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 (invited)**	**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	**<u>Moderator:</u> 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 **<u>Moderator:</u>** **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 **<u>Moderator:</u>** **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*