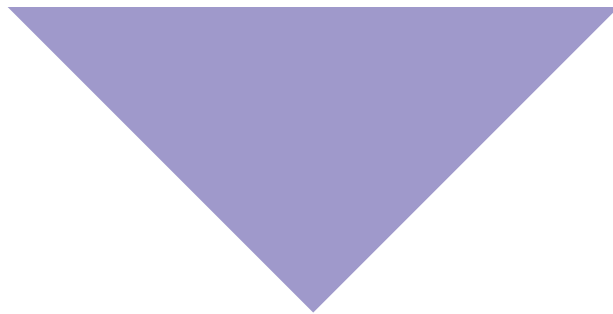




# 2019 ANNUAL REPORT



## Dear SMA Community,

Another year and another big step forward for the SMA community. It was in May that we saw the U.S. Food and Drug Administration (FDA) grant approval for Zolgensma – a gene therapy for SMA and the second approved therapy in less than three years. We are proud of the role Cure SMA has played in driving the research agenda that has led to two FDA-approved treatments—with more on the horizon—and we will forge ahead with research into new areas as we continue our pursuit of a cure for SMA.

With the rise in newborn screening leading to earlier diagnosis and the introduction of treatments extending lifespan, many children with SMA may be able to live longer and healthier lives. This changing landscape will require a new standard of care for SMA—not only for young children but also for teens and adults—if we are going to achieve our mission in support of the SMA community. Our comprehensive education programs for healthcare professionals, as well as the roll-out of the Cure SMA Care Center Network, will help to expand access to approved SMA treatments and the development of an evidence-based standard of care.

As we grow our care initiatives, we remain committed to the research that is transforming the future of SMA. Today we have two approved treatments, another one under review with the FDA, and nearly 20 additional ongoing research programs. In order to develop effective non-SMN targeted therapies that will work together with existing treatments to create the best possible outcomes for all ages and stages of SMA, Cure SMA will fund research grants that target new systems and processes in the body. This may include treatments that increase muscle strength or motor neuron function.

Finally, we continue our investment in the core tenant of Cure SMA's mission—to support individuals and families living with SMA and offer resources and services that allow them to live their lives to the fullest. The Annual SMA Conference remains the cornerstone of our community engagement initiatives and we now offer new locally driven Summit of Strength programs that are uniting our community around support, advocacy, and care. This year, we also expanded our support for teens and adults with SMA, creating and distributing a new care package that included helpful items that support daily tasks and enhance independence.

We know the highlights laid out in this report are a direct result of the hard work and dedication of our entire community – affected individuals, families, clinicians, researchers, and donors. Thank you for all you do, as we would not be where we are today without you or your continued support.

Best,



Kenneth Hobby  
President



Richard Rubenstein  
Chairman of the Board

# Research Breakthroughs Advance Treatment for SMA

Cure SMA has a rich history of supporting research aimed at improving quality of life, expanding treatment options, and advancing the understanding of SMA. In 2019, we saw the promise of our research investments continue to enhance the treatment landscape, which now has multiple therapeutic options available.



## **Zolgensma Approved for SMA in Patients Under 2 Years of Age**

On May 24, the U.S. FDA approved Zolgensma for the treatment of children less than 2 years of age with SMA.



## **FDA Grants Priority Review to Risdiplam for People Living with SMA**

On November 25, the U.S. FDA accepted the New Drug Application and granted Priority Review for risdiplam for the treatment of SMA.



## **Spinraza Continues Momentum with New Global Approvals**

As of September 2019, it is reported that more than 9,300 patients have been treated with Spinraza in over 40 countries.



# Nationwide Programs Offer Support for Families Affected by SMA

Each year, Cure SMA provides families affected by SMA with resources to help them live active, engaged, and hopeful lives. These programs provide tangible support for families affected by SMA, easing the difficulties of an SMA diagnosis, and showing each family that they are not alone. Our national support programs are available to all people affected by SMA. Most of these resources are provided at no cost.

- **Information packets** for newly diagnosed families, relatives, or friends, as well as healthcare providers looking to raise the standards of care for those affected by SMA.
- **Care packages** for newly diagnosed children, sent automatically and filled with toys and helpful items that have been recommended by other SMA parents.
- **Support packages** available upon request for teens and adults with SMA that includes items recommended by other adults living with SMA, including items that allow for increased independence with certain tasks they might not have been able to do on their own.
- An **equipment pool** where Cure SMA loans, free of charge, necessary equipment, such as adaptive strollers, wheelchairs, car beds, standers, and more.

