



ANNUAL REPORT



**cure
SMA**

2025

DEAR SMA COMMUNITY,

As we reflect on 2025, we are filled with gratitude and optimism for the progress our community has achieved together. This past year brought meaningful advances in treatment, research, and advocacy, creating new possibilities and renewed hope for individuals and families impacted by spinal muscular atrophy (SMA). These are breakthroughs built together.

One of the most significant milestones of the year was the broad FDA approval of a fourth treatment, Itvisma®, for children and adults ages 2 and older. This approval marks important progress in addressing the needs of older individuals living with SMA while expanding the options families have to manage the disease and improve quality of life.

We also celebrated FDA approval of a new Evrysdi® tablet for individuals ages 2 and older. Offering the same proven effectiveness as the oral solution in a more convenient form, this new option provides greater flexibility in daily routines and supports increased independence.

Together, these approvals showcase the power of collaboration across the SMA community, from researchers and clinicians to industry partners and advocates. Each new treatment expands choice, strengthens quality of life, and brings us closer to a future of more comprehensive, personalized care. Looking ahead, two additional treatments are under review in 2026, and we will keep the community updated as news emerges, sharing what each approval means for individuals and families living with SMA.

Our commitment to progress remained strong in 2025. We awarded \$750,000 in new research grants to accelerate discovery and drive the next generation of treatments. At the same time, nearly 150 community members traveled to Washington, D.C., to champion research priorities and improvements to disability policy. Their efforts helped secure inclusion of SMA in a Department of Defense biomedical research program, signed into law in early 2026. Together, these actions ensure that SMA remains a national priority, from the lab to Capitol Hill.

The energy and dedication of our community was on full display this year. Nearly 2,800 individuals, families, and researchers came together at the Annual SMA Conference, the largest gathering of its kind, to connect, learn, and share the latest scientific advancements. Across the country, Summits of Strength, Walk-N-Rolls, galas, and other events brought supporters together to raise awareness, build community, and provide critical support that drives continued breakthroughs.

The accomplishments of 2025 reflect the resilience, generosity, and collaboration of this community. To every individual living with SMA, every family, clinician, researcher, donor, advocate, and partner, thank you. The progress we celebrate is possible because of you, and together we will continue building what comes next.

Best,



Kenneth Hobby
President



Nick Farrell
Chairman of the Board



2025 IMPACT REPORT

BREAKTHROUGHS BUILT TOGETHER

In 2025, the SMA community turned progress into momentum. From expanded treatment options to national policy wins and investments in the next generation of research, each milestone reflects what is possible when we work together.

HERE IS A SNAPSHOT OF WHAT WE BUILT IN 2025:



BREAKTHROUGHS IN TREATMENT

- FDA broad approval of a fourth SMA treatment, Itvisma®, expanding options for adults and children ages 2+
- FDA approval of a new tablet formulation of Evrysdi®, increasing flexibility and independence
- Two additional therapies are under regulatory review in 2026



BREAKTHROUGHS IN COMMUNITY SUPPORT

- 939 support items sent to individuals and families
- 164 Newly Diagnosed Care Packages delivered
- 194 packages sent to adults with SMA
- 2,739 individuals and families attended the Annual SMA Conference
- 16 Summits of Strength held nationwide
- 37 chapters supported across the country



BREAKTHROUGHS IN RESEARCH

- \$750,000 awarded in new research grants
- Six new basic research grants funded
- 4th Annual State of SMA Report published
- Two peer-reviewed manuscripts accepted for publication



BREAKTHROUGHS IN FUNDRAISING & STEWARDSHIP

- \$5,402,819 raised in 2025
- 11% growth in fundraising revenue
- 10,721 individual donors
- 4,921 first-time donors
- \$10,922,659 invested directly into mission programs
- 77% of expenses directed to programs and services



BREAKTHROUGHS IN ADVOCACY

- 150 advocates participated in Hill Day
- 4,536 advocacy actions taken
- 361 letters, statements, and testimonies submitted
- SMA designated as a research priority within a Department of Defense biomedical research program, signed into law in early 2026

TOGETHER, WE BUILD WHAT'S NEXT

Every milestone in 2025 reflects the strength of this community — individuals living with SMA, families, researchers, clinicians, donors, partners, and advocates. Breakthroughs are not built alone. They are built together. And together, we will continue accelerating progress in 2026 and beyond.

