Title: The GM1 Natural History Data Sharing Collaborative: A roadmap for accelerating rare disease research

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Background:
GM1-gangliosidosis (GM1) is an ultra-rare neuronopathic lysosomal disease, that is uniformly lethal, and for which there is no approved therapy. Due to the rarity and severity of the disease, use of historical control data as an external comparator in interventional clinical treatment studies is essential, in addition to collection of prospective data.

Objectives:
The aim of this research project is to build a forum for collaboration and aggregation of natural history data that will increase understanding and establish meaningful clinical endpoints and a comparator control dataset suitable for clinical treatment trials and regulatory filings.

Methods:
The CureGM1 Foundation began a global initiative, The GM1 Natural History Data Sharing Collaborative, a network of disease experts, organizations and individuals conducting natural history studies in GM1. The Collaboration developed a Charter of principles that serves as the foundational framework for the practice of sharing data and identifying roles and responsibilities, including periodic formal data assessments prior to data analysis. The Critical Path Institute's Rare Disease Accelerator Platform, a U.S. Food and Drug Administration funded initiative, serves as central repository for storage, aggregation, interrogation and analysis of the data.

Results:
Investigators conducting 4 international natural history studies are early adopters, contributing deidentified data into a unified precompetitive database. Preliminary key GM1 data elements for supporting longitudinal data updates have been identified and are presented herein.

Conclusions:
The Collaboration is the first-ever precompetitive consortium for GM1 natural history data sharing. It provides a roadmap for identifying the right parties, confirming patient consent for broad data sharing, selecting the data platform, putting a suitable legal structure in place, establishing leadership and the appropriate goals for all constituents, and for keeping parties motivated. Importantly, this project has a broader application in that it provides a new model for accelerating clinical trial readiness for any rare disease.