ANNUAL REPORT

2024

MISSION AND VALUES

VISION AND MISSION STATEMENT

Cure SMA leads the way to a world where everyone impacted by spinal muscular atrophy (SMA)is empowered to lead independent, successful, and fulfilling lives. We strive to create a community where every individual is heard and feels welcomed. Cure SMA provides practical support programs for our community and advocates for their needs. We fund and direct comprehensive research that drives breakthroughs in treatment and we advance access to high quality care. We will not stop until we have a cure.

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.

DEAR SMA COMMUNITY,

As we reflect on the past year, we are filled with immense gratitude for the remarkable strides we've made together. Our collective efforts have led to significant achievements across advocacy, research, and support, bringing renewed hope and tangible progress to all those impacted by SMA.

In January 2024, we reached a monumental milestone: all 50 U.S. states now screen newborns for SMA ensuring that infants born with SMA receive an early diagnosis, allowing families to make timely decisions about treatments that can save lives and dramatically improve health outcomes. This achievement underscores the power of our community's persistent advocacy.

Our policy and advocacy efforts this year have yielded substantial progress. We published a comprehensive report highlighting the challenges faced by SMA caregivers, amplifying awareness and influencing legislation aimed at addressing these needs. Our collaborative advocacy also led to key victories in the FAA Reauthorization Act, improving wheelchair accessibility and passenger safety. Additionally, the U.S. Department of Transportation released a groundbreaking rule to make air travel safer for wheelchair users, marking a significant step toward equitable travel for individuals with SMA. Through our work with Congress, the National Institutes of Health was urged to prioritize research addressing the unmet needs of individuals with SMA, paving the way for future breakthroughs.

Our commitment to advancing research for new SMA treatments remains steadfast. This year, we awarded six new research grants totaling $750,000 to drive breakthroughs advancing muscle and nerve targeting approaches that could reverse symptoms, weakness and damage from SMA and combination therapies. We also launched Phase 9 of our SMA Industry Collaboration, bringing together pharmaceutical companies and nonprofit organizations to share information, ideas, and data to benefit the broader SMA community. Closing out 2024, we reached positive clinical trial results for multiple new treatment options that we are now working to get approved and hopefully available to individuals and the community in 2025.

The strength of our community was on full display at our 2024 Annual SMA Conference in Austin, Texas. This year's conference brought together over 1,900 individuals and families living with SMA, researchers, and healthcare professionals serving as a powerful reminder of resilience and unity within our community. The Annual SMA Conference continues to be a cornerstone event where we share the latest scientific advancements, provide educational resources, and celebrate the incredible bonds that make up the SMA community. In addition, Cure SMA hosted more than 80 local events, including Summits of Strengths, Walk-N-Rolls, and galas, that allowed individuals with SMA, their families and other supporters to learn, raise money, and raise awareness about SMA at the local level.

The great accomplishments of 2024 are a testament to the unwavering dedication, resilience, and collaboration of our community. Thank you to all individuals living with SMA, families, clinicians, researchers, donors, and corporate partners for all you do to advance our mission forward – the progress we've made as a community is a direct reflection of you and we are grateful for your support.

Best,

Kenneth Hobby
President

Nick Farrell
Chairman of the Board

COMMUNITY SUPPORT

NATIONAL SUPPORT

Each year, Cure SMA provides thousands of individuals and families affected by SMA with vital resources and support programs. From information packets to care packages, these programs provide tangible support to help our SMA community live independent, successful, and fulfilling lives.

The SMA community identified accessible travel as one of their top priorities, and while progress has been made, traveling is still not accessible for everyone. With this in mind, we launched some new travel-related support programs to make traveling with SMA easier. These resources include a new web-page with a variety of travel materials, as well as a travel support package for adults with SMA.

Our national support programs, including the travel support package, are available to all people affected by SMA who reside in the U.S., and all are provided at no cost.

LOCAL SUPPORT

As the largest organization dedicated to spinal muscular atrophy, Cure SMA is uniquely positioned to leverage the collective strength of our community for both national impact and local empowerment. Across the U.S., Cure SMA's 37 chapters offer personalized, local support to every individual and family affected by SMA who connects with us.

