

2023 ANNUAL REPORT

A project of NYU Grossman
School of Medicine's
Division of Medical Ethics

PGTME

The Working Group on
Pediatric Gene Therapy &
Medical Ethics

Dear Friends of PGTME,

Our working group celebrated its 4th birthday this year. This marks four years of our commitment to addressing ethical issues in the pediatric gene therapy research space through education, research, and policy guidance. This year we saw a handful of new GT approvals, and more are expected to come in 2024. As the field grows, so too does PGTME; we have expanded our scope to include fetal gene therapies, added new members with critical expertise, and hastened research initiatives focusing on the 'lived experience' of caregivers whose children are participating in clinical trials. We will continue convening conversations with diverse stakeholders around complex ethical topics and striving to be a valuable resource for all.

Thank you for following our work – we hope you have a wonderful holiday season!

Sincerely,

Alison Bateman-House & Lesha Shah
PGTME Co-Chairs

Cara Hunt
PGTME Program Coordinator

NEW MEMBERS

Rasha Alnaibari,
MBA - Parent
Project
Muscular
Dystrophy

**Marilyn Baffoe-
Bonnie,** MA -
Rutgers
University & NIH

**Carolyn
Chapman,** PhD -
Harvard Medical
School

**Liza-Marie
Johnson,** MD,
MPH, MSB, HEC-
C - St. Jude
Children's
Research
Hospital

4TH ANNUAL LUNCHTIME LECTURE SERIES

This year's LLS was a success, bringing in over 100 attendees from diverse backgrounds each day.

Monday's program "*The Ethics & Science of Controls in Gene Therapy Studies: Placebos, Shams, Natural History, & Delayed Crossovers*" received press coverage in:

- PinkSheet: [Gene Therapies Without Randomized Clinical Trials: Marks Outlines Rare Disease Development Path](#) (Derrick Gingery) and
- EndPoints News: [Marks: FDA open to using single-arm studies for accelerated approval of rare disease gene therapies](#) (Lei Lei Wu).



Recordings from all 5 days can be found on our [YouTube](#) channel—as are videos from prior years. We thank all of the experts who participated in this week-long event.

- ◆ **The Ethics & Science of Controls in Gene Therapy Studies: Placebos, Shams, Natural History, & Delayed Crossovers**
- ◆ **The Antibody Question: How Does it Impact Trial Enrollment?**
- ◆ **Trial on Hold: Balancing Expectations and Understanding of Clinical Investigations**
- ◆ **What Are We Talking About When We Talk About Risk?**
- ◆ **Fetal Gene Therapy**



POLICY & RESEARCH

POLICY

PGTME received funding from National Center for Advancing Translational Sciences (NCATS) and National Heart, Lung, and Blood Institute (NHLBI) to host a 1-day public conference entitled:
ANTIBODIES TO VIRAL VECTORS IN GENE THERAPY RESEARCH: SEEKING BEST PRACTICES FOR SPONSOR POLICIES AND COMMUNICATION.



You can watch the recording [here](#). Look out for our white paper in the new year!

ONGOING RESEARCH

- ◆ Members of our **Lived Experiences Subgroup** recently published a review of literature in this area. The TLDR: We need a better foundational understanding of how children/families experience clinical GT trials. Read the paper [here](#).
- ◆ We are recruiting for our **caregiver focus groups**. If you are a parent/caregiver who has experience with pediatric GT as a **current, past, or future** research opportunity, we want to hear from you!

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PGTME MEMBERS IN PRINT & AT THE MIC

Alison Bateman-House was a guest speaker on the episode, “Access to Novel Medical Products” of the *Clinical Trial Podcast*. She also accepted an invitation to serve as Program Consultant for NIH Somatic Cell Genome Editing (SCGE) Program.

Carolyn Chapman was a speaker and co-moderator for the December 2023 Bioethics Collaborative on “Gene Therapies: Probing the Ethics,” hosted by the Multi-Regional Clinical Trial Center of Brigham and Women’s Hospital and Harvard (MRCT Center).

Emma James was co-author of two studies: “Safety and efficacy of gene replacement therapy for X-linked myotubular myopathy (ASPIRO): a multinational, open-label, dose-escalation trial” (co-authored with LLS panelists Perry Shieh and Suyash Prasad) and “Effects of gene replacement therapy with resamirigene bilparvovec (AT132) on skeletal muscle pathology in X-linked myotubular myopathy: results from a substudy of the ASPIRO open-label clinical trial.” Additionally, she spoke at the 6th Gene Therapy for Rare Disorders Summit in Boston for panel discussions, “Navigating a competitive market and establishing a differentiated product,” and “Improving processes to ensure consent is informed.”

Alison Bateman-House, Lesha Shah, Rafael Escandon, Andrew McFadyen, and Cara Hunt co-authored, “Somatic Gene Therapy Research in Pediatric Populations: Ethical Issues and Guidance for Operationalizing Early Phase Trials” in *Pharmaceutical Medicine*.

Lisa Kearns and **Rafael Escandon** spoke on a panel on “Gene Therapy and Human Gene Transfer Research,” at WCG’s MAGI Clinical Research Conference—2023 East.

Rafael Escandon, Cara Hunt, and John Massarelli presented their research, “Assessment of Investigational Gene Therapy and Gene Editing Program Sponsor Compliance with 21st Century Cures Act and 42 CFR Part 11 Reporting Requirements” at the ASGCT Annual Meeting.

PGTME MEMBERS IN PRINT & AT THE MIC

Erin D. Paquette was a co-author of “Pediatric decision making: consensus recommendations,” published in *Pediatrics*. She also helped to coordinate and **Pat Furlong** and **Lesha Shah** spoke at an American College of Medical Genetics (ACMG)-sponsored educational series entitled “Gene Therapy: Ethical, Legal, and Social Issues in Gene Therapy Research.”

Patrick Moeschen was featured in an episode of *Joining Forces*, in which he discussed the importance of listening to and learning from the community and shed light on the pivotal regulatory role in advancing therapeutics for rare muscles diseases. He also participated in a lived-experience panel at the Limb-Girdle Muscular Dystrophy International Conference in Washington, DC.

John Lantos co-authored “The FDA’s dueling narratives: protector versus obstacle,” published in *Health Affairs Forefront*.

John Lantos spoke on a panel, “Should We Discuss Potential Benefits of Research with Clinical Trial Participants” at the 2024 Annual American Society for Bioethics and Humanities (ASBH) Conference. Also at ASBH, **Rafael Escandon** gave presentations on “Gene Therapy Trials and Pediatric Assent – Is it Time to Re-Think or Align with Organ Transplant?” and “Unzipping Ethical Dilemmas Regarding Parental Authority in Prenatal Gene Therapy.” Together, they published “Challenges in accelerated approvals for gene therapies” in *Molecular Therapy*.

Alison Bateman-House was quoted in this *STAT News* article, “A bellwether moment: Once a distant dream, gene therapy for Duchenne nears historic decision.”

Thank You!