Use of Neurophysiological Markers to Design and Enrich Clinical Trials in Rare Neurological Disorders

Chairs:
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Disclosures

Kemi Olugemo

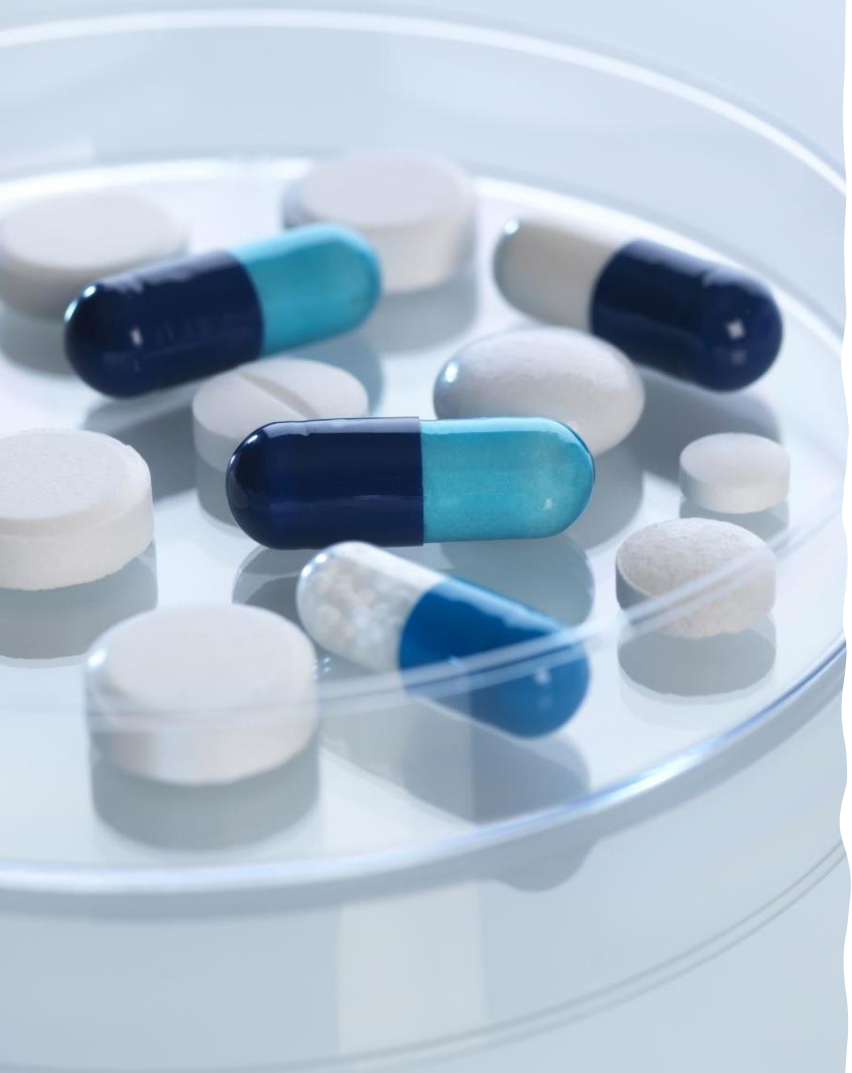
Employed by Korro Bio

Uma Vaidyanathan

Consulting fees with Wellcome Trust Consulting Fees with Woebot

Speakers

Allyson Berent, DVM, DACVIM,CSO
Elizabeth Berry-Kravis, MD, PhD
Justin Brooks, MD, PhD
David Matthews, PhD
Kemi Olugemo, MD, FAAN
Uma Vaidyanathan, PhD

