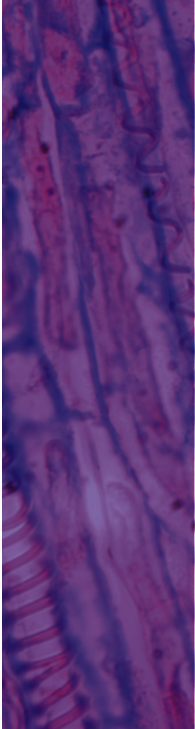
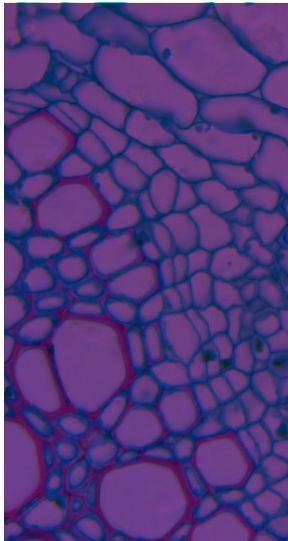
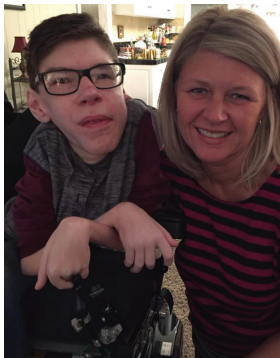
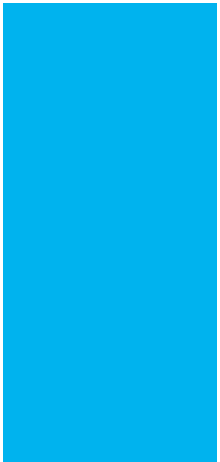
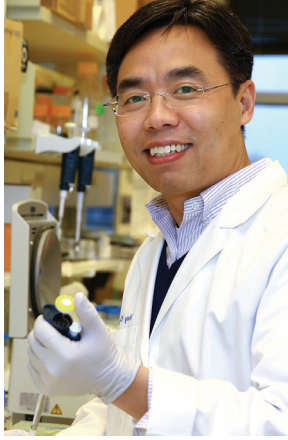
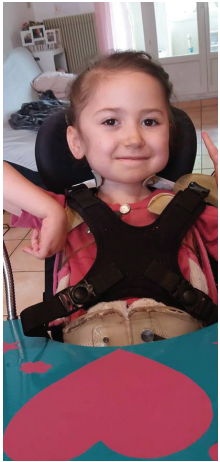
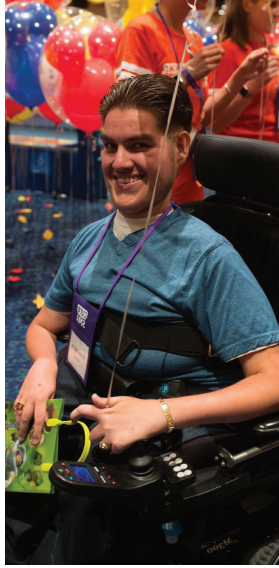
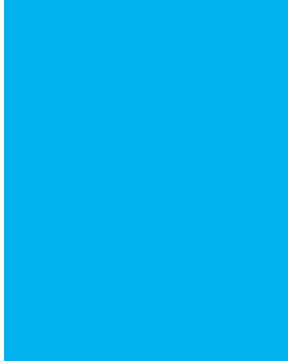
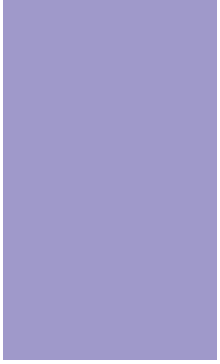


2020 ANNUAL REPORT





Dear SMA Community,

In what can only be described as a most unusual year, the spinal muscular atrophy (SMA) community was still able to make significant progress. Highest on the list was in August when the U.S. Food and Drug Administration (FDA) granted approval for Evrysdi, the third approved therapy for SMA in less than four years. We are proud of the role Cure SMA has played in driving a research agenda that has led us to this point, but it's imperative our community continue to press forward together, because our work toward a cure for SMA is not done.

