

CUPA

The NYU Grossman School of Medicine Working Group on Compassionate Use and Preapproval Access

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

2022 Annual Report

As CUPA enters its ninth year, we remain deeply grateful for your interest in and support of our work. Fulfilling our mission to advance research, education, and policy surrounding access to investigational medical products would be impossible without your collaboration and thoughtful engagement.

In 2022, CUPA welcomed several new faces as members, staff, interns, and a visiting scholar:

- Alen Agaronov, a post-doctoral fellow in Division of Medical Ethics, joined in May. Alen is primarily situated within CUPA's Ethics & Real-World Evidence Subgroup (ERWE).
- In March, Ramya Antony, a clinical analyst at the FDA's Oncology Center for Excellence's Project Facilitate, joined as an observing representative of FDA's Center for Drug Evaluation and Regulation (CDER).
- Doris Aucacama, a senior at the NYU Rory Meyers College of Nursing, joined in September as the second awardee of CUPA's Undergraduate Internship Program.
- In June, Ayden Eilmus, of the Division of Medical Ethics, became a CUPA project associate.
- In April, Dhriti Jagadish, a senior at Rocklin High School in California, began working with CUPA as a high school intern.
- Sandy Macrae, chief executive officer of Sangamo Therapeutics, joined in March.
- Tobias Polak, a PhD student at Erasmus University, Real-World Data Lead at myTomorrows, and a member of CUPA's Ethics & Real-World Evidence Subgroup (ERWE), was a visiting scholar with CUPA from September to December.

CUPA members often collaborate on issues related to preapproval access, but they also accomplished *a lot* on their own this year...

- Tom Watson was appointed chief executive officer of Bionical Emas, a clinical research and early access program organization in the United Kingdom, in August.
- Holly Fernandez Lynch was elected to the Hastings Center Fellows program in late 2021 and was named an Emerging Leader in Health and Medicine by the National Academy of Medicine in September.
- Sukhun Kang was hired as an assistant professor in UC Santa Barbara's Technology Management Department this fall. He is currently finishing his PhD at London Business School. In December, the LBS PhD program awarded Sukhun the 2022/23 Professor Sir James Ball PhD Prize in recognition of his excellent performance and the potential impact of his research.
- Mary Elizabeth Williams, author of [A Series of Catastrophes & Miracles: A True Story of Love, Science, and Cancer](#), about her experience with an immunotherapy clinical trial for metastatic melanoma, earned a certificate in conflict resolution and leadership at Drew University.

- Jennifer Miller has continued her work as director of Bioethics International, a non-profit that evaluates and ranks pharmaceutical, biotechnology, and medical device companies on their social responsibility and governance.
- CUPA co-chair Alison Bateman-House was a member of the Council for International Organizations of Medical Sciences (CIOMS) Working Group XI, which released the report [“Patient Involvement in the Development, Regulation and Safe Use of Medicines”](#) in June. Alison was also recognized by the Morris Principles team for helping “people living with ALS and caregivers become well-informed on matters of clinical trials and pre-approval access to investigational therapies.” The [Morris ALS/MND Principles](#) were led by longtime friend of CUPA Sandy Morris, who died in August from ALS and whom we mourn deeply.
- CUPA co-chair Arthur Caplan received the 2022 NYU Rory Meyers College of Nursing Humanitarian Award, in recognition of his outstanding humanitarian efforts.

CUPA’s research drives the education and advocacy work that we do. Selected pieces in high impact journals this year include:

Research

- Christopher Robertson co-authored, with Margaret Houtz, [“When desperate patients go to court for unproven treatments—the battle for hospital independence,”](#) featured in the *New England Journal of Medicine*.
- Jennifer Miller was joined by Joseph Millum for [“Ethical considerations in the international clinical trial site selection,”](#) published in *BMJ Global Health*.
- Jennifer Miller co-authored, with Tanvee Varma, Camara P. Jones, and Carol Oladele, [“Diversity in clinical research: public health and social justice imperatives,”](#) published in the *Journal of Medical Ethics*.
- Holly Fernandez Lynch and Jinsy Andrews co-authored, with Sandra Morris, [“Access to investigational drugs for patients with amyotrophic lateral sclerosis in the USA,”](#) which appeared in *Lancet Neurology* (sub req).
- Arthur Caplan co-authored, with Rafael Dal-Ré and Voo Teck Chuan, [“Participants’ informed consent in adaptive, platform drug trials in hospitalized COVID-19 patients: Not all approaches are ethically acceptable,”](#) which was published in the *European Journal of Internal Medicine*.
- Laura Kimberly, Holly Fernandez Lynch, and Alison Bateman-House were joined by Patrick Gould and Tasnim Salam on [“Perspectives of academic oncologists about offering expanded access to investigational drugs,”](#) which appeared in *JAMA Oncology*.
- Holly Fernandez Lynch was joined by former CUPA project manager Sage Gustafson and others on [“Estimated clinical trial capacity of sites participating in the Covid-19 convalescent plasma expanded access program,”](#) in *JAMA Infectious Diseases*.

In 2020, we pledged to devote more energy and attention to advancing education in access to investigational medical products. For 2022, we are proud to share some of the products of this pledge:

Education

- Alison Bateman-House taught, with Lisa Kearns, [LAW590A: Access to Investigational Medical Products: Clinical Trials, Expanded Access and Right to Try](#), an intensive online 6-week graduate-level course offered through the University of Arizona Health Law Program. Each year, CUPA and the University of Arizona award full scholarships to a small number of “Patient Advocate Scholars.” This year’s recipients of the scholarships were Oyebode Oluwaseyi Dosunmu and

Rebecca Oberman. For more information about the 2023 course and how to apply for a scholarship, contact law-healthcare@email.arizona.edu.

