WHAT IS CDKL5 DEFICIENCY DISORDER?

A rare disease affecting 1 in 40-50,000 people

Caused by de novo mutations in the CDKL5 gene on the X-chromosome

Complex neurological presentation starting soon after birth including epilepsy and neurodevelopmental delay

No approved drug. No effective treatment

The Loulou Foundation

A private non-profit UK foundation founded in 2015 by the parents of an affected child

Dedicated to advancing research into the understanding and development of therapeutics for CDKL5 Deficiency Disorder (CDD)

Our mission

To de-risk the disorder to enable industry interest and partner with all stakeholders in order to realize treatments for CDKL5 Deficiency Disorder by 2020 and cures by 2025.

HOW YOU CAN HELP

Registry. Join the new patient registry so that scientists and companies can better understand and treat CDD

Support. Support your national patient organization so that we are ready for clinical trials

Awareness. Share information and help find more patients with CDD in your country

loulou FOUNDATION
Tackling CDKL5 Deficiency

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A member of:

IRDiRC INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

RARE FOUNDATION ALLIANCE

TRAIN

Advancing research into the understanding and development of therapeutics for CDKL5 Deficiency Disorder

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FUNDING ACADEMIC RESEARCH

CDKL5 Program of Excellence through strategic partnership with Penn Medicine Orphan Disease Center since 2016 with annual Pilot Grant program:

- **31** research projects
- **41** labs at 30 leading institutions
- Over **120** active scientists

the orphan disease center

Also **direct funding** of additional priority areas such as development of research tools and specific disease-modifying strategies

BUILDING THE KNOWLEDGE AND TOOLBOX THAT WE NEED FOR NEW TREATMENTS

- Identification of CDKL5 **targets**
- Antibodies, **brain atlas, biomarkers**
- Open-access transgenic **mouse models**
- Collection of **patient-derived iPSC lines**
- Development of CDD **clinical endpoints**

ENABLING TREATMENTS

Access to **better anti-epileptic drugs** and treatments able to **improve other symptoms** while we progress towards cures:

- **Encouraging companies** that have promising compounds to consider CDD
- Facilitating **preclinical testing**
- **Assistance** with protocol design, center identification and **regulatory engagement**

ADVANCING CURES

Pursuing **multiple approaches** to develop cures through investment and partnering:

- Unlocking the inactive CDKL5 copy through **gene-specific X reactivation**
- Supply of new gene with **gene therapy**
- **Enzyme replacement** approaches
- Functional rescue of the **mutated CDKL5 gene**

BREAKING DOWN BARRIERS

- Annual **CDKL5 Forum research congress** (London and Boston) with record participation in 2017 of 190 attendees including 35 leading companies' representatives
- **www.cdkl5forum.org on-line portal** for research collaborations
- Working closely with the **CDKL5 Deficiency International Patient Alliance**
- **Partnering with IFCR** around Clinical Centers of Excellence and research
- **Working** with biotech and pharmaceutical companies as well as regulators to de-risk clinical trials and attract interest

We are currently actively engaged with over 15 bio-pharma companies, and preparing the field for 5-6 potential clinical trials during the next 2 years