COMMUNITY SUPPORT



COMMUNITY SUPPORT IMPACT

Throughout the year, Cure SMA's community support efforts delivered critical resources, education, and connection to individuals and families, fostering support and engagement across the SMA community. Here's the impact we made together:

- 939 support items sent to individuals and families
- 164 Newly Diagnosed Care Packages sent to provide guidance and resources for newly diagnosed families
- 194 packages sent to adults with SMA to support independence and daily life
- 2,739 families, individuals with SMA, and researchers attended the Annual SMA Conference
- 16 Summits of Strength were held nationwide, bringing together attendees to learn from experts and build connections within the SMA community
- Cure SMA supported its 37 chapters across the country to provide local, personalized support and strengthen connections within the SMA community

Each year, Cure SMA provides thousands of individuals and families impacted by SMA with vital resources and support programs. From information packets to care packages, these programs offer tangible assistance that helps families live independent, successful, and fulfilling lives. All programs are available at no cost to anyone in the U.S., ensuring that support is accessible to every family and individual who needs it.

ADAPTIVE SPORTS AND RECREATIONAL SUPPORT

The SMA community expressed interest in learning more about adaptive sports and recreational activities. In response, we launched new adaptive sports and recreation support programs to make exploring these opportunities easier. These resources include a 3-part webinar series and six new PDF guides on a variety of topics. A new dedicated webpage serves as a one-stop shop for easy access to the webinars and all of the new materials.

ANNUAL SMA CONFERENCE

Cure SMA's Annual SMA Conference is the largest SMA conference in the world, bringing together researchers, healthcare professionals, families, and adults to network, learn, and collaborate. It is the centerpiece of our national support program.

The 2025 Annual SMA Conference in Anaheim, CA, was an impactful weekend filled with community, research, care, and fun for more than 2,739 attendees. The weekend began with Thursday's Newly Diagnosed Program, Meet & Greet, and Researcher Relay Race. The four-day weekend included over 71 workshops on a variety of topics, special events for adults, teens, and children, and the Family-Friendly Researcher Poster Session, where attendees were encouraged to learn from and build connections with researchers.

In addition to these educational opportunities, conference attendees were able to unwind and engage in activities such as the Family PJ Party & Movie Night, and a variety of socials for teens, adults with SMA, parents, grandparents, and more.

SUMMIT OF STRENGTH PROGRAM

The Summit of Strength Program is a free, half-day event hosted by Cure SMA, designed for individuals of all ages and types of SMA, as well as their caregivers. These educational and networking gatherings are tailored to inform, empower, and connect the SMA community. They also provide an opportunity to network and learn from national and local experts about the latest advancements in support, treatment, care, and advocacy. This local engagement keeps our community vibrant through building connections and informative presentations. In 2025, we welcomed over 1,474 attendees to the 16 Summit of Strength programs held nationwide, a 17% increase over the prior year, with 369 SMA patients represented.

In 2025, Cure SMA Advocacy elevated the voices of individuals with SMA, their families, and supporters to advance policies that promote independence, access, and opportunity. Through education, engagement, and direct advocacy, the SMA community made meaningful progress at the federal, state, and local levels.



LEGISLATIVE PROGRESS & POLICY WINS

Sustained advocacy led to significant progress on top SMA community priorities.

- Congress and the President approved extensions of three ABLÉ account provisions, supporting long-term financial independence for individuals with SMA
- Congress designated SMA as a research priority within a Department of Defense biomedical research program
- Congress included language to prioritize SMA research at the National Institutes of Health
- Congress passed legislation to further incentivize rare disease research and development

The final three provisions were signed into law in February 2026, marking a historic win for the SMA community.