In late 2018, Cure SMA launched the Summit of Strength Program - a series of single-day, educational events for people with SMA, and their friends and families. These mini-conferences 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. Over the past six years, we've welcomed over 7,600 attendees to 112 Summits of Strength.

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 2024 Annual SMA Conference in Austin, TX, was an impactful weekend filled with community, research, care, and - most importantly - fun for the more than 1,975 attendees. As always, this incredible event began with Thursday's Newly Diagnosed Program, Meet & Greet, and Researcher Relay Race. The four-day weekend also included over 63 workshops covering a variety of topics, special events for adults, teens, and children, and our Family Friendly Researcher Poster Session, where attendees are encouraged to learn from and build connections with researchers.

In addition to these educational opportunities, conference attendees were given the chance to unwind, engage, and have fun through activities such as the Family PJ Party & Movie Night, a variety of socials for teens, adults with SMA, moms, dads, grandparents, and more!

ANNUAL SMA RESEARCH & CLINICAL CARE MEETING

The Annual Cure SMA Research & Clinical Care Meeting is held each year alongside the Annual SMA Conference. In 2024, over 584 SMA researchers and clinicians from around the world met in Austin, TX with the common goal to accelerate the pace of SMA research leading to new treatments, and achieve excellence in SMA clinical care.

The annual meeting provides an opportunity for scientists, doctors, physical therapists, and other experts in SMA research and clinical care to share their most recent discoveries and learnings. It facilitates cross-disciplinary discussions and serves as an incubator for collaborations.

2024 Community Support Impact

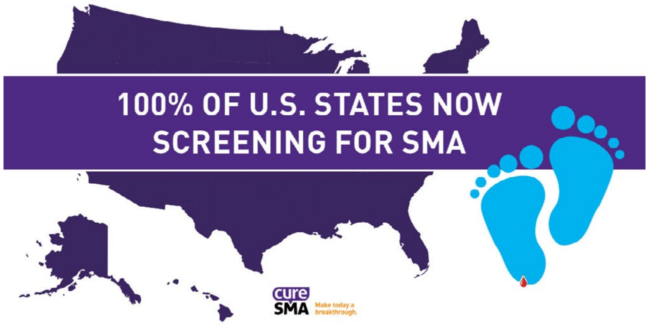
- **1,611 Support Items Sent**
- **174 Newly Diagnosed Care Packages Sent**
- **152 Packages Sent to Adults with SMA**
- **1,975 Families, Individuals with SMA & Researchers at the Annual SMA Conference**
- **16 Summit of Strength Local Events Held Nationwide**

ADVOCACY

During this fiscal year, Cure SMA Advocacy made significant progress in advancing the mission of Cure SMA by educating lawmakers and stakeholders about the needs and priorities of the SMA community. This work resulted in meaningful milestones and generated momentum that will continue to drive impactful change.

A YEAR OF EDUCATION AND AWARENESS

- **608,327** Congressional messages sent – a dramatic increase due to an advocacy campaign that went viral
- **535** Members of Congress contacted
- **170+** in-person & virtual congressional meetings, including directly with U.S. Senators Mike Braun (IN), Tim Kaine (VA), and Elizabeth Warren (MA) during Cure SMA's Hill Day
- **60+** Advocacy campaigns launched in support of SMA community priorities
- **15** SMA community members attended White House events as Cure SMA guests
- **20+** Cure SMA testimony, statements and reports submitted to Congress and the Administration, including a national report that highlighted the caregiving challenges and policy recommendations of individuals with SMA and their families
- **Expanded the Adult Advisory Council to 20 adults** with SMA who provide high-level advice and feedback on Cure SMA activities to serve the SMA adult community