## WHAT WE'VE ACHIEVED TOGETHER IN 2019

**450** NEWLY DIAGNOSED FAMILIES REACHED

**\$5.2MILLION**

INVESTED IN RESEARCH AND CARE

**2,400** FAMILIES, CLINICIANS, AND RESEARCHERS AT ANNUAL SMA CONFERENCE

**2,953** PATIENT SUPPORT ITEMS SENT

**22** DRUG PROGRAMS CURRENTLY IN PROGRESS

**1** NEWLY APPROVED THERAPY, MAKING **2** TREATMENT OPTIONS

**36** STATES TAKING ACTION ON NEWBORN SCREENING

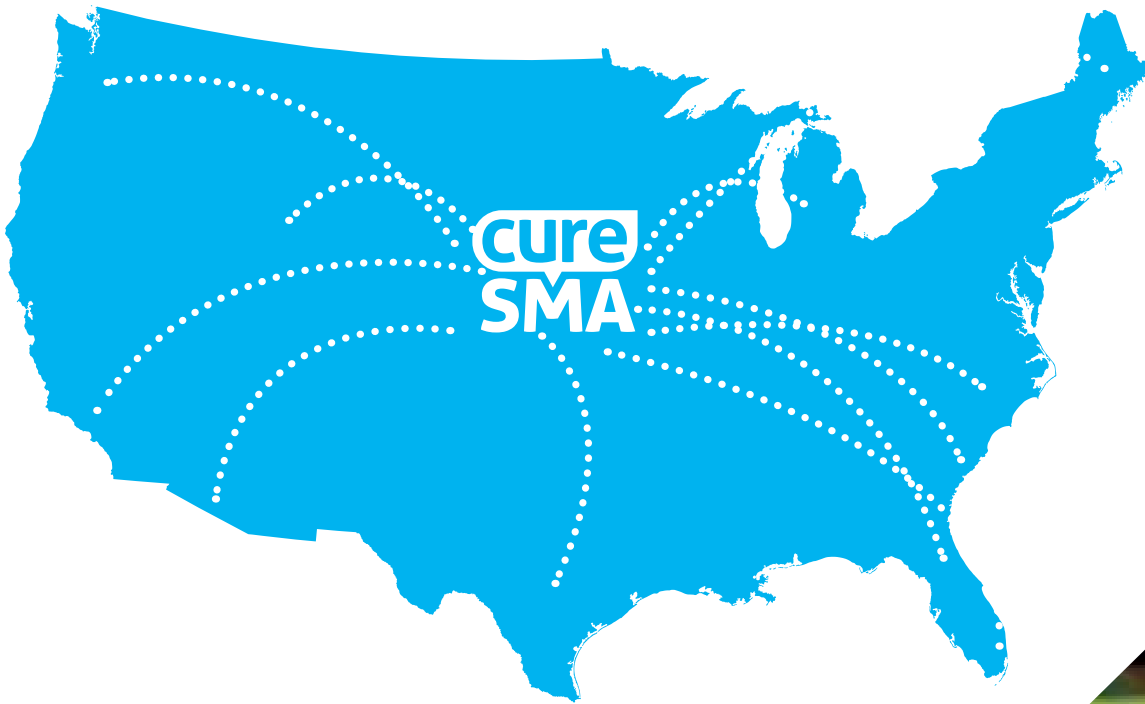
**18** CARE CENTERS IN THE CURE SMA CARE CENTER NETWORK



## Local Events Fosters Greater Connections in SMA Community

As the largest organization dedicated to SMA, Cure SMA is uniquely positioned to not only leverage the community's collective strength for a national impact, but also empower its 36 chapters across the country to offer personalized, local support to each individual and family affected by SMA. Whether joining a Walk-n-Roll, hosting a gala, golf, or special event, or organizing a local Family Social, chapters offer a variety of ways to connect as a community.

In late 2018, Cure SMA launched the Summit of Strength Program—a series of single-day, educational events providing information for people of all ages and types of SMA, as well as their loved ones and caregivers. These “mini conferences” give attendees the opportunity to network and learn from national and local experts about the latest advances in support, treatment, care, and advocacy. Along with the informative presentations, attendees can also meet their Cure SMA chapter leaders and connect with the rest of the local SMA community. This local engagement further strengthens the bonds that make our communities so vibrant!



In just the last 16 months, Cure SMA has hosted 36 Summits of Strength reaching 2,900 registered attendees.



# Cure SMA Care Center Network and SMA Clinical Data Registry Expand

Our primary focus is supporting care of the highest value to individuals with SMA. The mission of the Cure SMA Care Center Network is to provide the best care—including offering new therapies—as well as gather and disseminate new knowledge to advance the SMA standard of care for pediatric and adult persons with SMA.

