



## **ADVANCE Sanfilippo Scientific & Clinical Research Program**

### **Request for Proposals: February 2025**

[Cure Sanfilippo Foundation](#), a 501(c)3 non-profit patient advocacy and medical research organization, provides funding and collaborative support for a wide range of research programs. Areas of funding support include but are not limited to: basic and translational science, identification and development of disease modifying therapeutics including clinical trials, improving clinical care, understanding of human disease manifestations, and patient preference studies.

The Foundation's overarching research funding goal is to advance a pipeline of scientific discovery which leads to treatments and care strategies that improve the lives of individuals with all forms of Sanfilippo syndrome.

#### **Priority Areas:**

Priority areas of interest for this submission period include proposals seeking collaborative research with Cure Sanfilippo Foundation to:

- Advance the clinical understanding, diagnosis, measurement tools and management of movement disorders in individuals with Sanfilippo syndromes.
- Evaluate the feasibility of biomarkers related to mitochondrial function, oxidative stress, neurotransmitter profiles, autophagy, mitophagy functions in humans with Sanfilippo syndromes. Preference for clinically-available laboratory tests.
- Increase the evidence base of behavioral therapy (often referred to as Applied Behavioral Analysis) in individuals with Sanfilippo syndromes. May include case series which report on longitudinal data and validated assessment tools.
- Case studies comparing outcomes and/or biomarkers between siblings who have received enzyme restorative therapy vs an untreated sibling.
- Advance innovative translational research for one or more Sanfilippo syndromes which include a plan to begin a clinical trial within 2 years. May include combination therapies. May be subtype specific or basket studies.

Proposals outside these priority areas are also welcome.

#### **Instructions:**

Please follow the prompts in the ProposalCentral portal to enter the requested information. Attachments should be labeled as below. Required and optional attachments may vary between LOI and Full Proposal applications.

- Title the document with Investigator's last name-LOI (phase of application)-PD (type of information i.e. Preliminary Data as PD)-date (month year). For example, Jones-LOI-PD-Feb2025.
- Text in attachments should be written in 11-point Calibri (body) font.

**Considerations in Preparing a Budget:** Funding amounts vary depending on the scope of the project and will be considered on a case by case basis. For case study write-ups, the budget maximum is \$5,000 and may include publication fees.

- In the LOI period, only an estimated total budget is requested.
- Full Proposal Budget: Please specify the cost breakdown of the various components of the project.
  - Budgets should be written to align with clear scientific milestones and go/no timepoints in the research as applicable.
  - Costs per year should indicate both direct and indirect cost allocations.
  - Invoices should be submitted to Foundation based on achieving predefined milestones and reporting requirements. Please structure your budget to accommodate this framework.
  - Maximum allowable indirect costs/overhead equals no more than 5% of total approved grant amount, and cannot exceed \$10,000 regardless of total approved grant amount.
  - Travel is not a covered expense.
  - Publication costs should not be included in the budget. If there are funding needs for publication of CSF funded research, the investigator may request such support as a separate request at the time of manuscript submission/acceptance.

### **How Research Proposals are Evaluated:**

CSF utilizes the expert advice of its Scientific Advisory Board (SAB) members and independent external peer reviewers to evaluate the scientific merit of proposed research programs. Final funding decisions are made by CSF's Board of Directors. These decisions take into consideration the SAB's evaluation of scientific merit and recommendations along with CSF's funding strategy and ongoing programs, alignment with areas of patient need, and the budget available to commit to new project funding.

### **Review Process:**

1. LOI submission period: Feb 15- March 28, 2025
2. Notification of LOI review and invitation to submit full proposals for selected LOIs: April 11, 2025
3. Full Proposals Due: anticipated May 30, 2025
4. Peer Review
5. Feedback provided to applicant
6. Applicant Response period
7. SAB Review Meeting



**Deadline to Apply:** Submit your LOI using the ProposalCentral platform by 11pm ET on March 28, 2025. After review, selected LOIs will be invited to submit a full proposal.

**Reporting and Communication Requirements include:**

- Ongoing communication through confidential written reports are requested every 6 months to communicate project status to CSF, non-confidential brief plain language reports annually for broader community updates.
- Videoconference meetings are requested every 6-12 months following written reports to review progress and allow for richer collaboration.
- Other communication and collaboration may be requested on an as needed basis. More frequent communications will take place for clinical study collaboration projects.
- Dissemination of research results and progress are expected through peer-reviewed publications, poster presentations and/or other informal presentations.

**Contacts for More Information & Collaboration:**

For more information on this current funding opportunity and to view sample forms we invite you to visit us at: <https://curesanfilippofoundation.org/how-to-help/for-researchers/funding-opportunities/>

For additional questions or to discuss your research idea with our CSO, Dr. Cara O'Neill, please contact us at [Research@CureSanfilippoFoundation.org](mailto:Research@CureSanfilippoFoundation.org).