This year, we also saw great progress in implementing newborn screening for SMA across the U.S., with nearly 7 in 10 babies now being screened at birth. Based on the latest timelines, we hope to reach more than 90 percent of all newborns screened for SMA by the end of next year. The rise in newborn screening for SMA is leading to earlier diagnosis and the introduction of treatments sooner, which means children with SMA can live longer and healthier lives.

With this changing landscape in mind, we continued to forge ahead on our work to expand the Cure SMA Care Center Network, grow our knowledge through the SMA Clinical Data Registry, and ultimately develop an evidence-based standard of care. As we grow our care initiatives, we remain committed to directing and investing in new research that will lead to new symptomatic and muscle-targeting treatments that will work in combination with a disease-modifying genetic therapy.

Finally, as the SMA community remained focused on keeping safe and healthy throughout the ongoing global pandemic, Cure SMA moved rapidly to launch a series of virtual programs that have allowed us to come together for education and social engagement. Our first-ever Virtual SMA Conference and SMA Research & Clinical Care Meeting was a great achievement, and the engagement continued with our Summit of Strength webinars and series of community social events. We also launched financial and physical support packages to help the community during these times of isolation. Despite all these successful programs, we do hope that 2021 will bring us back together in-person.

The successes you will see highlighted in this report are a direct result of the hard work and dedication of the entire community—individuals with SMA, their families, clinicians, researchers, and donors. Thank you for all you do, as we would not be where we are today without you and your continued support.

Best,



Kenneth Hobby
President



Nick Farrell
Chairman of the Board



COMMUNITY SUPPORT

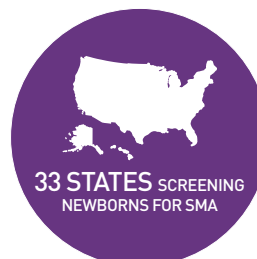
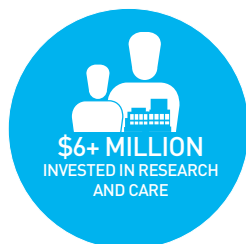
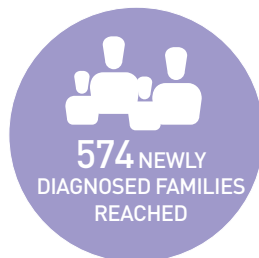
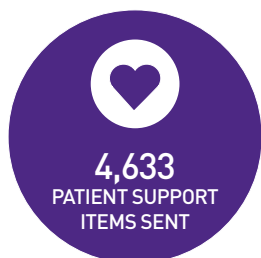
A Year of Virtual Engagement and Creative Community Building

Despite the 2020 reality of quarantines, remote working, and social distancing, Cure SMA made sure our work for the spinal muscular atrophy (SMA) community continued. With the rapid and unexpected changes, Cure SMA remained vigilant in monitoring and responding to the needs of the SMA community by maintaining our standard support programs (i.e., newly diagnosed and adult/teen support packages, equipment pool, etc.), hosting a Virtual 2020 Annual SMA Conference, and launching new support programs to help individuals with SMA and their families during the Coronavirus (COVID-19) pandemic. We also were able to expand our engagement among adults with SMA, working with the Adults with SMA Advisory Council to better understand their advocacy interests and determine new programs to support engagement on an ongoing basis.

