

Individualized Therapies on the RISE

November 20, 2025

9:00 am – 5:00 pm ET

Hybrid Public Meeting | National Press Club

Meeting Background and Objectives:

The current drug development paradigm and associated regulations were developed to test single medicines that treat numerous patients, long before the concept of precision medicine became a reality in the form of individualized therapies [e.g., genome editing products and antisense oligonucleotides (ASOs)]. Individualized therapies, for the purposes of this meeting, are defined as therapies tailored to the unique genetic variants of one or a very small number of individuals. While ASOs are not novel, their application as individualized therapy is. The first individualized ASO treatment was administered in 2018 to treat a form of Batten disease, and since then, many more patients with various conditions have received such treatments. More recently, individualized gene editing therapies have been developed, including one used to treat an infant with CPS1 deficiency. Over recent years, the U.S. Food and Drug Administration (FDA) has developed numerous guidance documents for gene therapies and ASOs, including for individualized ASOs. As individualized ASOs and gene editing products are entering the clinic, it is timely to evaluate whether current regulatory practices are optimal to support the development of individualized therapies.

This public workshop, co-convened by the Duke-Margolis Institute for Health Policy and the Rare Disease Innovation Hub at the FDA, will examine the emerging science and regulatory environment for these individualized medicine programs, including nonclinical data recommendations, clinical assessments, regulatory submission structure, and additional information necessary to support the development, evaluation, and potential commercialization of these treatments.

The workshop will also provide opportunities for members of the rare disease community, including researchers, sponsors, patients, patient advocates, and regulators, to share their experiences with individualized medicine approaches. The goal of this workshop is to enhance understanding of regulatory considerations for individualized treatments and to inform future policy around individualized treatments.

Agenda

9:00 am **Welcome**

Gerrit Hamre, Duke-Margolis Institute for Health Policy

9:05 am **Opening Remarks and Presentations**

Amy Comstock Rick, RDIH, U.S. Food and Drug Administration

Presentations:

Teresa Buracchio, CDER, U.S. Food and Drug Administration

Bart Rogers, CDER, U.S. Food and Drug Administration

Brian Stultz, CBER, U.S. Food and Drug Administration

10:10 am Session 1: Current Landscape of Individualized Therapies

Objective: This session will introduce the current advancements and accompanying challenges in delivering individualized therapies. Panelists will explore individualized applications of gene editing therapies and ASOs, focusing on the real-world application of these therapeutic approaches.

Moderator: **Gerrit Hamre**, Duke-Margolis Institute for Health Policy

Panelists:

Terry Jo Bichell, COMBINEDBrain, Vanderbilt University

Sarah Glass, n-Lorem Foundation

Michelle Hastings, University of Michigan Medical School

Sonia Vallabh, Broad Institute

Moderated Discussion and Q&A

11:00 am Break

11:10 am Session 2.1: Nonclinical Considerations

Objective: This session will examine the unique challenges and strategies for developing and implementing nonclinical studies for individualized therapies. Panelists will discuss the types of nonclinical data that can support the initiation of clinical studies, and approaches to dose exploration and selection, including the use of biomarkers, computational methods, and advanced modeling techniques to acquire safety and toxicity data to facilitate drug product risk assessment.

Moderator: **Charles Gersbach**, Duke University

Presentations:

Lauren Black, Charles River Laboratories

Tim Yu, Boston Children's, N=1 Collaborative

Additional Panelists:

Lois Freed, CDER, U.S. Food and Drug Administration

Val Myers, CBER, U.S. Food and Drug Administration

Neil Shneider, Columbia University

Fyodor Urnov, Innovative Genomics Institute, UC Berkeley

Moderated Discussion and Q&A

12:10 pm Lunch Break

1:15 pm Session 2.2: Clinical Considerations

Objective: Building on the previous, this session will explore the distinct challenges and strategies for initiating clinical studies for individualized therapies. Panelists will discuss patient selection, dose escalation, benefit and safety considerations based on drug target and route of administration, as well as expansion of the treatment population.

Moderator: **Scott Winiecki**, CDER, U.S. Food and Drug Administration

Presentations:

Rebecca Ahrens-Nicklas, Children's Hospital of Philadelphia
Richard Finkel, St. Jude Children's Research Hospital

Additional Panelists:

Shelby Elenburg, CBER, U.S. Food and Drug Administration
Emily Freilich, CDER, U.S. Food and Drug Administration
Amy Simon, Beam Therapeutics

Moderated Discussion and Q&A

2:15 pm Break

2:25 pm Session 3: Generating Evidence from Clinical Experience and Integrating Existing Data

Objective: This session will discuss innovative study designs and approaches to scale existing efforts. Panelists will explore approaches such as the use of master protocols, integration of individual case experiences, and ways to evaluate individual patient responses.

Moderator: **Brian Canter**, Duke-Margolis Institute for Health Policy

Presentations:

Klaus Romero, Critical Path Institute (C-Path)
Marshall Summar, Uncommon Cures

Additional Panelists:

Ashley Munchel, CBER, U.S. Food and Drug Administration
Michael Pacanowski, CDER, U.S. Food and Drug Administration
Michael Panzara, Neurvati Neurosciences, GRIN Therapeutics
Charlene Son Rigby, Global Genes

Moderated Discussion and Q&A

3:40 pm Break

3:50 pm Session 4: Opportunities and Pathways Forward

Objective: In this session, panelists will discuss challenges and opportunities to support the advancement of individualized therapies from inception to market. Panelists will explore potential enhancements to the IND process, mechanisms to support patient access, including reimbursement, and opportunities for future collaboration. This discussion will encompass multiple perspectives on potential paths forward for individualized therapies.

Moderator: **Michelle Campbell**, CDER, U.S. Food and Drug Administration

Panelists:

Scott Demarest, Children's Hospital Colorado

Sadik Kassim, Danaher Corporation

Michael Lehmicke, Alliance for Regenerative Medicine (ARM)

Dan O'Connor, Association of the British Pharmaceutical Industry

Mark Trusheim, Tufts Medical Center, Co-Bio Consulting, LLC

Julia Vitarello, Mila's Miracle Foundation, N=1 Collaborative, EveryONE Medicines

Moderated Discussion and Q&A

4:55 pm Concluding Remarks

Gerrit Hamre, Duke-Margolis Institute for Health Policy

5:00 pm Adjournment

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