



cureSHANK

accelerating life-transforming therapies

Rare Entrepreneur Bootcamp

April 27, 2023





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WHO WE ARE

CureSHANK is a **volunteer army** of parents, whose children are living with the disastrous symptoms of Phelan-McDermid syndrome, who are determined to accelerate the development of treatments for shankopathies.

Founded July 2019

OUR PARTNERS





Abby, Treasurer
Accounting, regulatory, fundraising



Talya, Vice President
Engagement, comms, fundraising



Paulina, Secretary
Governance, strategy, science



Geraldine, President
Strategy, oversight, science

CureSHANK Operations

- Working board functions as executive team, weekly Zoom meeting to discuss ops
- Responsibilities divided by our areas of strength and expertise
- Commitment to the mission – helping our kids get better



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Patient Identification

- Genetic Testing “Gates”

- Pre/Post-natal

- Cardiac or renal malformation

- Infants

- Hypotonia
 - Feeding difficulties
 - Delays in motor milestones

- Toddlers/preschoolers

- Signs of autism
 - Developmental delays

- Teens & adults

- Catatonia
 - Mood cycling disorder

Opportunity 1: Increasing diagnosis of newborns and infants



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Legislative advocacy:
Project Baby Dillo



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Opportunity 1: Increasing diagnosis of newborns and infants

Opportunity 2: Improving diagnosis of children and teens

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Collaborative projects:
Project FIND-Out &
Other



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Opportunity 3: Finding newly diagnosed people in “other places”



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Opportunity 3: Finding newly diagnosed people in “other places”

Discord, Reddit, Twitch

TikTok, Triller, IG Reels

Mommy apps, momfluencers

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Strategic & Scientific Advisors

- Avoid conflicts of interest
- Include a variety of perspectives:
 - Investors, companies, scientists, clinical investigators.
- Consider:
 - the challenges in your disease area
 - the kinds of expertise that will be needed to solve those challenges.



Fundraising

- **People want to help!** Tap into your personal networks
- Patient community – get creative about engagement
- Beyond fundraising:
 - Seek sponsorships for events
 - Evaluate grant opportunities carefully
 - Ask academic and industry partners for support of specific activities (not just \$, but in-kind donation of services)
- Also, streamline admin expenses
 - Negotiate discounted rates for professional services, such as legal or consulting services
 - TechSoup for reduced cost software
 - Can a committed volunteer help? **People want to help!**



Prioritizing how funds are used

1. Is it in your strategic plan?
2. Does it address a critical barrier in your disease?
3. Is there another funder?
4. What would happen if you didn't fund the project?



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is bridging the



Translational Research Funding Gap

**Basic
Research**

**Drug
Discovery
& Design**

**Pre-clinical
Development**

**Early
Phase
Clinical
Trials**

**Late
Phase
Clinical
Trials**

**FDA
Application
and
Approval!**

**Research Grant
EPILEPSY
in PMS
\$125,000**

**CureSHANK
Consortium
BIOMARKER
Development
\$250,000**

**CureSHANK
RESOURCES
for
RESEARCHERS
Workshops**

**Autism Science
Foundation
Neuropsychiatric
REGRESSION
\$10,000**

**CureSHANK
Consortium
OUTCOME
MEASURE
Development
\$250,000**

**Research Grant
Developmental
REGRESSION
in PMS
\$125,000**

**EL-PFDD
meeting
for PMS
\$80,000** ✓

**ORCA
Validation
-ongoing-**

**Autism Science
Foundation
SLEEP
Measurement
\$10,000**

**Phelan-McDermid
syndrome
CONCEPTUAL
MODEL
\$25,000** ✓

**VOICE
of the
PATIENT
Report
\$20,000** ✓

**REAL
FAMILY
STORIES
-ongoing-
\$20,000**

CureSHANK's 2022-2023 Projects as of April 2023



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Tracking projects we fund

- Grant management: annual reporting (consider: Altum, Wizehive)
- Contracts: milestones
- Other projects: pre-specify your metrics of success

Engaging with industry

- Precompetitive Consortium – biomarkers and outcome measures

Lessons

- Make the ask (people want to help!)