A PARADIGM SHIFT IN COLLECTING RWD FOR GM2 GANGLIOSIDOSIS

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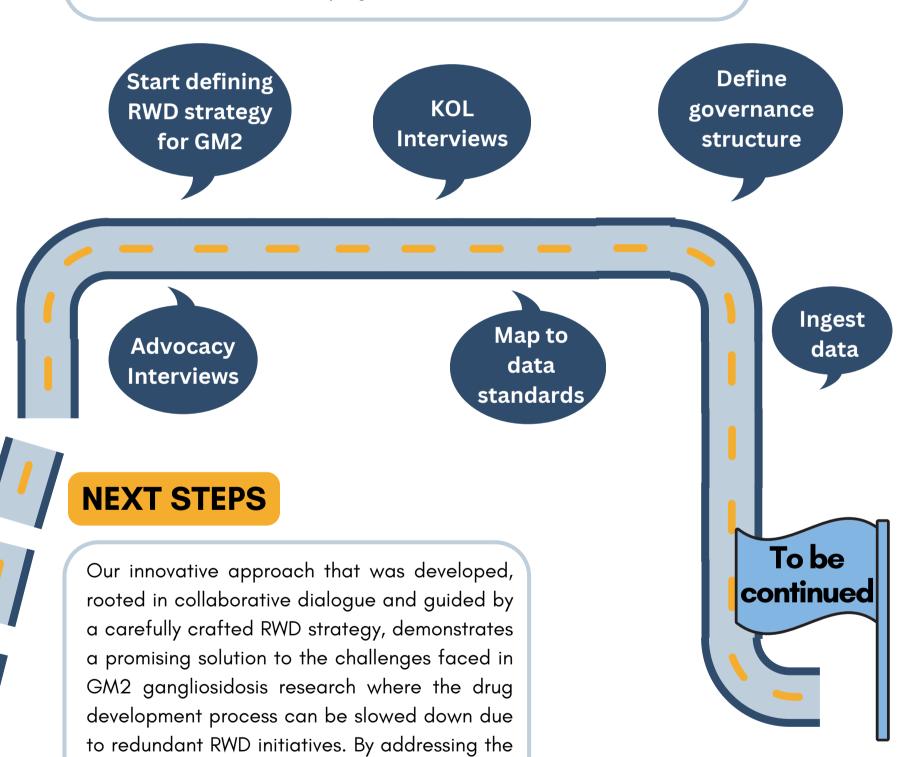
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BACKGROUND

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, patient-reported data. This and fragmentation hampers comprehensive and collaborative insights advancements in addressing GM2 gangliosidosis as treatments explored for the disease. Nevertheless, recent FDA guidance 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.



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

gangliosdosis 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.

siloed data sources

Review of

Identify key missing data



Start

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.