A YEAR OF MOMENTUM AND RESULTS

- Achieved 100 percent newborn screening of SMA in all 50 states with the addition of Hawaii and Nevada in 2024
- Implemented and managed new advocacy initiatives and activities, including Do-It-Yourself Advocacy webinars, advocacy workshops, and Adult Advisory Council meetings
- Secured a provision in the National Institutes of Health budget in support of new SMA research to address the unmet needs of children and adults with SMA
- Earned passage of key disability transportation improvements in federal aviation law and lavatory regulation that will make air travel safer and more accessible for passengers with SMA who fly in wheelchairs
- Hosted or participated in Washington, D.C. events with key Administration and Congressional leaders, including U.S. Health and Human Services Secretary Xavier Becerra, U.S. Transportation Secretary Pete Buttigieg, National Institute of Neurological Disorders & Strokes (NINDS) Director Dr. Walter Koroshetz, U.S. Senator Tammy Duckworth (IL), U.S. Representative Debbie Dingell (MI-6), U.S. Representative Greg Stanton (AZ-4), and U.S. Representative Dina Titus (NV-1)

RESEARCH

CURE SMA SHARES UPDATES WITH FDA

As part of our ongoing relationship and dialogue with regulatory authorities, Cure SMA is committed to sharing updated data regarding the SMA population and their voice. In 2023 FDA officials were sent the latest State of SMA report that highlighted key demographics and unmet needs of the SMA community. Several key FDA representatives from the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) responded to this update thanking the SMA community for sharing their information and voice.

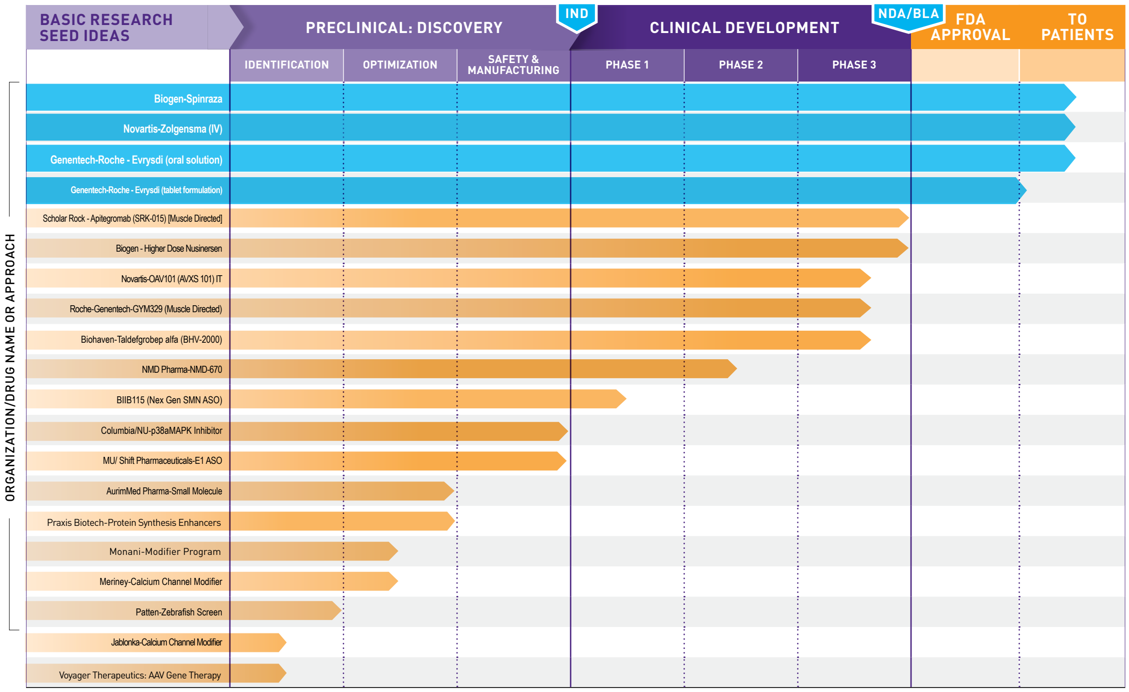
BASIC RESEARCH

Cure SMA is proud to have funded early-stage research for the three FDA approved drugs for SMA. While these treatments represent unprecedented advances for individuals with SMA, we know that there remains much to do and learn to ensure maximally effective treatments for every individual living with SMA.

As part of our continued investment in basic research, Cure SMA awarded 6 new basic research grants totaling $750,000 in early 2024. Thanks to the generosity of our community, we are pleased to announce another $750,000 in awards for the current grant cycle. Announcement of those awards will be made in early 2025.

