



CUPA

The Working Group on Compassionate Use & Preapproval Access

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

August 2024

Happy Autumn/Spring, Friends of CUPA,
Here's what we have been doing and following over the past six weeks. We would love to hear about *your* work in preapproval access.

###

TRANSPLANT ETHICS AND POLICY CONFERENCE

NYU's Division of Medical Ethics and the NYU Langone Transplant Institute are presenting a daylong in-person/virtual conference on emerging and critical ethical issues in research, medicine, and policy of organ transplantation on Oct. 17, 8:30 am–4 pm ET. A current issue of interest to CUPA is the fact that xenotransplantation, in which tissue or organs from one species are transplanted to another—most famously and recently genetically modified pig kidneys and hearts have been transplanted in humans—is regulated under FDA's Expanded Access pathway. Register and find more information, including the agenda and list of speakers, [here](#).

###

SAVE THE DATE FOR THE NEXT CUPA WEBINAR

Speaking of xenotransplantation, CUPA's Advanced Topics fall webinar will be devoted to expanded access oversight of xenotransplantation and will take place on Nov. 20, 12–1 pm ET. More information is coming soon, but please mark your calendars!

###

PRIM&R WEBINAR

Public Responsibility in Medicine and Research is holding a webinar on "[IRBs and Expanded Access: Ethical Considerations and Implementing FDA Guidance](#)" on Tues., Nov. 12, 1–2:30pm ET. A panel of representatives from the FDA, industry, and academia—including CUPA's Holly Fernandez Lynch and Christine MacCracken, head of patient strategies and solutions at Johnson & Johnson—will discuss recent regulatory and policy developments in expanded access, as well

as ethics issues involved. Find more information, including how to obtain CE credit, and register through the link above.

###

FDA

“[FDA Rare Disease Innovation Hub to enhance and advance outcomes for patients](#)” describes a new initiative by CDER and CBER (FDA’s centers for drug and for biologics evaluation and research) to spur development of treatments for rare diseases. Led by Patrizia Cavazzoni and Peter Marks, the directors of CDER and CBER, respectively, the Hub “will work across rare disease but will especially focus on products intended for smaller populations.” Read more through the link above.

On the latest edition of the “Q&A with FDA” podcast, which discusses recent regulatory topics, the Division of Drug Information talks about “[Real-World Data and Evidence Generation](#)” with FDA CMO Dr. Hilary Marston. On the same topic, the agency recently released criteria for “[Real-World Evidence Submissions to the Center for Biologics and Research & the Center for Drug Evaluation and Research](#).” (See related article below.)

###

BABE RUTH, EARLY ACCESS PIONEER

We recently learned that baseball great—to some, the greatest—George Herman Ruth received an experimental drug to treat the nasopharyngeal cancer that claimed his life at age 53. In his 1948 biography, Ruth wrote, “I realized that if anything was learned about that type of treatment, whether good or bad, it would be of use in the future to the medical profession and maybe to a lot of people with the same trouble. So I took the shot.” Read more in Howard Markel’s [PBS article](#) and Theodore Pappas and Nadim Bikhazi’s [research article](#) in Ear, Nose & Throat Journal.

###

CUPA RECORDINGS ON OUR YOUTUBE CHANNEL

ICYMI, the recording of our 5th annual Expanded Access 101 webinar, “An introduction to Preapproval Access,” is now up on our [YouTube channel](#). This year’s event was co-sponsored by the International Society for Cell & Gene Therapy (ISCT) Expanded Access Working Group and, as always, covers the basics of EA for all stakeholders.

The latest video from our biannual CUPA Con conference, which was held in late January in New York (and online), is also now on the CUPA [YouTube channel](#). “What’s Special About Expanded Access for Gene and Cell Therapies?” joins FDA commissioner Robert Califf’s keynote address, “Beyond Expanded Access: Legislative Approaches to Access”; an interview about [Project Facilitate](#); panels on “Balancing Access & Evidence Generation” and “The Ethics and Business Cases for and Against Expanded Access”; and the “The Patient Narrative,” with Lucy

and Mike Carroll, founders of the advocacy group [Ollie's Army](#), and Adrienne Nicole of Adrienne Nicole Productions. Keep checking back for more.

