Who we are. What we do. How we operate.

We are Cure Sanfilippo Foundation and we have a clear mission: to advocate for and fund research that could give children with Sanfilippo syndrome a chance at a better life by finding a treatment or cure for the disease. And we remain passionately focused on that goal. The results and progress made so far have been largely thanks to partner-families joining our Foundation around the country and the world.

We recognize every family of a child with Sanfilippo syndrome is on their own unique journey. This difficult journey can often be a lonely and isolating one. Cure Sanfilippo Foundation is a place to join with other parents and families going through the same, and be part of changing the fate for children with Sanfilippo. We do this together, primarily through advocacy, spreading awareness, and fundraising. Cure Sanfilippo is proud of our laser-focused mission, professionalism, demonstrated results, transparency, collaboration, and information-sharing. We seek families that would like to join these efforts to better the lives of children.

We believe that working together creates exponential power and opportunity to find a treatment or cure for Sanfilippo. Voices raised together are heard more loudly. Work done together is done faster. In addition, it is empowering for a family to take a stand against this disease that currently has no treatment options. A family will see their community rally in honor of their child and their circle of awareness and influence grow. This upswell of support and action truly makes a tangible difference that is seen through development of more clinical trials and faster movement toward approved, effective, and available therapies for all children with Sanfilippo.

This is why we hope every family of a child with Sanfilippo will consider partnering with Cure Sanfilippo Foundation and joining this mission. If this sounds like something you would want to be part of, we hope you’ll reach out to us to become a partner-family. We welcome you.

Please read more about how we can work together in four areas.
SUPPORT AND CONNECTING FAMILIES

We support and empower families dealing with Sanfilippo syndrome. We work diligently to ensure research funded by the Foundation is well rounded and includes the study of all disease subtypes and stages. We believe every child with Sanfilippo syndrome, no matter the type or the age, deserves a chance at more good days.

We believe in equipping partner families for however they want to fight back against Sanfilippo syndrome. Whether they choose to share their story publicly, to hold fundraisers multiple times a year, or simply benefit from the research information that we share regularly. Together, we brainstorm, collaborate, and advocate.

We help partner-families tell and share their individual stories as a gateway to attract people's attention and raise awareness of the need for continued research to find a treatment or cure for Sanfilippo.

Every single family is welcome, valued equally, and respected. While it should go without saying, we would like to be clear in writing: Cure Sanfilippo Foundation is passionate about non-discrimination on all levels. All races, genders, religions, education levels, occupations, family structures, political beliefs, personal beliefs, and orientations are welcome and respected equally.

If it appears some families are working "more" with the Foundation, it is only because those are the families actively reaching out to the Foundation with new advocacy, awareness, or fundraising ideas to further the mission to help children. We welcome this. However, fundraising and advocacy in no way favors any family or child for clinical trial participation. The Foundation does not have any involvement in the selection process for clinical trials. Those decisions are made by the primary investigators (clinical doctors) for the clinical trials.

We provide parents with the latest scientific information regarding research from around the world and help families tell their unique story to elevate awareness of Sanfilippo syndrome. We share scientific and medical expertise to support your decision-making for your child's unique needs. We also lend support to partner-families interested in fundraising for the mission, with resources and assistance. Additionally, we coordinate multiple collective fundraisers throughout the year to provide families an array of choices from which they can choose to participate, if any fit their interest.

This group of partner-families allows for information and experience sharing, advice, and questions asked and answered, from one family to another. Strong and lifelong bonds and partnerships are formed. Having the opportunity to discuss the highs, lows, and everything in-between with the only people who can truly understand, because they are going through (or have been through) the same, can be an important source of support on this important journey.
Fundraising is a priority for the Foundation. It has to be because the dollars fund the research that moves us closer to effective treatments. Fundraising — along with partnering with families, advocating with regulators, collaborating with researchers and scientists, and spreading awareness — is critically necessary fuel for achieving our mission. Without it, progress towards a cure slows down, if not stops. And a cure can’t wait.