SMA DRUG PIPELINE

We're funding and directing research with more breadth and depth than ever before. We know what we need to do to develop and deliver new therapies, which could also work in combination, to reach our goal of treatments for all ages and types. And we're on the verge of further breakthroughs that will continue to change the course of SMA, and eventually lead to a cure.



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BLA = Biologics License Application **IND** = Investigational New Drug **NDA** = New Drug Application Last updated: February 2025

CLINICAL RESEARCH

The Clinical Research and Care Department made significant progress in advancing clinical research, care and data collection, analysis and reporting. Accomplishment highlights include:

CLINICAL RESEARCH

- Key learnings report from the 2023 Cure SMA Clinical Trials survey to assess clinical trial readiness and capacity at SMA clinical trial sites
- Enhanced SMA clinical trial awareness and access for the SMA patient and healthcare provider community through the SMA Registry and Clinical Trial Finder – a searchable database, and resources including Screening Visit Checklist, Decoding Informed Consent, and Travel Planners: Air and Ground (available in 4 languages)

To learn more about the Clinical Research and Care journey, please scan the QR code or visit www.curesma.org/cure-sma-clinical-trials/.

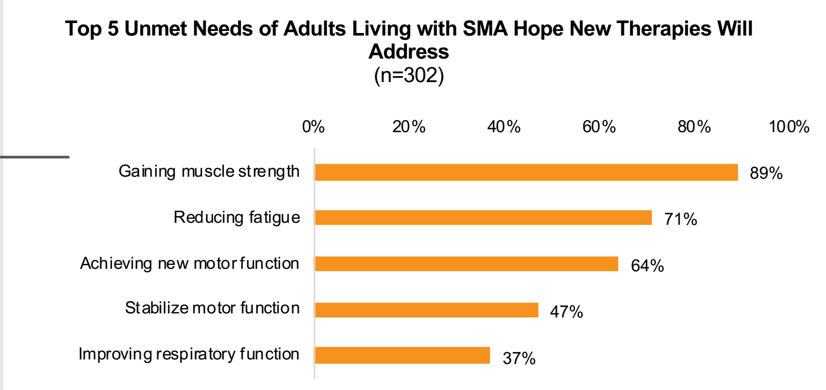
- Publication of the 3rd Annual State of SMA Report sharing highlights from Cure SMA databases including the Membership Database containing patient-reported outcomes, the SMA Clinical Data Registry containing electronic medical record sourced data, and the SMA Newborn Screening Registry containing parent-reported outcomes. Highlights include:
  - Cure SMA estimates **9,000-9,500** individuals with SMA are living in the U.S.
  - **50%** of people living with SMA in the U.S. are adults
  - **60-70%** of individuals with SMA are estimated to have received an FDA-approved treatment
  - A higher percentage of individuals with 2-3 *SMN2* copies that initiated their first treatment before 30 days old were reported to walk alone at 2 years of age compared to individuals that initiated treatment later
  - The majority of adults with SMA report they hope new therapies will help them gain muscle strength


STATE OF SMA

Cure SMA hopes data from this report will foster future research, programs, and development of additional therapies. Please scan the QR code to access the 2023 State of SMA report.

- Launch of the 8th Annual Cure SMA Community Update Survey which collects data and information on the SMA community's experiences and daily challenges from people living with SMA and caregivers of people with SMA.

Responses from adults regarding their unmet needs from the 8th Annual Cure SMA Community Update Survey.


