loulou FOUNDATION

Tackling CDKL5 Deficiency

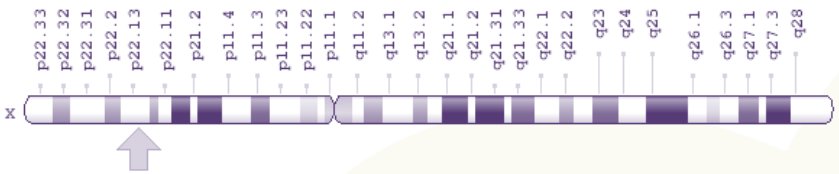
How can a non-profit advance research into a specific rare disease?

Majid Jafar
Co-Founder, Loulou Foundation

ECRD 2018
12 May 2018

“Just one letter”

CDKL5 deficiency disorder



- Rare, X-linked disorder defined by spontaneous loss-of-function mutations in one copy of the **CDKL5 gene**.
- **Incidence 1 in 40-50,000**. One of the most common causes of genetic epilepsy.
- **Intractable infantile seizures** within the first months after birth.
- Severe **developmental delay** leading to intellectual disability, along with speech, visual, and motor deficits.
- **No effective treatment available**. 1 Orphan Drug Designation.

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- **Genesis and Strategy** – De-risking CDKL5 Deficiency Disorder
- **Multiple Approaches** – Treatments and Cures
- **Parallel Objectives**:
  - Funding Academic Research
  - Engaging and Enabling Industry
  - Breaking Down Barriers
  - Plugging the Gaps – Translational Toolkit/ Regulatory

The Loulou Foundation

http://www.louloufoundation.org/

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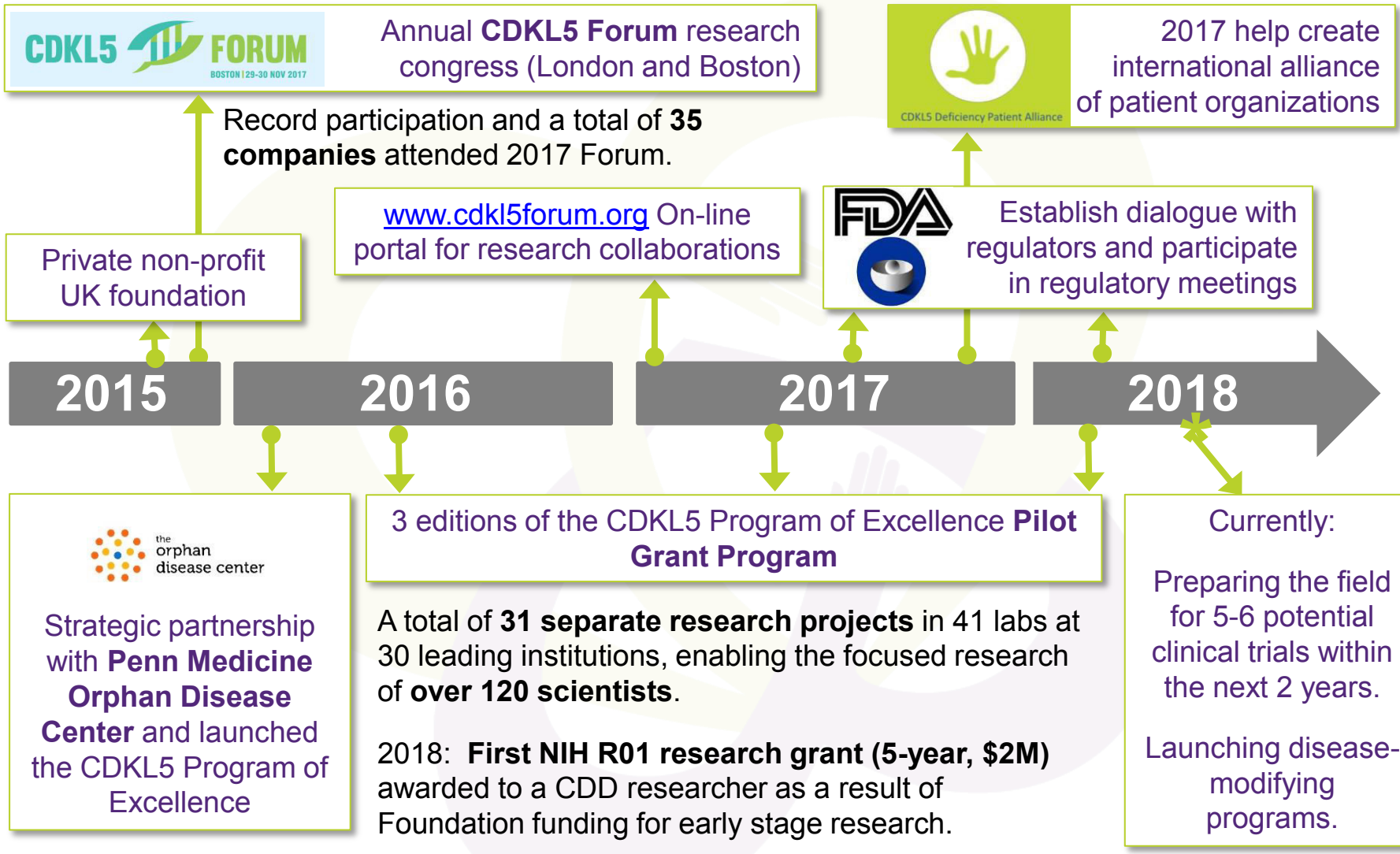

