Annual Report 2024

CURE HSPB

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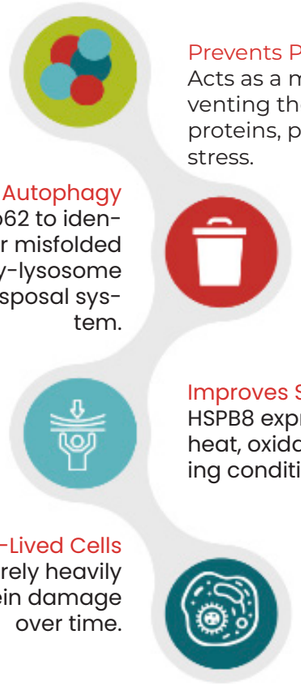
Uniting against rare challenges

What is HSPB8 myopathy?

HSPB8 Myopathy is an ultra-rare, autosomal dominant, slowly progressing, adult-onset muscle wasting condition. It is a type of myofibrillar myopathy first identified by Ghaoui et al, in 2015. It is caused by mutations in the HSPB8 gene with 26 cases identified worldwide.



Prevents Protein Aggregation
Acts as a molecular chaperone, preventing the accumulation of misfolded proteins, particularly during cellular stress.

Promotes Autophagy
HSPB8 partners with BAG3 and p62 to identify and degrade damaged or misfolded proteins via the autophagy-lysosome pathway—a cellular waste disposal system.

Improves Stress Resilience
HSPB8 expression increases during heat, oxidative stress, or other challenging conditions to ensure cell survival.

Key for Long-Lived Cells
Neurons and muscle cells rely heavily on HSPB8 to mitigate protein damage over time.

HSPB8 stands for heat shock protein beta-8 and is essential for cellular stress response. A molecular multitasker, HSPB8 maintains protein stability and protects proteins during stress. Notably, HSPB8 is expressed across several tissues but is especially abundant in muscle, brain, and liver. Its role is especially prominent during cellular stress, ensuring vital functions remain intact.

HSPB8 Myopathy is not routinely included in genetic myopathy panels and therefore heavily underdiagnosed. Patients typically start experiencing muscle weakness between their 20s and 40s. With no treatment available, patients inevitably worsen and experience extremely poor quality of life.

Cure HSPB8

Cure HSPB8 is the only charitable project and advocacy group dedicated to overcoming the challenges posed by HSPB8 Myopathy. We have brought together a multidisciplinary team of patients and families, advocates, researchers and healthcare professionals to treat and to cure this debilitating condition.

The mission of Cure HSPB8 is to improve the lives of all people affected by HSPB8 Myopathy and their families. We do this by accelerating the drug development process, building a strong and empowered community and advocating for the HSPB8 community. Our goal is to find a treatment and a cure using state-of-the-art technologies and advances in science. To achieve this mission, we focus on three pillars:

Research and drug development

We support research to uncover the underlying mechanisms of HSPB8 Myopathy and develop innovative tools that will expedite the drug development process.

Awareness and advocacy

We are dedicated to raising awareness about HSPB8 Myopathy in the medical and scientific communities, with the goal of reducing the time between the onset of symptoms and correct diagnosis through more widespread testing and education. By working to identify the true number of patients affected worldwide, we aim to strengthen the visibility of this rare disease and prioritize it within healthcare systems.

Community

We believe that an informed, connected, and empowered community is vital to overcoming the challenges of HSPB8 Myopathy. Our efforts focus on building and fostering a global network of patients, families, and expert clinicians, providing critical resources, and offering ongoing support to those navigating this journey. At Cure HSPB8, our vision is life free of HSPB8 Myopathy and all its burden.

Words of Founder

Dear Friends, Supporters, and Advocates,

Even though this year was our very first year of existence, it has been nothing short of extraordinary. What began as a simple mission—to bring hope and answers to everyone affected by HSPB8 Myopathy—has already grown into a thriving movement of families, researchers, clinicians and advocates united by a shared purpose.

At Cure HSPB8, we believe that progress is not just measured in milestones, but in the lives we touch and the momentum we build. Every step we take brings us closer to understanding this disease and, ultimately, to finding treatments. The strides we have made this year—growing our community, advancing research, and amplifying our voice—are a testament to the power of collaboration and determination.

None of this would be possible without you. Your support, whether through advocacy, donations, or simply sharing our mission fuels the work ahead. While challenges remain, we face them with optimism. We know that each breakthrough, each discovery, and each connection moves us toward a future filled with hope.

Thank you for being part of this journey. Together we are not just imagining a better future—we are building it.

