

Working Group on Compassionate Use & Preapproval Access

NYU Grossman School of Medicine Division of Medical Ethics

2024 Annual Report

The Working Group on Compassionate Use & Preapproval Access (CUPA) celebrated a significant milestone this year: 2024 was our 10th year of advancing research, education, and policy surrounding ethical issues in preapproval access to medical products! We are deeply grateful to our colleagues, collaborators, funders, and supporters, whose contributions have been instrumental to CUPA's success—not the least of whom are the members comprising CUPA.

I. Membership

In 2024, CUPA welcomed several new members:

- Lea Ann Browning-McNee, MS, Director of Communication & Engagement, Reagan-Udall Foundation for the FDA
- Ken Menkhaus, PhD, Professor of Political Science, Davidson College, ALS advocate, person living with ALS
- Tobias Polak, PhD, former CUPA visiting scholar, expanded access and real-world data specialist

Avessie Annan, a first-year student in the NYU Master of Arts in Bioethics program, also joined CUPA, as a volunteer.

We look forward to their collaborations with longer-term members:

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| • Sharad Adegkar, MD, PhD, CIP | • Sandy Macrae, PhD, ChB, MB |
| • Jinsy Andrews, MD, MSc | • Regine Nshimiyimana Maniraho, DNP, PharmB, FNP |
| • Alison Bateman-House, PhD, MPH, MA | • John Massarelli, BA |
| • Hayley Belli, PhD, MS | • Andrew McFadyen, MHSc, BEd |
| • Arthur Caplan, PhD | • Lindsay McNair, MD, MPH, MS |
| • Pat Furlong, MS, RN, BSN | • Jennifer Miller, PhD |
| • Cláudia Hirawat | • Kenneth Moch, MBA |
| • Kay Holcombe, MS | • Barbara Redman, PhD, MBE, RN |
| • Sukhun Kang, PhD, MS | • Christopher Robertson, JD, PhD |
| • Lauren Karel, PharmD, BCPS | • David Scheer, MS |
| • Lisa Kearns, MS, MA | • Lesha Shah, MD |
| • Laura Kimberly, PhD, MSW, MBE | • David Wallach, MPH, CIP |
| • Richard Klein | • Tom Watson, BSc |
| • Connie Law, PharmD | • Mary Elizabeth Williams |
| • Holly Fernandez Lynch, JD, MBE | |

Alen Agaronov, PhD, a postdoctoral fellow who researched real-world evidence in expanded access, has completed his fellowship and left the Division of Medical Ethics. We thank Alen for his valuable contributions to CUPA and wish him well in his future endeavors.

In Memoriam

We want to acknowledge the passing this year of our dear friend Nancy Neveloff Dubler. Nancy was an original and cherished CUPA member and a bioethics powerhouse. The Division of Medical Ethics has named its longtime ethics lecture series “The Nancy N. Dubler Bioethics Lunch Talk Series” in her memory. We will miss her wisdom, passion, and kindness.

Over its 10-year span, CUPA has mourned the loss of one other active CUPA member, [Jane Reese-Coulbourne](#). We hold dear our memories of Nancy and Jane.

II. Activities & Accomplishments

Below we look back and offer highlights of our work fulfilling CUPA's commitment to research, education, and policy over the past year.

A. Research

Our members' academic work drives CUPA's efforts in education and advocacy. In 2024:

- Sharad Adekar co-authored “[Current opportunities and outcomes in rare disease clinical trials](#)” in a WCG Trends & Insights blog post.
- Arthur Caplan and colleagues published “[Adequate information about clinical trial results must be given to participants](#)” in *Nature Medicine*.
- Sukhun Kang co-authored “[When do firms provide early access to investigational drugs? Evidence from expanded access in the drug market 1990–2020](#)” in *Social Science Research Network*.
- Jennifer Miller and colleagues published “[Reporting of demographics & subgroup analyses in premarketing studies of FDA approved high-risk cardiovascular devices, 2014-2022](#)” in *Medical Devices: Evidence and Research*.
- Sukhun Kang co-authored “[The complexity of funding rare disease research: An IRDiRC \[International Rare Diseases Research Consortium\] assessment of the landscape](#)” in *Rare Disease and Orphan Drugs Journal*.
- Alison Bateman-House and co-authors published “[The state-of-the-art of N-of-1 therapies and the IRDiRC \[International Rare Diseases Research Consortium\] development roadmap](#)” in *Nature Reviews Drug Discovery*.
- John Massarelli and colleagues published “[Data professionals' attitudes on data privacy, sharing, and consent in healthcare and research](#)” in *Digital Health*.

If you are unable to access an article of interest, contact us!

B. Policy

In 2024, CUPA continued to work to improve the legal and regulatory policies surrounding preapproval access to investigational medicines. Here are some highlights:

- Alison Bateman-House and Holly Fernandez Lynch's First Opinion piece "[Trump gave patients a 'right to try.' It hasn't helped them](#)" appeared in *STAT*.
- Jinsy Andrews spoke about the [ACT for ALS Act](#) and the opportunities and challenges of implementing expanded access programs in the ALS field at Working to End ALS: The State of Research and Care in the U.S., hosted by the Congressional ALS Caucus.
- Xinping Hu presented a paper co-authored with CUPA's Alison Bateman-House, Christopher Robertson, and Holly Fernandez Lynch, "Legal pathways to promote access to safe and effective psychedelic medicines," at the 2024 Petrie-Flom Center Annual Conference: Law and Policy of Psychedelic Medicine.
- Arthur Caplan talked to the United Nations Women's International Forum about "The Ethical Challenges of Rare Diseases."
- Arthur Caplan spoke about "Public Health Emergencies & Compassionate Use" at the World Medical Association North America Regional Meeting for the Revision of the Declaration of Helsinki.
- Arthur Caplan and Alison Bateman-House were quoted in "[FDA reports sparse use of Trump's Right to Try program](#)" in *Politico Pro*.
- Alison Bateman-House was quoted in "[Choices for the terminally ill: FDA's Expanded Access program or Right to Try Act?](#)" for the MedShadow Foundation.