###

DON'T YOU WANT TO BE A PART OF IT ALL?

We *know* you like the webinars, analysis, conferences, advocacy, and outreach we do, because you're reading this! Due to funding issues, CUPA has been unable to sponsor an undergraduate intern this fall. This is an opportunity for us to introduce students from groups historically underrepresented in bioethics both to the field and to the research, education, and policy work we do in preapproval access.

A contribution of any amount will help us continue our mission (and, of course, **keep these monthly updates flowing to your inboxes**). Contact Lisa Kearns at lisa.kearns@nyulangone.org for information about how to support CUPA with a gift large or small.

###

Upcoming Events:

Alison Bateman-House will discuss "The Ethics of Phase 1 Trials" at the [American College of Clinical Pharmacology Annual Meeting](#) on Sept. 10 in N. Bethesda, MD.

Holly Fernandez Lynch will be on a panel for PRIM&R's November webinar on "[IRBs and Expanded Access: Ethical Considerations and Implementing FDA Guidance](#)"; find details in the separate item up top.

*If you'd like to request a speaker for your group or event, contact Lisa Kearns:
Lisa.Kearns@nyulangone.org*

###

CUPA Members in Print and at the Mic:

Art Caplan spoke about "Public Health Emergencies & Compassionate Use" at the World Medical Association North America Regional Meeting for the Declaration of Helsinki on Aug. 15, Washington, DC.

Alison Bateman-House's article "[Somatic Gene Therapy: Ethics and Access](#)" was published in the August issue of Annual Review of Genomics and Human Genetics.

Holly Fernandez Lynch was quoted in "['Jerking families around': Canceled Roche rare disease trial devastates parents, angers researchers](#)" (Stat+ sub req)

Alison Bateman-House spoke about “Clinical Trials & Non-Trial Access to Investigational Medical Products” to the second-year students at NYU Grossman Long Island School of Medicine on Sept. 3.

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 on Aug. 2

Art Caplan and Alison Bateman-House were quoted in “[FDA reports sparse use of Trump’s Right to Try program](#)” (Politico Pro sub req)

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Articles of Interest:

(Note: Articles below reflect current discussions in preapproval access and should not be considered CUPA endorsements.)

Gardner FDA Law Firm, “[FDA unveils criteria for reporting real-world evidence \(RWE\) submissions](#)”

A Wesevich, M Ratain, “[Accelerated Approval Program versus NCCN guidelines as mechanisms for early drug access](#)”

C Bobier, D Hurst, D Rodger, “[Xenotransplantation under the Food and Drug Administration’s Expanded Access pathway](#)” (American Journal of Transplantation sub req)

N Emmot, M D’Ambrosio, et al, “[Applying real-world data and real-world evidence for accelerated approvals and coverage decisions](#)”

Duke Margolis Institute for Health Policy, “[2024 State of Real-World Evidence Policy](#)” (agenda, slide deck, and speaker bios)

A Conti, “[In marked shift, the FDA is increasingly tolerant of single pivotal trials to support approval](#)”

J Weishaupt, P Körtvélyessy, et al, “[Tofersen decreases neurofilament levels supporting pathogenesis of the SOD1 p.D91A variant in amyotrophic lateral sclerosis patients](#)”

C Belsterling, “[University Hospitals implants first-in-human patient-specific 3D printed tracheal T-tube \[via expanded access pathway\]](#)”

B Adlin, “[Federal court hears doctor’s dispute with DEA over giving psilocybin to terminally ill cancer patients](#)”

C Cirruzzo, B Leonard, “[Trump’s pat on the back for Right to Try](#)”

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Thanks very much for following our work.

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