CUPA is proud to share its 2020 accomplishments in its fifth annual report. During this year of public health crises and political turmoil—and a very visible spotlight on access to investigational medicines and the inequities that plague it—our work and mission have been more important than ever. We continue to seek to advance research, policy, and education regarding the ethical issues surrounding access to investigational medical products, with the goal of ensuring the fair and transparent treatment of all patients.

Research

CUPA members continued to publish widely in the academic literature and mainstream press, advancing the conversation about ethical issues related to preapproval access. Some of our members’ most influential pieces are listed below.

- Former Division of Medical Ethics researcher Jamie Webb and CUPA’s Lesha Shah and Holly Fernandez Lynch argued that “patients seeking access to unproven COVID-19 drugs should receive lower priority for allocation when they decline to participate in clinical trials, either of the requested drug or other investigational products,” in the American Journal of Bioethics on Aug. 25. Their target article, “Ethically Allocating Covid-19 Drugs via Pre-approval Access and Emergency Use Authorization,” ignited a spirited conversation in responses and comments, which you can read through the link above.

- Former CUPA project manager Kelly McBride Folkers, along with Alison Bateman-House and Christopher Robertson, wrote “Paying for Unapproved Medical Products,” which looked at specific payment mechanisms for products that have not been approved by the FDA.


Although most of our attention was focused on Covid-19-related issues, we have continued to evaluate where things stand two years after passage of the federal Right to Try law.

- Former CUPA member Caroline Riley Chapman and former Division researcher Jared Eckman co-authored “Oversight of Right-to-Try and Expanded Access Requests for Off-Trial Access to Investigational Drugs” with CUPA co-chair Alison Bateman-House, for the Jan.–Feb. issue of Ethics & Human Research. They reviewed the regulatory and IRB oversight requirements of the two pathways, ultimately concluding that such oversight is in the interest of patient welfare while acknowledging that the IRB review process could benefit from improvement.

- Alison Bateman-House joined Jeremy Snyder and Leigh Turner in “Is Right to Try Being Tried? Using Crowdfunding Data to Better Understand Usage of Nontrial Pre-Approval Access Pathways,” which confirmed CUPA’s previous analyses that Right to Try is being used much more as an ideology than it is as a regulatory pathway for access to unapproved products.
**Education**

CUPA has made the commitment to focus on education during 2020, as it had received less attention than the research and policy components of our mission in prior years. That commitment was well timed, as bioethics and questions about use of investigational products have never been more in the public spotlight. CUPA members published in popular media, spoke on podcasts and in public webinars, and gave interviews in widely televised programs.

- CUPA co-chair Art Caplan became a regular analyst on CNN early in the pandemic, giving a very public voice and face to the role that bioethics plays in a global public health emergency and access to investigational medicines.

Last January, we hosted the first of an ongoing series of CUPA conferences. This daylong event convened patients and patient advocates, members of the biopharmaceutical industry, clinical researchers, regulators, clinicians, and academics for Non-Trial Preapproval Access to Investigational Medical Products: Lessons Learned and Practical Advice Moving Forward. More people than we could accommodate wanted to register for this series of panel discussions and lunchtime breakout sessions, and, in post-event feedback, attendees said they found the conference valuable but wished it had been longer. Covid-permitting, our next conference will be in January 2022.

CUPA partnered with the University of Arizona Health Law & Policy program to put on the first ever CUPA academic course. This 6-week summer session allowed students in a number of different programs to interact with experts in the field of preapproval access and get an in-depth look at the historical, regulatory, and ethical issues while earning academic credit. We are excited that we were able to offer free enrollment in the course to an inaugural CUPA Patient Advocacy Scholar. Please stay tuned to learn how to apply to become a future Scholar and to participate in this course.

The University of Arizona partnership also co-created a three-part Healthcare and Research Ethics for Covid-19 webinar series. The first episode was “Possible Treatments and Vaccines.” The second episode, “Taking Care of Chronic and Acute Non-Covid Patients During the Pandemic,” was presented in memory of longtime friend of CUPA and a founder of the Max Cure Foundation Richard Plotkin. Perspectives on Real-World Evidence from Expanded Access during Covid-19, “the third episode, featured keynote speaker Michael Joyner, MD, principal investigator for the Expanded Access to Convalescent Plasma for the Treatment of Patients with COVID-19 program at Mayo Clinic, and members of CUPA’s Ethics and Real World Evidence (ERWE) project.

