THE WORKING GROUP ON
PEDIATRIC GENE THERAPY & MEDICAL ETHICS

2024

2024 ANNUAL REPORT

PGTME

DEAR FRIENDS OF PGTME,

As we wrap up the group's 5th year, we want to say thank you for following our work. A handful of new gene therapy (GT) products were approved for use in pediatric populations in 2024, and more are expected next year. With the increasing number of GTs approved for use in children, PGTME has expanded its remit from gene therapy clinical trials to also include use of these products in the clinic. This year we added new members with critical expertise, continued important research initiatives related to the lived experience of participating in a gene therapy trial, expanded the reach and scope of our hallmark events, and fostered new collaborations. In 2025 we will continue to convene conversations with diverse stakeholders about the complex ethical issues in this space and strive to be a resource for all.

Thank you for following along, and happy holidays!

Sincerely,

Alison Bateman-House & **Lesha Shah**
PGTME Co-Chairs

Ayden Eilmus
PGTME Program Coordinator

MEMBER UPDATES

Welcome to our newest members:

Allyson Berent, DVM - Foundation for Angelman Syndrome Therapeutics

Laura Kimberly, PhD, MSW, MBE - NYU Grossman School of Medicine

Ayden Eilmus, Program Coordinator

PGTME says farewell to its outgoing members and thanks them for their valuable work:
Alen Agaronov, Ryan Fischer, Cara Hunt

PGTME activities are supported in part by a generous gift from Parent Project Muscular Dystrophy

5th Annual Lunchtime Lecture Series

PGTME hosted its 5th annual LLS December 2nd-6th. The virtual conference drew approximately 100 guests each day of the series and brought together patients and parents, clinicians, academics, regulators, and more. Recordings of all five days can be found on our YouTube channel, along with videos from prior years. We thank all who participated in this weeklong event.

MONDAY – The ABCs of Genetic Medicine: What's an AAV Anyway?
Timothy Cripe, MD, PhD, Nationwide Children's Hospital
Kathrin Meyer, PhD, Alcyone Therapeutics

TUESDAY – Balancing Risks and Benefits: Gene Therapy Across Diverse Diseases
Emma James, PhD, Encoded Therapeutics (Moderator)
Barry Byrne, MD, PhD, University of Florida
Liza-Marie Johnson, MD, MPH, MSB, St. Jude Children's Hospital
Andrew McFadyen, MHSc, The Hospital for Sick Children; The Isaac Foundation

WEDNESDAY – Ethical Questions in Genetic Screening and Testing
Holly Tabor, PhD, Stanford Center for Biomedical Ethics (Moderator)
James Griffin, Author, "Breaking Silence: Living With Sickle Cell Anemia"
Stephen Kingsmore, MD, DSc, Rady Children's Hospital San Diego
Dylan Simon, MS, EveryLife Foundation for Rare Diseases

THURSDAY – What's Up with Long-Term Follow-UP? Ethical, Regulatory, and Operational Challenges
Carolyn Chapman, PhD, MS, Multi-Regional Clinical Trials Center of BWH and Harvard (Moderator)
Najat Bouchkouj, MD, Office of Clinical Evaluation, Office of Therapeutic Products, CBER, FDA
Priya Stephen, MD, FAAP, Pediatric Associates of Greater Salem; Wiskott-Aldrich Foundation
Aimee Talleur, MD, St. Jude Children's Hospital
Himal Thakar, MD, bluebird bio

FRIDAY– Data Sharing in the Gene Therapy Space
Rebecca H. Li, PhD, Vivli (Moderator)
Pat Furlong, MS, RN, BSN, Parent Project Muscular Dystrophy
Kanwaljit Singh, MD, MPH, MBA, Critical Path Institute
Timothy Yu, MD, PhD, Boston Children's Hospital

THE WORKING GROUP ON PEDIATRIC GENE THERAPY & MEDICAL ETHICS

2024

PGTME

EDUCATION

Our second annual Early Career Scholars Meeting was a success! In November, PGTME convened 30 international junior scholars working on ethical issues relevant to genetics, clinical research, and/or pediatrics for a virtual conference. Registrants were invited to submit abstracts, and five were selected for flash presentations. Congratulations to our 2024 speakers:

Oluwatobi Adeniji: "Exploring Knowledge Gaps and Ethical Considerations in Gene Therapy and Editing in Nigeria and Africa"

