Advancing Pediatric Cell and Gene Therapy Clinical Trials: Scientific, Ethical, Regulatory and Practical Considerations

U.S. FOOD & DRUG ADMINISTRATION

Alliance for Regenerative Medicine

April 09, 2026
9:00 AM – 4:30 PM

AGENDA

Hybrid Workshop: FDA Great Room, White Oak Campus | FDA YouTube Live stream
10903 New Hampshire Avenue, Silver Spring, MD 20903

Time	Topic	Speaker(s)
9:00 – 9:10 AM	Welcome and Introduction	**Vijay Kumar, MD** *Acting Director, Office of Therapeutic Products (OTP), Center for Biologics Evaluation and Research (CBER), FDA* **Mike Lehmicke, MSc** *Sr. Vice President, Science and Industry Affairs, Alliance for Regenerative Medicine (ARM)*
9:10 – 9:35 AM	Ethical Considerations: Balancing Protection and Promise **Moderator:** **David Wendler, MA, PhD** *Head, Section on Research Ethics, NIH*	**Tom Whitehead** *Co-Founder, Emily Whitehead Foundation* **Sharon King** *Chief Operating Officer, National MPS Society*
9:35 – 10:40 AM	***Panel Discussion 1***	
	Regulatory and Scientific Challenges and Opportunities in Pediatric Cell and Gene Therapy (CGT) Development **Moderator:** **Najat Bouchkouj, MD** *Associate Director for Pediatrics, OTP, CBER, FDA*	**Lynne Yao, MD** *Director, Division of Pediatric and Maternal Health Center, Center for Drug Evaluation and Research (CDER), FDA* **Crystal Mackall, MD** *Founding Director, Stanford Center for Cancer Cell Therapy, Stanford University* **Ronald J. Bartek** *Co-Founder and President, Friedreich's Ataxia Research Alliance* **Anne-Virginie "AV" Eggimann, MSc** *Vice President/Chief Development Officer, Lilly Regenerative Medicine*
10:40 – 11:00 AM	**BREAK**	
11:00 AM – 1:15 PM	***Case Studies:*** *Real-World Examples of Pediatric CGT Development*	
	Case Study: Adrenoleukodystrophy	**Florian Eichler, MD** *Director, Center for Rare Neurological Diseases, Massachusetts General Hospital*
	Case Study: Sickle Cell Disease	**Lydia Pecker, MD** *Director of Research & Advocacy, Sickle Cell Center for Adults, Johns Hopkins University*
	Case Study: Systemic Lupus Erythematosus	**Shaun Jackson, MD, PhD** *Attending Physician, Pediatric Nephrology and Rheumatology, Seattle Children's Hospital*

	Case Study: Mucopolysaccharidosis type I	**Robert Sikorski, MD, PhD** *Chief Medical Officer, Immusoft*
	Case Study: Rett Syndrome	**Andrew Mulberg, MD** *Senior Vice President, Neurogene, Inc.*
	Case Study: Inborn Errors of Immunity	**Fyodor Urnov, PhD** *Director of Therapeutic R&D, Innovative Genomics Institute*
	Q&A Session	**Moderator: Nancy Myers, JD** *CEO, Catalyst Healthcare Consulting*
1:15 – 2:15 PM	**LUNCH**	
2:15 – 3:15 PM	***Panel Discussion 2***	
	Prospect of Direct Benefit and Pre-Trial Data Requirements **Moderator:** **Rosa Sherafat-Kazemzadeh, MD** *Acting Deputy Director, Office of Clinical Evaluation (OCE), OTP, CBER, FDA*	**Melanie Bhatnagar, MD** *Associate Director for Pediatric Education and Outreach, Office of Pediatric Therapeutics (OPT), FDA* **Nirali Shah, MD, MHSc** *Senior Investigator, Pediatric Oncology Branch, National Cancer Institute (NCI)* **Louise R. Rodino-Klapac, PhD** *President, R&D and Technical Operations, Sarepta Therapeutics* **Brett Kopelan, MA** *Executive Director, DEBRA of America* **Rebecca Ahrens-Nicklas, MD, PhD** *Associate Chief for Research, Division of Human Genetics, Children's Hospital of Philadelphia* **Marshall Summar, MD** *Chief Executive Officer, Uncommon Cures, LLC*
3:15 – 4:15 PM	***Panel Discussion 3***	
	Earliest Acceptable Disease Stage for Pediatric CGT Trial Enrollment **Moderator:** **Shelby Elenburg, MD** *Acting Director, General Medicine, Branch 1, DCEGM, OCE, OTP, CBER, FDA*	**Patroula Smpokou, MD** *Director, Division of Clinical Evaluation General Medicine (DCEGM), OTP, CBER, FDA* **Sam Barone, MD** *Chief Medical Officer, Nanoscope Therapeutics* **Donald B. Kohn, MD** *Distinguished Professor, Microbiology, Immunology and Molecular Genetics, UCLA Stem Cell Research Center* **Lejla Vajzovic, MD** *Professor of Ophthalmology, Duke University School of Medicine* **Kelly Brazzo, MS** *Co-Founder and CEO, CureLGMD2i Foundation*
4:15 – 4:30 PM	Closing Comments and Next Steps	**Megha Kaushal, MD, MSc** *Acting Deputy Director, OTP, CBER, FDA*