- In January, we hosted our second biennial CUPA Conference (“CUPACON”): Lessons Learned and Practical Advice Moving Forward.” The daylong event featured representatives from industry, academia, patient advocacy, clinical medicine, news/media, law, and drug regulation. Sessions from the event are posted on our [YouTube page](#). Stay tuned for more info on CUPACON 2024!
- In April, CUPA hosted “Where’s Our Operation Warp Speed? Ethical and Practical Considerations When Adjusting Pandemic Approaches to Non-Pandemic Diseases,” a one-hour webinar featuring CUPA members Holly Fernandez Lynch and Arthur Caplan, as well as Novartis’ Paul Aliu and ALS advocate Gwen Petersen. If you missed it, watch the recording [here](#).
- Each year, CUPA sponsors an “Introduction to Preapproval Access: Expanded Access, Compassionate Use, and Right to Try” webinar. The 2022 event featured an all-CUPA panel of Regine Nshimiyimana Maniraho, Lisa Kearns, Richard Klein, Jinsy Andrews, and Ken Moch. You can watch the recording [here](#).
- CUPA said goodbye to its first undergraduate intern, Rei Marshall, in May and welcomed its second, Doris Aucacama, in September. The Undergraduate Internship Program provides paid, real-world bioethics exposure and work experience to college juniors and seniors from underrepresented populations. Find more information about the internship program [here](#).

CUPA continued to work to ensure that the rules and regulations that guide preapproval access to investigational medicines are just and human. The new Rapid Response Subgroup aims to increase the frequency of and speed at which we publish response statements to new federal or regulatory policies. Look for more “rapid responses” in 2023! Here are some highlights from our work on policy in 2022:

Policy

- Arthur Caplan is a member of the World Health Organization work group that issued “[WHO guidance on the ethical conduct of controlled human infection studies](#).”
- Alison Bateman-House commented on the FDA’s accelerated access program in “[How to make experimental treatment less of a gamble](#),” a Guest Essay in the *New York Times*.
- Sanket Dhruva, from CUPA’s subgroup on Ethics & Real World Evidence (ERWE), was joined by Reshma Ramachandran and Joseph Ross on “[Medicare’s national coverage determination for aducanumab—a one-off or a pragmatic path forward?](#),” in the *New England Journal of Medicine*.
- Alison Bateman-House was a co-author on Critical Path’s “[Rare Diseases Cures Accelerator Data and Analytics Platform \(RDCA-DAP\) best practices and recommendations for FAIR data, toward alignment with international regulatory agencies](#).”
- Holly Fernandez Lynch took part in “[Comparing legal approaches to accessing psychedelics](#),” a webinar for Harvard Law Petrie-Flom Center’s Project on Psychedelics Law and Regulation.
- Arthur Caplan and Ken Moch published “[Breakthrough Therapies Act: Good idea, wrong solution](#),” about a proposed [federal bill](#) that seeks to provide access to potentially beneficial but highly regulated substances like psilocybin and LSD for therapeutic purposes.

CUPA’s voice was heard far and wide in 2022 at conferences, workshops, seminars, guest lectures, grand rounds, and on podcasts and radio shows, including:

- ALS Association/Northeast ALS Consortium
- American Society for Bioethics and Humanities (ASBH) Annual Conference

- Ann & Robert H. Lurie Children’s Hospital of Chicago Pediatric Grand Rounds
- ASGCT Annual Conference
- Biotechnology Innovation Organization (BIO) Annual Conference
- Bioethics Club, Rye Country Day School
- Bioethics Grand Rounds at Toronto’s SickKids: The Hospital for Sick Children
- Brocher Summer Academy in Global Population Health
- Columbia University’s Mailman School of Public Health
- Connecting ALS podcast
- Drug Information Association (DIA) Annual Conference
- Everything ALS
- Gene Therapy Patient Engagement Summit
- Global Health Network’s Epidemic Ethics Series
- Harvard Law Petrie-Flom Center’s Project on Psychedelics Law and Regulation (POPLAR)
- Harvard-MIT Center for Regulatory Science’s Global Conference
- New York Academy of Science
- NORD’s Rare Diseases and Orphan Products Breakthrough Summit
- Operationalize Expanded Access Programs
- PRIM&R Annual Conference
- Professional Patient Advocates in Life Sciences (PPALS) Patient Advocacy Certificate Training Course
- RAD: Research Administration Demonstration Series
- Society for Clinical Trials Annual Meeting
- Stonybrook University’s Center for Medical Humanities, Compassionate Care and Bioethics Grand Rounds
- STAT’s Rare Disease Research: A Prescription
- The Readout LOUD, STAT’s Biotech Podcast
- The Revisionist History Podcast
- Treuman Katz Center for Pediatric Bioethics’ Annual Conference
- WCG MAGI Clinical Research Conference — 2022 West
- World Orphan Drug Congress USA

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

- ABC News
- Amp&rsand – The PRIM&R Blog
- AP News
- Acta Paediatrica
- American Journal of Bioethics
- Bloomberg Law
- BMJ Global Health
- Endpoint News
- Epidemic Ethics Blog
- European Journal of Internal Medicine
- Frontiers in Pharmacology
- Health Affairs
- Inside Precision Medicine
- JAMA Infectious Diseases
- JAMA Oncology
- Journal of Medical Ethics
- Lancet Neurology
- Medical Ethics Advisor
- New York Times
- New England Journal of Medicine
- Petrie-Flom Bill of Health Blog
- Pink Sheet/Pharma Intelligence
- Stat

Thank you for supporting our work over the past year. 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 support CUPA—which operates solely on donations—please contact Lisa Kearns (lisa.kearns@nyulangone.org). Here’s to another productive year in 2023!

Members

* - denotes affiliation with NYU Grossman School of Medicine

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