

A PARADIGM SHIFT IN COLLECTING RWD FOR GM2 GANGLIOSIDOSIS

Coolidge K¹; Araujo R²; Lewi D¹; Gwadry-Sridhar F¹

¹Pulse Inframe, Canada ²Sanofi, USA

Email: kcoolidge@pulseinframe.com

BACKGROUND

Collecting real-world data on rare diseases presents well-documented challenges. Despite numerous initiatives in GM2 gangliosidosis to gather RWD, the persisting challenge lies in the fragmented nature of the data from diverse stakeholders. A significant proportion of these data are isolated, focusing solely on particular facets of the disease. Unfortunately, they often lack essential components such as clinical information, genomic data, biomarkers, treatment history, and patient-reported data. This fragmentation hampers comprehensive insights and collaborative advancements in addressing GM2 gangliosidosis as treatments are explored for the disease. Nevertheless, the recent FDA guidance on considerations for utilizing Real-World Data (RWD) underscores the regulatory significance of establishing a unified data set for collaborative efforts.

APPROACH

In response to this pressing unmet need, we have devised and executed an innovative approach aimed at benefiting all stakeholders, necessitating a paradigm shift. Recognizing the paramount importance of achieving a comprehensive understanding of the disease through collaborative efforts, we have undertaken a detailed exploration of the barriers involved. By conducting in-depth interviews with Key Opinion Leaders (KOLs) and patient advocacy, we have strategically outlined a RWD strategy for GM2 gangliosidosis that adheres to regulatory guidelines. This strategic approach will ensure that pertinent data can be systematically harmonized and mapped to a standardized data framework for use in developing treatments for the disease.

Start defining RWD strategy for GM2

KOL Interviews

Define governance structure

Advocacy Interviews

Map to data standards

Ingest data

To be continued

NEXT STEPS

Our innovative approach that was developed, rooted in collaborative dialogue and guided by a carefully crafted RWD strategy, demonstrates a promising solution to the challenges faced in GM2 gangliosidosis research where the drug development process can be slowed down due to redundant RWD initiatives. By addressing the pitfalls of duplicative efforts and emphasizing transparency, we not only navigate the intricacies of rare disease data collection but also pave the way for enhanced trust and efficiency in drug development that mitigates risk. This methodology not only showcases the potential for comprehensive insights into GM2 gangliosidosis but also sets a precedent for the future of rare disease research, offering a glimpse into a more streamlined and impactful approach to scientific advancement in this critical field.

SUMMARY

Steering through the intricacies of a RWD strategy for a rare disease involves numerous complexities; however, adopting a collaborative and transparent methodology can effectively streamline RWD process, propelling advancements in treatment modalities and creating alignment for all stakeholders.

Start

Review of siloed data sources

Identify key missing data