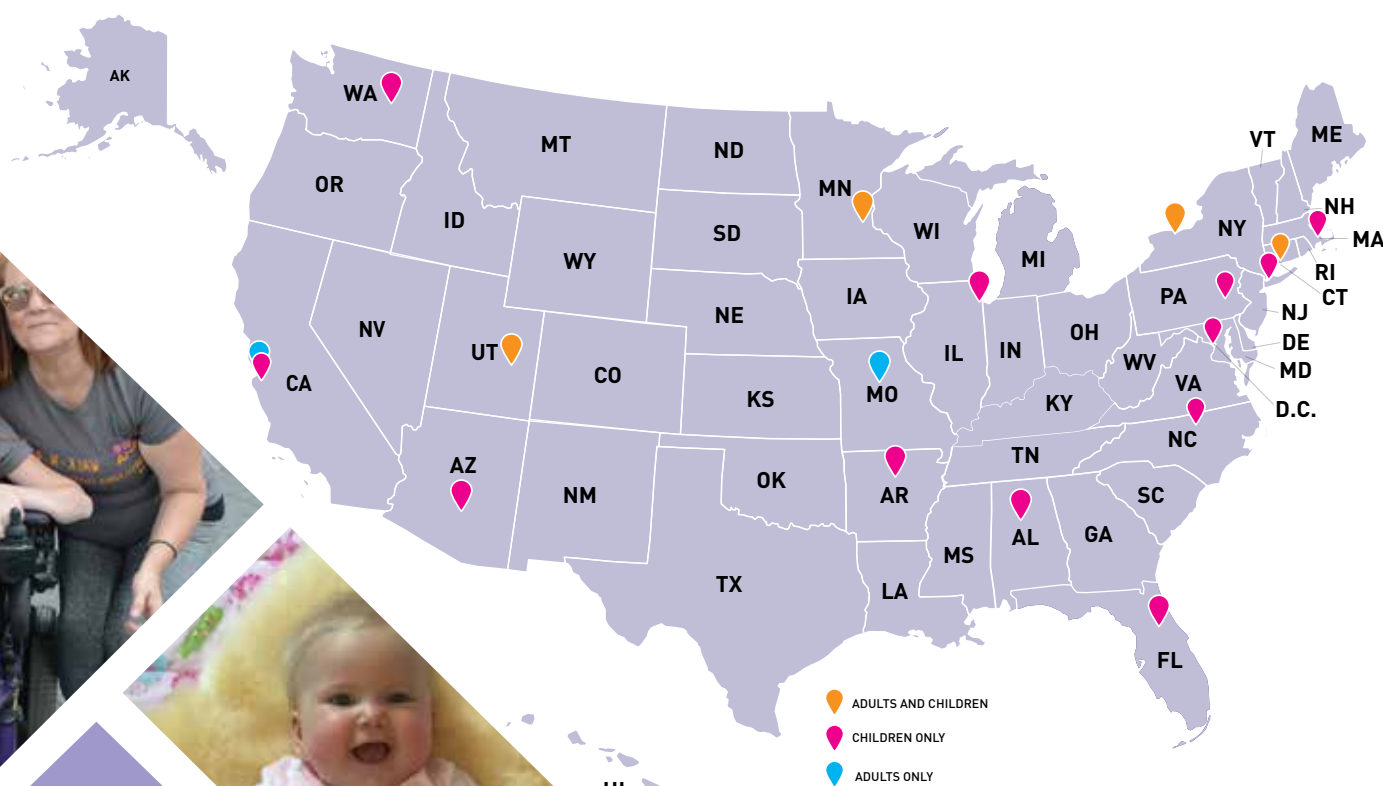
The care for people with SMA is more important than ever to ensure timely access to new treatments and optimized outcomes. The Cure SMA Care Center Network has expanded to 18 partners, as of December 2019, with the plan to expand to 24 Care Centers in 2020. These Centers across the country represent pediatric and adult care centers, provide multidisciplinary care, and contribute consented SMA patient data electronically to the SMA Clinical Data Registry. This real-world data will lead to the creation of evidence-based guidelines for care, better characterization of the changing SMA phenotype, and move care and new treatments forward.

