



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?



Million
Dollar
Bike Ride

Programs
of
Excellence

JumpStart
Program



Samantha Charleston
Director, Programs & Community Engagement



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

Total Pilot Investment	\$17,582,547
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

Total Pilot Investment	\$17,582,547
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)

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

MPS Diseases Est. 2011

Total Pilot Investment	\$11,536,996
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**