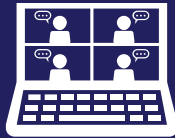


## Promoting Patient-Focused Drug Development

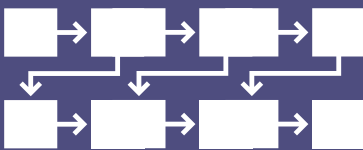
Externally-Led  
Patient-Focused  
Drug Development  
**EL-PFDD meeting for PMS**  
before the FDA



**\$80,000**

- held in November 2022
- nearly 300 families participated
- more than 150 written comments
- attended by 13 Companies  
30 Researchers &  
16 FDA Regulators

Phelan-McDermid syndrome  
**CONCEPTUAL MODEL**



**\$25,000**

- data extracted from 15 peer-reviewed publications
- 96 disease signs and symptoms
- represented 13 observer-reportable patient impacts
- 31 caregiver impacts

Created two new  
**REAL FAMILY STORIES** videos



**\$10,000**

- self-injurious behaviors
- severe hypotonia

## Accelerating Translation of Research to Therapeutics



CureSHANK

**Biomarkers and Outcome Measures Consortium**  
**\$100,000**



Retained a  
project manager  
and legal team

Engaged with  
22 potential industry  
partners

Developed  
governance and  
terms of participation

## PMS Community Engagement

**Two Community Webinars**  
updating & engaging the  
PMS community on CureSHANK's work