Top 5 Unmet Needs of Adults Living with SMA Hope New Therapies Will Address
(n=302)

- 2 manuscripts accepted for publication: Assessment of Barriers to Referral and Appointment Wait Times for the Evaluation of SMA: Findings from a Web-Based Physician Survey, and Evaluating Perceived Fatigue within an Adult Spinal Muscular Atrophy Population
- 6 abstract presentations at national meetings

CARE

- The 2024 SMA Clinical Care Session of the Annual SMA Research & Clinical Care Meeting had 584 attendees who were offered 5.5 CME/CE credit hours of SMA care information to improve their clinical practice.
- The SMA Care Center Network and Clinical Data Registry collected data on over 1200 patients from 25 care centers across the U.S. These centers have partnered with Cure SMA to improve care for people living with SMA. The collected data contributed to the State of SMA report.
- Analysis of the SMA Care Center Network care quality measures survey results identified 7 essential structural components of SMA care delivery

- Updates and development of resources to address insurance challenges limiting equitable access to SMA treatment and care
- Analysis of SMA newborn screening time to treatment across multiple real world data sources showed decreasing time to treatment year over year
- 3 manuscripts accepted for publication: SMA Update in Best Practices: Recommendations for SMA diagnosis and Recommendations for SMA treatment considerations, and SMA Newborn Screening and Birth Prevalence
- 4 abstract presentations at national meetings

BIOGEN
GENENTECH / ROCHE
NOVARTIS
cure SMA
REAL WORLD EVIDENCE
COLLABORATION

NOVARTIS
BIOGEN
SCHOLAR ROCK
BIOHAVEN PHARMACEUTICALS
GENENTECH/ROCHE
ALCYONE THERAPEUTICS
NMD PHARMA
SMA EUROPE
cure SMA
INDUSTRY
COLLABORATION

ACKNOWLEDGEMENT:

Funding was provided by the Cure SMA Industry Collaboration and the Cure SMA Real World Evidence 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 2023 & 2024 SMA-IC included Cure SMA, Alcyone Therapeutics, Biogen, Biohaven Pharmaceuticals, Epirium Bio, Genentech/Roche, NMD Pharma, Novartis, Scholar Rock, and SMA Europe.

The Cure SMA Real World Evidence Collaboration was established in 2021 to leverage the experience, expertise and resources of pharmaceutical and biotechnology companies and nonprofit organizations involved in development of SMA therapeutics to guide the future direction of real world evidence collection and use in SMA. Members included Cure SMA, Biogen, Genentech/Roche, and Novartis.

FUNDRAISING

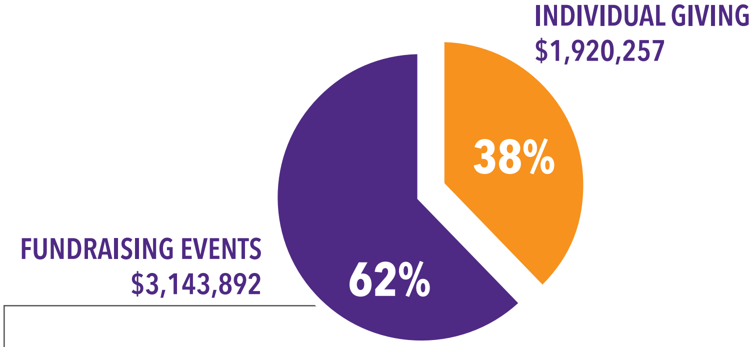
INTRODUCTION

Fundraising is the driving force behind our mission at Cure SMA, enabling us to propel groundbreaking research, provide critical support programs, and drive advocacy efforts that bring hope and tangible progress to the SMA community. In FY24, the dedication and generosity of our donors, corporate partners, volunteers, and event participants enabled us to expand vital resources and invest in the future of care and treatments. Every dollar raised reflects the unwavering commitment of our community to create a world where everyone impacted by SMA can lead full and independent lives. We are deeply grateful to each supporter who has joined us on this journey, and together, we are making a profound and lasting impact.

FY24 FUNDRAISING FINANCIALS

In Fiscal Year 2024, Cure SMA experienced another extraordinary year of generosity, raising $5,064,149 through more than 50 events and donor giving campaigns. These numbers tell a powerful story—but beyond the figures are the countless moments where our community united with a common purpose: to drive progress and offer hope to those impacted by SMA. Each fundraiser, donor, corporate partner, and volunteer played a vital role in advancing our mission, strengthening the bonds of support, and accelerating the search for a cure. As we reflect on this year's achievements, we celebrate not only the dollar raised but the collective impact of the SMA community determined to change the future of SMA.