DIRECT ENGAGEMENT WITH POLICYMAKERS

- Congressional and federal leaders participated in Cure SMA's Hope on the Hill and Summit of Strength events
- SMA community members met in-person with 155 Congressional offices from 34 states, including directly with 10 U.S. Senators and Representatives, to advocate for bipartisan SMA priorities

EXPANDING STATE & LOCAL ADVOCACY

Cure SMA launched a new State and Local Advocacy Toolkit to empower advocates beyond Washington, D.C.

EDUCATING LAWMAKERS & AMPLIFYING COMMUNITY VOICES

SMA advocates worked throughout the year to educate Congress and key stakeholders about SMA priorities and persistent unmet needs across healthcare, research, transportation, education, caregiving, and financial independence. Here's what we achieved together:

- 150 SMA community members participated in Cure SMA's 2025 Hill Day, meeting directly with congressional offices
- 4,536 advocates took action by signing letters, joining advocacy campaigns, or completing surveys
- 361 letters, statements, and testimonies were submitted by Cure SMA to Congress and other stakeholders



RESEARCH

UPDATES FOR THE FDA

Cure SMA continued to elevate the voice of the SMA community in discussions with the FDA during a critical time for new and emerging therapies. In 2025, we shared our latest State of SMA Report and new research on unmet needs among adults living with SMA. We also provided first-person perspectives from individuals and families to help inform regulatory decision-making.

These efforts ensure that the lived experiences of the SMA community remain central as new therapies and treatment approaches are reviewed.

BASIC RESEARCH

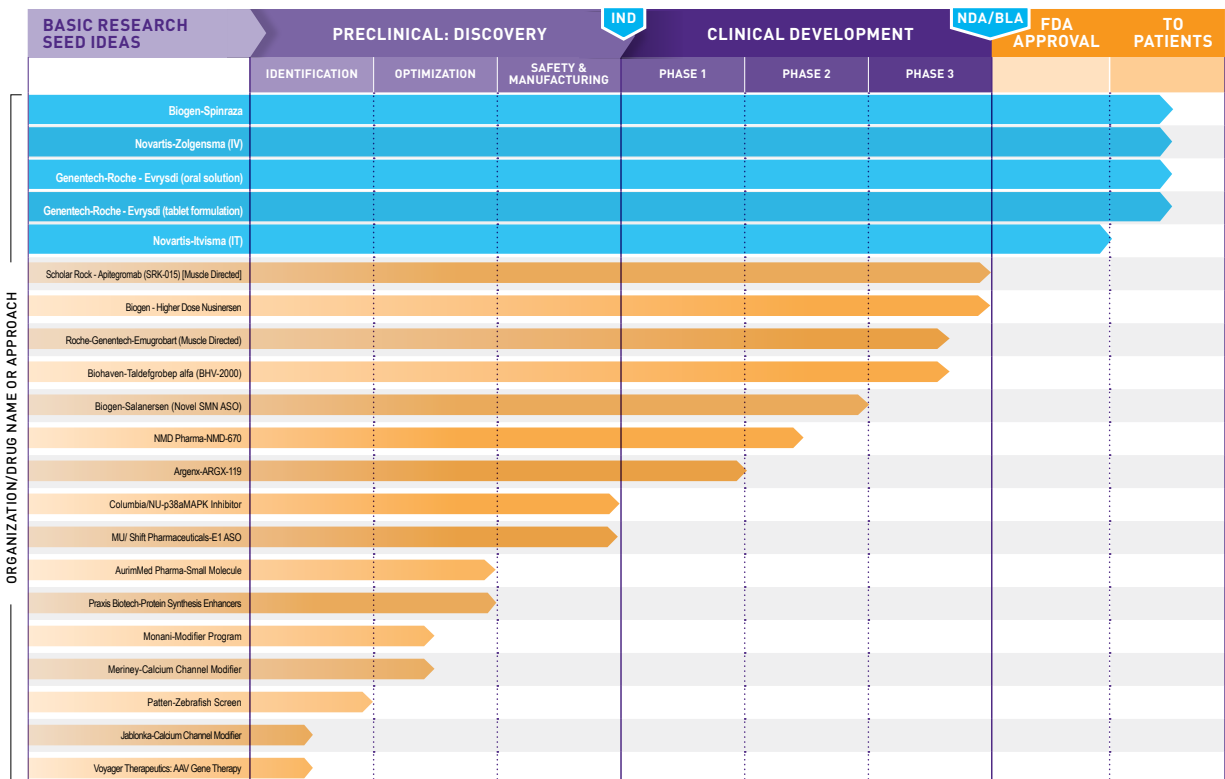
Cure SMA is proud to have supported early-stage research that contributed to the development of the four FDA-approved treatments for SMA. While these therapies represent extraordinary progress, important work remains to ensure treatments are as effective as possible for every person living with SMA.