Speaker Biographies

Rebecca Ahrens-Nicklas, MD, PhD
Director, Gene Therapy for Inherited Metabolic Disorders Frontier Program, Children's Hospital of Philadelphia

Dr. Rebecca Ahrens-Nicklas is an Associate Professor of Pediatrics and Associate Chief for Research in the Division of Human Genetics at The Children's Hospital of Philadelphia (CHOP) and the University of Pennsylvania. She directs the Gene Therapy for Inherited Metabolic Diseases Frontier Program at CHOP. She completed training in Pediatrics, Clinical Genetics, and Metabolism. She cares for children with rare diseases—particularly neurometabolic disorders—and combines clinical work with translational research to accelerate therapy development. Her laboratory investigates the molecular and cellular mechanisms that drive rare disease pathology, with the goal of translating those insights into targeted treatments. Most recently, she co-led the development of the first personalized in vivo gene editing approach for a patient with a rare urea cycle defect.

Sam Barone, MD
Chief Medical Officer, Nanoscope Therapeutics

Samuel Barone, MD, is Chief Medical Officer at Nanoscope Therapeutics where he leads the clinical development of optogenetic gene therapies for retinal diseases. His experience spans regulatory, academic, and industry settings, including service as a Senior Medical Officer in the FDA's Office of Cellular, Tissue, and Gene Therapies. Dr. Barone has held multiple C-suite leadership roles, including CMO positions at both Gemini Therapeutics and Avalanche Biotechnologies. Trained in ophthalmology, he has served as a physician at Retina Associates and as an Adjunct Clinical Assistant Professor at Stanford University. Dr. Barone began his medical career as a flight surgeon in the United States Air Force and completed a research associateship at the Wilmer Eye Institute.

Ronald J. Bartek
President and Co-founder, Friedreich's Ataxia Research Alliance (FARA)

Ronald J. Bartek is President and Co-Founder of the Friedreich's Ataxia Research Alliance (FARA) and is Co-Founder of both the Pediatric Inclusion Alliance and the NCATS Alliance. He is former chair of the board of the National Organization for Rare Disorders and serves on the boards of the Alliance for a Stronger FDA and the Critical Path Institute. He has served on th Board of Directors of the Alliance for Regenerative Medicine and on the NIH National Advisory Councils of both the National .Institute for Neurological Disorders and Stroke and the National Center for Advancing Translational Sciences Throughout the course of his career, he has served in a number of federal agencies, including the U.S. Army, the CIA, and the State Department. He and his FARA colleagues continue to collaborate with other patient organizations in working closely with government agencies and congress to accelerate progress in developing life-changing therapies.

Melanie Bhatnagar, MD
Associate Director for Pediatric Education and Outreach, Office of Pediatric Therapeutics (OPT), FDA

Melanie Bhatnagar, MD, is the Associate Director for Pediatric Education and Outreach within the Office of Pediatric Therapeutics (OPT) at the U.S. Food and Drug Administration (FDA). In this role, she oversees OPT's Pediatric Education and Outreach Program and works to strengthen strategic partnerships that advance pediatric scientific and policy priorities. Prior to her current position, she served as a pediatric clinical consultant in CDER's Division of Pediatrics and Maternal Health and as a pediatric ethicist and Acting Team Leader in OPT's Pediatric Ethics Program. Dr. Bhatnagar earned her medical degree from George Washington University.

