



# Overview

## The Orphan Disease Center at UPenn

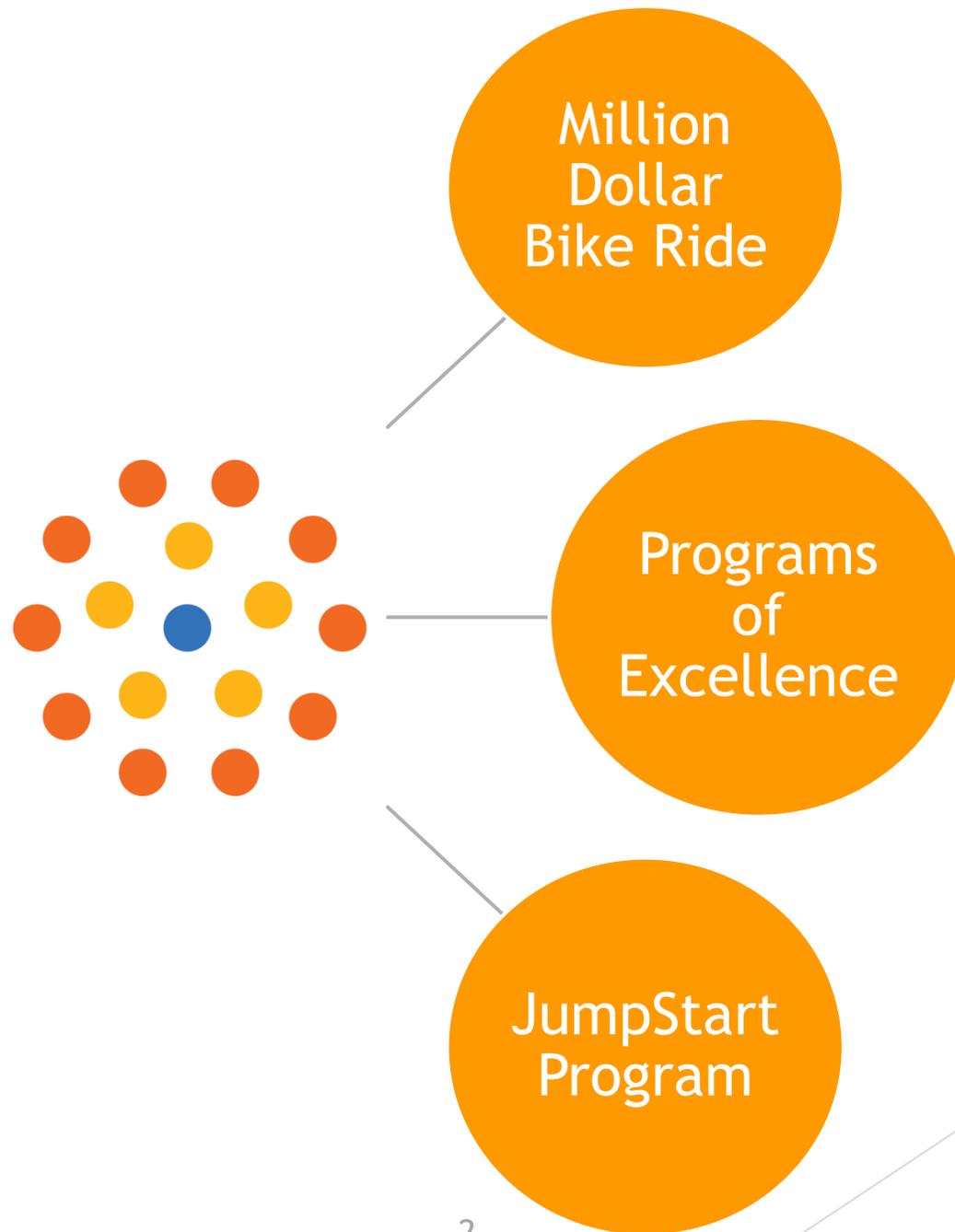


the  
orphan  
disease center

Deborah Requesens, PhD  
Director, JumpStart  
Orphan Disease Center  
University of Pennsylvania

April 28, 2023

# What is the ODC?





Samantha Charleston  
Director, Programs & Community Engagement



# Million Dollar Bike Ride Pilot Grant Program

## GRANT PHASES AND TIMELINES

# 10<sup>TH</sup> ANNUAL 2023



### 1

**Teams:**  
Write 1- paragraph research focus to be included in RFA

**ODC:**  
Finalizes RFA with application guidelines and timelines. RFA is posted and distributed by August 15, 2023. Letters of intent (LOIs) are due by September 18, 2023. LOIs provide a brief, 1-page overview of the research plan

**TEAM PARAGRAPH  
DUE BY APR 1, 2023**  
**DISTRIBUTE FULL RFA  
BY AUG 15, 2023**

### 2

**Teams:**  
Ensure all final team donations are submitted to Penn

**ODC:**  
Tallies all checks, credit card payments and bank wires to determine total team fundraising, and calculates matching funds to post final grant amounts in Request for Applications (RFA)

**BY JUNE 30, 2023**

### 3

**Teams:**  
Provide ODC with list of experts in disease area to serve as reviewers for grant applications

**ODC:**  
Reaches out directly to suggested names to review the full applications. Reviews include scoring of scientific merit and written comments for funding recommendations

