



## Working Group on Pediatric Gene Therapy and Medical Ethics

A Project of the NYU Grossman School of Medicine Division of Medical Ethics

### 2021 Annual Report

PGTME was proud to celebrate its second birthday in 2021. We are very grateful to Parent Project Muscular Dystrophy for its continued funding support. As a multi-stakeholder working group with members from the fields of bioethics, patient advocacy, drug development, clinical research, law, and medicine, we continue to work collaboratively on the evolving ethical issues surrounding clinical trials of gene therapies in pediatric patients. We remain grounded by our collaborative spirit and committed to improving the gene therapy research experience for families and caregivers. Hearing from and involving patients and families is at the center of our work because it has ripple effects that improve gene therapy trial design, policy, and protocols for all other stakeholders involved.

This second annual report highlights some of the projects, publications, and other engagements we worked on this past year. Five key 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.

\*\*\*

#### 2nd Annual Lunchtime Lecture Series

PGTME's work is perhaps best exemplified by our [annual free lecture series](#) that is held in late fall. This year's series, *Critical Discussions: Multistakeholder Perspectives on the Ethics of Pediatric Gene Therapy Research* brought together panels of diverse experts that broadly addressed the theme of 'uncertainty' from a variety of angles. All the recordings are now on our [YouTube](#) channel—as are videos from our 2020 inaugural series. We thank all of the experts who participated in this week-long event:

#### [Pediatric Gene Therapy Research in the Context of Uncertain Risk-Benefit](#)

- Moderator: Katherine Beaverson, Senior Director, Patient Advocacy Lead, Pfizer
- Adam Hartman, Program Director, Division of Clinical Research, NINDS/NIH
- Cláudia Hirawat, Executive Chair, VOZ Advisors
- Ryan Fischer, Chief Advocacy Officer, Parent Project Muscular Dystrophy
- Safiyya Gassman, Director, Policy & Public Affairs (Rare Disease & Gene Therapy), Pfizer

#### [Trust & Transparency for Trial Participants and Families](#)

- Moderator: Lesha Shah, Assistant Professor of Psychiatry, Mount Sinai Icahn School of Medicine; PGTME Co-Chair
- Aisha Langford, Assistant Professor, Department of Population Health, NYU Grossman School of Medicine
- Andrew McFadyen, Executive Director, The Isaac Foundation
- Heather Lau, Executive Director, Global Clinical Development, Ultragenyx
- Valder Arruda, Researcher, Division of Hematology, Children's Hospital of Philadelphia

#### [The Many Facets of Hope](#)

- Moderator: Moshe Cohn, Clinical Ethicist, Pediatric Intensivist and Palliative Care Physician, Faculty Affiliate, Division of Medical Ethics, NYU Grossman School of Medicine
- Jennifer Siedman, Director of Family Engagement, Courageous Parents Network
- Laura Palmer, Chaplain, Cancer Center and Center for Fetal Diagnosis and Treatment, Children's Hospital of Philadelphia
- Rafael Escandon, Clinical Research Consultant & former SVP, Medical Affairs, Policy & Patient Engagement, BridgeBio
- Tartania Brown, Clinical Assistant Professor, Department of Family and Social Medicine, Albert Einstein College of Medicine

#### [The Lived Experience: Social, Emotional, and Practical Aspects](#)

- Moderator: Patrick Moeschen, Coordinator, Adult Advocacy & Engagement, Parent Project Muscular Dystrophy
- Cecelia Valerie, Director, Health Psychology Doctoral Program, Virginia Commonwealth University
- Diana Bharucha-Goebel, Assistant Professor, Children's National Hospital; Clinical Research Fellow, NIH
- Gretchen Egner, Parent Project Muscular Dystrophy Connect Coordinator, Wisconsin
- Isaac McFadyen, MPS VI Gene Therapy Trial Participant

#### [Accountability and Collaboration with Patient Communities](#)

- Moderator: Alison Bateman-House, Assistant Professor, Department of Population Health, NYU Grossman School of Medicine; PGTME Co-Chair
- Lakshmanan Krishnamurti, Chief of Pediatric Hematology and Oncology, Yale University
- Liza Marie Johnson, Program Director, Oncology Hospitalist Medicine, St. Jude Hospital; Bioethics Consultant
- Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy
- Sandra Reyna, Vice President, Global Medical Affairs; Head, Therapeutic Area, Novartis Gene Therapies

#### **Viral Vector Antibody Testing Project**

PGTME spent significant time on the use of commercial antibody tests by individuals seeking predictive insights into a patient's potential eligibility for gene therapy clinical trials. We held two listening sessions and a closed-door meeting on this topic and look forward to sharing our findings and recommendations this spring.