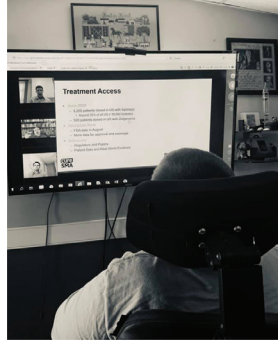
From the beginning of the global health crisis, Cure SMA was proactive in its response and set out to create new programs and resources that supported the SMA community, including:

- **COVID-19 Information Center & Community Webinars:** Provided the most up-to-date information and resources in support of the health and advocacy needs of the entire SMA community, including individuals, families, and healthcare providers.
- **COVID-19 Support Packages:** Helped members of the SMA community obtain essential items that are difficult to locate, such as PPE, hand sanitizer, and antibacterial wipes. Two versions of the package were launched in April and November.
- **COVID-19 Assistance Program:** To help alleviate some of the financial burden for those in the SMA community who may be experiencing hardships due to the pandemic, we offered a \$50 gift card to help with purchasing items such as meals, groceries, or other essential supplies.
- **Summit of Strength (SOS) Webinar Series:** Considering SOS event cancellations, these weekly virtual seminars brought experts in to speak on a variety of educational topics tailored to help individuals with SMA and families during this life in quarantine.
- **SMA Community Social Events:** Virtual community engagement events for various segments within the SMA community—such as adults with SMA, families, and children/teens—provided that much-needed social boost that we have been missing from one another. Virtual events geared toward adults with SMA were especially popular, including the adult hang-outs and trivia nights. In 2021, we will see even more adult-focused virtual events, such as coffee dates and book clubs.

What We've Achieved Together In 2020



COMMUNITY SUPPORT



Hosting the First-Ever Virtual SMA Conference

As we entered 2020 and prepared to meet up in Orlando in June, no one could have imagined what the world was about to face and how our lives would be changed. And, when it was clear that it was safest for everyone to stay home, Cure SMA quickly pivoted its plans for the 2020 Annual SMA Conference and SMA Research & Clinical Care Meeting, moving everything virtual.

The 2020 Virtual SMA Conference took place June 8-12, 2020, and although we were unable to come together in-person, individuals with SMA, their families, clinicians, and SMA researchers virtually attended the conference from 77 countries around the globe. It was an impactful week of online workshops, networking, and community-building—**with more than 6,600 participants taking part in 28 offered live events.**

The Virtual SMA Conference kicked off on Monday, June 8, with the Opening Session, Clinical Care & Research Update Session, and a Newly Diagnosed Virtual Session. This set the stage for a week of education and engagement, where the SMA community heard presentations on topics such as Aquatic Therapy, Women's Health in SMA, Education, Medical Management of Adults, Breathing Basics, and more.

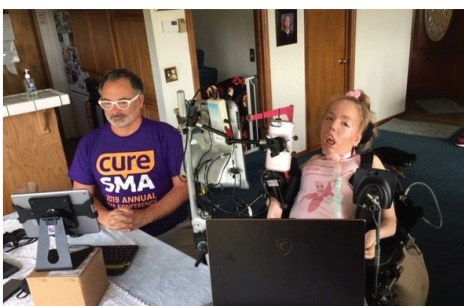
We also hosted virtual networking and social events, including an SMA grandparents "virtual mingle" and several Adults with SMA social events. These evening events were an opportunity to gather online in a friendly setting and connect via Zoom.

The conference concluded with the special celebration, where all members of the SMA community were invited to share messages of unity on their social channels. In an enthusiastic display of our community's strength, **more than 5,000 people liked and shared Cure SMA's content** or posted their own messages to show how we were all "United, At Home." We look forward to coming back together for the 2021 Annual SMA Conference in June.

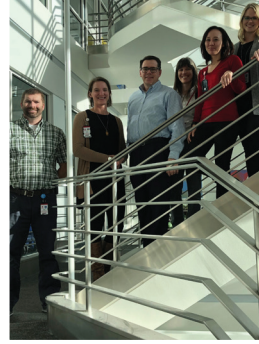
Virtual SMA Research & Clinical Care Meeting

On Wednesday, June 10, Cure SMA kicked off the 2020 Virtual SMA Research & Clinical Care Meeting with a Clinical Care CME Session that shared updates on innovations in clinical care and the impact of new therapies. It also provided a valuable opportunity to share knowledge and earn continuing education credits for 423 clinical attendees. This session also emphasized the value and importance of multidisciplinary care teams in SMA care and created meaningful dialogue between care providers across the globe.

