Best Data Practices for Rare Disease Patient Foundations and Researchers

Project Scope
This project aims to create a guidance about best data practices for patient foundations and for researchers in the form of decision trees, graphics, short printable materials or videos.

Project Statement
The purpose of this Working Group is to create awareness and education about best practices for collecting regulatory quality data from the industry perspective, improving data quality, empowering patients and patient groups with knowledge and information, and building bridges between industry, academics and patient communities.

Project Impact
This project will create greater partnerships between industry, researchers and the patient community. It will also help improve data collection, with the goal of the data being useful to the industry.

Project Leads	Email
Sophia Zilber, *Alexion AstraZeneca Rare Disease, Cure Mito Foundation*	sophia.zilber@alexion.com
Danielle Boyce, *Johns Hopkins University*	dboyce3@jhu.edu
Paul Howard, *Amicus Therapeutics*	phoward@amicusrx.com
Nicola Newton, *PHUSE Project Assistant*	nicky@phuse.global

CURRENT STATUS **Q1 2024**

- Drafted a lay language summary of recent FDA guidance

Objectives & Deliverables	Timelines
Decide on a strategy to share all three resources, possibly through PHUSE Education webinars	Q2 2023

Published Deliverables	
Ensuring Registry Data Relevance and Reliability for Regulatory Use	4 April 2024
Myths VS Facts About Patient Registries	18 May 2023
Patient Foundations Guide to Starting a Registry	28 April 2023
Patient Registry Transparency Checklist For Patient Foundations	28 April 2023