In early 2025, Cure SMA awarded six new basic research grants totaling \$750,000 to advance promising scientific discoveries. Thanks to the continued generosity of our community, an additional \$750,000 in research funding will be awarded in the current grant cycle, with recipients to be announced in early 2026.

SMA DRUG PIPELINE

Cure SMA continues to fund and guide research across a broad and expanding pipeline of potential therapies. Our strategy supports the development of new treatments, including approaches that may work in combination, with the goal of improving outcomes for individuals of all ages and SMA types.

With multiple therapies advancing through clinical development, the pace of progress continues to accelerate toward even more effective treatment options.



BLA = Biologics License Application

IND = Investigational New Drug

NDA = New Drug Application

Last updated: November 2025

CLINICAL RESEARCH

In 2025, Cure SMA made significant progress in advancing clinical research, strengthening data collection, and improving access to care. Key accomplishments include:

- Increasing awareness of clinical trials and expanding access through the Cure SMA Registry and Clinical Trial Finder, a searchable online database
- Publishing the 4th Annual State of SMA Report, highlighting data collected to better understand trends and unmet needs within the SMA community

Report findings include:

- An estimated 1 in 16,200 babies born in the U.S. between 2020–2023 have SMA
- 51% of people living with SMA in the U.S. are adults
- Approximately 75% of individuals with SMA have received an FDA-approved treatment
- The average time from diagnosis to first treatment was 18 days for individuals diagnosed in 2024
- The proportion of individuals requiring a feeding tube has decreased nearly 20% over the past five years
- The mortality rate for SMA has declined nearly 80% over the past decade

- Launching the 9th Annual Cure SMA Community Update Survey to better understand the experiences and daily challenges of individuals living with SMA and their caregivers
- Launching the 2025 Adult-Onset Survey to better understand the diagnostic journey and treatment use among individuals living with SMA Type 4
- Updating the Cure SMA Health Insurance Roadmap to support families and providers navigating coverage denials and appeals
- Maintaining and expanding the Find a Treatment Center Tool, improving access to specialized SMA care
- Launching a roundtable series, Advances in SMA Treatments: Insights for Neurologists, in partnership with NeurologyLive to educate providers on evolving standards of care
- Securing acceptance of two peer-reviewed manuscripts focused on community priorities for future therapies and improved measurement of function and fatigue in teens and adults with SMA
- Presenting six research abstracts at national scientific meetings

2025 ANNUAL SMA RESEARCH & CLINICAL CARE MEETING

Held June 25–27 in Anaheim, CA, the Annual SMA Research & Clinical Care Meeting brought together more than 600 researchers and healthcare professionals from around the world.