$5,064,148 RAISED THROUGH
FUNDRAISING EVENTS AND INDIVIDUAL GIVING



Individual Giving = $1,572,014
Workplace Giving = $97,694
Planned Giving = $30,279
Honors & Memorials = $133,144
Walk-n-Roll = $1,597,911
Distinguished Events = $1,122,710
Endurance = $146,985
Do-It-Your Way Fundraising = $276,287

TOTAL = $5,064,148

CURE SMA WALK-N-ROLL

During this fiscal year, the Cure SMA Walk-n-Roll program united communities across the country, raising an impressive $1,597,911 to drive forward life-changing research, essential resources, and critical programming for those impacted by SMA. Over 6,000 participants and 500 teams joined us in multiple cities across the United States and Puerto Rico, spreading awareness and making a powerful impact.

We are incredibly grateful to everyone who participated—your dedication fuels progress and brings us closer to achieving our mission. Thank you for walking, rolling, and making a difference!

Total raised: $1,597,911
Total participants: 6,125
Total teams: 505
Average $ raised by participant: $179

FINANCIAL BREAKDOWN

STATEMENT OF FINANCIAL POSITION

FOR THE YEAR ENDED JUNE 30, 2024 AND 2023

ASSETS	2024	2023
Cash, cash equivalents, and investments	$6,100,092	$6,454,699
Receivables and other assets	4,442,283	5,873,747
Fixed assets, net	195,240	384,858
Total Assets	***$10,737,615***	***$11,297,459***

LIABILITIES	2024	2023
Accounts payable and accrued expenses	$3,161,418	$4,195,716
Research and grants payable	1,421,221	1,724,857
Total Liabilities	4,582,639	5,920,573

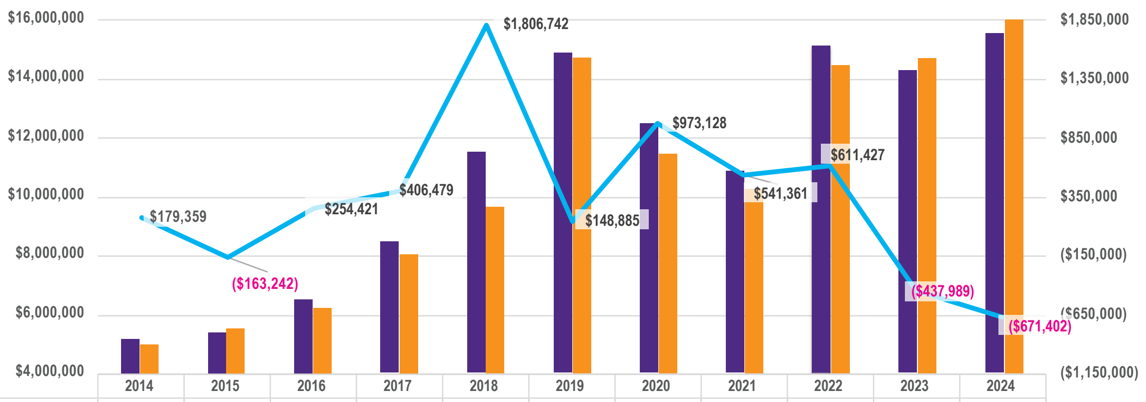
NET ASSETS	2024	2023
Unrestricted	$1,424,129	$1,566,462
Temporarily restricted	2,558,327	3,301,558
Permanently restricted	1,138,873	1,924,711
Total Net Assets	6,121,329	6,792,731
Total Liabilities and Net Assets	***$10,703,968***	***$12,713,304***

STATEMENT OF ACTIVITIES

FOR THE YEAR ENDED JUNE 30, 2024 AND 2023

SUPPORT AND REVENUE	2024	2023
Contributions:		
Contributions and grants	$4,939,282	$6,378,572
Special events, net	2,802,600	3,266,484
Program revenue	6,958,513	3,981,929
Other revenue	348,365	258,614
Total Revenue	15,048,760	13,885,599