The research portion of the meeting continued Thursday, June 11, with a session on Basic and Clinical Research, and on Friday, June 12, with a session on Clinical Drug Development. The goal here was to create open communication of scientific data, in hopes of accelerating the pace of research. The meeting also sought to build productive collaborations—including cross-disciplinary dialogue, partnerships, integration of new researchers and drug companies, and educational opportunities for junior researchers.



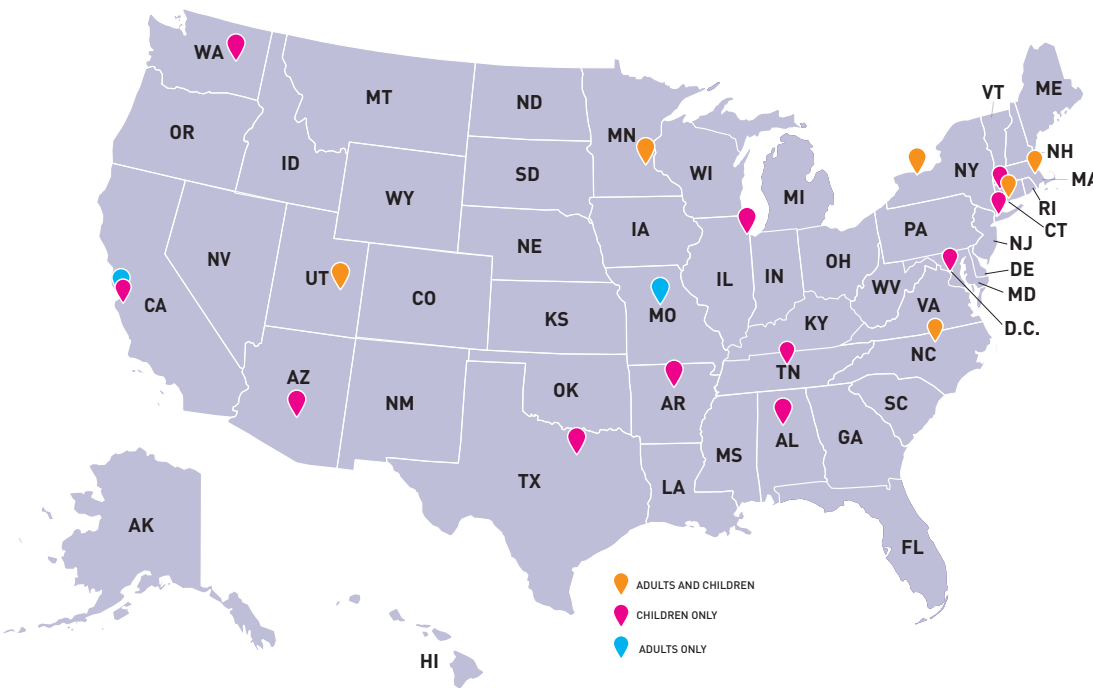
CLINICAL CARE



Continued Support of Clinical Care for SMA

Therapies approved by the U.S. Food and Drug Administration (FDA) are extending lifespans and, as a result, increasing the number of individuals living with spinal muscular atrophy (SMA). The care for people with SMA is more important than ever to ensure timely access to these treatments and to achieve the best possible outcomes.

The SMA Care Center Network empowers families and healthcare providers to collaborate by connecting centers to one another and utilizing a united SMA registry to collect and share information. Cure SMA partners with established neuromuscular care centers across the U.S. to establish best care and practices for all people with SMA. As of the end of 2020, the Cure SMA Care Center Network included 19 centers that provide SMA care and all FDA approved treatments. These SMA Care Centers are diverse in geographic location, number of patients, and types and ages of patients.



