**Fair Access and Equity of Individualized Interventions for Ultrarare Genetic Conditions**

**Date and time:** Friday, September 8, 2023 | 12pm ET US / 9am PT US

**Speakers:** Alison Bateman-House, PhD, MPH & Ingrid Holm, MD, MPH

**Moderator:** Meghan Halley, PhD, MPH

**Biographies**

**Moderator:** Meghan Halley, PhD, MPH

**Biography**: <https://elsihub.org/directory/meghan-c-halley>

**Panelist:** Alison Bateman-House, PhD, MPH

**Biography:** <https://elsihub.org/directory/alison-bateman-house>

**Panelist:** Ingrid Holm, MD, MPH

**Biography:** <https://www.childrenshospital.org/directory/ingrid-holm>

**ELSIhub Collections**

ELSIhub Collections are essential reading lists on fundamental or emerging topics in ELSI, curated and explained by expert collection editors. Please use the link to access *Paying for Cures: The Ethics and Economics of Gene Therapies for Rare Diseases* curated by Meghan Halley, PhD, MPH, Senior Research Scholar, Center for Biomedical Ethics, Stanford University School of Medicine.

[https://elsihub.org/collection/paying-cures-ethics-and-economics-gene-therapies-rare-diseases](https://nam02.safelinks.protection.outlook.com/?url=https%3A%2F%2Felsihub.org%2Fcollection%2Fpaying-cures-ethics-and-economics-gene-therapies-rare-diseases&data=05%7C01%7Cts3508%40cumc.columbia.edu%7C242b793f2c85424bfd3008dbaef9eb76%7Cb0002a9b0017404d97dc3d3bab09be81%7C0%7C0%7C638296160650292899%7CUnknown%7CTWFpbGZsb3d8eyJWIjoiMC4wLjAwMDAiLCJQIjoiV2luMzIiLCJBTiI6Ik1haWwiLCJXVCI6Mn0%3D%7C3000%7C%7C%7C&sdata=GMZn8t4O14GOxJvF45f1eQ%2FVAXNMrHfKJEWxPRKrTJQ%3D&reserved=0)

**Meghan Halley, PhD, MPH**

Link to “Scientists Designed a Drug for Just One Patient. Her Name Is Mila” (2019): <https://www.nytimes.com/2019/10/09/health/mila-makovec-drug.html>

Link to “Patient-Customized Oligonucleotide Therapy for a Rare Genetic Disease” (2019): <https://doi.org/10.1056/NEJMoa1813279>

Link to “Drug Regulation in the Era of Individualized Therapies” (2019): <https://doi.org/10.1056/NEJMe1911295>

**Ingrid Holm, MD, MPH**

Link to “Preparing n-of-1 Antisense Oligonucleotide Treatments for Rare Neurological Diseases in Europe: Genetic, Regulatory, and Ethical Perspectives” (2022): <https://doi.org/10.1089/nat.2021.0039>

Link to “A framework for individualized splice-switching oligonucleotide therapy” (2023): <https://www.nature.com/articles/s41586-023-06277-0>

Link to “Personalized medicine is having its day” (2023): <https://www.nature.com/articles/s41587-023-01724-9>

Link to “Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy” (2017): <https://www.nejm.org/doi/full/10.1056/nejmoa1702752#:~:text=Infants%20who%20received%20nusinersen%20had%20a%20significantly%20higher%20likelihood%20of,receiving%20ventilatory%20support%20at%20baseline>.

Link to “Patient-Customized Oligonucleotide Therapy for a Rare Genetic Disease” (2019): <https://www.nejm.org/doi/full/10.1056/nejmoa1813279>

Link to “Antisense therapies: A new approach to tackling challenging targets in areas of high unmet medical need” (2023): <https://www.openaccessgovernment.org/antisense-therapies-tackling-challenging-high-unmet-medical/156151/>

**Alison Bateman-House, PhD, MPH**

Link to n-lorem Foundation: <https://www.nlorem.org/>

Link to “IND Submissions for Individualized Antisense Oligonucleotide Drug Products for Severely Debilitating or Life-Threatening Diseases: Chemistry, Manufacturing, and Controls Recommendations Guidance for Sponsor-Investigators”: <https://www.fda.gov/media/154664/download>

Link to “Gene therapy death not caused by CRISPR, investigators confirm” (2023): [https://www.statnews.com/2023/05/18/gene-therapy-death-not-caused-by-crispr-investigators-confirm/](https://www.statnews.com/2023/05/18/gene-therapy-death-not-caused-by-crispr-investigators-confirm/#:~:text=Gene%20therapy%20death%20not%20caused%20by%20CRISPR%2C%20investigators%20confirm,-By%20Jason%20Mast&text=Terry%20Horgan%2C%20the%2027,deliver%20the%20treatment%2C%20investigators%20concluded)

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**Audience Contributions**

Link to “Individualized interventions for rare genetic conditions and the research-treatment spectrum: Stakeholder perspectives” (2023): <https://doi.org/10.1016/j.gim.2023.100832>

Link to “Oversight of Right-to-Try and Expanded Access Requests for Off-Trial Access to Investigational Drugs” (2020): <https://doi.org/10.1002/eahr.500038>

Link to N=1 Collaborative: <https://www.n1collaborative.org/>