## CURE SMA CARE CENTER NETWORK COMMITTEE:

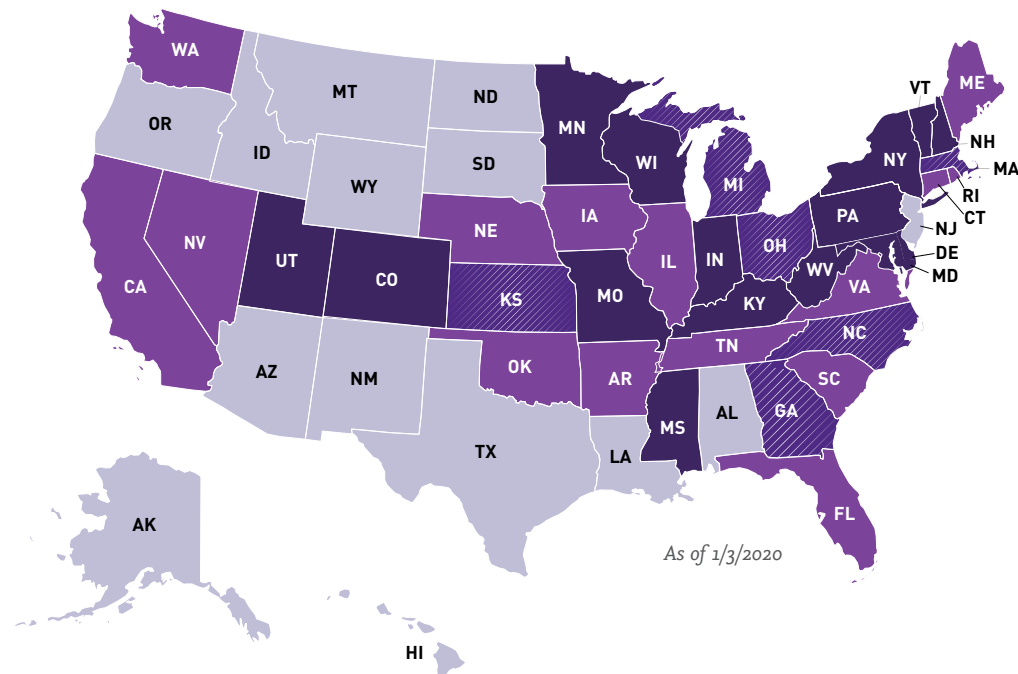
Mary Schroth, MD, Chair  
Shelley Coskery, RN, MSN, CRNP-AC  
Kapil Arya, MD  
Lauren Camarda, MD  
Randal Richardson, MD  
Raghav Govindarajan, MD  
Basil Darras, MD  
Diana Castro, MD  
Gyula Acsadi, MD, PhD

## SMA CLINICAL DATA REGISTRY COMMITTEE:

Mary Schroth, MD, Chair, MD  
Diana Bharucha-Goebel  
Jacqueline Montes, PT, EdD, NCS  
Russell Butterfield, MD, PhD  
Richard Finkel, MD  
Michael McDermott, PhD  
Saunder Bernes, MD  
Emma Cialfoni, MD



# States Continue to Make Progress Toward SMA Newborn Screening Adoption and Implementation



*Dr. Francis Collins, Director, National Institutes of Health, gave the keynote address at Cure SMA's 9th Annual "Hope on the Hill" Congressional Dinner, December 3, 2019, in Washington D.C.*

Cure SMA continues its work with our community of individuals, families, and partners across the country to push for the state-level adoption and implementation of newborn screening. The complexities of state implementation for newborn screening require a sophisticated, multifaceted approach, and Cure SMA has been working on newborn screening through several different avenues, bringing our full resources to ensure that babies born with SMA can receive the most effective treatment.

We also remain engaged at the federal level, ensuring that federal newborn screening programs are adequately funded, particularly those designed to help states begin screening for newer conditions like SMA. In 2019, we provided nearly \$1 million in grants to support state-level implementation of newborn screening for SMA.

The upcoming 2020 legislative sessions are key to continuing progress toward nationwide SMA newborn screening implementation. Cure SMA is working actively in states that are holding legislative sessions this year to advocate for SMA screening action.



# Cure SMA Continues to Advance a Comprehensive Research Program

In FY19 (July 1, 2018-June 30, 2019), Cure SMA awarded approximately \$5.2 million in new research funding to accelerate work that focuses on developing treatments and improving care for all types, ages, and stages of SMA, including symptomatic patients. To do this, we have directed our research funding beyond survival motor neuron (SMN) to include new biological systems, pathways, and processes—like the neuromuscular circuitry—which hopefully will serve as the basis of novel SMA drugs or combination therapies. In addition to the funding of grants for the exploration of novel drug targets, Cure SMA continues to lead the SMA Industry Collaboration (SMA-IC) to move drug development forward in a way that incorporates the patient voice into this process.