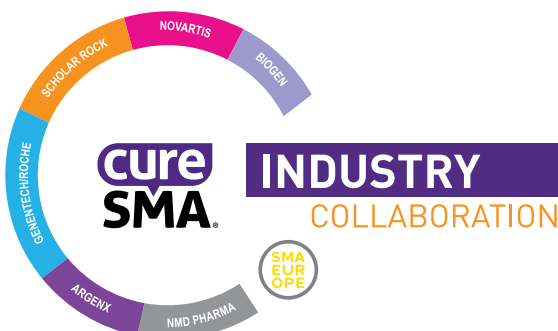
The meeting fostered collaboration across disciplines, encouraged integration of new investigators and industry partners, and accelerated progress in both research and clinical care. Attendees also engaged directly with individuals and families through joint events such as the Meet and Greet and Family-Friendly Researcher Poster Session.

Research sessions included dedicated tracks in Basic Research, Clinical Research, and Clinical Therapeutic Development.

The 2025 Clinical Care Session welcomed 617 attendees and offered 5.75 continuing education credit hours to support excellence in SMA clinical practice.

ACKNOWLEDGEMENT

Funding was provided by the Cure SMA Industry Collaboration. The Cure SMA Industry Collaboration (SMA-IC) was established in 2016 to leverage the experience, expertise, and resources of pharmaceutical and biotechnology companies, as well as other nonprofit organizations involved in the development of spinal muscular atrophy (SMA) therapeutics to more effectively address a range of scientific, clinical, and regulatory challenges. Members of the 2025 SMA-IC included Cure SMA, Biogen, Novartis, Scholar Rock, Genentech/Roche, argenx, NMD Pharma, and SMA Europe.



FUNDRAISING

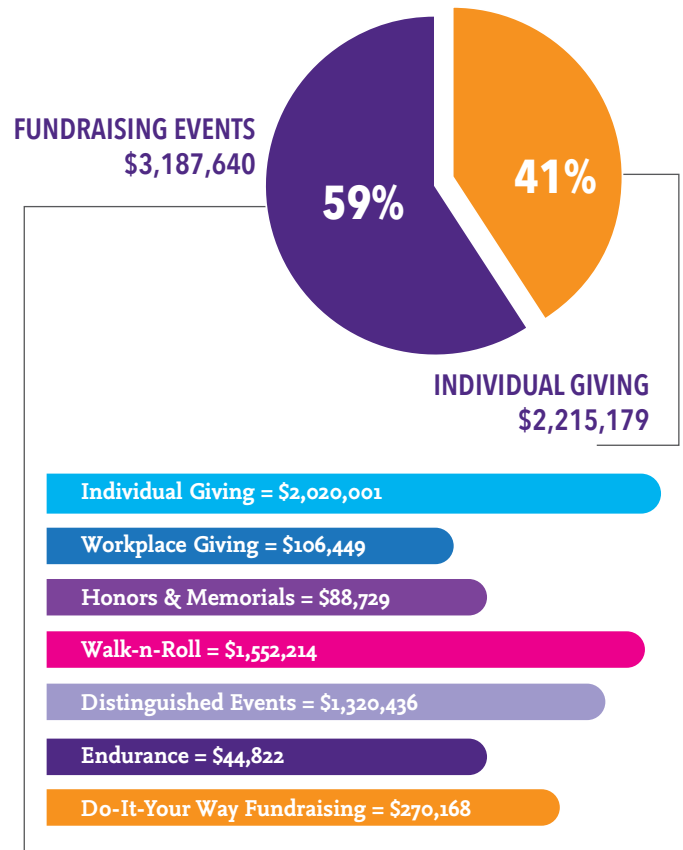
POWERED BY COMMUNITY. DRIVING IMPACT.

The progress we are making toward our mission is powered by a community that refuses to give up. Across the country, individuals and families impacted by SMA, dedicated volunteers, generous donors, and committed corporate partners came together this year to fuel meaningful progress for the SMA community.

Through fundraising events, personal campaigns, corporate partnerships, and individual giving, thousands of supporters united around a shared purpose: advancing research, expanding support programs, and strengthening advocacy efforts that improve the lives of people living with SMA. Every fundraiser organized, every mile walked or rolled, and every gift made reflects the compassion and determination of a community working together to change the future of SMA.

