

# 2022 Gene Therapy Symposium

8 a.m. to 5 p.m. | Nov. 2, 2022

John G. Rangos Sr. Research Center | UPMC Children's Hospital of Pittsburgh

## Overview

The 2022 Gene Therapy Symposium provides an opportunity for interested health care professionals who are committed to the care of patients with conditions that may benefit from gene therapy treatments.

The 2022 Gene Therapy Symposium is presented by the Center for Rare Disease Therapy (CRDT) at UPMC Children's Hospital of Pittsburgh. The CRDT consists of international experts focused on treating children with rare diseases, defined by leading standards of care, pioneering protocols, and individualized services in a world-class environment. It is one of 31 National Organization for Rare Disorders (NORD) Centers of Excellence throughout North America.

## Agenda

8–9 a.m. Breakfast

9–10 a.m.

Gene Therapy: Transitioning From Clinical Trials to Clinical Care  
Jerry Vockley, MD, PhD | UPMC Children's Hospital of Pittsburgh

10–11 a.m.

New Genomic Therapies To Treat Methylmalonic Acidemia (MMA)  
Charles Venditti, MD, PhD | National Institutes of Health

11–11:30 a.m. Break

11:30 a.m.–12:30 p.m.

Gene Therapy for Pompe Disease: State of the Art  
Dwight Koeberl, MD, PhD | DukeHealth

12:30–2 p.m. Lunch

2–3 p.m.

Gene Therapy To Cure Diabetes Mellitus  
George Gittes, MD | UPMC Children's Hospital of Pittsburgh

3–4 p.m.

Gene Therapy for Neurodegenerative Lysosomal Storage Disorders: Opportunities and Challenges  
Deepa Rajan, MD | UPMC Children's Hospital of Pittsburgh

4–5 p.m. Reception/Meet the Speakers