**Since the inception of the SMA-IC in 2016, we have seen tremendous growth in the breadth of projects with our seven pharmaceutical and biotechnology partners. In 2019, efforts were directed toward several critical projects, including:**

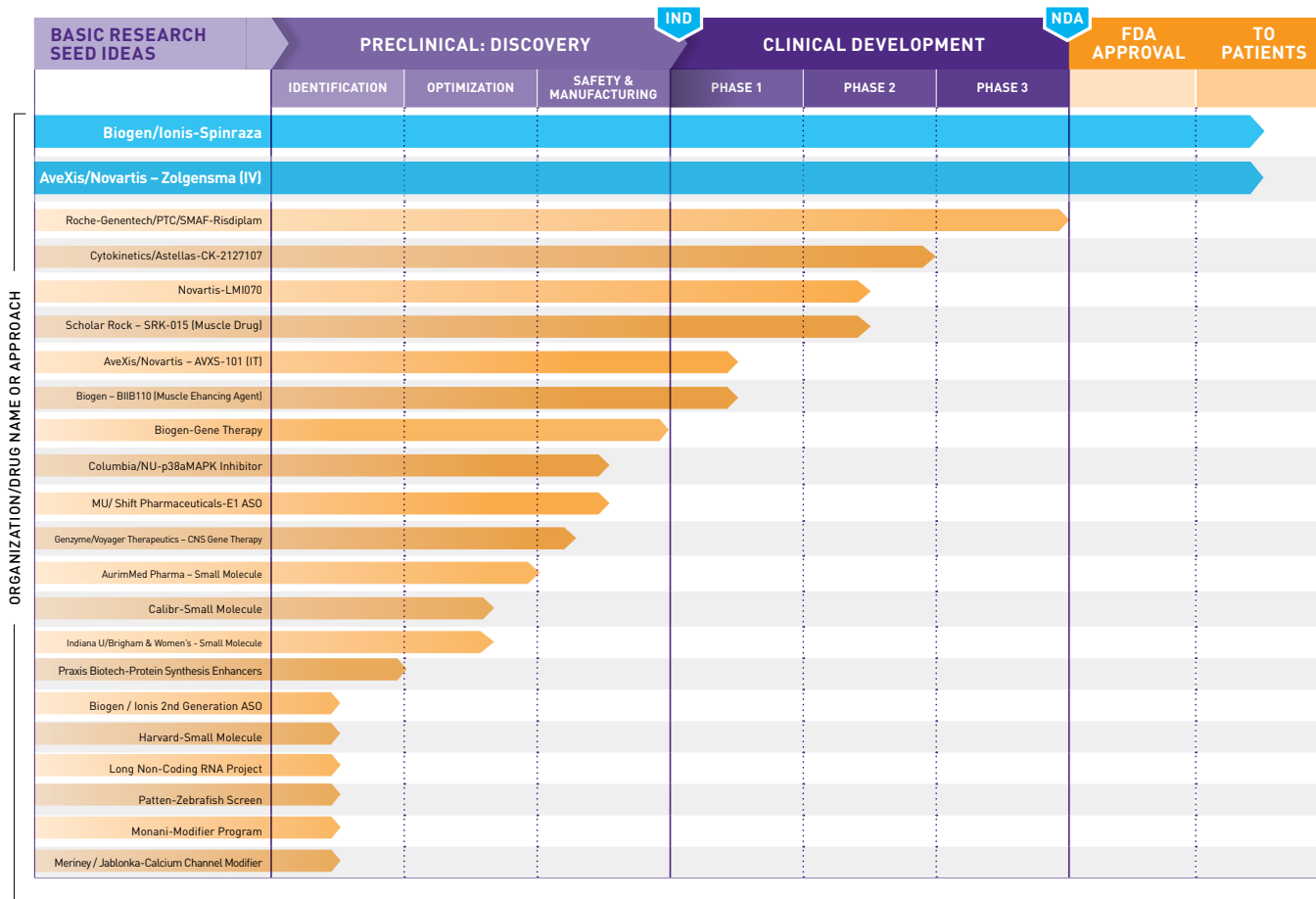
**Clinical Trial Readiness:** To increase clinical trial site capacity, Cure SMA launched the Clinical Trial Readiness Program, which included a series of educational resources (e.g., best practice toolkits, in-person workshops and webinars, online resource hub, etc.) that help to enhance a site's ability to conduct effective, patient-centric clinical trials in SMA. We expect 15 new sites to have been completed the program by the end of 2019.

**SMArt Moves Education Campaign:** In late 2018, Cure SMA launched SMArt Moves, a disease awareness campaign to empower new parents and healthcare professionals to promptly recognize and diagnose the early signs of SMA. In 2019, to promote the resources for healthcare professionals (e.g., diagnostic toolkit, instructional videos, CMEs), we partnered with key stakeholders in the rare disease space and academia—NORD, CNS, AAN, Global Genes, PPMD, Genetic Alliance, and AMDA—to engage more than 5,400 healthcare providers this year.

