PGTME celebrated its third birthday in 2022. Evolving alongside the field of gene therapy, our group remains grounded in the issues that are front and center for patients and their families. But grappling with ethically complex issues necessitates an all-hands-on-deck approach: this is PGTME.

- We are committed to fostering multi-stakeholder dialogue and producing actionable research, education, and policy guidance that serves the many people – clinicians, researchers, industry representatives, psychiatrists, advocates – involved.
- We believe in the interdisciplinary process of engagement and problem-solving, and feel firmly that it is the bedrock upon which to build better gene therapy trial designs, policies, and protocols.
- As always, we are immensely grateful to Parent Project Muscular Dystrophy for its continued funding support, as well as to other donors who support our thriving undergraduate internships program that empowers college students to learn about bioethics and gene therapy, as well as the academic research process.

This third annual report highlights some of the projects, publications, and other engagements we worked on this past year. Five key (overlapping) topics in pediatric gene therapy research serve as a framework for PGTME’s efforts: 1) risks and benefits 2) equity 3) immunogenicity and toxicity 4) informed consent, and 5) the lived experience of patients, families, and caregivers.
PGTME’s work is perhaps best exemplified by our **annual free lecture series** that is held in late fall. All the recordings are now on our **YouTube** channel—as are videos from 2021 and our 2020 inaugural series. We thank all of the experts who participated in this week-long event:

**Day 1: Pediatric Gene Therapy: State of the Field and Future Directions**
Moderator: **Alison Bateman-House**, PhD, MPH, MA, Assistant Professor, Department of Population Health, NYU Division of Medical Ethics, Grossman School of Medicine; Co-chair, PGTME  
**Phillip J. Brooks**, PhD, Acting Director, Division of Rare Diseases Research Innovation, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health [BHA1]  
**Terence R. Flotte**, MD, Provost and Executive Deputy Chancellor; Dean; Celia and Isaac Haidak Professor and Professor of Pediatrics, University of Massachusetts T.H. Chan School of Medicine  
**Louise Rodino-Klapac**, PhD, Head of R&D, Chief Scientific Officer, Sarepta Therapeutics

**Day 2: Unrealistic Expectations and Decision-making Support**
Moderator: **Karen Weintraub**, Health Reporter, USA TODAY  
**Djurdja Djordjevic**, MD FRCP, Pediatric Neurologist, Neuromuscular Fellow, Hospital for Sick Children  
**John Masembe**, BS, Clinical Patient Navigator, Fred Hutchinson Cancer Center  
**Andrew McFadyen**, BEd, Executive Director, The Isaac Foundation; Member, PGTME  
**Cynthia J. Tifft**, MD, PhD, Deputy Clinical Director for the National Human Genome Research Institute (NHGRI)

**Day 3: Gene Therapy for Sickle Cell Disease**
Moderator: **Rafael Escandon**, PhD, DrPH, MPH, Consultant; Member, PGTME  
**Sheila Cintron**, PHM, Sickle Cell Disease Advocate  
**Lori E. Crosby**, PsyD, Professor of Pediatrics, Cincinnati Children’s Hospital Medical Center  
**Lewis Hsu**, MD, PhD, Professor of Pediatric Hematology, Director of Pediatric Sickle Cell Program, University of Illinois at Chicago  
**Akshay Sharma**, MBBS, Bone Marrow Transplant Physician, St. Jude Children’s Research Hospital

**Day 4: Ask an Industry Member + Regulator**
Moderator: **Cláudia Hirawat**, Executive Chair, VOZ Advisors  
**Wilson Bryan**, MD, Director, Office of Tissues and Advanced Therapies (OTAT), Food and Drug Administration (FDA)  
**Emma James**, PhD, MFPM (Hon), Vice President, Medical & Patient Affairs, Encoded Therapeutics; Member, PGTME  
**Gregory LaRosa**, PhD, Vice President and Head of Scientific Research, Rare Disease Research Unit (RDRU), Pfizer, Inc.

**Day 5: Ask a Family Member**
Moderator: **Lesha D. Shah**, MD, Assistant Professor, Psychiatry, Mount Sinai Icahn School of Medicine; Co-chair, PGTME  
**Maria Jose Contreras**, PhD, Associate Professor, Theatre, Columbia University; patient mother  
**Danielle Jonas**, MSW, LCSW, Clinical Social Worker/Therapist  
**Gabriel McFadyen**, Patient Sibling  
**Isaac McFadyen**, MPS VI gene therapy trial participant & patient advocate  
**Matthew Vo**, BS, Nursing Assistant; former Intern, PGTME
The Lived Experiences subgroup, chaired by Cincinnati Children’s pediatric hospitalist Jennifer deSante-Bertkau, is dedicated to furthering research, education, and policy about ethical issues in pediatric gene therapy research from the vantage point of the people who live through these encounters. This spring, member Laura Kimberly virtually presented “The ‘Lived Experience’ of Pediatric Gene Therapy—A Scoping Review” at ELSIcon 2022.

