of NIH.



Kiran Musunuru, MD, PhD, MPH, ML, MRA

Barry J. Gertz Professor for Translational Research; Co-Director of Penn Medicine/CHOP Orphan Disease Center; Director of Genetic and Epigenetic Origins of Disease Program Cardiovascular Institute; Scientific Director of Center for Inherited Cardiovascular Disease, Perelman School of Medicine

Dr. Musunuru earned his bachelor's degree from Harvard College, his PhD in biomedical sciences from Rockefeller University and his MD from Weill Cornell Medical School. He also has a Master of Public Health in epidemiology from Johns Hopkins Bloomberg School of Public Health, Master of Law degree from the University of Pennsylvania Law School, and Master of Regulatory Affairs from the Perelman School of Medicine at the University of Pennsylvania. A practicing cardiologist, Dr. Musunuru's research focuses on developing gene editing therapies for treating cardiovascular and metabolic diseases. He is a co-founder of Verve Therapeutics and serves as the company's Scientific Advisor. He co-led the development of the first personalized in vivo gene editing therapy for a patient with a rare urea cycle disorder. His work has been recognized with numerous awards, including *TIME*100 Most Influential People in Health 2026.

Speaker Biographies



Janet Woodcock, MD

Retired, Director of the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA)

Dr. Janet Woodcock received her undergraduate degree in chemistry from Bucknell University and earned her MD from Northwestern University Feinberg School of Medical. She had a long career at the FDA, where she served in numerous senior positions, most recently as Director of the CDER. During the COVID pandemic, Dr. Woodcock served as the therapeutic lead for Operation Warp Speed. She also worked with industry and Congress on the passage of legislation related to biosimilars and generic drugs.



Timothy Yu, MD, PhD

Attending Physician in the Division of Genetics and Genomics at Boston's Children's Hospital, Associate Professor of Pediatrics at Harvard Medical School, Associate Member at the Broad Institute of MIT and Harvard

Dr. Yu earned his undergraduate degree in biochemistry and molecular biology from Harvard College, and his MD and PhD from University of California, San Francisco. He completed his neurology residency at Massachusetts General Hospital and Brigham and Women's Hospital, and fellowship at Massachusetts General Hospital and Boston's Children's Hospital. Dr. Yu is a neurologist at Boston's Children's Hospital in the Division of Genetics and Genomics. His research focuses on genomics, bioinformatics and developing individualized treatments for rare genetic neurological diseases. He gained international recognition for developing "milasen", a personalized antisense oligonucleotide therapy created in record time to treat a young patient with a fatal, ultra-rare form of Batten disease.



Sonia Vallabh, PhD, JD

Director of Prion Therapeutic Science at the Broad Institute of MIT and Harvard

Dr. Vallabh earned her undergraduate degree from Swarthmore College, her law degree from Harvard Law School, and PhD in biological and biomedical sciences from Harvard University, where she trained under Dr. Stuart Schrediber at the Broad Institute. Her research focuses on developing therapies for prion disease. A new siRNA drug candidate developed in her lab is currently being evaluated for safety and tolerability in an ongoing Phase 1 clinical trial.

Speaker Biographies



Brian Stultz, MS

Chief of Gene Therapy Branch 3 at the U.S. Food and Drug Administration (FDA)
Center of Biologics Evaluation and Research (CBER) Office of Therapeutic
Products (OTP)

Mr. Brian Stultz received a Master of Science degree with training in biochemistry and molecular biology from the University of Virginia. He joined the FDA in 2000, where his research focused on regulation of TGF- β signaling pathway and identifying critical quality attributes of mesenchymal stem cells. He later transitioned from laboratory research to regulatory review and has over 20 years of experience in cell and gene therapy as a CMC product reviewer. He currently serves as Chief of Gene Therapy Branch 3 at the FDA.



Jacob Bitterman, PhD, MS

Gene Therapy CMC Product Reviewer at the U.S. FDA

Dr. Jacob Bitterman earned a BS in chemistry from Cornell University, a Master of Science degree in chemistry from the California Institute of Technology, and a PhD in pharmacology from Weill Medical College of Cornell University. He completed a postdoctoral fellowship at the NIH. Dr. Bitterman joined the FDA in 2015, first at the Center for Veterinary Medicine before transitioning to CBER. Dr. Bitterman is currently a team lead and CMC reviewer in CBER's Office of Therapeutic Products.



