



IFCR

International Foundation
for **CDKL5** Research

WE ARE IFCR

TIPS FOR SHARING ABOUT OUR ORGANIZATION
AND THE WORK WE DO FOR CDKL5

HOPE • LOVE • CURE



TABLE OF CONTENTS

- Talking Points
 - About CDKL5
 - About the IFCR
 - Our Budget
 - IFCR Patient Advocacy
 - About IFCR Research
- Flier: CDKL5 Centers of Excellence
- Donation Solicitation Sample Letters (editable versions available)

HOPE • LOVE • CURE



Thank you for your interest in fundraising for the IFCR! We put together this packet to provide you with talking points that you can use to share about our organization. We rely on donations to further our mission. Your efforts are so important and we are appreciative! Thank you for all you do!

ABOUT CDKL5

CDKL5 Deficiency Disorder (CDD) is a rare genetic disease affecting approximately 1 in 40,000 – 60,000 live births. It is a developmental and epileptic encephalopathy. This means that a genetic change causes epileptic activity as well as severe impairment of development. CDD is characterized by early-onset, intractable epilepsy and complex neurodevelopmental disability which impacts cognitive, motor, speech, and visual function.

ABOUT THE IFCR

The International Foundation for CDKL5 Research (IFCR) is the leading patient advocacy group for CDKL5 Deficiency Disorder (CDD).

Our Board of Directors is made up of passionate and dedicated CDKL5 parent volunteers. The IFCR's mission is to treat and cure CDKL5 Deficiency Disorder by funding scientific research, while helping affected individuals and their families to thrive.

The IFCR Board of Directors has created a thriving research, clinician, and patient network. Our Science Director, Heidi Grabenstatter, has a doctorate in neuroscience and is an expert in epilepsy research. She leads an esteemed Scientific Advisory Board, which is comprised of scientists and medical professionals who understand the landscape of CDKL5 research and ensures that the IFCR makes strategic decisions on the research they fund.



ABOUT THE IFCR (con't)

The IFCR was founded in 2009 by parents of children affected by CDKL5. They understand the journey and have dedicated themselves to helping all those affected by CDD. The IFCR's trained volunteer Family Support Reps offer emotional support and understanding to families. The IFCR's website, www.CDKL5.com, has a comprehensive resource library of information to help families understand what they may need to provide the best care for their loved one. The IFCR connects with CDKL5 families through social media, bi-weekly newsletters, virtual support groups, and family conferences.

The IFCR is passionate about its mission and collaborative in its approach, believing that a unified community is paramount to moving CDKL5 forward.

OUR BUDGET

Our work is funded primarily by generous donations from our CDKL5 families and their personal networks. We are proud to be championed by our CDKL5 community!

IFCR PATIENT ADVOCACY

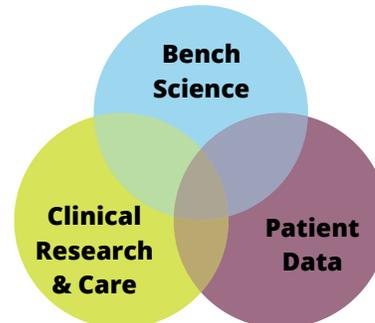
We believe that collaboration benefits us all and that patients should always have a seat at the table. Some of our CDKL5 specific collaborative efforts are with the ICCRN, the LouLou Foundation, and the CDKL5 Alliance.

Additionally, we are involved in multiple collaborative efforts across the epilepsy field and in the larger rare disease space. Because we know there is strength in numbers, we partner with these organizations and advocate for favorable policies that work to get treatments to patients who desperately need them.

ABOUT IFCR RESEARCH

The IFCR's investment in CDKL5 research has laid the foundation for all the exciting CDKL5 science happening today!

See our "Mission Milestones" at www.CDKL5.com/ifcr-timeline/



The IFCR has been leading the field of CDKL5 research since we began in 2009 and we are proud of our accomplishments.

- We funded the development of the first biological models for CDKL5 (mouse models, zebrafish, iPS cell lines, brain bank)
- We establish and fund CDKL5 Centers of Excellence throughout the US
- We fund patient data collection in two ways:
 - ICDD – The *International CDKL5 Disorder Database* (2012) is the most robust patient database for CDKL5 in existence and has been collecting CDD patient data for a decade now. From the information within this database, there have been over 20 research publications.
 - Connect CDKL5 (2019) is the IFCR's own patient contact registry. We collect information on the incidence and prevalence of CDKL5 as well as how to contact families, including those who might otherwise be resistant to participating in research.

The IFCR has created 8 Centers of Excellence (COEs) for the treatment and study of CDKL5 Deficiency Disorder (CDD). These COEs engage in important clinical research that is vital to ultimately being able to cure CDD.

