

# 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

### 2020 Annual Report

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.
- J. Russell Teagarden and Art Caplan wrote about the role reversal in regulating drug makers in “[Hell Hath Frozen Over: The Pharmaceutical Industry Stands in for a Politically Impaired FDA](#),” in a first opinion for Stat.

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.
- The use of experimental treatments by prominent individuals sparked a wave of publications by and featuring CUPA members, including: “[Trump’s Regeneron Treatment Is a Tangled Ethical Mess](#),” for Barron’s, by Alison Bateman-House, Sage Gustafson, and Art Caplan; “[Trump Gets Regeneron Covid-19 Treatment under ‘Compassionate Use.’ Is that Ethical?](#),” by J. Russell Teagarden; “[What Trump’s ‘Compassionate Use’ of a Covid-19 Drug Use Means—and Doesn’t Mean](#),” by Lisa Kearns, Alison Bateman-House, and Art Caplan; and “[A Bioethicist on Trump’s Treatment Plan](#),” featuring Holly Fernandez Lynch.

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

CUPA members spoke at the following events and for the following groups:

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

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](mailto:sage.gustafson@nyulangone.org)). If you would like to support CUPA—which operates solely on donations—please contact Lisa Kearns ([lisa.kearns@nyulangone.org](mailto: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  
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*Aisha Langford  
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*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  
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*Christopher T. Robertson  
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*David I. Scheer  
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*J. Russell Teagarden*

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

*Mary Elizabeth Williams  
Journalist and Author*

Jan 1, 2021

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