However, **partner-families are NEVER pressured or obliged to fundraise for the Foundation.** We believe in providing opportunities for families interested in doing so. When you tell us you are ready, we provide advice, support, and ready-to-use materials to help those interested. We are cheerleaders and helpers in executing campaigns, collaborators to brainstorm ideas, but never commanders.

We believe that any efforts to raise funds for research for Sanfilippo, whether on behalf of Cure Sanfilippo Foundation or another organization or group, are helpful. Being a partner-family does not bind you to only advocating or fundraising with Cure Sanfilippo Foundation.

We are relentless in our pursuit of research that will open the next door to finding a treatment or cure.

We fund research into Sanfilippo syndrome, and we also build it. We are constantly searching for promising research in need of funding. Additionally, our scientific experts review research happening around the world in related fields and use it to build new scientific ideas specific to Sanfilippo syndrome to pursue. If there’s an idea that doesn’t exist, we have the network to create collaborations to make these new, potential therapeutic paths happen.

We are able to do this because we combine the rare “unified perspective” of medical professional, scientific expert, and caregiver. We not only possess it, but we are working to create it in others. We are leading several projects to better integrate the caregiver perspective and priorities in research and testing. We are working to improve the medical community’s knowledge of Sanfilippo syndrome so children can be diagnosed earlier and have access to supportive interventions as soon as possible. Facilitating earlier diagnosis will also help increase the opportunities for children to participate in clinical trials as early as possible. Also, we continue to bring forward clinical trial options for better quality of life for children with all disease subtypes and stages.
Our unified perspective and thought-leadership makes Cure Sanfilippo Foundation a respected and sought-after collaborator in the Sanfilippo research community. We collaborate with other nonprofits in the MPS and rare disease space in the United States and around the world. We are the sole funders of many research projects and co-funders and collaborators on others. We enjoy working closely with industry organizations (biotechs), scientists, researchers, regulatory agencies such as the U.S. Food & Drug Administration, and fellow rare disease foundations.

We receive research proposals year-round from clinicians and scientists around the world. There is an abundance of ideas about treating Sanfilippo syndrome that are in need of funding. Each one is carefully evaluated by our experienced and independent Scientific Advisory Board, external scientific reviewers, and our internal team, which helps the Board of Directors decide which programs to fund. Research programs are selected based on scientific merit, ability to impact patient care in a timely manner, and if they fill critical gaps in our understanding of Sanfilippo that could lead to effective treatments.

All contracts for research funding or partnerships are reviewed by an experienced law firm with expertise in nonprofit law, life-science contracting, technology transfer, and more. This ensures the research deliverables are being met through milestone reporting, which triggers Cure Sanfilippo to release the grant payments as the project progresses over time. Our research accountability process ensures that precious dollars are not wasted. It also enables us to confidently report progress and updates to families and supporters, based on science and facts.

Additionally, our research contract grants may include the potential for Cure Sanfilippo to receive investment income or royalty payments, at a future date, based on the success of the company involved or the licensing of the research. This type of funding strategy is called “venture philanthropy,” and has been highly successful for many rare disease organizations. These potential future dollars are used by the Foundation to fund more research and further advance the mission.

**OUR ETHICS & VALUES**

We are an established, professional, non-profit organization with proven impact and an experienced and passionate staff, partner-families, volunteers, and advisors. We manage operational costs very carefully and always weigh the benefit of every expense against how it helps achieve the mission.

We believe in transparency to our partner-families, donors, and collaborators. We go through an official and independent audit annually by a certified public accountant (CPA). We proactively report progress regularly to each of these valued groups, focused on results and impact, through email and social media. We also have partner-family video conferences to discuss updates, brainstorm, and provide space for Q&A. We also share much of the information on our website, such as:
“There’s no such thing as false hope. Because hope is not conditional on an outcome. Hope is actually just love, on a mission.”

— Cara O’Neill, Chief Science Officer