Aimee Magnarelli, DO

Medical Officer at the U.S. FDA

Dr. Aimee Magnarelli earned a BS in biology from Syracuse University and her DO from New York Tech College of Osteopathic Medicine. She is a pediatric allergist and immunologist. Dr. Magnarelli currently serves as a Medical Officer at the FDA.

Speaker Biographies



Dan O'Connor, MD, PhD, MS

Executive Directory Regulatory at the Association of the British Pharmaceutical Industry

Dr. Dan O'Connor earned his MD from Leicester Medical School, a PhD in tumor suppressor genes from Imperial College London and a Master of Science in oncology from the Institute of Cancer Research. He served as Deputy Director of the UK Medicines and Healthcare Products Regulatory Agency (MHRA)'s Innovation Accelerator with special interests in rare diseases, early access, health innovation, drug repurposing, and patient-reported outcomes and engagement. Dr. O'Connor is currently Executive Director Regulatory at the Association of the British Pharmaceutical Industry.



Deanna Portero, MPH, MBA

Vice President of Partnerships and Innovation at Orphan Therapeutics Accelerator

Ms. Deanna Portero earned a BA in philosophy and psychology from Dartmouth College, an MPH from Johns Hopkins Bloomberg School of Public Health and an MBA from Johns Hopkins University Carey Business School. She is Vice President of Partnerships and Innovation at Orphan Therapeutics Accelerator; a non-profit biotech company focused on advancing "shelved" therapies for ultra-rare diseases.



Jessica Galens, PharmD, MBA

Chief Financial Officer of the University of California San Francisco (UCSF) Benioff Children's Hospitals

Dr. Jessica Galens received her BS in physiology from Queen's University, an MBA from the University of Southern California Marshall School of Business and PharmD/MBA from the University of Southern California. She is the Chief Financial Officer of the University of California San Francisco Benioff Children's Hospital.

Speaker Biographies



Jack Tyndall, MPH

Service Line Director, Children's Center for Cancer, Blood Disorders, Cellular Therapies at the UCSF Benioff Children's Hospitals

Mr. Jack Tyndall earned a BA in biology from Pitzer College and an MPH from the University of California Los Angeles Fielding School of Public Health. He is the Service Line Director, Children's Center for Cancer, Blood Disorders, Cellular Therapies at the University of California San Francisco Benioff Children's Hospital.



Mitchell Zack, MBA

Executive Director, Payer Contracting and Strategy at UCSF Health

Mr. Mitchell Zack received his BA in biology from UCLA and an MBA in healthcare from the University of California, Irvine Paul Merage School of Business. He is the Executive Director of payer contracting and strategy at the University of California San Francisco Health.



Caroline Larson, JD

Co-lead for the Cell and Gene Therapy Access Model at the Centers for Medicare and Medicaid Services (CMS) in the Center for Medicare and Medicaid Innovation (CMMI)

Ms. Caroline Larson received a BS in engineering from Duke University and her JD in health law from Harvard Law School. She joined 2023 as a health insurance specialist in their Innovation Center and currently serves as a co-lead for the Cell and Gene Therapy Access Model in the Center for Medicare and Medicaid Innovation.

Speaker Biographies



Shyam Patel, PhD

Associate VP of Patient Access at the California Institute for Regenerative Medicine (CIRM)

Dr. Shyam Patel earned his BS and PhD in bioengineering from University of California Berkeley. He has held numerous positions at CIRM since joining in 2016 and currently is the Associate VP of patient access.



Winston Yan, MD, PhD

Director of the Center for Therapeutic Genetics (CTG) at the Broad Institute of MIT and Harvard, Founding President of the N=1 Collaborative

Dr. Winston Yan completed his undergraduate degree in physics from Harvard College, his MD from Harvard Medical School, PhD in biophysics from Harvard University. He worked on early CRISPR genome editing technology development at the Broad Institute. A physician-scientist and genome engineer, he focuses on creating sustainable models to treat patients with rare and ultra-rare genetic diseases. Dr. Yan is currently the director of the Center for Therapeutic Genetics at the Broad Institute after transitioning from industry where he was co-founder and most recently the Director of Clinical Development at Arbor Biotechnologies. He is also the Founding President of the N=1 Collaborative, an international consortium dedicated to the development of individualized genetic medicines through sharing data and learnings.