C. Education

CUPA remains dedicated to addressing the need for preapproval access education among all stakeholder groups. Some highlights from 2024:

- Our flagship educational event, the biannual CUPA Conference, took place at NYU Langone Health's Tisch Hospital and online on January 29th and 30th. More than 200 stakeholders assembled to discuss the most pressing ethical issues in preapproval access. For those who missed it, recordings of each CUPA Con 2024 session have been posted to the [CUPA YouTube page](#). Topics that had not previously been subjects at CUPA-hosted events included: pharmacists' role in preapproval access and preapproval access for xenotransplantation and phage therapies. You can find a full presenter list and agenda from the 2-day event [here](#). Thank you to the generous sponsors of CUPA Con 2024: Bionical Emas, Buchanan Ingersoll & Rooney PC, Voz Advisors, and WCG.
- In May, CUPA partnered with the International Society for Cell & Gene Therapy (ISCT) Expanded Access Working Group to host our annual "Expanded Access 101" webinar. The one-hour panel discussion was moderated by Johnson & Johnson's Christine MacCracken and featured CUPA members Jinsy Andrews and David Wallach and ISCT EA Working Group members Bambi Jo Grilley and Laertis Ikonomou. A recording of the event can be found [here](#).
- In November, CUPA hosted its annual "Advanced Topics" webinar. This year's topic was "Expanded Access for Xenotransplantation: Current Regulations and Path Forward." The

one-hour panel discussion was moderated by Arthur Caplan and featured Karen Khalil, Jayne Kim, and Tamar Schiff of NYU Langone Health and Vijay Kumar of FDA's Center for Biologics Evaluation and Research (CBER). A recording of the event can be found [here](#).

- Alison Bateman-House taught "Clinical Trials & Non-Trial Access to Investigational Medical Products" to second-year students at NYU Grossman Long Island School of Medicine.
- CUPA program coordinator John Massarelli discussed the ethics and logistics of expanded access during a clinical ethics course he led for NYU undergraduate students.
- Andrew McFadyen co-moderated a grand rounds discussion entitled "Capturing a Broader Assessment of Clinical Benefit in Heterogeneous Complex Rare Disease." The October event was sponsored by Toronto's Hospital for Sick Children Department of Bioethics.

This year CUPA's expertise was on display far and wide at conferences, workshops, seminars, guest lectures, grand rounds, and on podcasts and radio shows, including:

- American Academy of Neurology Annual Meeting
- American College of Clinical Pharmacology Annual Meeting
- American Society for Bioethics and Humanities Annual Conference
- The Biocentury Show
- DIA Global Annual Meeting
- International Rare Diseases Research Consortium
- NYU Grossman Long Island School of Medicine
- Operationalize: Expanded Access Programs Summit
- Parent Project Muscular Dystrophy Annual Summit
- Petrie-Flom Center Annual Conference
- Precision Child Health: From Technology to Translation Conference
- Professional Patient Advocates in Life Sciences
- Public Responsibility in Medicine and Research (PRIM&R)
- Shattuck Labs
- United Nations Women's International Forum
- Working to End ALS: The State of Research and Care in the U.S.
- World Medical Association North America Regional Meeting
- World Orphan Drug Congress
- Yale Innovation Summit

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

- Annual Review of Genomics and Human Genetics
- Biospace
- Digital Health
- Endpoints News
- KFF Health News
- Medscape
- Medshadow Foundation
- Nature Medicine
- Nature Reviews Drug Discovery
- Politico Pro
- Rare Disease and Orphan Drugs
- Salon
- Social Science Research Network
- STAT
- WCG Trends & Insights

III. In closing

One crucial part of CUPA's work is creating educational content (for example, our free monthly newsletters); another is ensuring our online resources, including our comprehensive FAQ page, reflect the latest news and ideas in preapproval access; and still another is trying to reach new audiences (for example, by recently offering live translation of a webinar in Spanish). We are delighted to announce that we were recently awarded a "Rare Giving" grant by the [EveryLife Foundation](#) to help us continue this work, including the translation our FAQs into Spanish.

If you would like to know more about CUPA, please visit our website. We are also on [X \(Twitter\)](#) and [YouTube](#). To receive our free monthly newsletter, contact program coordinator John Massarelli (john.massarelli@nyulangone.org). To request a speaker for your organization—or to help support CUPA, which operates solely on donations and gifts—please contact Lisa Kearns (lisa.kearns@nyulangone.org).

Last but absolutely not least: Thank you for being part of our past 10 years! We look forward to many more and hope to have you with us.

Sincerely,

Alison Bateman-House, PhD, MPH, MA
CUPA co-chair
NYU Grossman School of Medicine

Arthur L. Caplan, PhD
CUPA co-chair
NYU Grossman School of Medicine

John Massarelli
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