Care Center Sites

- Advocate Children's Hospital, Park Ridge, IL
- Arkansas Children's Hospital, Little Rock, AR
- Boston Children's Hospital, Boston, MA*
- Children's of Alabama, Birmingham, AL
- Children's National, Washington, DC
- Columbia University Medical Center, New York, NY*
- Connecticut Children's Medical Center, Hartford, CT
- Duke University Medical Center, Durham, NC
- Gillette Children's Specialty Healthcare, St. Paul, MN
- Phoenix Children's Hospital, Phoenix, AZ
- Seattle Children's Hospital, Seattle, WA
- Stanford Children's Health, Palo Alto, CA*
- Stanford Healthcare, Palo Alto, CA*
- University of Missouri Health Care, Columbia, MO
- University of Rochester Medical Center, Rochester, NY
- University of Utah, Utah Program for Inherited Neuromuscular Disorders, Salt Lake City, UT
- University of Texas Southwestern/Children's Health, Dallas, TX
- Vanderbilt University Medical Center, Nashville, TN
- Yale Pediatric Neuromuscular Disorder Clinic, New Haven, CT

*Pediatric Neuromuscular Clinical Research (PNCRN) Sites

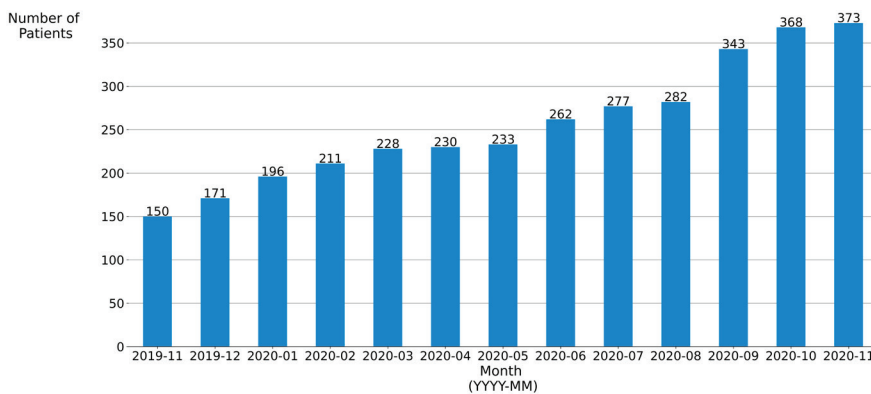
These Care Centers across the country represent pediatric and adult care centers, contributing consented SMA patient data electronically to the SMA Clinical Data Registry. This real-world data will lead to the creation of evidence-based guidelines for care, better characterization of the changing SMA phenotype, and move care and new treatments forward.



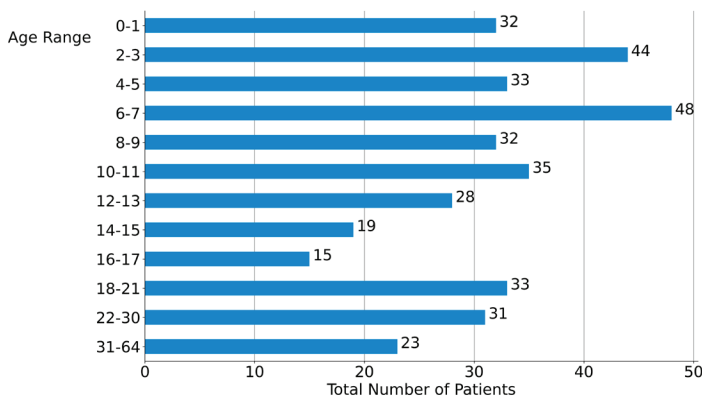
Update on the SMA Clinical Data Registry

The core of the Cure SMA Care Center Network is the SMA Clinical Data Registry, which secures electronic medical record information from individuals with SMA who receive care at a Network site. The SMA Clinical Data Registry continues to grow as new patients are consented at Cure SMA Care Center Network sites across the country. As of December 15, 2020, there were 380 patients in the Registry, representing a diverse cross-section of individuals with SMA in the U.S.