These 8 COEs are located across the United States at leading children's hospitals. **Boston Children's Hospital** (opened in 2013), **Children's Hospital Colorado** (opened in 2013), **Cleveland Clinic** (opened in 2014), **St. Louis Children's Hospital** (opened in 2016), **Texas Children's Hospital** (opened in 2018), **NYU Langone Health Center** (opened in 2018), **Children's Hospital of Philadelphia** (opened in 2019), **UCLA Mattel Children's Hospital** (opened in 2019).



ABOUT IFCR RESEARCH (con't)

What is the purpose of a COE?

- *CDKL5 specific clinical care.* The COEs help affected families access a team of multi-disciplinary specialists who understand CDD. The COEs ease the burden on families by bringing specialists together in one place. This saves families time and hassle, while ensuring that their medical team can easily collaborate. Each COE team consists of a variety of specialties, including but not limited to, epilepsy, genetics, gastroenterology, pulmonology, and physical medicine. The COEs also provide guidance on CDD to physicians and health systems internationally.
- *CDKL5 clinical research.* The COEs conduct clinical research to continually advance the understanding of CDD. They research how the condition unfolds so that standards of care can be developed for this rare disease. Additionally, they collaborate as part of the [International CDKL5 Clinical Research Network \(ICCRN\)](#) to advance CDKL5 clinical trial readiness. They do this by collecting uniform data that can be openly shared with other data repositories and stakeholders, and by developing validated “clinical outcome measures” that can measure treatment effectiveness in CDKL5 Deficiency Disorder. Without these outcome measures, treatments such as gene therapy cannot go to clinical trial in CDKL5.

What are the IFCR's other research interests?

In addition to clinical research, the IFCR looks to fund scientific research that furthers understanding of the CDKL5 gene and fills in gaps that exist in the larger CDKL5 research landscape. We accept Letters of Intent (LOIs) on an ongoing basis through our website.



IFCR

International Foundation
for **CDKL5** Research

CDKL5 Centers of Excellence

The IFCR has created 8 **Centers of Excellence** (COEs) for the treatment and study of CDKL5 Deficiency Disorder. These COEs are vital to ultimately being able to cure this rare disease.

Our COEs are located across the US at leading children's hospitals.



Texas Children's Hospital

NYU Langone Health Center

Children's Hospital of Philadelphia

Boston Children's Hospital

UCLA Mattel Children's Hospital

Children's Hospital Colorado

Cleveland Clinic

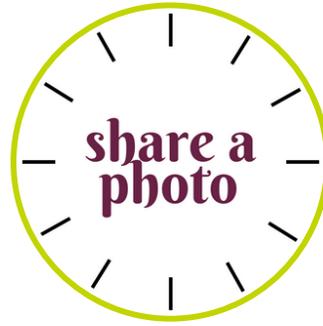
St. Louis Children's Hospital



Our CDKL5 Centers of Excellence (COEs):

- 1) Help affected families access a team of multi-disciplinary specialists who understand CDKL5 Deficiency Disorder *and*
- 2) Conduct critical **research** to advance our understanding of this rare disease and to prepare for life-changing clinical trials of the future.

learn more at CDKL5.COM



Dear _____,

I'm writing to share with you the story of my **son/daughter** _____ and to ask for your support to improve **his/her** life. **Share a short 1-3 sentences about your child and their life with CDKL5.**

CDKL5 Deficiency Disorder is considered a rare disease. One of the challenges of being "rare" is that the burden of fundraising for needed research is often left to the families affected.

Our CDKL5 community is fortunate to have a foundation that is leading the way towards a cure for this disease. The [International Foundation for CDKL5 Research \(IFCR\)](#) was started in 2009 by a group of passionate and dedicated parents who dreamt of a healthier future for their children. Their mission is to treat and cure CDKL5 Deficiency Disorder by funding scientific research, while helping affected individuals and their families to thrive.

The IFCR's work means the world to our family and gives us hope for _____'s future. Will you help us by making a tax-deductible donation? You can do so online at CDKL5.COM or via mail to "IFCR" at **PO Box 926, Wadsworth, OH 44282.**

Thank you for learning about my child. If you would like to hear more, I'd love to speak with you. I can be reached at **000-000-0000.**

With gratitude,

Your Name Here
City, State



Dear _____,

This time last year, I wrote to you asking for your support by making a tax-deductible donation to the [International Foundation for CDKL5 Research \(IFCR\)](#) in honor of my **son/daughter** _____. Please know that your gift made it possible for children with CDKL5 to access medical experts at CDKL5 Centers of Excellence across the US, for newly diagnosed families to find support, and for research to move forward in this rare disease.

For our family, this past year brought...**Share a short 1-3 sentences about your child and their life with CDKL5.**

Your gift eases the fundraising burden that CDKL5 families face. We are grateful for your generosity and humbly ask if you will support our cause again.

The IFCR's work means the world to our family and gives us hope for _____'s future. You can make your donation online at [CDKL5.COM](#) or via mail to "IFCR" at **PO Box 926, Wadsworth, OH 44282.**

Thank you for your kindness towards my child and our family. If you would like to hear more, I'd love to speak with you. I can be reached at **000-000-0000.**

With gratitude,

Your Name Here
City, State