The Working Group on Pediatric Gene Therapy and Medical Ethics (PGTME) was formed in mid-2019 to study the myriad ethical issues surrounding clinical trials of gene-based therapies in pediatric patients. PGTME is chaired by Alison Bateman-House, PhD, MPH, MA, an assistant professor in the NYU Grossman School of Medicine Division of Medical Ethics, and Lesha D. Shah, MD, an assistant professor of psychiatry and the medical director of Child, Adolescent and Family Services at the Icahn School of Medicine at Mount Sinai. PGTME is a multi-stakeholder group with members who have experience in the fields of bioethics, the biopharmaceutical industry, clinical research, law, medicine, and patient advocacy.

PGTME is proud to share its accomplishments from 2020 in our first annual report. This year, we look forward to continuing to implement our mission: to advance research, policy, and education regarding the ethical issues surrounding gene therapy trials in children. We seek to promote improved understanding of challenges and nascent best practices for ethical research across the evolving landscape of gene-based technologies.

We initially focused on hearing firsthand from various stakeholders in the gene therapy space, including clinicians, academic researchers, industry representatives, parents, and patient advocates. It is our core belief that interdisciplinary dialog is essential to understanding the complex, multifactorial dynamics of pediatric gene therapy research and related endeavors. Through listening, the members of PGTME gained an understanding of the ethical issues at hand and could begin formulating feasible solutions and identifying best practices.

We heard from speakers including parents of children with genetic diseases, founders and members of rare genetic disease advocacy organizations, industry members in the gene therapy research space, and clinicians active in pediatric research. In partnership with the EveryLife Foundation, PGTME convened two listening sessions—one with patient advocates and another with representatives from gene therapy product developers—during the 2020 Rare Disease Week events in Washington, D.C.

In late November/early December, PGTME hosted a free weeklong conference series on Ethical Issues in Pediatric Gene Therapy Clinical Trials. We brought together diverse experts to discuss ethical issues related
to risks and benefits, equity, immunogenicity and toxicity, the experience of being a patient or caregiver, and informed consent. The webinars are available on our newly inaugurated PGTME channel:

- **Risks + Benefits in Pediatric Gene Therapy Research**, featuring Dr. Peter Marks, director of the FDA’s Center for Biologics Evaluation and Research, with a response by Ms. Pat Furlong, founder and president of Parent Project Muscular Dystrophy

- **Equity Issues in Pediatric Gene Therapy Research**, featuring Dr. Pilar Ossorio, Professor of Law and Bioethics at University of Wisconsin Law School, with a response by Dr. Rafael Escandon, Senior Vice President of Medical Affairs, Policy, and Patient Engagement at BridgeBio Pharma Inc.

- **Ethical Challenges of Immunogenicity and Toxicity in Pediatric Gene Therapy Research**, featuring Dr. James Wilson, Rose H. Weiss Professor and Director of the Orphan Disease Center and Gene Therapy Program at the University of Pennsylvania Perelman School of Medicine, with a response by Dr. Timothy Cripe, Chief of Pediatric Hematology and Oncology and Blood and Marrow Transplantation at Nationwide Children’s Hospital

- **Lived Experiences: The Personal + Family Impact of Participating in Pediatric Gene Therapy Research**, featuring Mr. John F. Crowley, Chairman and CEO of Amicus Therapeutics, with a response by Mr. Patrick Moeschen, teacher and Muscular Dystrophy patient advocate

- **Operationalizing Informed Consent in Pediatric Gene Therapy Research**, featuring Dr. Sandy Macrae, CEO & President of Sangamo Therapeutics, Inc., with a response by Dr. Arthur Caplan, Founding Director of the NYU Grossman Division of Medical Ethics

**Additional Speaking Engagements**

PGTME members participated in a wide variety of conferences and webinars and were invited to speak to several organizations.

Lisa Kearns discussed “Ethical Issues in Individualized Gene Therapies for Ultrarare Diseases” at the Orphan Drugs & Rare Diseases Global Congress 2020 Americas—East Coast meeting and spoke about “Individualized Therapies for Ultra-Rare Diseases: What Are the Ethical Issues?” to the NYU Grossman School of Medicine Department of Population Health.

Katherine Beaverson spoke on “Advances in Rare Disease Therapies” at the [Rare Drug Development Symposium](#) hosted by Global Genes and the Orphan Disease Center.

Pat Furlong and Andrew McFadyen participated in a webinar on “[Ethics & Patients in Research](#),” as part of the University of Toronto’s Translational Research Program.

Pat Furlong and Alison Bateman-House discussed clinical research–related issues during a panel discussion on “Taking Care of Chronic and Acute Non-COVID Patients during the Pandemic,” a webinar sponsored by the NYU Grossman School of Medicine Division of Medical Ethics Working Group on Compassionate Use and Preapproval Access ([CUPA](#)) and the [University of Arizona Health Law and Policy Program](#).

Alison Bateman-House spoke about “Early Forays into Individualized Therapeutics: Ethical Considerations” at the [Oligonucleotide Therapeutics Society (OTS) annual meeting](#) and “IRB/Ethics Committee Requirements for Pediatrics and How These May Affect Drug Research” at the [International Society for CNS Clinical Trials and Methodology 2020 meeting](#).

Alison Bateman-House and Lisa Kearns joined a panel of experts at [The Isaac Foundation’s annual Rare Disease Patient Symposium](#).

PGTME co-chair Lesha Shah, a member of the American Society of Gene & Cell Therapy Ethics Committee, assisted in planning for the [ASGCT Policy Summit](#), which highlighted ethical and regulatory issues in cell and gene therapy.

Lisa Kearns, Rafael Escandon, Alison Bateman-House, and Lesha Shah presented a panel discussion on “A Survey of the Ethical Landscape of Pediatric Gene Therapy Research” at the annual meeting of the [American Society of Bioethics and Humanities](#).

Lesha Shah gave a talk on “[Immunogenicity in Gene Therapy Trials: Impact on Pediatric Eligibility and Participation](#)” while Pat Furlong presented on “[Gene Therapy and Seroprevalence: Community Impact and Worries](#)” at the [NCATS-FDA Workshop on Systemic Immunogenicity Considerations on AAV-mediated Gene Therapy](#).
In the Media

In an interview with Relias Media for an article titled “New Working Group to Produce Guidance for Pediatric Gene Therapy,” PGTME co-chair Lesha Shah explained the group’s mission and the five topics it has prioritized for study: equity in trial recruitment; lived experiences of patients, parents, and families; informed consent; risks and benefits; and vector immunity. Jamie Webb, former PGTME project manager, and Alison Bateman-House co-authored an op-ed in STAT on the current deficiencies of ClinicalTrials.gov and suggested changes that would improve understanding of available trials.

We are grateful for your interest in and support of PGTME’s 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 for next year. To receive our quarterly newsletter, contact Cara Hunt.

We’re excited to share our work in 2021!

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