Patient Accrual (over the last year)

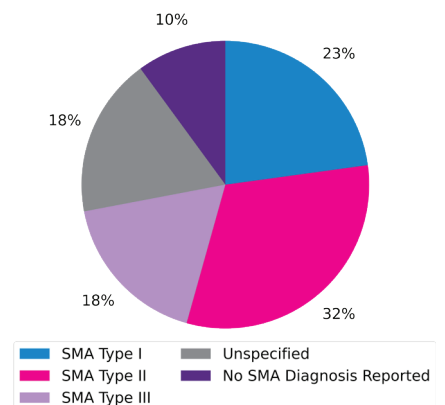


Age Distribution of Patients



Age Breakdown
3 months Youngest
64 years Oldest
9 years Median

SMA Type



SMA Type I	84
SMA Type II	116
SMA Type III	65
Unspecified	66
No SMA Diagnosis Reported	37

Treatment Highlights



Spinraza Clinical Trials Focus on Unmet Clinical Needs

Globally launched clinical trials (e.g., DEVOTE, RESPOND) to improve outcomes for all people with SMA. 11,000+ people worldwide have received Spinraza.



Evrysdi Approved for SMA in Patients Over 2 Months

On August 7, the U.S. FDA approved Evrysdi to treat adults and children 2 months of age and older in the U.S. To date, 275+ patients have received Evrysdi.



Zolgensma Maintains Momentum with New Global Approvals

900+ individuals with SMA globally have been treated with Zolgensma, including in clinical trials, commercially, or through managed access programs.