**Patient Reported Data Project and Annual Community Survey:** This project aims to both better understand the natural history of SMA through the patient view and to document the changes in SMA as new treatments become available. Data collected when new patients first contact us and respond to the annual community survey is used to better understand real-world experiences of people living with SMA. This data informs healthcare providers, regulators, and payers on SMA and its changing landscape.

**Enhance and Advance Drug Development:** By bringing the voice of the patient to regulators, we can improve their understanding of the shifting burden of SMA and what remains clinically meaningful for affected individuals and caregivers. Several projects in 2019 that supported this effort include the development of a resource booklet on the complex topic of combining treatments, the publication of research findings on the “Benefit-Risk Community Survey in SMA” in Clinical Therapeutics, and the development of a survey on clinical meaningfulness in teens and young adults.





IND = Investigational New Drug    NDA = New Drug Application    Last updated: November 2019

We're funding and directing research with more breadth and depth than ever before, aiming to target new pathways, systems, and processes within the body that may help treat SMA. The SMA drug pipeline reflects the SMA community's progress in gaining access to Spinraza and Zolgensma, as well as the progress of additional research programs currently in development or under review with the FDA.

## The Cure SMA drug pipeline identifies four possible treatment targets:

1. Replacement or correction of the faulty SMN1 gene.
2. Modulation of the low functioning SMN2 "back-up gene."
3. Neuroprotection of the motor neurons affected by loss of SMN protein.
4. Muscle protection to prevent or restore the loss of muscle function in SMA.



# Annual SMA Conference Attendance Continues to Grow and Offer Value to Community

Cure SMA's Annual SMA Conference is the largest SMA conference in the world, bringing together researchers, healthcare professionals, and families to network, learn, and collaborate. It is the centerpiece of our national support program. The 2019 Annual SMA Conference at the Disneyland Hotel in Anaheim, Calif., was an impactful weekend filled with community, research, care, and fun for the more than 2,400 attendees—our largest conference to date!

The 4-day weekend included a variety of workshops, research discussions, and specials events for all attendees – children, teens, and adults. The Conference began on Friday with the Newly Diagnosed Program, Meet & Greet, and Researcher Relay Race. These events brought together new and returning families, clinicians, and researchers (as well as a host of Disney characters) in a fun, relaxed setting before the start of conference workshops. The weekend brought family workshops and the Family Friendly Researcher Poster Session,

where families had direct access to the latest information about research, and researchers had the opportunity to build personal connections with the patients who will benefit from their work.

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, an Evening in the Park, and the newly-established “Adults with SMA Lounge” that provided a space for adults with SMA to mingle and relax.

The 2020 Annual SMA Conference will take place June 11-14, 2020, as we head to Disney's Yacht & Beach Club Resort in Orlando, Florida! The SMA Research and Clinical Care Meeting will begin on June 10, 2020.

## SMA Clinical Care Meeting

In conjunction with the Annual SMA Conference, Cure SMA hosted its 2nd Annual SMA Clinical Care Meeting. This meeting provided a valuable opportunity for healthcare providers to share knowledge around SMA patient care and network among the 350 clinical attendees representing 19 countries and 70 different institutions. Attendees also had the opportunity to network with other members of the SMA community, including persons with SMA, families, and researchers.

## SMA Researcher Meeting

The SMA Researcher Meeting is held each year alongside the Annual SMA Conference. With more than 700 attendees, it is the largest international SMA research meeting in the world. Its goal is to create open communication of scientific data to accelerate the pace of research. It also helps to build productive collaborations – including cross-disciplinary dialogue, partnerships, integration of new researchers and drug companies, and educational opportunities for junior researchers.



# 2019 ANNUAL SMA CONFERENCE





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 Nick Farrell, Vice Chair  
 Gillian Mullins, Secretary  
 Peter Statile, Treasurer

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 Corey Braastad  
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 Shannon Shryne  
 Shannon Zerzan  
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Allie Balleau

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Established in 2019, the Adult Advisory Council is a group of volunteers offering guidance, advice, and feedback to Cure SMA based on members' experiences as individuals living with SMA. The council makes recommendations on topics related to support programs, patient care, engagement, independence, advocacy, accessibility, education, transition to adulthood, and much more.