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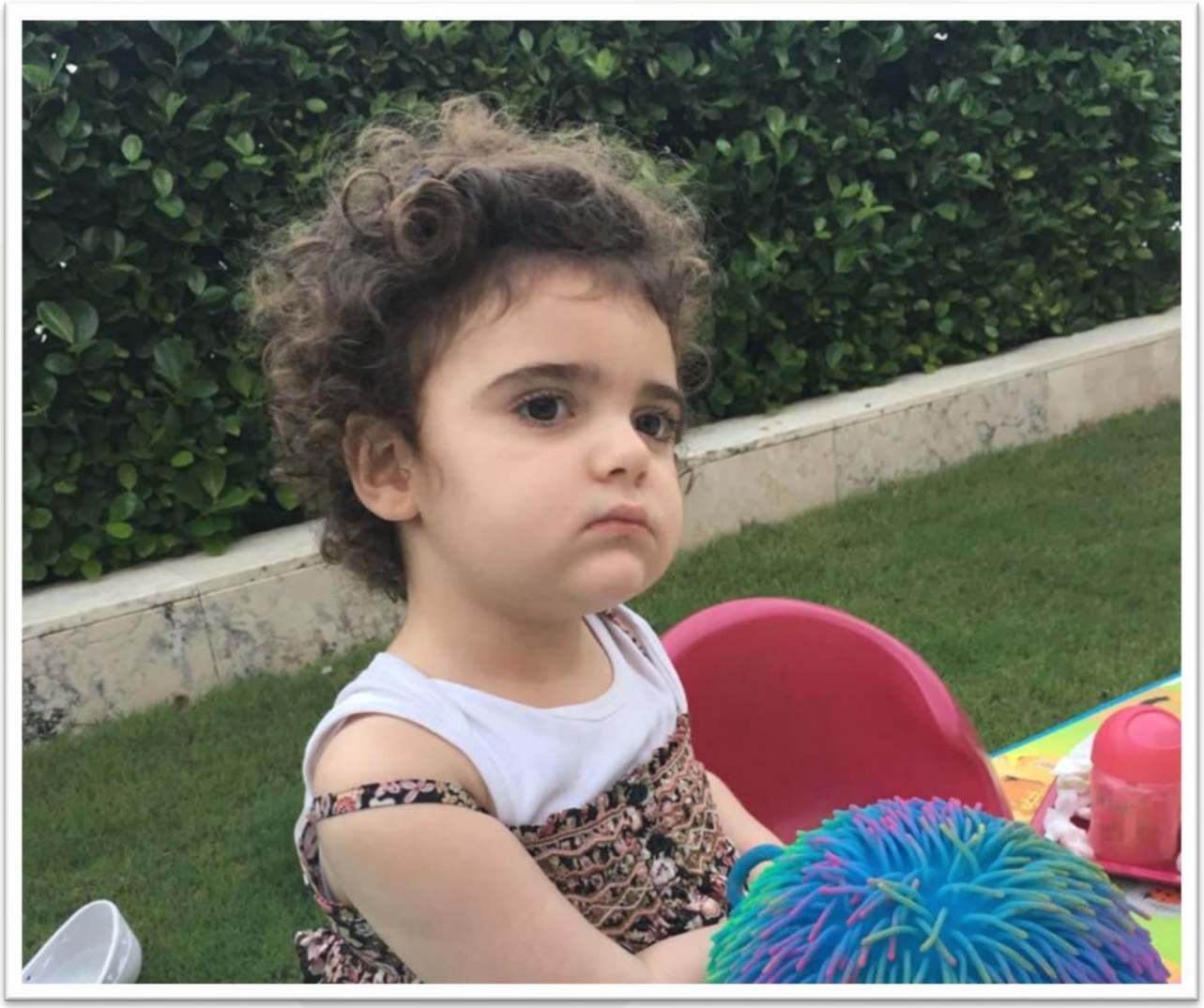

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