



Working Group on Compassionate Use & Preapproval Access

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

2023 Annual Report

Happy New Year, Friends of CUPA!

This is a milestone year for the working group—2024 marks our 10th year of work in preapproval access! Reflecting upon all that we have been able to do since CUPA was launched in 2014, we feel a deep sense of gratitude for all of our colleagues, collaborators, and supporters, without whom our work would not be possible. As we enter the new year, we remain dedicated to our mission of advancing research, education, and policy surrounding access to investigational medical products, both in and outside of clinical trials.

We are especially excited to kick off our 10th year with our biannual conference, CUPA Con 2024, on January 29th and 30th in-person in NYC and worldwide via Zoom livestream. Head to our [conference website](#) for registration and more info. We hope to see you there!

Before going forward, we wanted to take this opportunity look back and highlight our work from 2023. Without further ado, we present CUPA's year-in-review.

In 2023, CUPA welcomed several new faces to the working group:

- Lauren Karel, a drug information pharmacist at the Children's Hospital of Philadelphia
- Sharad Adekar, Lead Medical Chair at WCG IRB
- Treena Williams, a recent graduate of the Master of Science in Bioethics program at Columbia University, is a CUPA visiting scholar.
- Ayalah Rose, a junior at Howard University, is CUPA's 3rd intern as part of the [Undergraduate Internship Program](#).

Outside of CUPA, our members accomplished much in their own careers this year. Here are some highlights:

- Barbara Redman's new book, "[Reconstructing Research Integrity: Beyond Denial](#)," was published.
- Jennifer Miller was made Co-Director of the Program for Biomedical Ethics at Yale School of Medicine.
- CUPA co-chair Alison Bateman-House was appointed to the HHS Secretary's Advisory Committee on Human Research Protections.
- Holly Fernandez Lynch was promoted to Associate Professor of Medical Ethics at the University of Pennsylvania, effective July 2024.

- Tobias Polak, a former CUPA visiting scholar and member of CUPA's Ethics & Real-World Evidence subgroup (ERWE), defended his PhD dissertation—titled “Unlocking the Value of Expanded Access: Statistical, Ethical, and Policy Considerations”—with distinction at Erasmus University in Rotterdam.
- Lindsay McNair re-launched Equipoise Consulting, LLC.
- Alen Agaronov, a Division postdoctoral fellow and member of CUPA's Ethics & Real-World Evidence subgroup (ERWE), received a grant from Pfizer Inc. to study how stakeholders differentially perceive evidence of functional motor outcomes for new therapies for Duchenne muscular dystrophy.
- [L'Accès aux Médicaments à Titre Compassionnel](#) [Compassionate Use Access to Medicines], a new book by Carole-Anne Baud, CUPA's inaugural visiting scholar, was published by Schulthess.

Research

CUPA's research drives the education and advocacy work that we do. Here are some selections from 2023:

- Jennifer Miller and co-authors published “[Metrics, baseline scores, and a tool to improve sponsor performance on clinical trial diversity: Retrospective cross sectional study](#)” in *BMJ Medicine*.
- “[The ethics of expanded access research](#),” a Viewpoint article by Holly Fernandez Lynch and Tobias Polak was published in *JAMA Network*.
- Tobias Polak and colleagues published “[Results from expanded access programs: A review of academic literature](#)” in *Drugs*.
- Tobias Polak and colleagues published “[Augmenting treatment arms with external data through propensity-score weighted power-priors: An application in expanded access](#)” in *arXiv*.
- Andrew McFadyen and co-authors' article, “[Ethical challenges and opportunities in the development and approval of novel therapeutics for rare diseases](#),” was published in the *Journal of Medicine Access*.
- Holly Fernandez Lynch provided commentary titled “[Devil in the details: Physician duties and expanded access](#)” on the article “[Do physicians have a duty to discuss expanded access to investigational drugs with their patients? A normative analysis](#),” by Stefan Vermeulen et al. in the spring 2023 issue of the *Journal of Law, Medicine & Bioethics*.
- Barbara Redman's article, “[Rebalancing commercial and public interest in prioritizing biomedical, social, and environmental aspects of health through defining and managing conflicts of interest](#),” was published in *Frontiers in Medicine*.
- Tobias Polak and colleagues published “[Expanded access to investigational drugs in psychiatry: A systematic review](#)” in *Psychiatry Research*.
- Christopher Robertson co-authored “[Silencing the FDA's Voice — Drug Information on Trial](#)” in *New England Journal of Medicine*.