ADVOCACY

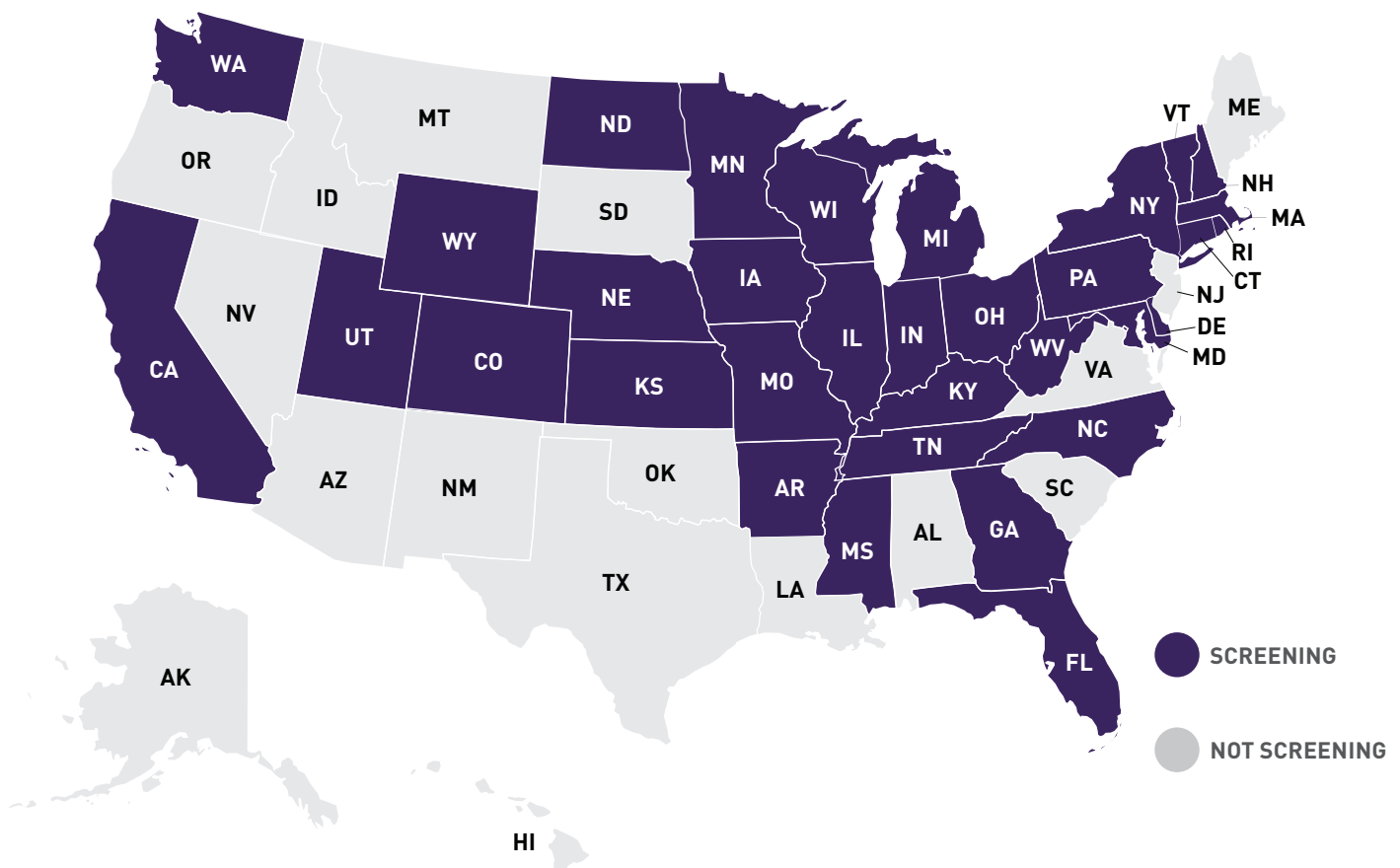


SMA COMMUNITY ADVOCACY MEANS 2 IN 3 U.S. BABIES ARE NOW SCREENED FOR SMA

We are proud that approximately 2.6 million babies born in the U.S. are now screened for spinal muscular atrophy (SMA), according to a nationwide newborn screening status report. This report was released by Cure SMA at the two-year anniversary of SMA being added to the Federal Recommended Uniform Screening Panel (RUSP).

As of the end of 2020, there are 33 states now screening newborns for SMA through permanent and pilot programs. Together, these SMA screening states represent more than two-thirds (68%) of all U.S. births. The percentage of newborns screened increased considerably in 2020, with the implementation action by large states, such as California, Florida, Illinois, and Washington State. Cure SMA expects the percentage of newborns screened through a state program will increase to close to 90 percent by the end of 2021 when Texas, which represents more than 10 percent of all U.S. births, and other key states start screening, based on current Cure SMA estimates.

Cure SMA will continue working with SMA families and partners in all non-screening states to help expedite implementation of newborn screening for SMA to ensure babies in every state have the best chance at a healthy and full life.





Cure SMA Advocates for SMA Community During COVID-19 Pandemic

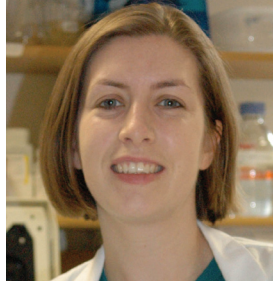
From the start of the Coronavirus (COVID-19) public health emergency, Cure SMA was actively educating policymakers at the federal and state level about the needs, concerns, and impacts of the pandemic on the SMA community. **Our advocacy efforts, which have been informed by direct conversations and survey responses from the SMA community, have included:**

- Cure SMA letters to Congress, Governors, and key state health care officials on the unique care, treatment, equipment, and other needs of children and adults with SMA and their families.
- Letter downloads for the SMA community to send directly to their state officials on their specific COVID-19 related needs.
- Grassroots action alert to Congress to ensure safe and ongoing access to community services, including in-home care and personal care attendants.
- Participation in sign-on letters with other national organizations seeking protections for people with disabilities in vaccine allocation once a COVID-19 vaccine is made available.
- Direct advocacy and collaboration with other national organizations to seek priority access and protections for people with disabilities in vaccine allocation once a COVID-19 vaccine is made available.