## ADULT ADVISORY COUNCIL

Colleen McCarthy O'Toole, Chair,  
 Cure SMA VP of Events & Family Support  
 Angela Wrigglesworth  
 Angie Lee  
 Brad Nunemaker  
 Brynne Willis  
 Carolyn Barrett  
 Collin Pollock  
 Dany Sun

Doug McCullough  
 Emma Lockwood  
 Kevin Schaefer  
 Lyza Weisman  
 Mary McHale  
 Sandy Spoonmore  
 Shannon O'Brien  
 Shannon Zerzan



# Financial Breakdown

## STATEMENT OF FINANCIAL POSITION FOR THE YEAR ENDED JUNE 30, 2019 AND 2018

ASSETS	2019	2018
Cash, cash equivalents, and investments	\$5,584,223	\$5,908,022
Receivables and other assets	3,834,150	1,544,464
Fixed assets, net	844,177	580,930
Other Assets	113,736	117,862
<b>Total Assets</b>	<b>\$10,376,286</b>	<b>\$8,151,278</b>

LIABILITIES	2019	2018
Accounts payable and accrued expenses	\$2,215,545	\$1,703,132
Research and grants payable	3,055,935	1,492,224
<b>Total Liabilities</b>	<b>5,271,480</b>	<b>3,195,356</b>

NET ASSETS	2019	2018
Unrestricted	\$497,975	\$1,357,096
Temporarily restricted	3,440,835	2,522,697
Permanently restricted	1,165,996	1,076,129
<b>Total Net Assets</b>	<b>5,104,806</b>	<b>4,955,922</b>

<b>Total Liabilities and Net Assets</b>	<b>\$10,376,286</b>	<b>\$8,151,278</b>
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## STATEMENT OF ACTIVITIES FOR THE YEAR ENDED JUNE 30, 2019 AND 2018

SUPPORT AND REVENUE	2019	2018
Contributions:		
Contributions and Grants	\$8,470,475	\$7,662,812
Chapter Events, net	3,680,607	2,471,474
Conference	2,007,413	1,159,409
Other Revenue	119,505	198,560
<b>Total Revenue</b>	<b>14,278,000</b>	<b>11,492,255</b>

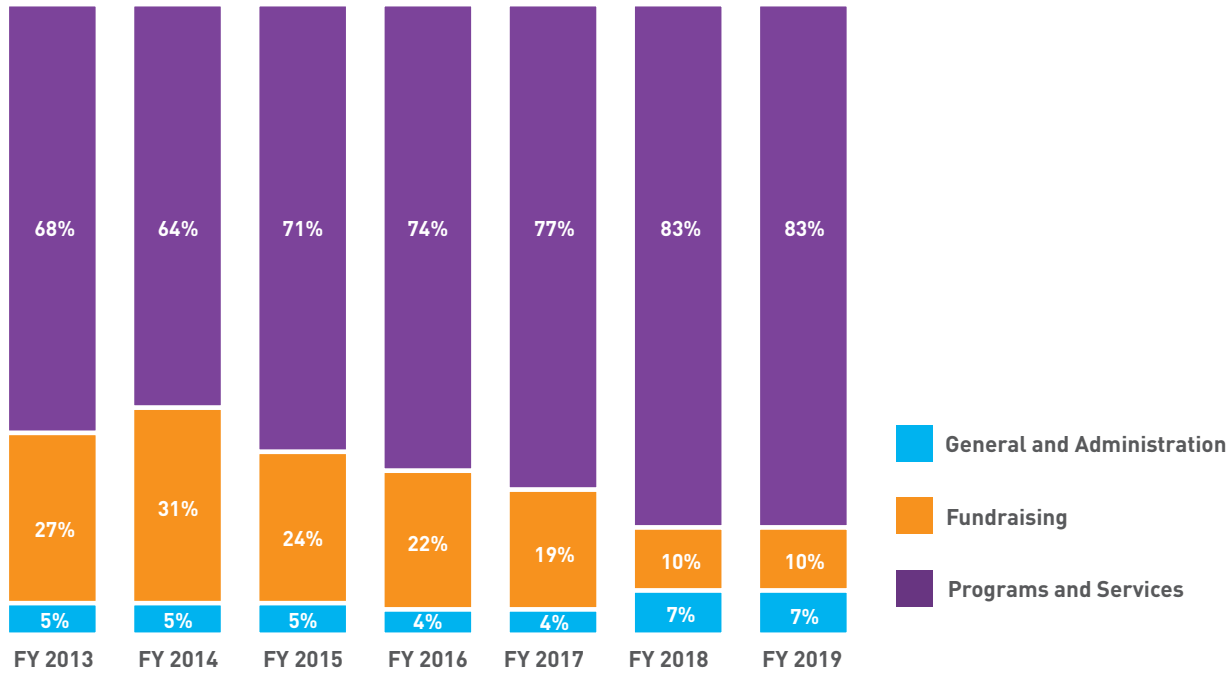
EXPENSES	2019	2018
Research	\$5,155,498	\$4,760,605
Patient Services	3,313,869	1,349,632
Family Support	2,462,687	1,296,855
Awareness	763,974	593,677
Management & General	1,010,788	669,278
Fundraising	1,422,300	1,015,466
<b>Total Expenses</b>	<b>14,129,116</b>	<b>9,685,513</b>