**BY JULY 11, 2023**

### 4

**ODC:**  
LOIs are reviewed internally and invitations to submit a full 5-page application are sent. Decisions are based on whether LOIs meet the research aims of the RFA

**LOIs DUE BY  
SEPT 18, 2023**  
**FULL APPS DUE  
BY OCT 20, 2023**

### 5

**ODC:**  
Internally processes reviewer comments/-scores, & funding recommendations are made to teams in advance of ODCs final decisions

**Teams:**  
Provide feedback on ODCs award funding recommendations

**ODC:**  
ODC will send Notices of Award (NOA) by December 24, 2023

**REVIEWS DUE  
BY NOV 10, 2023**

### 6

**ODC:**  
ODC staff distributes funds to awardees and monitors research progress. Awardees submit a 6-month interim and a final scientific report at the end of the grant period. Teams may request a copy of all non- confidential grant reports

**FUNDING PERIOD:  
BY FEB 1, 2024 -  
JAN 31, 2025**

# 2023 Participating Teams



# MDBR Grant Programs

## Est. 2014

<b>Total Pilot Investment</b>	<b>\$17,582,547</b>
Total Awards	289
Total Institutions	132
Total Countries	16
Total Individual PIs Funded	234

# Million Dollar Bike Ride & RARE Drug Development Symposium

**9th Annual MDBR**  
**Saturday, June 11, 2022**  
**31 Disease Teams**  
**600+ cyclists/volunteers**  
**186 virtual cyclists**  
**34 states, 4 countries**  
**\$2.6M for 34 awards**



**10th Annual MDBR**  
**Saturday, June 10, 2023**  
**39 Disease Teams**

**2022 RARE Drug Development Symposium**  
**Thurs-Fri, June 9-10, 2022**  
**200 participants**



**2023 RARE Drug Development Symposium**  
**Tues-Wed, May 2-3, 2023**  
**~200 participants**



# The JumpStart Program

## Scientific Outreach

Identify and connect with  
KOLs & researchers

Identify and connect with  
other foundations

Connections with Genetic  
Counselors & Med  
Students

## Research Tools

Cell lines/Animal models

Patient Registries /  
Natural History Studies

## Think Tank Support

Organization /  
Agenda support /  
Technical support

Identify KOLs and  
participants

## Grants Support

RFA best practices

NOA templates

Scientific advisors  
and/or reviewers

Award management

# JumpStart Partners



# JumpStart Grant Programs Est. 2019

<b>Total Pilot Investment</b>	<b>\$17,582,547</b>
Total Awards	289
Total Institutions	132
Total Countries	16
Total Individual PIs Funded	234

# Programs of Excellence

- Dedicated Director
- Sustainable source of funding identified
- Partnership with a rare disease foundation(s)
- Clear path towards understanding basic biology
- Research tools available or within reach

Daniel J. Lavery, PhD  
Director, CDKL5 PoE



Cara M. Weismann, PhD  
Director, MPS Diseases PoE



## CDKL5 Deficiency Disorder Est. 2016

(in partnership with the Loulou Foundation)

<b>Total Pilot Investment</b>	<b>\$13,451,796</b>
Total Awards	103
Total Institutions	40
Total Countries	9
Total Individual PIs Funded	59
Total Publications	23
Total Patents	5

## MPS Diseases Est. 2011

<b>Total Pilot Investment</b>	<b>\$11,536,996</b>
Total Awards	70
Total Institutions	22
Total Countries	6
Total Individual PIs Funded	35
Publications:	30
Patents:	8

# Pilot Grant Programs

Est. 2011

1-2 years

\$40-150k/year

Category	Total
Total Pilot Grant Investment:	\$46M+
Total Awards	493
Unique Institutions	174
Unique Countries	23
Unique PIs	349
Unique Reviewers	230+

# Global Reach

We fund research in:

Australia  
Canada  
Chile  
United Kingdom  
Egypt  
Estonia  
France  
Germany  
Ireland  
Israel  
Italy  
Japan  
Netherlands  
Portugal  
Spain  
Switzerland  
United Kingdom  
United States

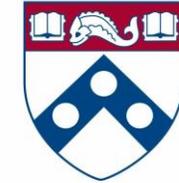


We engage scientists and patient groups from:

Argentina  
Australia  
Brazil  
Canada  
China  
Colombia  
Costa Rica  
UAE/Dubai  
Egypt  
Europe  
India  
Israel  
Japan  
Mexico  
Singapore  
South Korea  
United Kingdom  
United States

# Other Activities

- ❖ Penn Rare Disease Interest Group
- ❖ Collaborations with Hispanic Community
- ❖ Genetic Counseling Student Exchange

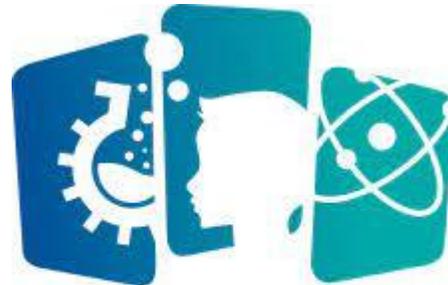


Perelman  
School of Medicine  
UNIVERSITY of PENNSYLVANIA



GCSX

# Thank you!



**RARE ENTREPRENEUR  
BOOTCAMP**