#### **Lived Experience Subgroup**

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. It has several research projects underway and looks forward to sharing them publicly in 2022.

### **PGTME Intern**

This year PGTME was proud to launch a paid undergraduate internship program for students underrepresented in the discipline of bioethics.

We thank and congratulate Ryan Dieudonné, our first intern, on his productive tenure with PGTME. Among other accomplishments, he was selected to receive a scholarship to attend the 2021 annual meeting of the American Society for Bioethics and Humanities. We wish him well in his last years at Emory University and then in medical school.

We are also thrilled to introduce our new intern, Matthew Vo. Matthew is a third year Public Health student at the University of Washington interested in clinical-based work, ethics, and pediatric medicine. We'll tell you more about Matthew in our quarterly newsletters.

We are profoundly grateful to Pfizer for supporting this internship. We would also like to thank NYU Langone Health's Clinical & Translational Science Institute for additional support. If your organization would like to help support PGTME's undergraduate internship with a gift, please contact Alison Bateman-House ([Alison.Bateman-House@nyulangone.org](mailto:Alison.Bateman-House@nyulangone.org)).

\*\*\*

### *Notable member activities*

PGTME members contributed to a wide variety of events and publications. Here's just a sampling...

**Emma James** was co-author on "[DRAVET ENGAGE. Parent caregivers of children with Dravet syndrome: Perspectives, needs, and opportunities for clinical research](#)" in *Epilepsy & Behavior* and presented "A Patient-Oriented Approach to Integrating Families' Experiences in Clinical Trials Design and Clinical Care for Dravet Syndrome" at the American Epilepsy Society Annual Meeting.

**Alison Bateman-House, Lesha Shah, Alix Hall, and Rafael Escandon** participated in a panel on "Gene Therapy Research in Pediatric Populations: Ethical Issues" at the 2021 DIA Conference.

**Lesha Shah** and **Pat Furlong** participated in a panel on "Prioritizing Patients in Gene Therapy Development" at the American Society of Gene + Cell Therapy (ASGCT) Policy Summit.

**Pat Moeschen** participated in a webinar with the employees of Sarepta Therapeutics in December to discuss developing improved end points for clinical trials in Duchenne/Becker/limb-girdle muscular dystrophy for those who are no longer ambulatory. Moeschen also leads the Parent Project Muscular Dystrophy patient adult advisory committee (PAAC), a group of 25 men living with DMD/BMD/LGMD who take a holistic approach to living life with a chronic illness. In February of 2022, he will speak in Massachusetts

at the opening of the new Dyne Therapeutic center for genetic research; his remarks will focus on older adults and increasing access to gene therapy trials.

**Richard Finkel, Lesha Shah, Alison Bateman-House**, and Jennifer Kwon were interviewed for “Ethics meets practice in gene therapy for neuromuscular disease,” in a rare neurological disease special report for *Neurology Reviews*.

**Lesha Shah** co-chaired the Pediatric Research Ethics session at the Annual Meeting of the American Academy of Child and Adolescent Psychiatry.

**Cara Hunt** presented “Ethical Issues in Emerging Technologies: Considerations for Pediatric Gene Therapy Research” for a panel at the 11th Annual Western Michigan University Medical Humanities Conference in September.

Former PGTME project manager Jamie Webb, **Lesha Shah**, and **Alison Bateman-House** co-authored “Siblings and Discordant Eligibility for Gene Therapy Research: Considering Parental Requests for Non-Trial ‘Compassionate Use’” in *Clinical Ethics*.

**Lisa Kearns**, Carolyn Riley Chapman, Kenneth Moch, **Arthur Caplan**, Tom Watson, **Andrew McFadyen**, **Pat Furlong**, and **Alison Bateman-House** published “[Gene therapy companies have an ethical obligation to develop expanded access policies](#)” in *Molecular Therapy*.

\*\*\*

On behalf of all the members of PGTME, thank you for your interest in and support of our mission over this past year. The ethical issues surrounding pediatric gene therapy are of vital import to patients, industry, clinicians, and the public, and we look forward to ongoing exploration, education, and advocacy. Follow our [PGTME YouTube channel](#) and visit our [webpage](#) to stay updated during 2022. To receive our quarterly newsletter, contact [Cara Hunt](#).

We’re excited to share our work in 2022!

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