TKG RARE DISEASE

TKG RARE DISEASE QUARTERLY Newsletter

The rare disease drug development process faces several challenges from finding enough patients who fit inclusion and exclusion criteria, to designing a trial that is simplified and flexible to enroll enough patients and ensuring quality clinical trial outcomes data.

SOCIAL MEDIA IN RARE DISEASE DRUG DEVELOPMENT

Social media data currently influences the rare disease drug development process as a method to enable researchers to:

- Discover unmet needs
- Describe disease burden (humanistic, economic, quality of life)
- Assess caregiver burden
- Discover factors that potentially determine patient preferences
- Recruit and survey hard-to-find patient populations through online communities
- Reveal potential information gaps

However, using social media in rare disease drug development requires caution. Manufacturers must navigate how to:

- Protect patient privacy by efficiently de-identifying personal health information
- Assure quality, constant, and reliable data
- Prevent the unbinding of patient group assignment

In a recent study for an ultra-rare pediatric disease, researchers recruited and surveyed the ***largest reported contemporary cohort*** of 671 people born with a single functional ventricle in their hearts by using ***Facebook, Twitter, and other social media platforms***.

TKG's collaboration with an artificial intelligence partner may provide an opportunity to create innovative solutions for our clients.

ARTIFICIAL INTELLIGENCE OPPORTUNITIES

Given the limited clinical research and knowledge among rare diseases, artificial intelligence (AI) presents several unique opportunities to bring innovative solutions to the space.

AI-powered social listening can monitor and analyze data from hundreds of sources and identify patterns in the data. For rare diseases where information is typically scarce, this can play a pivotal role in the identification of patients, uncovering unmet needs, and guiding drug development and clinical trials.

TKG's ongoing collaboration with an AI partner seeks to improve quality of life by shortening the time it takes to appropriately identify conditions and leveraging the voices of patients at risk of hitting the 'four walls' of health care through social listening and AI.

For more information about this partnership, please contact Mindy Olivarez, ***mindyo@thekinetixgroup.com***

The FDA's Center for Drug Evaluation and Research (CDER) announced the creation of the Accelerating Rare disease Cures (ARC) program.

RARE DISEASE DIGEST

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Through the ARC Program, the FDA aims to support innovation and quality in the drug development pipeline for rare diseases. Priorities for ARC include strengthening partnerships with internal and external stakeholders and engaging with external experts to better identify solutions to challenges in rare disease drug development, incorporate the patient's perspective in clinical outcome assessment measures, and build clinical trial readiness in the pre-competitive space.

While rare disease drug development is on the rise, there is still a significant unmet need in FDA-approved therapies. The introduction of the ARC program solidifies the FDA's commitment to supporting the development new treatments for patients with rare diseases. For more information, visit https: //www.fda.gov/drugs/regulatory-science-research-and-education/rare-disease-cures-accelerator.