Education

As part of its founding mission, CUPA has dedicated itself to addressing the need for preapproval access education within all stakeholder groups. We are proud to share some of the products of this pledge from the last year:

- In April, CUPA hosted its annual “Expanded Access 101” webinar with the theme “Empowerment Through Education.” The one-hour panel discussion featured CUPA members Christopher Robertson (moderator), Cláudia Hirawat, Kay Holcombe, Kenneth Moch, and Sukhun Kang. A recording of the event is available in both English and Spanish on [CUPA’s YouTube page](#).
- In October, CUPA hosted its annual “Advanced Topics” webinar, this year focusing on “Expanded Access Policies and Compliance in an Ever-Changing Environment.” The discussion featured CUPA members Cláudia Hirawat, Sukhun Kang, and Kenneth Moch, Rachel Sher of Manatt, Phelps & Phillips, LLP, and CUPA Program Coordinator John Massarelli (moderator). Watch a recording of the event [here](#).
- CUPA said goodbye to its 2022–2023 undergraduate intern, Doris Aucacama, in May and welcomed Ayalah Rose in October as the 2023–2024 CUPA intern. The Undergraduate Internship Program provides paid, real-world bioethics exposure and work experience to college juniors and seniors from underrepresented groups. Find more information about the CUPA Undergraduate Internship Program [here](#).

Policy

In 2023, CUPA continued working to improve the policies governing preapproval access to investigational medicines. Here are a few highlights from our policy work in 2023:

- Holly Fernandez Lynch was quoted in “[How a controversial US drug policy could be harming cancer patients worldwide](#),” in *Nature*. In October, she testified during the Senate Committee on Aging’s hearing on the Promising Pathway Act in a session titled “[Unlocking Hope: Access to Therapies for People with Rare, Progressive, and Serious Diseases](#).” She also co-authored, with Reshma Ramachandran, a [letter to members of Congress](#) opposing the Promising Pathway Act.
- Several members of CUPA took part in the “Oncology Clinical Trial Site Selection Project” — a collaboration between the NYU Division of Medical Ethics and FDA’s Oncology Center of Excellence (OCE). OCTSS featured a series of virtual workshops in which key stakeholders discussed what FDA’s role ought to be in ensuring diversity in and fairness in access to the clinical studies it oversees, culminating in a white paper submitted to the OCE.
- Holly Fernandez Lynch, Lisa Kearns, Kenneth Moch, and Art Caplan’s article “[What’s the worst that could happen? A toothless FDA](#)” appeared in *Health Affairs Forefront*.

CUPA’s expertise was on display far and wide in 2023 at conferences, workshops, seminars, guest lectures, grand rounds, and on podcasts and radio shows, including:

- American Society for Bioethics and Humanities (ASBH) Annual Conference
- American Society of Clinical Oncology (ASCO) Annual Meeting
- American Society of Gene and Cell Therapy (ASGCT) Annual Conference
- Aspen Ideas Festival 2023
- Association of Clinical Research Professionals (ACRP) Annual Meeting
- The Clinic & The Person Podcast
- ELSI Friday Forum
- Harvard Medical School Center for Bioethics Health Policy & Bioethics Consortia

- JAMA Network
- Manatt Health
- Newport Beach Public Library Foundation
- NORD's Rare Diseases and Orphan Products Breakthrough Summit
- Operationalize Expanded Access Programs
- Professional Patient Advocates in Life Sciences (PPALS) Annual Patient Advocacy Certificate Training Seminar
- Society for Clinical Trials (SCT) Annual Meeting
- University of Colorado Anschutz Center for Bioethics and Humanities Research Rounds
- University of Vermont Larner College of Medicine
- WCG MAGI Clinical Research Conference — 2023 East
- Witte Lectures 2023
- World Orphan Drug Congress USA

CUPA members were published or quoted in many journals and news outlets, including:

- ArXiv
- ASGCT.org
- Association of Clinical Research Professionals Blog
- Bloomberg.com
- Boston Globe
- Clinical Trials Arena
- The Clinical Trial Podcast
- Drugs
- Frontiers in Medicine
- Health Affairs Forefront
- Journal of Law, Medicine & Bioethics
- Journal of Medicine Access
- MedPage Today
- Medscape
- MIT Technology Review
- Nature
- NCODA PQI Podcast
- New England Journal of Medicine
- New Yorker
- Precision Medicine Online
- Public Understanding of Science
- Salon
- Stat
- Frontiers in Medicine

Thank you for supporting our work in 2023. If you or someone you know would like to receive our free monthly newsletter, contact John Massarelli (john.massarelli@nyulangone.org). If you would like to help support CUPA—which operates solely on donations—please contact Lisa Kearns (lisa.kearns@nyulangone.org). Here's to another productive year in 2024!

Sincerely,

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Alison Bateman-House (CUPA co-chair)
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John Massarelli (CUPA program coordinator)
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