Najat Bouchkouj, MD
Associate Director for Pediatrics Office of Therapeutic Products (OTP), Center for Biologics Evaluation and Research (CBER), FDA

Najat Bouchkouj, MD, serves as Associate Director for Pediatrics in the Office of Therapeutic Products (OTP) at the FDA's Center for Biologics Evaluation and Research (CBER), where she leads regulatory, scientific, and policy initiatives for cell and gene therapy development in pediatric populations. Before joining the FDA in 2016, Dr. Bouchkouj was an attending physician at Boston Children's Hospital and MedStar Georgetown University Hospital and a guest researcher at the National Cancer Institute. She earned her medical degree from Damascus University and completed a fellowship in pediatric hematology/oncology at Children's National Medical Center, where she continues to provide clinical care as a consulting oncologist.

Kelly Brazzo, MS
Co-Founder and CEO, CureLGMD2i Foundation

Kelly Brazzo is Co-Founder and CEO of the CureLGMD2i Foundation and a passionate advocate inspired by her daughter, Sammy, who is living with Limb-Girdle Muscular Dystrophy (LGMD) 2I/R9 - an ultra-rare and progressive muscle-wasting condition with significant unmet need. Combining personal experience with 24 years in the healthcare and special education settings, Kelly brings an authentic and compelling voice to discussions on rare disease advocacy, patient-centered care, and the urgency of advancing research and therapeutic development. She leads CureLGMD2i's mission to raise awareness, strengthen advocacy efforts, and accelerate progress toward a cure. Prior to her work in the nonprofit sector, Kelly served at Lancaster-Lebanon IU 13 as a speech-language pathologist and feeding team consultant and was Director of Rehabilitation at EnduraCare, where she led multidisciplinary care teams to improve the quality of life for individuals and families navigating complex medical needs. Kelly holds a Master of Science in Speech-Language Pathology from Columbia University and a Bachelor of Science from Old Dominion University.

Anne-Virginie "AV" Eggimann, MSc
Vice President and Chief Development Officer at Lilly Regenerative Medicine

Anne-Virginie "AV" Eggimann, MSc, is an executive leader with 25 years of experience advancing the development and regulatory approval of innovative cell and gene therapies. Currently VP and Chief Development Officer of Lilly Regenerative Medicine at Eli Lilly & Co., she provides strategic leadership across a portfolio of cutting-edge gene therapy programs. Prior to Lilly, she served as Chief Regulatory Officer at Tessera Therapeutics where she oversaw program management & leadership and led the first-ever IND clearance for a TPRT-based in-vivo genome editing therapy for AATD. Before that, she spent a decade at bluebird bio, where her team achieved marketing approvals for four gene therapies in severe genetic diseases and oncology. She began her career at Voisin Consulting, contributing to landmark authorizations including the first ATMP EU approval.

A driving force in global regulatory policy, AV helped shape the EU ATMP regulation and the FDA RMAT designation under the 21st Century Cures Act and has championed platform technology frameworks globally. She serves as Industry Representative on the FDA's CTGT Advisory Committee and on the Board of Directors of ARM. She received the 2025 ASGCT Catalyst Award and holds an M.S. from UCLA and a B.S. in Chemical Engineering from Caltech.

Florian Eichler, MD
Professor of Neurology, Harvard Medical School

Director, Center for Rare Neurological Diseases, Massachusetts General Hospital

Florian Eichler, MD, is a Professor of Neurology at Harvard Medical School and serves as Director of the Center for Rare Neurological Diseases, Co-Director of the Precision Therapeutic Unit, and the Katherine B. Sims Chair in Neurogenetics at Massachusetts General Hospital (MGH). Dr. Eichler has received multiple recognitions from the NIH for his work on metabolic brain disorders, and his research focuses on monogenic lipid metabolism disorders of the nervous system. He received his medical degree from the University of Vienna and completed specialized fellowship training in neurogenetics and metabolism.