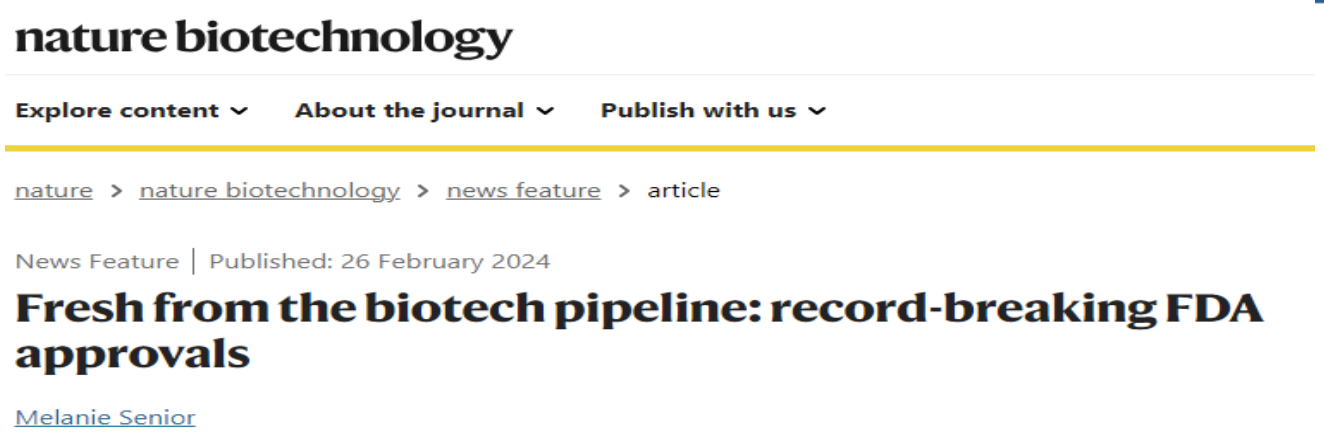


Why this session is needed

- Orphan disease drug development is challenging for many reasons – disease heterogeneity, paucity of natural history data, few and geographically dispersed patients, lack of regulatory precedents, etc.
- Need more sensitive, reliable, non-invasive endpoints and biomarkers to assess disease progression and treatment response
- EEG, Polysomnography and other neurophysiology tools under-utilized
- Findings and methodology applicable to rare and more prevalent diseases

Precision and personalized medicine is the north star

New drug approvals reached an all-time high in 2023, with five gene therapies, the first CRISPR–Cas9-edited therapy and a disease-modifying Alzheimer's drug.



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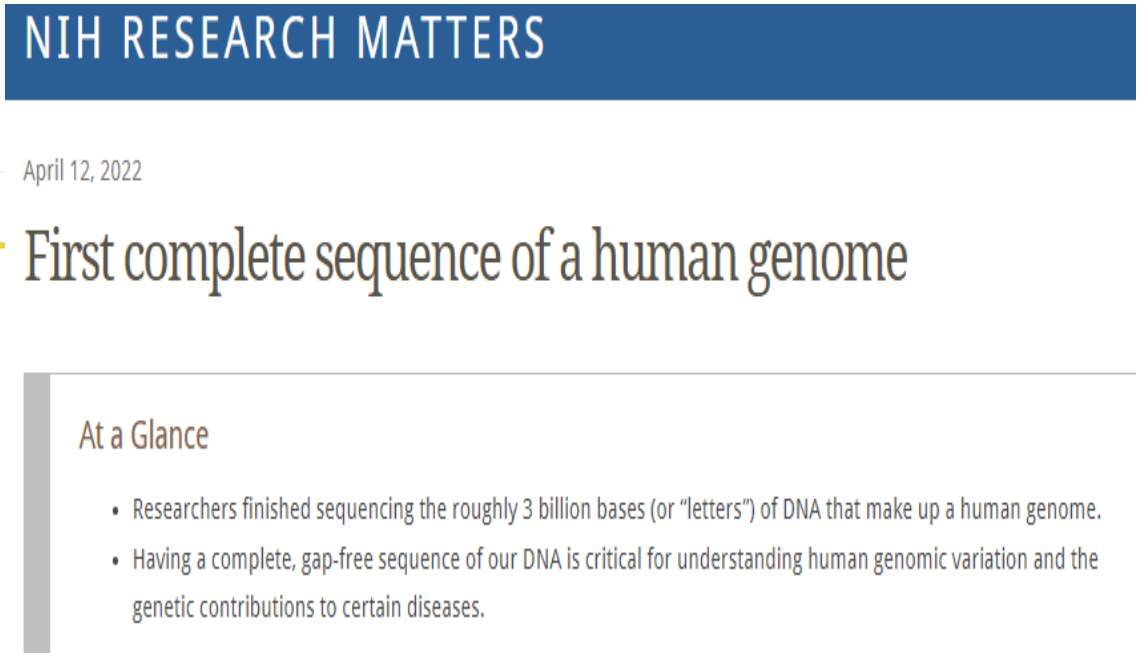
News Feature | Published: 26 February 2024

Fresh from the biotech pipeline: record-breaking FDA approvals

Melanie Senior



NIH RESEARCH MATTERS

April 12, 2022

First complete sequence of a human genome

At a Glance

- Researchers finished sequencing the roughly 3 billion bases (or "letters") of DNA that make up a human genome.
- Having a complete, gap-free sequence of our DNA is critical for understanding human genomic variation and the genetic contributions to certain diseases.

Vertex and CRISPR Therapeutics Announce US FDA Approval of CASGEVY™ (exagamglogene autotemcel) for the Treatment of Sickle Cell Disease

– First-ever approval of a CRISPR-based gene-editing therapy in the U.S. –

Session Topics ~ 3 Hours

- The parents' journey through drug development: Making the impossible possible for Angelman syndrome
- EEG as a marker of direct mouse-to-human translation in clinical trials in neurodevelopmental disorders
- Beyond PSG: Sleep assessment endpoints, considerations and recommendation
- Computational approaches to unlocking EEG and PSG for biomarker discovery in neurodevelopmental and sleep disorders
- Panel discussion with regulatory considerations