David Tischler, MBA

Co-Founder and CEO of CureTrust

Mr. David Tischler earned his undergraduate degree in biology at the University of Michigan and his Master of Business Administration from the University of California, Berkeley Haas School of Business. He is a co-founder and the CEO of CureTrust, a private company that helps insurers manage the financial risks associated with high-cost treatments for rare diseases while supporting patient access to these therapies.

Speaker Biographies



Jayson Slotnik, MPH, JD

Partner at Health Policy Strategies, Inc.

Mr. Jayson Slotnik earned his undergraduate degree from the University of Rochester, Master of Public Health from George Washington University and JD in health law from the University of Maryland Francis King Carey School of Law. He is a partner at Health Policy Strategies, Inc., a consulting firm representing innovative biotech, pharmaceutical, device, and diagnostic companies. Mr. Slotnik specializes in and counsels clients on the impact that Medicare and Medicaid coverage, payment, reimbursement, coding, and government policies have on commercial success.



Philip J. (P.J.) Brooks, PhD, MA

Acting Director of Division of Rare Diseases Research Innovation (DRDRI) in NCATS at the NIH

Dr. P.J. Brooks received a BA in psychology and neuroscience from Boston College, an MA in psychology and neuroscience from the University of Toronto, and a PhD in neurobiology from the University of North Carolina at Chapel Hill. He serves as Acting Director of the Division of Rare Diseases Research Innovation in NCATS. Dr. Brooks is also the working group co-coordinator for the NIH Common Fund program on SCGE, co-chair of the Bespoke Gene Therapy Consortium (BGTC), and one of the leaders of the Platform Vector Gene Therapy (PaVe-GT) program. He provides NIH oversight to Building Evidence and Collaboration for GenOmics in Nationwide Newborn Screening (BEACONS) initiative. In addition, Dr. Brooks was recently elected to the Board of Directors at American Society of Gene and Cell Therapy (ASGCT).



Anna Kwilas, PhD

Chief, Team Lead, and Product Reviewer in the Gene Therapy CMC Branch at the U.S. FDA CBER Office of Tissues and Advanced Therapies (OTAT)

Dr. Anna Kwilas earned her BS in animal science from West Virginia University and her PhD in integrated biomedical sciences from the Ohio State University. She completed her postdoctoral research at the National Cancer Institute, where she investigated the efficacy of cancer vaccines. Dr. Kwilas joined the FDA in 2015 and has over 10 years of experience as a gene therapy CMC product reviewer. She currently serves as gene therapy CMC team lead and branch chief.

Speaker Biographies



Rebecca Ahrens-Nicklas, MD, PhD

Associate Professor of Pediatrics (Human Genetics); Director of Gene Therapy for Inherited Metabolic Disorders Frontier Program, Children's Hospital of Philadelphia (CHOP); Associate Chief for Research, Division of Human Genetics, CHOP and University of Pennsylvania

Dr. Ahrens-Nicklas earned a BS in pharmacology from Duke University, her PhD and MD from Weill Cornell Medical College. She is an attending physician whose research focuses on developing gene editing therapies for inherited metabolic disorders. Dr. Ahrens-Nicklas works closely with patient advocacy groups to advance natural history and biomarker studies that support clinical trial readiness and the development of meaningful outcome measures. She co-
led the development of a groundbreaking personalized in vivo gene editing therapy for a patient with a rare urea cycle disorder. Her work has been recognized with numerous awards, including *TIME*100 Most Influential People in Health 2026.

Acknowledgements

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Thank you to our co-chairs Dr. Kiran Musunuru and Dr. P.J. Brooks, to our excellent speakers, and to everyone that was involved in this event. We would like to specifically thank the following people for their time and efforts in the creation of this workshop:

- Kiran Musunuru
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- Shyam Patel
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- Gabe Gonzales
- Linda Ho
- Andrew Wright
- Mindy Dwinell
- Angela Lemke
- Tristan Maroske
- Allison Endsley
- Karen Clark
- Becky Schilling
- Kristin Busse

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