Shelby Elenburg, MD
Clinical Team Lead / Acting Branch Chief, Office of Therapeutic Products (OTP), Center for Biologics Evaluation and Research (CBER), FDA

Shelby Elenburg, MD, is a Clinical Team Lead and Acting Branch Chief within the Office of Therapeutic Products (OTP) at the FDA's Center for Biologics Evaluation and Research (CBER), where she leads clinical reviews of cell and gene therapy products, with a primary focus on treatments for rare diseases. During her tenure at the Agency, she has served as a Committee Chair and Clinical Reviewer for Biologics License Applications (BLAs) and has been named as a contributor to the Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC). Prior to joining the FDA, she worked in an allergy and immunology practice for six years. Dr. Elenburg focused on pediatric medicine during her residency at Phoenix Children's Hospital and continued this focus during her fellowship at the University of Tennessee Health Science Center. She earned her medical degree from the University of Cincinnati College of Medicine and is board-certified in Allergy and Immunology.

Shaun Jackson, MD, PhD
Attending physician, Pediatric Nephrology and Pediatric Rheumatology, Seattle Children's Hospital

Professor of Pediatrics, University of Washington School of Medicine

Shaun Jackson, MD, PhD, is an attending physician in Pediatric Nephrology and Pediatric Rheumatology at Seattle Children's Hospital and a Professor of Pediatrics at the University of Washington School of Medicine. His research is focused on the immune mechanisms of autoimmune diseases, particularly systemic lupus erythematosus (SLE) and the development of novel cell therapies for pediatric autoimmunity. He is currently leading a clinical trial to assess the safety and feasibility of using CD19 CAR T-cell therapy to treat pediatric and young adult patients with severe lupus. Dr. Jackson earned his medical and doctoral degrees from the University of Cape Town in South Africa and completed postdoctoral research in vascular biology at Harvard Medical School. He completed his pediatric residency at Boston Children's Hospital and a dual fellowship in Pediatric Nephrology and Pediatric Rheumatology at Seattle Children's.

Megha Kaushal, MD, MSc
Acting Deputy Director, Office of Therapeutic Products (OTP), Center for Biologics Evaluation and Research (CBER), FDA

Dr. Kaushal is a pediatric hematology oncologist. She received her M.D. from Rush University, Chicago and completed her residency training in Pediatrics at Medical College of Georgia and her fellowship in Pediatric Hematology Oncology at Children's National in DC. Following fellowship, she joined the Division of Bone Marrow Transplant at Children's National prior to joining the FDA in 2014. As a clinical reviewer, she was responsible for the review and regulatory oversight of several benign and malignant hematology Investigational New Drug (IND) and Biologic Licensing Applications (BLAs). She has served as the Branch Chief for Benign Hematology. She currently serves as the Acting Deputy Director for the Office Therapeutic Products in CBER which regulate cell and gene therapy products.

Sharon King
Chief Operating Officer, National MPS Society

Sharon King is Chief Operating Officer of the National MPS Society and a nationally recognized rare disease advocate. Following her daughter's diagnosis with CLN1 Batten disease, Ms. King to co-found Taylor's Tale, a organization designed to accelerate research and raise awareness for patients and families affected by this rare and fatal neurodegenerative condition. She previously served as Senior Lead of Advocacy at Aldevron, where she focused on aligning patient experiences with advanced cell and gene therapy development. Ms. King has also held leadership roles with the North Carolina Advisory Council on Rare Diseases and the Emily Whitehead Foundation Board, and served as a National Institutes of Health Patient Ambassador. She received her Bachelor's Degree from Meredith College.

Donald B. Kohn, MD
Distinguished Professor, Microbiology, Immunology and Molecular Genetics, UCLA Broad Stem Cell Research Center

Donald B. Kohn, MD, is a Distinguished Professor at UCLA in Microbiology, Immunology & Molecular Genetics, Pediatrics, and Molecular & Medical Pharmacology. Dr. Kohn's work focuses on the genetic modification of hematopoietic stem cells to treat congenital immune deficiencies, hemoglobin disorders, and other blood cell diseases. Before joining UCLA, he completed a fellowship in Pediatric Immunology within the Metabolism Branch of the National Cancer Institute at the NIH, which he followed with over two decades at Children's Hospital Los Angeles. Dr. Kohn is a founding member and past President of the American Society of Gene and Cell Therapy and has served as President of the Clinical Immunology Society and Chair of the NIH Recombinant DNA Advisory Committee. He earned his BS in Biology and MS in Microbiology from the University of Illinois at Urbana-Champaign and his MD from the University of Wisconsin Medical School, where he also completed his pediatric residency.

