00:32:41 Tiana Sepahpour: Welcome to ELSI Friday Forum! We are glad you will spend the hour with us. We ask that all attendees please abide by the code of conduct, which can be found here: https://elsihub.org/news/code-conduct

Following the webinar will be an opportunity to continue the conversation in another Zoom meeting. Please consider joining us then at this link, which will also be sent towards the end of the hour: https://columbiacuimc.zoom.us/j/92213824621

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00:34:59 Tiana Sepahpour: Are you familiar with MePan? My adult son is one of only approximately 30 patients diagnosed worldwide. I know there is research being conducted. Which seem most promising?

00:35:07 Tiana Sepahpour: 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

00:35:32 Tiana Sepahpour: Please use the “cc” button at the bottom of the screen to access closed captioning.

Use the Q&A function to ask questions. “Like” a question in the Q&A to push it towards the top of the queue. We will post links to references in the chat and please feel free to add your own. A recording of this session and all references posted in the chat will be available on ELSIhub.org. If you have questions, please email: info@elsihub.org

00:36:26 Tiana Sepahpour: Moderator: Meghan Halley, PhD, MPH

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

00:37:43 info@elsihub.org - Dounya Alami-Nassif (she/her): Today's presentations can be accessed here and will be posted on ELSIhub following the webinar.

00:41:35 Tiana Sepahpour: Panelist: Ingrid Holm, MD, MPH

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

00:41:58 Tiana Sepahpour: Panelist: Alison Bateman-House, PhD, MPH

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

00:43:30 Tiana Sepahpour: 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

00:44:00 Tiana Sepahpour: Link to “Patient-Customized Oligonucleotide Therapy for a Rare Genetic Disease” (2019): https://www.nejm.org/doi/full/10.1056/nejmoa1813279

00:45:15 Tiana Sepahpour: 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/

00:54:45 Tiana Sepahpour: Link to N-Lorem Foundation: https://www.nlorem.org/

01:01:20 Tiana Sepahpour: 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

01:04:59 Tiana Sepahpour: 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/

01:09:49 Tiana Sepahpour: Email: Alison.Bateman-House@nyulangone.org

Twitter: @ABatemanHouse

01:12:33 paul vrana: As someone who works in the (ultra) rare disease space, it occurs to me that there's another issue here- if you have an N=1/ handful of patients with variants in said gene, you don't have a lot of evidence for the gene-disease relationship. That raises another ethical question in designing the therapy - you could successfully employ an oligo towards the variant of question, but what if that is not actually the causal variant?

01:21:08 Paul Appelbaum: Per the issue mentioned earlier regarding whether use of these therapies for ultra-rare conditions are research or treatment, see: Individualized interventions for rare genetic conditions and the research-treatment spectrum: Stakeholder perspectives. Genetics in Medicine, https://www.gimjournal.org/article/S1098-3600(23)00845-6/fulltext

01:21:13 Alyx Vogle: I think this is exceptionally difficult hearing from people who seemingly don't belong to typically undeserved communities

01:21:28 Alyx Vogle: underserved\*

01:25:49 Carolyn Chapman: Paper on oversight of requests for investigational drugs outside of clinical trials https://onlinelibrary.wiley.com/doi/abs/10.1002/eahr.500038?casa\_token=KTUkHgmuHJ4AAAAA:WiLA-zenC\_duoCOpQ6LcFolp9NGjPDm3pSZtyyyCX1IFs\_4y33MQkaco\_hEksJl-5XXuZ0UzTx61Dw

01:30:37 Tiana Sepahpour: We hope you will consider joining us at the link below to continue the conversation: https://columbiacuimc.zoom.us/j/92213824621

We would appreciate your feedback. Please fill out our survey to discuss your experience and to make suggestions for topics and speakers: https://cumc.co1.qualtrics.com/jfe/form/SV\_3JftvNFzeRJq3Vc

We invite you to register for our next ELSI Friday Forum - “The Genomics of PTSD Risk: Scientific and Ethical Perspectives” on November 10 at 12pm ET: https://us06web.zoom.us/webinar/register/WN\_wB3qqCC4RqiLRgDD-7jecQ#/registration

We invite you to browse ELSIhub Collections. ELSIhub Collections are essential reading lists on fundamental or emerging topics in ELSI, curated and explained by expert Collection Editors, often paired with ELSI trainees. https://elsihub.org/resources/collections

01:33:17 Alix Hall: Thanks so much for holding this session, everyone, was a really insightful discussion.