What I Learned At

World Orphan Drug Congress Boston, 2024

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What is WODC?

- **World Orphan Drug Congress (WODC)** is a conference for stakeholders in the orphan drug and rare disease space.
- Strategies for escalating development of drugs, treatments & therapies are discussed, as well as increasing access for patients.
- Attendees include biopharma companies, government & regulatory authorities, patients, patient advocacy groups & solution providers.

Rare Disease

a disease or condition that affects less than 1 in 2,000 people.

300 MILLION

Monetizing Hope

Orphan Drug

a drug used to treat, prevent or diagnose a rare disease or condition

- Panelists included Matt Salo of **SALO HEALTH**, Patroski Lawson of **THE KPM GROUP DC**, Kevin Bagley of **BAGLEY STRATEGIC CONSULTING** & James Griffin, **SICKLE CELL PATIENT ADVOCATE**
- Discussion: access, affordability & coverage for patients & the FDA's role in the Medicaid space.
- Emphasis on the importance of clear communication & relationship building between all stakeholders to drive best outcomes for patients.
- Innovations being available does not mean they are easily accessible to patients because state's have fixed medical budgets for Medicaid.

AI & Social Listening

- Personalized Endpoints in Clinical Trials: Exploring Goals with AI & Social Listening
- Presented by Chere Chapman of **ARDEA OUTCOMES** & Max Flurie of **TREND COMMUNITY**
- Discussion: how AI & social listening can accelerate the GAS (Goal Attainment Setting) process. GAS uses quantitative & qualitative data to help clinical researchers learn more about patients in clinical trials.
- Focus on anecdotal data from Facebook groups for Prader-Willi Syndrome. Conversation maps helped evaluate what is important to the community.

Engaging with FDA Ombuds

Ombuds

work with individuals or groups to provide a safe space to discuss concerns and resolve conflicts

- Navigating Difficult Pathways in Rare Drug Development: How, When & Why Sponsors Should Engage with FDA Ombuds
- Presented by Jenn McNary of **ONE RARE** & Sherry Lard, PhD, **US FDA**
- Discussion: how Ombuds work to foster good communication between parties for conversations that can impact clinical study design & help all parties meet in the middle.
- Types of Dispute Resolutions: Informal have more opportunity for engagement. Formal have built-in timeframes. Choose one: they can't be done simultaneously.

Radical Inclusion

- Roundtable Discussion moderated by Richie Kahn of **Canary Advisors** & Donna Mackey of **Akouos Inc.**
- Conversation with rare disease stakeholders from across the ecosystem about strategies that best work for rare disease & ultra rare disease clinical trials.
- Patients with ultra-rare conditions often know one another; word of mouth can help share information about clinical trials & process.
- Considerations: Compensation & structure. Some clinical trial participants may risk losing their health insurance if/when compensation comes into play. In-home trials can reduce patient & caregiver/care partner burden. Other challenges? Scaling diversity & navigating medical waivers.

Making Data Inclusive

- Keynote: AI & Digital Health for Rare Diseases: Making Data Inclusive
- Panelists included Harsha Rajasimha of **INDOUSRARE**, Joshua Resnikoff of **TMA PRECISION HEALTH** & Spencer Huggett of **SYNAPZE**
- A discussion around how ChatGPT & other AIs have unlocked insights about how patient data is handled & can best be protected.
- The use of AI as a tool in rare disease diagnosis & promoting data sharing & collaboration across international borders.

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SOURCES

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