Brett Kopelan, MA
Executive Director, DEBRA of America

Board Member, Alliance for Regenerative Medicine (ARM)

Brett Kopelan, MA was initiated into the world of rare diseases eighteen years ago when his daughter was born with a severe form of recessive dystrophic epidermolysis bullosa (RDEB). He became the Executive Director of debra of America in 2011 after a three-year tenure on its board of directors. As Executive Director, Brett has worked closely with industry and the FDA in shepherding three drugs to approvals, including the first re-dosable gene therapy and the first autologous gene mediated cell therapy. He also works extensively with elected officials in the House and Senate to ensure policies and legislation for those with rare diseases are enacted. Brett has served on a number of Boards of Directors including the Foundation for Cell & Gene Medicine, the Alliance for Regenerative Medicine, the Wound Care Collaborative Community, Debra International, the National Organization for Rare Disorders (NORD) and the Coalition of Skin Diseases. Prior to his work in rare disease, Brett was an accomplished entrepreneur, starting three companies raising more than $30 million in venture financing, where he led business development and marketing. He has a graduate degree from Columbia University and an undergraduate degree from New York University.

Vijay Kumar, MD
Acting Director, Office of Therapeutic Products (OTP), Center for Biologics Evaluation and Research (CBER), FDA

Vijay Kumar, MD, is Acting Director of the Office of Therapeutic Products (OTP) at the U.S. Food and Drug Administration's Center for Biologics Evaluation and Research (CBER), where he oversees regulatory review and policy for innovative biologic therapies. Before joining the FDA, Dr. Kumar was founding partner of Southwest Kidney Institute, Medical Director of Home Dialysis of Tempe, and Chairman of the Department of Medicine at Banner Desert Medical Center. Dr. Kumar began his medical training with a rotating internship at Mysore Medical College & Research Institute in India and has devoted his career to bridging clinical practice and regulatory leadership.

Crystal Mackall, MD
Professor of Pediatrics and Internal Medicine, Stanford University

Founding Director, Stanford Center for Cancer Cell Therapy

Crystal Mackall, MD, is the Ernest and Amelia Gallo Family Professor of Pediatrics and Internal Medicine at Stanford University, the Founding Director of the Stanford Center for Cancer Cell Therapy, and the Director of the Parker Institute for Cancer Immunotherapy at Stanford. She is an international leader in pediatric cancer immunotherapy and engineered T cell therapies and is pioneering efforts to use CAR T cells to treat solid tumors, including pediatric brain tumors. In addition to her academic work, Dr. Mackall has founded three biotechnology companies. She has received numerous national awards for her contributions to cancer immunotherapy, including having been elected to the National Academy of Medicine and chosen to serve as both a Fellow of the AACR Academy and the American Association of Physicians.

Andrew Mulberg, MD
Senior Vice President and Head of Regulatory Affairs, Neurogene Inc.

Andrew Mulberg, MD, FAAP, CPI, is Senior Vice President and Head of Global Regulatory Affairs at Neurogene Inc., a company developing therapeutics for rare pediatric neurological diseases, including Rett syndrome. Prior to his current role, he served as Senior Vice President of Global Regulatory Affairs at Amicus Therapeutics, where he was responsible for the approval of Galafold® (migalastat) for Fabry disease and contributed to registration planning for AT-GAA for Pompe disease. Dr. Mulberg also held leadership roles at the U.S. Food and Drug Administration as Division Deputy Director of Gastroenterology and Inborn Errors Products within CDER from 2010 to 2016. He earned his undergraduate degree from Columbia University and his MD from the Mount Sinai School of Medicine and completed his pediatrics residency at the Children's Hospital of Philadelphia. Dr. Mulberg also serves as Adjunct Professor of Pediatrics at the University of Maryland School of Medicine.