EXPENSES	2024	2023
Program Services		
Research	$2,976,714	$3,723,718
Patient services	2,342,300	2,595,626
Community support	6,461,052	4,534,772
Awareness	713,191	669,641
Total program services	12,493,257	11,523,757
Supporting services		
Management & general	1,352,444	1,272,690
Fundraising	1,874,461	1,527,141
Total supporting services	3,226,905	2,799,831
Total Expenses	15,720,162	14,323,588
Increase (decrease) in net assets	***($671,402)***	***($437,989)***



	2014	2015	2016	2017	2018	2019	2020	2021	2022	2023	2024
▬ Revenue	$5,181,409	$5,390,573	$6,530,711	$8,485,733	$11,492,255	$14,885,074	$12,480,715	$10,864,199	$15,130,215	$14,276,205	$15,477,20
▬ Expenses	$5,002,050	$5,553,815	$6,276,290	$8,079,254	$9,685,513	$14,736,189	$11,507,587	$10,322,838	$14,518,788	$14,714,194	$16,148,610
— Net Income (Loss)	$179,359	($163,242)	$254,421	$406,479	$1,806,742	$148,885	$973,128	$541,361	$611,427	($437,989)	($671,402)

Financial information presented has been summarized from financial statements audited by Warady & Davis LLP dated June 30, 2024 and 2023.
**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:

RESEARCH 34%

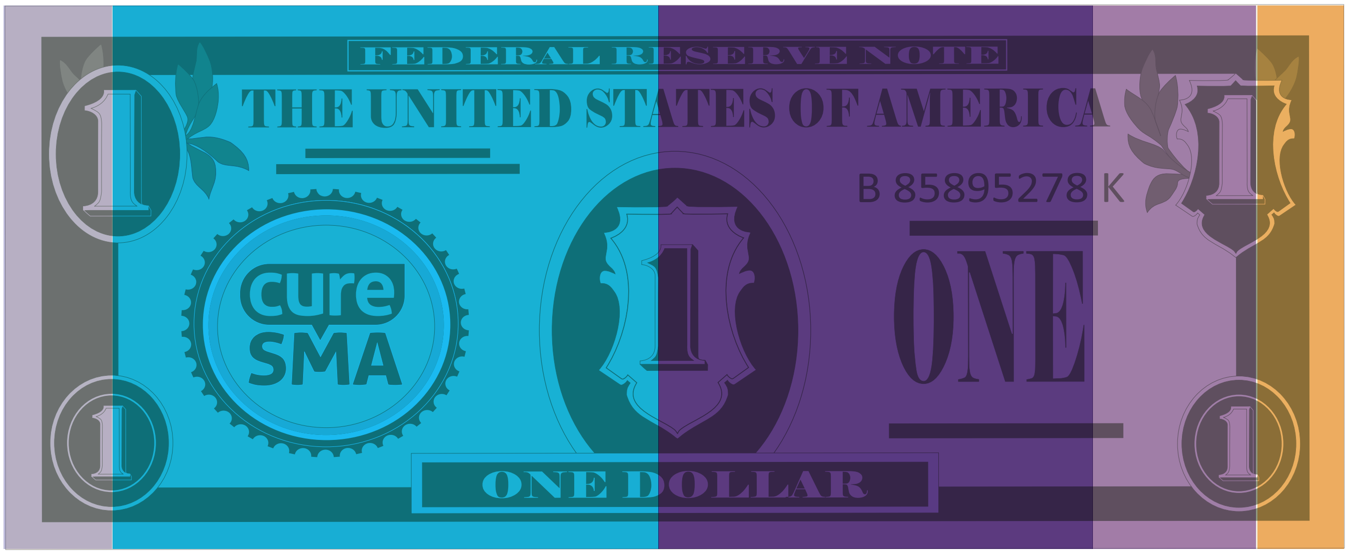
Advancing scientific discovery by funding basic research to understand the biology of SMA, identifying new targets and effective strategies for drug development, and supporting clinical trials and research to secure approval and ensure access to practical new treatments.

MANAGEMENT & GENERAL 9%

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

FUNDRAISING 12%

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



COMMUNITY SUPPORT 41%

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.

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.

Note: The figures above are taken from the 2024 fiscal year. Our complete financial information can be found at [www.curesma.org/annual-reports-financials/.](www.curesma.org/annual-reports-financials/)

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