<b>Total Net Income</b>	<b>\$148,884</b>	<b>\$1,806,742</b>
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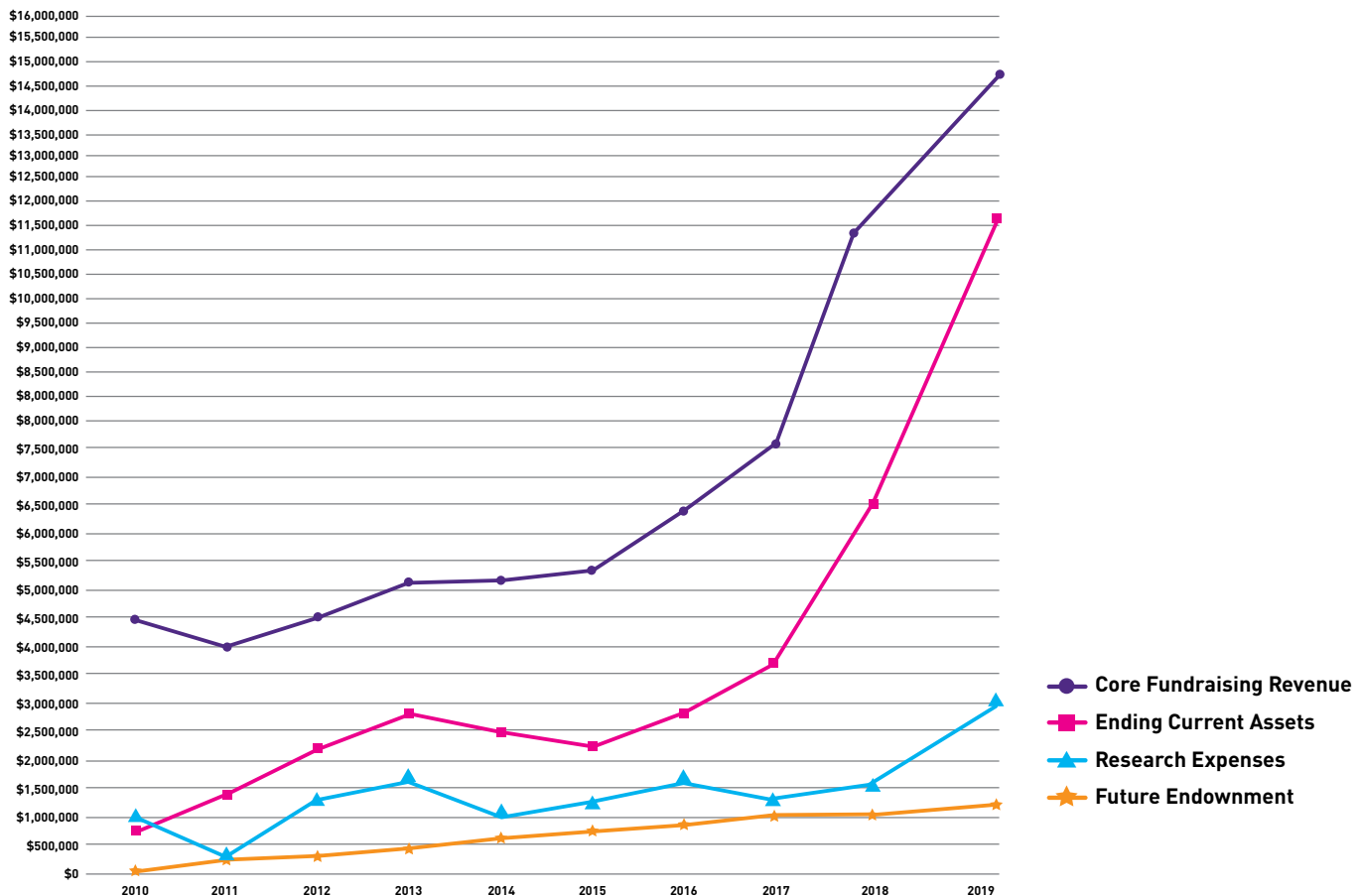
Financial information presented has been summarized from financial statements audited by Warady & Davis LLP dated June 30, 2019.

## Where Your Contributions Go

83 percent of every dollar spent in 2019 funded, research, patient services, family support, and awareness.



### Historical Trends





[twitter.com/cureSMA](https://twitter.com/cureSMA)



[@curesmaorg](https://www.instagram.com/curesmaorg)



[facebook.com/cureSMA](https://facebook.com/cureSMA)



[youtube.com/user/FamiliesofSMA1](https://youtube.com/user/FamiliesofSMA1)



[www.linkedin.com/company/families-of-sma](https://www.linkedin.com/company/families-of-sma)



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