These collective efforts raise critical resources powering real impact. The generosity of our supporters helps accelerate research toward new and improved treatments, ensures families have access to vital support programs and resources, and strengthens advocacy efforts that create lasting change for individuals living with SMA.

The strength of Cure SMA's mission is sustained by a diverse network of fundraising efforts led by passionate supporters across the country. From national fundraising events to individual giving and grassroots community initiatives, every contribution plays a vital role in fueling progress for the SMA community.



\$5,402,819
TOTAL RAISED THROUGH INDIVIDUAL GIVING AND FUNDRAISING EVENTS



“Cure SMA has changed Luke's life, changed our life. Cure SMA has stood by us since the very beginning of this daunting diagnosis. Through care packages, educational summits, conferences and connecting us with other families, Cure SMA has provided physical and emotional support to our family and made us feel less alone. Most importantly, Cure SMA was there before we knew we needed them. They had been advocating for decades for the development of the life-saving therapies from which Luke now benefits. Because of Cure SMA, we are hopeful, even optimistic about Luke's future. Because of Cure SMA, Luke is walking. I encourage others to join me in supporting Cure SMA so that they can continue to fight for all those affected by SMA, those that know they need their support and those yet to know.

- Joanna Whittler, mother to Luke, living with SMA, and Bodhi (pictured left)

FINANCIALS

STATEMENT OF FINANCIAL POSITION FOR THE YEAR ENDED JUNE 30, 2025 AND 2024

ASSETS	2025	2024
Cash, cash equivalents, and investments	\$7,154,260	\$6,100,092
Receivables and other assets	3,430,318	4,408,636
Fixed assets, net	60,673	195,240
Total Assets	\$10,645,251	\$10,703,968

LIABILITIES	2025	2024
Accounts payable and accrued expenses	\$1,842,564	\$3,161,418
Research and grants payable	1,380,198	1,421,221
Total Liabilities	3,222,762	4,582,639

NET ASSETS	2025	2024
Unrestricted	\$2,609,833	\$1,424,129
Temporarily restricted	2,670,455	2,558,327
Permanently restricted	2,142,201	2,138,873
Total Net Assets	7,422,489	6,121,329