**Policy**

CUPA members have been involved in a wide range of policy actions this year. In late August the FDA issued the proposed rule “Annual Summary Reporting Requirements Under the Right to Try Act” and requested public comments. CUPA flagged several ethical concerns in its public comment to the FDA, including that the reporting timeline is unnecessarily long and that the rule’s definition of “known
serious adverse events” is too passive and fails to require drug manufacturers to take responsibility for learning about such events.

Holly Fernandez Lynch and Alison Bateman-House commented on the Promising Pathway Act, which would establish a limited provisional approval pathway for certain drugs and biologics. They argued that the act would likely undercut patients’ abilities to get robust evidence-based treatment in “Facilitating both Evidence and Access: Improving FDA’s Accelerated Approval and Expanded Access Pathways,” for the Journal of Law, Medicine & Ethics.

Other notable CUPA policy action is listed here.

- Art Caplan joined the World Health Organization panel that is implementing the organization’s Monitored Emergency Use of Unregistered and Investigational Interventions (MEURI) protocol during the coronavirus pandemic.
- Alison Bateman-House was part of a public workshop, “Facilitating End-to-End Development of Individualized Therapeutics,” hosted by the FDA’s Center for Biologics Evaluation and Research (CBER), in Silver Spring, MD, in March. The purpose of the event: “to foster development of individualized therapeutic products for the treatment of one individual or a very small number of patients, based on engineering a product aimed at the specific molecular mechanism underlying a patient’s (or small group of patients’) illness.” Here is a link to a video of the daylong event.

This year at a glance

CUPA members were quoted in, wrote for, and had work cited in the following publications:

- ABC News
- Axios
- BBC
- Barron’s
- BioCentury
- Bioethics Forum
- Bloomberg Law
- Boston Globe
- Business Insider
- CBS News
- CNN
- Daily Beast
- E&E News
- Endpoint News
- Endpoints in Focus
- Factcheck.org
- Forbes
- The Guardian
- Health Affairs
- Health Affairs Blog
- The Hill
- Journal of Law, Medicine & Ethics
- Kaiser Health News
- Life Science Leader
- Los Angeles Times
- MedCity News
- Medscape
- MIT Technology Review
- NBC News
- New York News Daily
- New York Times
- Philadelphia Inquirer
- PRIM&R’s Ampersand Blog
- Pink Sheet/Informa Pharma Intelligence
- Politico
- Popular Science
- S&P Global Market Intelligence
- Salon
- San Jose Mercury News
- Science Magazine
- Scientific American
- Stat
- TMZ
- USA Today
- Washington Post
CUPA members spoke at the following events and for the following groups:

ACRP New York Metropolitan Chapter’s 12th Annual Research Symposium
American Society of Bioethics and Humanity
BIO Digital
Buchanan Ingersoll-Rooney Legal Podcast
Chan Zuckerberg Biohub’s Covid-19 Ethics Initiative
DIA Global Annual Meeting
Early and Managed Access Programs Europe
FDA CBER Public Workshop
Festival of Biologics USA
Global Bioethics Initiative
Harvard Medical School Center for Bioethics
Hastings Center Bioethics Forum
Institute for Mathematical and Statistical Innovation
Institute of Medical Ethics’ European Paediatric Bioethics Conference
Jerusalem Ethics Forum
Medical Affairs Congress
National Academies of Sciences, Engineering, and Medicine
Northeast Amyotrophic Lateral Sclerosis Consortium
Oligonucleotide Therapeutics Society
Orphan Drugs & Rare Diseases Global Congress 2020 Americas—East Coast
Outspoken Oncology Podcast
PRIM&R’s Annual Advancing Ethical Research Conference
Radio.com
Stanford Center for Biomedical Ethics
The Atlantic
The Guardian
The Hill
The New Yorker
The Scientist
Third Annual Expanded Access Summit
Time Magazine
University of Arizona James E. Rogers College of Law’s Health Law & Policy
University of Colorado Anschutz Medical Campus Center for Bioethics and Humanities
University of Minnesota Consortium on Law and Value in Health, Environment & the Life Sciences
University of Pennsylvania Research Ethics and Policy Series
Vanguard Leadership Dialogue series
WCG Webinar Series
WGBH Radio
Wall Street Journal
Washington Post
World Congress of Bioethics
World Orphan Drug Congress USA