Recently, we have received IRB approval to conduct focus groups about the issues, themes, and concerns that are most important to caregivers of children who have, are, or may participate in gene therapy (GT) clinical trials. Stay tuned for more information as we begin to recruit for this exciting study!

We have had three outstanding interns since the launch of our internship program in spring of 2021. Ryan Dieudonne, was recently awarded the Emory 100 Senior Honorary, recognizing 100 seniors who have greatly impacted the Emory community. Matthew Vo, recently graduated from Washington University and is working as a certified Nursing Assistant at Seattle Children’s Hospital while applying to medical school. He also spoke on the “Ask a Family Member” panel during the 2022 Lecture Series.

Anya Hamil, our current intern, is an undergraduate junior at Rutgers University-New Brunswick, working towards a B.S. in Biological Science and aspiring to apply to medical or PA school to pursue pediatric surgery.

We are profoundly grateful to Pfizer for supporting this internship at its inception. We would also like to thank NYU Langone Health’s Clinical & Translational Science Institute for additional support. To help support PGTME’s undergraduate internship, please contact Alison Bateman-House (Alison.Bateman-House@nyulangone.org).

A project of the Division of Medical Ethics at:

Alison Bateman-House gave the keynote address at Reuter’s 3rd Advocacy in Hemophilia event.


Patrick Moeschen hosted “How to Empower Your Child by Fostering Independence and Self Reliance (and avoiding ‘Learned Helplessness’) for Somebody To Talk To (STTT), a non-for-profit charitable organization offering free training and supportive group discussions exclusively for Duchenne parents and caregivers.

Alison Bateman-House, Cara Hunt, Andrew McFadyen, and Sal Rico presented “A Briefing of the Ethical Landscape of Pediatric Gene Therapy Research” at the World Congress of Bioethics.

Tim Cripe was lead author of “Leveraging gene therapy to achieve long-term continuous or controllable expression of biotherapeutics,” published in Science Advances.


Pat Furlong presented “Participant Challenges—Making the Decision” at the pre-conference meeting to ASGCT’s 25th Annual Meeting, where Alison Bateman-House spoke about the “Ethics of Recommending a Clinical Trial” and Tim Cripe spoke about “Drug Modulation of the Myeloid Tumor Microenvironment to Enable Oncolytic HSV Immunotherapy” in pre-meeting workshops.

Katherine Beaverson wrote a piece, “The Fierce Urgency of ’Now’ for Rare Disease” on LinkedIn.

Parent Project Muscular Dystrophy submitted an updated community draft guidance for Duchenne, Becker, and related Dystrophinopathies to FDA.

Richard Finkel’s paper “Spinal Muscular Atrophy“ was published in Nature Reviews Disease Primers.

Emma James presented “Considerations for Ethical and Patient-Focused Gene Therapy Clinical Research” at the 9th Clinical Trials Strategic Summit in San Francisco.

Rafael Escandon and Emma James discussed their experiences navigating complexities of conducting gene therapy clinical trials at the WCG/MAGI Clinical Research Conference in Las Vegas.

Aisha Langford gave a talk, “Enhancing Diversity, Equity, Inclusion, and Accessibility in Clinical Trials,” at NIH’s Rare Disease Day.

Aisha Langford and Art Caplan spoke at the “Equity and Diversity in Clinical Research” roundtable for the final webinar of the Advancing Healthcare Equity with Medical Humanities series at NYU Grossman School of Medicine.
On behalf of all the members of PGTME, thank you for your interest in and support of our mission over this past year. Follow our PGTME YouTube channel and visit our webpage to stay updated. To receive our quarterly newsletter, contact Cara Hunt (cara.hunt@nyulangone.org).

We’re excited to share our work in 2023!

Sincerely,

Alison Bateman-House, PhD, MPH, MA
Co-Chair, PGTME
Assistant Professor,
Division of Medical Ethics
NYU Grossman School of Medicine

Lesha D. Shah, MD
Co-Chair, PGTME
Assistant Professor, Department of Psychiatry
Icahn School of Medicine, Mount Sinai