Nancy Myers, JD
CEO, Catalyst Healthcare Consulting

Nancy Bradish Myers, JD is the President and CEO of Catalyst Healthcare Consulting, a dynamic regulatory policy and regulatory affairs partner which has helped clients advance innovative healthcare solutions that benefit patients throughout its twenty-year lifespan. Ms. Myers has worked to shape policies that enhance patient access to life-changing therapies, leveraging her extensive expertise across federal agencies, industry associations, health insurance, and investment research. Her career has included key leadership roles in the biopharmaceutical industry, financial sector, and government affairs. Before founding Catalyst, Ms. Myers served in multiple roles at the U.S. Food and Drug Administration (FDA), most recently as Special Assistant and Senior Strategic Advisor in the Office of the Commissioner. She continues to maintain strong relationships with FDA leadership and staff, actively supporting the agency through her work with nonprofit organizations and advocacy coalitions - including the Alliance for a Stronger FDA and the FDA Alumni Association.

Lydia Pecker, MD
Associate Professor of Medicine / Director of Research & Advocacy, Sickle Cell Center for Adults at Johns Hopkins

Lydia Pecker, MD, **MHS,** is an Associate Professor of Medicine and Director of Research & Advocacy at the Sickle Cell Center for Adults at Johns Hopkins. She founded and currently serves as Director of the Young Adult Clinic at the Johns Hopkins Sickle Cell Center for Adults. Dr. Pecker's research and clinical expertise is in reproductive health for girls and women with sickle cell disease, which includes advising on standardizing measures and counseling regarding late-effects of transformative therapies, including gene therapy. Dr. Pecker has been a member of the Johns Hopkins School of Medicine faculty since 2016, following advanced fellowship training at Children's National Medical Center. She completed her undergraduate studies at Brown University and earned her medical degree from the University of Pennsylvania School of Medicine.

Louise R. Rodino-Klapac
President, R&D and Technical Operations, Sarepta Therapeutics

Louise R. Rodino-Klapac, PhD, is President of R&D and Technical Operations at Sarepta Therapeutics, where she leads the company's gene therapy research, development, and manufacturing strategy. She has held successive leadership roles since joining the company in 2018, including Senior Vice President of Gene Therapy, Chief Scientific Officer, and Head of R&D. Prior joining Sarepta, Dr. Rodino-Klapac served as head of the Laboratory for Gene Therapy Research at Nationwide Children's Hospital and co-founded Myonexus Therapeutics. Dr. Rodino-Klapac is an elected NIH Fellow. She holds a Bachelor of Science in biology from King's College and a PhD in molecular genetics from The Ohio State University.

Nirali Shah, MD, MHSc
Senior Investigator, Pediatric Oncology Branch, National Cancer Institute (NCI)

Nirali Shah, MD, MHSc, is an Associate Research Physician in the Pediatric Oncology Branch of the National Cancer Institute. She has held leading roles in implementing and conducting Phase I and II trials for relapsed/refractory pediatric acute lymphoblastic leukemia, specifically advancing CD22-targeted and combinatorial CAR-T cell therapies. Prior to her major appointment, Dr. Shah received her M.D. from the University of Illinois College of Medicine and completed a dual residency program in Internal Medicine and Pediatrics at the Harvard Combined Residency Program. She completed her Pediatric Hematology and Oncology Fellowship through a joint National Cancer Institute-Johns Hopkins University training program. In addition to her MD, Dr. Shah holds a Master of Health Science in Clinical Research from the joint NIH-Duke University School of Medicine. Dr. Shah is board-certified in General Internal Medicine, General Pediatrics, and Pediatric Hematology Oncology.

Rosa Sherafat-Kazemzadeh, MD
Deputy Director, Office of Clinical Evaluation (OCE), OTP, CBER, FDA

Rosa Sherafat-Kazemzadeh, MD, is a board-certified pediatric endocrinologist, serving as the deputy office director in the Office of Clinical Evaluation (OCE), overseeing clinical development of cell and gene therapies at the FDA's Center for Biologics Evaluation and Research (CBER), Super Office of Therapeutic Products (OTP). Dr. Sherafat formerly served as the branch chief and lead the clinical reviewer of a diverse range of cellular, tissue, and gene therapy products across a broad clinical portfolio, including neurology, dermatology, and pulmonary indications for rare diseases. Before joining the FDA in 2018, Dr. Sherafat spent over a decade as an Associate Professor of Pediatrics at MedStar Georgetown University Hospital. She earned her medical and graduate degrees from Tehran University of Medical Sciences and completed her pediatric residency and fellowship training at the University of Illinois and Cincinnati Children's Hospital, respectively.