Isabelle Pirson: "Is There a Prospect of Direct Benefit in First in Human Pediatric Gene Therapy Trials? An Ethical Analysis"

Angel Prabakar: "Ethical Challenges of AI-Driven Decision-Making in Pediatric Neurological Care"

Jannieke Simons: "Key Stakeholders' Moral Attitudes on Somatic Gene Editing for Inherited Cardiomyopathy"

Ambria Williams: "Sci-Fi Fantasy and Gene Therapy: Developing a Sci-Fi Fantasy Entertainment-Education Patient Decision Aid for Pediatric Sickle Cell Disease"

POLICY AND RESEARCH

PGTME hosted two virtual listening sessions this year for industry representatives and clinicians and patients and advocates. These closed-door meetings allowed key stakeholders to tell us directly their most important ethical concerns regarding pediatric gene therapy clinical trials past, present, and future. Key findings from these sessions will inform the direction of PGTME's work in 2025 and will be shared publicly in the new year.

STUDENT VOLUNTEERS

Thank you to our 2024 PGTME volunteers:

Valeria Pasuizaca, New York University

Samantha Rose, Millburn High School

To be added or removed from our mailing list, if you have questions about PGTME, or **if you want to donate to support our work,** please contact ayden.eilmus@nyulangone.org

PGTME MEMBERS IN PRINT AND AT THE MIC

Carolyn Chapman presented on ethical issues in cell and gene therapy at a webinar, "Science Applications and Ethics of Cell and Gene Therapy," sponsored by the Institute for Advanced Clinical Trials for Children (I-ACT).

Carolyn also served on a panel on Community Engagement and Accessibility at the Rosamund Stone Zander Translational Neuroscience Center (RSZ TNC) at Boston Children's Hospital's fifth research symposium, entitled "Paving the Path: Therapeutic Development Readiness for Rare Neurogenetic Disorders."

Marilyn Baffoe-Bonnie was selected to be on the National Academies of Science, Engineering, and Medicine's committee on Sickle Cell Disease in Social Security Disability Evaluations.

Marilyn also published "A scoping review exploring cure definitions and language for inherited hemoglobinopathies," in *Genetics in Medicine Open.*

Liza-Marie Johnson and co-authors published "Listening to patients and parents with sickle cell disease: the totality of gene therapy risks may outweigh the perceived benefits," in *Blood Advances.*

Liza-Marie also presented "Attitudes Toward Medical Research Among Adolescent Patients and Parents of Children with Sickle Cell Disease: It's All About Trust and Human Relationships" at the American Society for Bioethics and Humanities annual conference.

Allyson Berent presented "The Parent's Journey Through Drug Development: Making the Impossible Possible for Angelman Syndrome" at the International Society for Cell and Gene Therapy autumn conference.

THE WORKING GROUP ON PEDIATRIC GENE THERAPY & MEDICAL ETHICS

2024

PGTME

PGTME MEMBERS IN PRINT AND AT THE MIC

Rafael Escandon and **Art Caplan** published an op-ed titled "Gene therapy trials should emphasize transparency, not secrecy," in *STAT.*

Erin Talati Paquette launched the *Journal of Health Advocacy*, and serves as Editor in Chief.

PGTME co-chairs **Alison Bateman-House** and **Lesha Shah** and co-authors published "Lived experience of patients and caregivers in rare genetic neurological gene therapy clinical trials in children," in *Pediatric Neurology.*

Alison Bateman-House authored "Somatic gene therapy: Ethics and access," in *Annual Review of Genomics and Human Genetics.*

Katherine Beaverson, **Alison Bateman-House**, and co-authors published "Clinician perspectives of gene therapy as a treatment option for Duchenne muscular dystrophy," in the *Journal of Neuromuscular Diseases.*

Rafael Escandon, **Richard Finkel,** and **Pat Furlong** were co-authors of "Draft guidance for industry: Duchenne muscular dystrophy, Becker muscular dystrophy, and related dystrophinopathies — Developing potential treatments for the entire spectrum of disease," in the *Journal of Neuromuscular Diseases.*

Richard Finkel co-authored "Bulbar function in Spinal Muscular Atrophy (SMA): State of the art and new challenges," in *Neuromuscular Disorders.*