Total Liabilities and Net Assets	2025	2024
	\$10,645,251	\$10,703,968

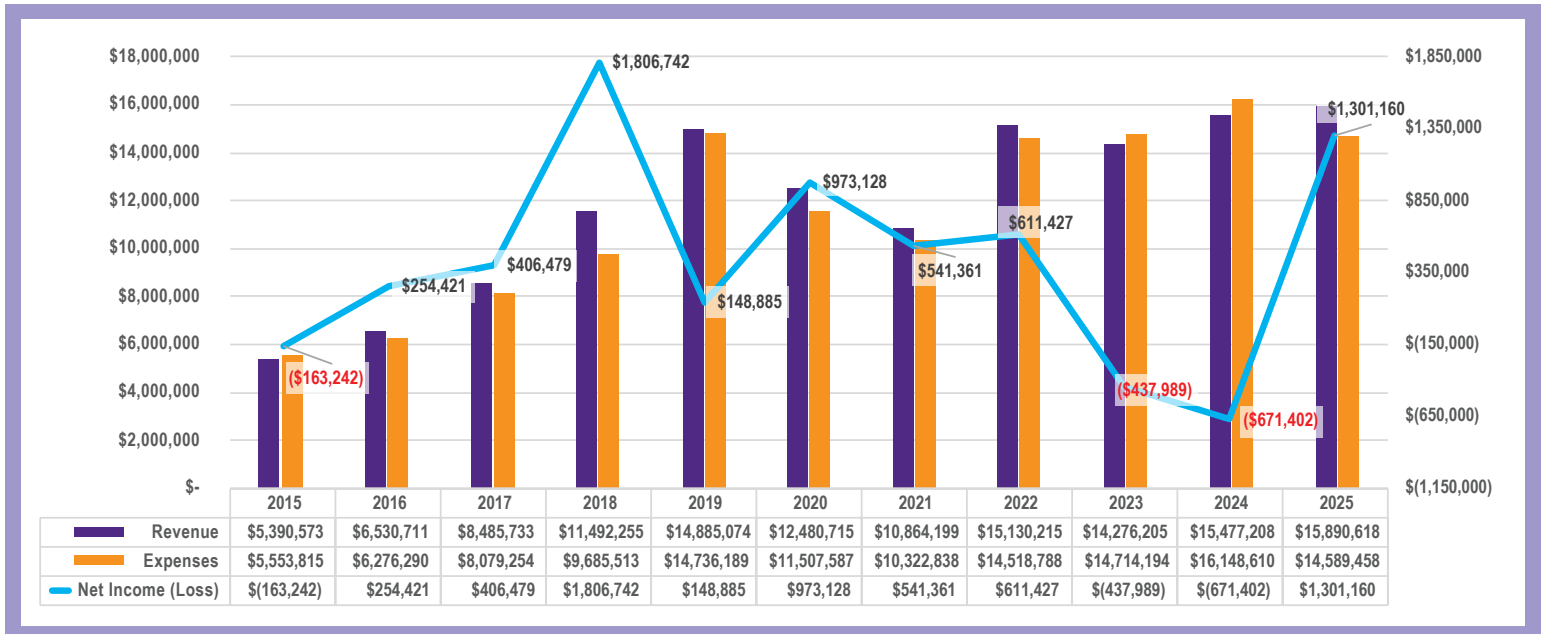
STATEMENT OF ACTIVITIES FOR THE YEAR ENDED JUNE 30, 2025 AND 2024

SUPPORT AND REVENUE	2025	2024
Contributions:		
Contributions and grants	\$5,027,258	\$4,939,282
Special events, net	2,856,366	2,802,600
Program revenue	6,912,298	6,958,513
Other revenue	611,627	348,365
Total Revenue	15,407,549	15,048,760

EXPENSES	2025	2024
Program Services		
Program Services	10,922,659	12,493,257
Total program services	10,922,659	12,493,257
Supporting Services		
Management & general	1,305,150	1,352,444
Fundraising	1,878,580	1,874,461
Total supporting services	3,183,730	3,226,905
Total Expenses	14,106,389	15,720,162

Increase (decrease) in net assets	2025	2024
	\$1,301,160	(\$671,402)

REVENUE AND EXPENSES



Financial information presented has been summarized from financial statements audited by Warady & Davis LLP dated June 30, 2025 and 2024.

*Variation between financials and the chart due to the deduction of "Benefit to Donor."

WHERE YOUR CONTRIBUTIONS GO

Cure SMA is deeply grateful for every gift we receive. Your generosity fuels groundbreaking research, drives critical advocacy efforts, and provides essential support programs that the SMA community relies on. Every dollar you donate is put to work, making a lasting impact by funding initiatives that bring us closer to a cure.