With gratitude and determination,

Todd King
President & Founder, Cure HSPB8

The Team

Dr. Ania Kordala
Program Director

Dr. Karolina Chwalek
Strategic Advisor

Matt McLeod
Scientific Advisor

Khosiyat Makhmudova
Outreach Officer

Julia Mielcarz
Brand Manager

Accomplishments of 2024

Since its foundation under the auspices of Social and Environmental Entrepreneurs in April 2024, Cure HSPB8 quickly established itself as a driving force in the fight against HSPB8 Myopathy. Our impact is reflected in the following key achievements:

Research achievements:

- In collaboration with the Coordination of Rare Diseases at Sanford (CoRDS), we launched an international registry to collect data on disease progression, paving the way for clinical trial readiness.
- We organize monthly Cure HSPB8 Research Meetings bringing together patients and leading researchers worldwide to collaborate and share insights on HSPB8 Myopathy, making sure patients have a voice in research.
- We partnered with Everlume Bio to develop iPSCs & a humanized mouse model for HSPB8 myopathy. Together, we also conducted and finalized an amenability study, marking a significant step toward identifying potential therapeutic approaches
- We initiated cutting-edge autophagy research with Dr. Wenli Zhou from XYZ Laboratories, to explore biomarkers of the disease.

Advocacy milestones:

- We built a strong team by bringing together passionate individuals who share our vision.
- Cure HSPB8 proudly joined the Global Genes Alliance, amplifying our voice in the rare disease community.
- HSPB8 Myopathy is now recognized on the Muscular Dystrophy Association (MDA) website as a form of myofibrillar myopathy. We were officially acknowledged by both Orphanet and the National Organization for Rare Disorders (NORD).
- We attended the MDA Conference in Orlando, FL, and the Unlocking Hope for Ultrarare Conference in Warsaw, Poland, presenting a poster on Cure HSPB8's mission and initiatives.

Community efforts:

- We advance our mission to unite and empower the HSPB8 Myopathy community through our global patient registry.
- We host regular patient meetings, providing a platform for connection, shared experiences, and ongoing support.
- We created a comprehensive website providing resources for patients, researchers, and clinicians, complemented by a bi-monthly newsletter.
- We established a presence on Twitter, Facebook, and LinkedIn, supported by engaging social media campaigns to raise awareness and foster community connections.

Priorities for 2025

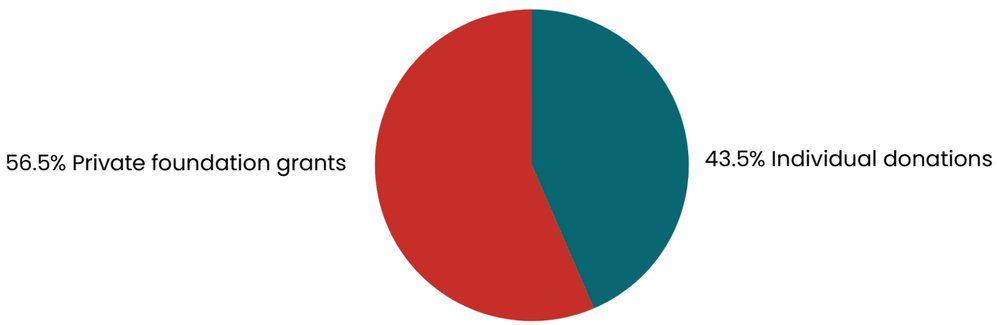
As we look ahead to 2025, CureHSPB8 is focused on three key priorities:

- Driving Research: Advancing efforts to develop treatments that can improve lives of those affected by HSPB8 myopathy.
- Raising Awareness: Spreading the word about HSPB8 myopathy to connect with more patients, families, and advocates.
- Identifying Patients: Building our community by finding individuals affected by this rare condition and ensuring their voices are heard.

Financial summary

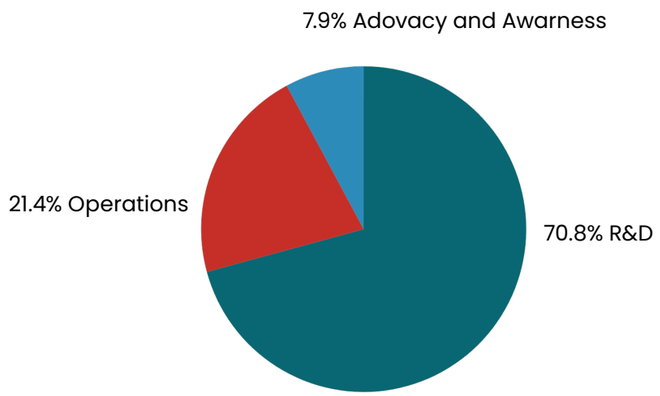
Total incomes: $487,020.80

- Individual donations: $212,020.80
- Private foundation grants: $275,000.00



Total expenses: $280,346.99

- R&D: $ 198,400.00
- Operations: $59,923.24
- Advocacy & awareness: $22,023.75



Connect with us

Thank you for reading and for your support of the HSPB8 Myopathy community! Stay connected by subscribing to our newsletter at curehspb8.org and following us on LinkedIn, X, and Facebook for the latest news, updates, and ways to get involved.

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