Robert Sikorski, MD, PhD
Chief Medical Officer, Immusoft

Robert Sikorski, MD, PhD, is a drug developer who currently serves as Head of the Scientific Advisory Board and consulting Chief Medical Officer for Immusoft. In this role, he has advanced ISP-001, the first engineered B cell therapy to enter human clinical trials for treating rare genetic disorders. Beyond his work with Immusoft, he is the Managing Director of Woodside Way Ventures. Dr. Sikorski previously served as Chief Medical Officer at FivePrime Therapeutics, leading the company through its acquisition by Amgen. His earlier career highlights include spearheading the development of the oncology drug Vectibix and serving as an editor for the journals *Science* and *JAMA*. He earned his MD and PhD from Johns Hopkins University and completed his medical residency at Massachusetts General Hospital.

Patroula Smpokou, MD
Director, Division of Clinical Evaluation General Medicine (DCEGM), OTP, CDER, FDA

Patroula Smpokou, MD is the Director of CBER's Division of Clinical Evaluation General Medicine under the Office of Therapeutic Products. In this capacity, Dr. Smpokou oversees the clinical review and regulation of cell and gene therapy products for rare and non-rare conditions under OTP's purview. She is a board-certified pediatric clinical geneticist with experience managing the unique challenges of pediatric genetic medicine. Prior to her tenure at the FDA, she served as a practicing clinical geneticist at Children's National Hospital in Washington,D.C.,and held an academic appointment as an Assistant Professor of Pediatrics at The George Washington University School of Medicine. Dr. Smpokou earned her medical degree from the University of South Florida College of Medicine in 2006. She completed her pediatric residency at Yale-New Haven Children's Hospital and her fellowship in Clinical Genetics and Genomics at Boston Children's Hospital and Harvard Medical School-affiliated hospitals.

Marshall Summar, MD
Chief Executive Officer, Uncommon Cures, LLC

Marshall Summar, MD, is a board-certified pediatrician, clinical geneticist, and biochemical geneticist who serves as Chief Executive Officer of Uncommon Cures, LLC, a rare disease clinical trials company. Prior to his current role, Dr. Summar served as Chief of Genetics and Metabolism at Children's National, where he founded the Rare Disease Institute. Dr. Summar's work spans basic discovery, diagnostics, and therapeutic development for patients with rare genetic diseases, with more than 170 peer-reviewed publications and over 120 international patents. He earned a BS in Molecular Biology from Vanderbilt University and an MD from the University of Tennessee Health Science Center. Dr. Summar completed his pediatrics residency and fellowship training in genetics, biochemical genetics, and clinical genetics at Vanderbilt University.

Fyodor Urnov, PhD
Director of Therapeutic R&D, Innovative Genomics Institute

Professor of Molecular Therapeutics, UC Berkeley

Fyodor Urnov is a Professor of Molecular Therapeutics at UC Berkeley and a Director for Therapeutic R&D at its Innovative Genomics Institute (IGI). He co-developed the initial toolbox of human genome and epigenome editing, co-named genome editing, and was on the team that advanced all of its first-in-human applications to the clinic. He also led the effort that identified the genome editing target for an approved medicine to treat sickle cell disease and beta-thalassemia. A major goal for the field of genome editing and a key focus of Fyodor's work is expanding access to CRISPR therapies for genetic disease. As part of that effort Fyodor directs the Danaher-IGI Beacon for CRISPR Cures - a first-in-class academia-industry partnership developing and advancing to the clinic CRISPR-based platform approaches to treat severe Mendelian diseases of the immune system. Fyodor was the IGI lead on the CHOP/Penn-led effort to develop the world's first on-demand engineered CRISPR therapy for a newborn with a severe metabolic disorder. He also directs the CZI-IGI Center for Pediatric CRISPR Curesthat aims to expand the "CRISPR on-demand" approach exemplified in that effort to multiple additional pediatric patients with severe genetic diseases.