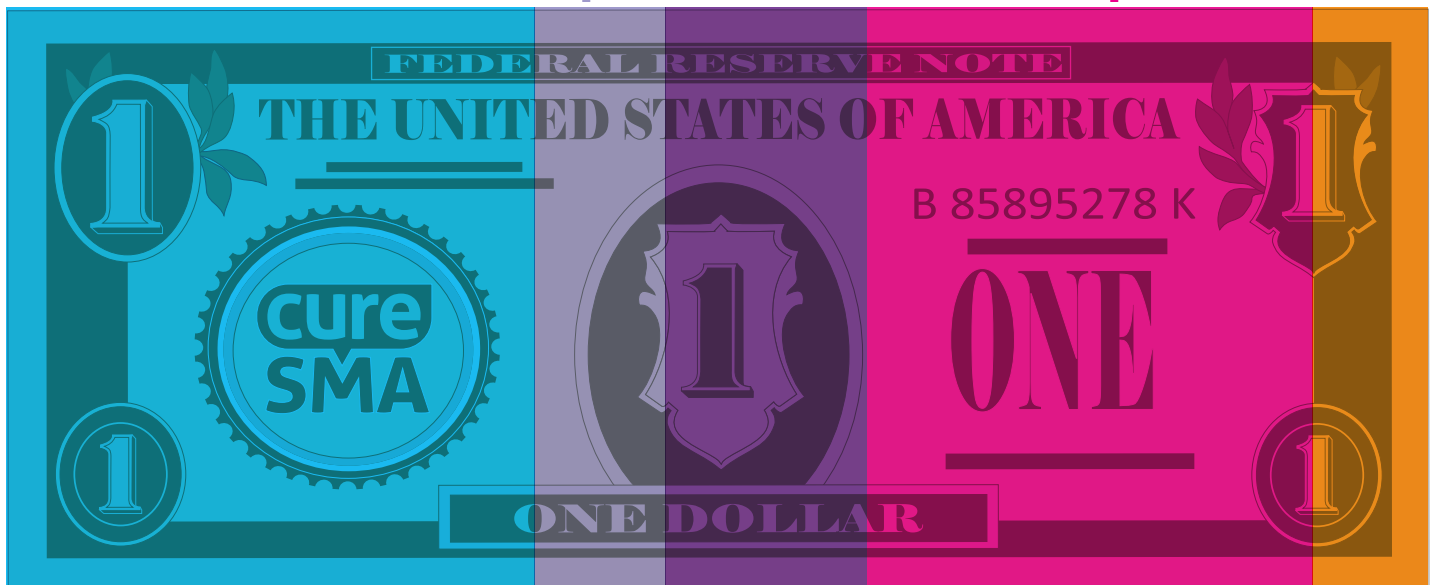
HERE'S HOW YOUR SUPPORT IS MAKING A DIFFERENCE:

MANAGEMENT & GENERAL 9%

Overseeing Cure SMA's operations to ensure efficiency, accountability, and long-term sustainability.

COMMUNITY SUPPORT 34%

Providing tangible support programs for today, expanding local outreach and uniting communities, and providing customized resources that meet the diverse needs of everyone impacted by SMA.



RESEARCH 38%

Advancing scientific discovery by funding basic research to understand the biology of SMA, identifying new targets and effective strategies for drug development, and supporting clinical trials and research to secure approval and ensure access to practical new treatments.

FUNDRAISING 13%

Planning and executing fundraising campaigns and events that generate critical resources to drive research, support programs, and advocacy efforts for the SMA community.

ADVOCACY 5%

Championing policies and initiatives that improve access to treatments and care, remove barriers that impact daily living, and drive change for increased independence and participation for individuals and families impacted by SMA.

MISSION & VALUES

Our mission is to drive research for treatments and a cure for SMA, and to support and empower everyone impacted by SMA today.

OUR VALUES:

INNOVATION

Our commitment to a treatment and cure is not just about seeking solutions—it's also about creating them. We're working with some of today's sharpest minds to advance a diversity of approaches and champion the most promising discoveries and methods.

BALANCE

As relentlessly as we pursue a treatment and cure, we are also strategic. We know the fastest way to a future without SMA is to take a comprehensive, unbiased approach to research and maintain a balance of optimism and realism.

COLLABORATION

Our community is everything to us. We would not have made it this far in our fight without the invaluable contributions of our researchers, doctors, and families. Together, we are—and always will be—stronger than SMA.

RESPECT

There is no “right way” to live with a disease like SMA. Every person's experience is different, and it's every family's right to decide what SMA means for them.

COMPASSION

Thanks to the Cure SMA community, no person is ever alone in facing this disease. We offer unconditional support to people with SMA and their families, and communicate openly and honestly, giving them clear and accurate information.

DETERMINATION

Our work is not done until we have a treatment and cure, and we'll remain strong in our fight no matter what challenges come our way.



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