THE GM1 NATURAL HISTORY DATA SHARING COLLABORATIVE: A ROADMAP FOR ACCELERATING RARE DISEASE RESEARCH

Christine Waggoner¹, Kathleen Kirby¹, Vanessa Rangel Miller¹, Roberto Giugliani², Jeanine Jarnes³, Cara Weismann⁴, David Whiteman¹, James Wilson⁵, Rafael Escandon¹, Collin Hovinga⁶

¹Cure GM1 Foundation, Albany, CA, USA; ²Casa dos Raros, Porto Alegre, Brazil; ³University of Minnesota, Minneapolis, MN, USA; ⁴Orphan Disease Center, University of Pennsylvania, Philadelphia, PA, USA; 5Institute for Life Changing Medicines, Philadelphia, PA, USA; 6Critical Path Institute, Tucson, AZ, USA





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.

Objective

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 Cure GM1 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. [FIGURE]

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 will be detailed in future publications.

Discussion

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.

Identifying key stakeholders Leadership and obtaining their commitment **Motivation & Engagement Throughout**



Legal

- This step can take the longest
- Select attorney with multi-party experience
- Draft agreement template





Charter

- Create outline with critical sections
- Group review and revision
- Set data sharing heiarchy
- Establish data management/security
- Set licensing standards
- REF: insert publication
- REF: Mascalzoni DataSharingCharterDevelopment 2015 EuJourHumGen



Sharing Platform

Effective Collaboration

- Specify data formats
- Set access controls
- Plan data assessments / analyses



Patient Consent



- Enable broad data sharing in consent language
- If updating is needed, revise consent, seek necessary approval, obtain re-consent



Projects & Collaborations

- Secure funding from industry members early on
- Consider grant applications
- Define data objectives
- Identify resource needs
- Plan deliverables



Additional thoughts and ideas in support of this roadmap