Michael Lehmicke, MSc
Senior Vice President, Scientific Affairs, Alliance for Regenerative Medicine (ARM)

Michael Lehmicke, MSc, joined ARM in 2019 as its first Director of Science and Industry Affairs. Michael has over 20 years of R&D experience in biomaterials, medical devices, and regenerative medicine. He has led product development teams for class II devices, human cell and tissue-based products, and drug/device combination products. He is a creator and an inventor with multiple U.S. patents to his name. Michael has an MSc in /Biomedical Engineering, with a focus on tissue engineering, from Drexel University. Michael's areas of expertise include cell-based tissue engineering, bioceramics, biodegradable polymers, project management, strategic pipeline development, and business development. He is passionate about regenerative medicine and believes that it represents our best hope for meeting many unmet clinical needs and thereby improving patients' lives.

Lejla Vajzovic, MD
Professor of Ophthalmology, Duke University School of Medicine

Lejla Vajzovic, MD, is Professor of Ophthalmology, Pediatrics and Biomedical Engineering with Tenure at Duke University School of Medicine and a vitreoretinal surgeon specializing in adult and pediatric retinal diseases. Dr. Vajzovic serves as principal investigator on numerous national clinical trials spanning early to late stages of development and is co-director of the Duke Pediatric Retina and Optic Nerve Center and the Duke Center for Artificial and Regenerative Vision. She directs Duke's Vitreoretinal Surgical Fellowship and Continuing Medical Education programs and has received multiple honors recognizing her contributions to ophthalmology and vision science. Dr. Vajzovic earned her MD from the Mayo Clinic Alix School of Medicine, and completed her ophthalmology residency and ophthalmic pathology fellowship at the Bascom Palmer Eye Institute at the University of Miami.

David Wendler, MA, PhD
Acting Chief, Department of Bioethics, NIH Clinical Center

David Wendler, MA, PhD, is Acting Chief of the Department of Bioethics at the National Institutes of Health Clinical Center. His work focuses on the ethical design and conduct of clinical research, particularly involving individuals who are unable to provide informed consent, including children and other vulvnerable populations. Dr. Wendler has been a leading voice in research ethics for over two decades, contributing extensively to scholarship on informed consent, risk-benefit assessment, and the ethical inclusion of underrepresented groups in clinical research. He joined NIH in 1993 as a postdoctoral fellow in the Clinical Bioethics program and has since held multiple leadership roles including heading the Unite on Vulnerable Populations and serving as a senior investigator. He earned his BA from the university of Pennsylvania and his MA and PhD from the university of Wisconsin-Madison, and has also held a fellowship in ethics at Harvard University.

Tom Whitehead
Co-founder, Emily Whitehead Foundation

Tom Whitehead is a keynote speaker, author, and journeyman lineman for an energy company. He is also the proud father of Emily and the co-founder of the Emily Whitehead Foundation, which is dedicated to raising awareness and funds for pediatric cancer immunotherapy research. Tom, his wife Kari, and their daughter Emily founded the Emily Whitehead Foundation to share their story and support families facing pediatric cancer. At age five, Emily was diagnosed with an aggressive form of leukemia that did not respond to standard treatments. As a last resort, she was enrolled in a groundbreaking clinical trial and became the first pediatric patient in the world to receive CAR T-cell therapy. The treatment was a success—Emily has been cancer-free for over 13 years and is now considered cured.

Lynne Yao, MD
Director, Division of Pediatric and Maternal Health Center, Center for Drug Evaluation and Research (CDER), FDA

Lynne Yao, MD, is the Director of the Division of Pediatric and Maternal Health at the FDA's Center for Drug Evaluation and Research (CDER). She has worked at FDA for over 18 years, having progressed through leadership roles including Clinical Team Leader and Associate Director within the Office of New Drugs. In her current capacity, she leads the development of regulatory policies and scientific initiatives to ensure that medical products are safe and effective for pediatric and maternal populations. Her work has focused on advancing drug labeling standards and clinical trial designs that specifically address the unique needs of children and pregnant individuals. Before joining the FDA in 2008, Dr. Yao practiced as a Pediatric Nephrologist at Inova Fairfax Hospital for Children for over seven years. She began her academic career at Yale University, where she earned a Bachelor of Science in Biology. Dr. Yao received her medical degree from The George Washington University School of Medicine and Health Sciences.