As we wrap up our sixth year, CUPA would like to extend our sincere gratitude to members who have been here since the group’s inception: David Curry, Nancy Dubler, Laura Kimberly, Kenneth Moch, Barbara Redman, and Russell Teagarden. We are so grateful for their ongoing time commitment and expertise. CUPA is better because of their contributions.

Although this year has forced us to “meet” our new members in more distant ways, please join us in thanking and welcoming CUPA’s newest members: Aisha Langford, Sage Gustafson, and Regine Nshimiyimana Maniraho.
We appreciate your continued interest in and support of CUPA’s mission! We believe that our work on the ethical issues surrounding preapproval access is of vital, practical import to patients, industry, and the public. If you or someone you know would like to receive our free monthly newsletter, contact Sage Gustafson (sage.gustafson@nyulangone.org). If you would like to support CUPA—which operates solely on donations—please contact Lisa Kearns (lisa.kearns@nyulangone.org).

We’re excited for what we have planned for next year, and for what 2021 will bring for all of us!

Alison Bateman-House (co-chair)
Assistant Professor, Division of Medical Ethics, NYU Grossman School of Medicine

Arthur L. Caplan (co-chair)
Drs. William F. and Virginia Connolly Mitty Professor of Bioethics, Founding Director, Division of Medical Ethics, NYU Grossman School of Medicine

M. Sage Gustafson (CUPA Project Manager)
Research Data Associate, Division of Medical Ethics, NYU Grossman School of Medicine

Jinsy A. Andrews
Assistant Professor of Neurology, Columbia University Division of Neuromuscular Medicine

Hayley M. Belli
Assistant Professor, Division of Biostatistics, NYU Langone Health

David R. Curry
Co-Founder and President, GE2P2 Global Foundation

Nancy N. Dubler
Consultant for Ethics, New York City Health and Hospital Corporation
Adjunct Professor, Division of Medical Ethics, NYU Grossman School of Medicine

Holly Fernandez Lynch
John Russell Dickson, MD, Presidential Assistant Professor of Medical Ethics, Perelman School of Medicine, University of Pennsylvania

Pat Furlong
Founding President and CEO, Parent Project Muscular Dystrophy

Cláudia Hirawat
Executive Chair, VOZ Advisors

Kay Holcombe
Senior Adviser, Milken Institute

Lisa Kearns
Senior Research Associate, Division of Medical Ethics, NYU Grossman School of Medicine

Laura Kimberly
Assistant Research Scientist, Hansjörg Wyss Department of Plastic Surgery, NYU Langone Health

Richard Klein
Director, Expanded Access Programs and Policy, GE2P2 Global Foundation

Aisha Langford
Assistant Professor, Department of Population Health, NYU Langone Health

Andrew McFadyen
Executive Director, The Isaac Foundation

Lindsay McNair
Chief Medical Officer, WIRB-Copernicus Group

Jennifer Miller
Assistant Professor, Yale School of Medicine

Kenneth I. Moch
Senior Advisor to the Chairman, Center for Global Health Innovation and the Global Health Crisis Coordination Center

Regine Nshimiyimana Maniraho
Adjunct Faculty, Thomas Jefferson University and Jefferson Health

Barbara Redman
Associate, Division of Medical Ethics, NYU Grossman School of Medicine

Christopher T. Robertson
N. Neal Pike Scholar and Professor of Law, Boston University

David I. Scheer
President, Scheer & Company, Inc.

Lesha D. Shah
Assistant Professor of Psychiatry, Icahn School of Medicine, Mount Sinai

J. Russell Teagarden

David Wallach
Director of Research Regulatory Services, NYU Langone Health

Tom Watson
Executive Vice President for Early Access Programs, Bionical Emas

Mary Elizabeth Williams
Journalist and Author