

CUIESHANK

accelerating life-transforming therapies

Rare Entrepreneur Bootcamp April 27, 2023





CURESHANK 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

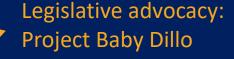


- Genetic Testing "Gates"
 - Pre/Post-natal
 - Cardiac or renal malformation
 - Infants
 - Hypotonia
 - Feeding difficulties
 - Delays in motor milestones
 - Toddlers/preschoolers
 - Signs of autism
 - Developmental delays
 - Teens & adults
 - Catatonia
 - Mood cycling disorder

Opportunity 1: Increasing diagnosis of newborns and infants



- 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



Legislative advocacy: Project Baby Dillo

- 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

Opportunity 2: Improving diagnosis of children and teens



- 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

Opportunity 2: Improving diagnosis of children and teens

Collaborative projects:
Project FIND-Out &
Other

Legislative advocacy:

Project Baby Dillo



Legislative advocacy: Project Baby Dillo

- 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

Opportunity 2: Improving diagnosis of children and teens

Collaborative projects: Project FIND-Out

Opportunity 3: Finding newly diagnosed people in "other places"



- 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

Legislative advocacy: Project Baby Dillo

Collaborative projects: Project FIND-Out

Opportunity 2: Improving diagnosis of children and teens

Opportunity 3: Finding newly diagnosed people in "other places"

Discord, Reddit, Twitch

TikTok, Triller, IG Reels

Mommy apps, momfluencers



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?





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

for

RESEARCHERS

Workshops

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

CureSHANK

Autism Science CureSHANK Foundation **RESOURCES** Neuropsychiatric **REGRESSION**

\$10,000

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

> Autism Science SLEEP

CureSHANK

Measurement

\$10,000

Research Grant REGRESSION

in PMS

\$125,000

Phelan-McDermid syndrome

CONCEPTUAL MODEL

\$25,000

EL-PFDD meeting for PMS

\$80,000

VOICE of the **PATIENT** Report

\$20,000

ORCA Validation -ongoing-

REAL **FAMILY STORIES** -ongoing-

\$20,000



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!)