Cure SMA also joined other national healthcare, rare disease, and disability organizations in letters urging federal and state officials to stop the discrimination of individuals with underlying health conditions, such as SMA, in accessing healthcare and equipment (e.g., ventilators) during the public health emergency. Separately, Cure SMA wrote to all 50 state Governors demanding that they rescind any discriminatory rationing of care policies. This collective effort has resulted in policy reversals and gubernatorial statements supportive of equal access to care and equipment during this crisis.

Cure SMA Works to Ensure Access to Treatments

Cure SMA continues to educate public and private insurers on the ongoing needs of the SMA community and the need for access to life-altering medications. Cure SMA contacted all major private and public payers following the recent U.S. Food and Drug Administration (FDA) approval of Evrysdi. In addition, our advocacy continues to promote full access to Spinraza and Zolgensma. Our outreach highlights the clinical evidence of treatments, and asks for full, unrestricted access to treatments for all individuals in accordance with each treatments' respective FDA label. In addition, Cure SMA has presented testimony and provided public comment in support of full and unrestrictive access to treatments, therapies, equipment, and services.



Cure SMA Met with U.S. FDA for Critical Path Innovation Meeting

In August, Cure SMA met with the FDA in a Critical Path Innovation Meeting (CPIM), where key leaders at the FDA had the opportunity to communicate with clinical investigators, members of academia, industry, scientific groups, and patients about how to improve efficiency and success in the next stages of drug development for SMA. At the virtual CPIM, Cure SMA worked to enhance the understanding among these regulatory leaders of the most significant unmet medical needs of children and adults with SMA. Discussions could affect the design of future clinical trials, expectations for future therapies, and help to better meet the changing needs of our community.

Comprehensive Research Program Continues to Propel SMA Treatment and Care

Cure SMA has a rich history of supporting research aimed at improving quality of life, expanding treatment options, and advancing the understanding of spinal muscular atrophy (SMA). In FY20 (July 1, 2019-June 30, 2020), we awarded approximately \$3.2 million in new research funding to accelerate work that focuses on developing treatments and improving care for all people with SMA.

This year, Cure SMA also committed \$2.2 million in funding for the Pediatric Neuromuscular Clinical Research Network (PNCRN), a network of highly skilled clinical trial investigators, clinical evaluators, clinical coordinators, statisticians, and data management personnel. Originally funded by The SMA Foundation in 2004, the PNCRN established a team of SMA clinical trial experts that have integrated clinical research, education, and care to achieve the best possible SMA clinical trial outcomes. Cure SMA has collaborated as co-sponsor of the PNCRN since 2018.

In recent years, the PNCRN has been involved in the seminal clinical trials that have led to the U.S. Food and Drug Administration (FDA) approvals of breakthrough SMA treatment options that have forever changed the natural history of the disease. We see our support of the PNCRN leading to more ground-breaking collaboration in SMA research and clinical care.

Six sites comprise the PNCRN for SMA, including Boston Children's Hospital, Boston, MA; Children's Hospital of Philadelphia, Philadelphia, PA; Columbia University Irving Medical Center, New York, NY; Nemours Children's Health System, Orlando FL; Stanford University, Palo Alto, CA; and the data coordinating center at the University of Rochester, Rochester, NY. These clinical research and treatment sites have also now been integrated into the established Cure SMA Care Center Network, which will lead to real-world evidence that increases access to approved treatments for SMA and improves care for individuals and families affected by SMA.

In addition to these research programs, we have continued our work in the Cure SMA-led Industry Collaboration. The Collaboration is a multi-faceted partnership that brings together pharmaceutical companies, Cure SMA, and other nonprofit organizations to address scientific, clinical, and regulatory topics that are critical to advancing drug development in SMA. The three collaboration topics groups are moving forward work to expand access to clinical trials; educate parents and physicians about SMA to reduce time to diagnosis; and engage with regulators to help them understand the unmet needs in SMA and the economic burden of the disease. In FY20, members of the Industry Collaboration included Genentech/Roche Pharmaceuticals, Novartis Gene Therapies, Biogen, Cytokinetics, and Scholar Rock.



NOVARTIS



Biogen



ScholarRock

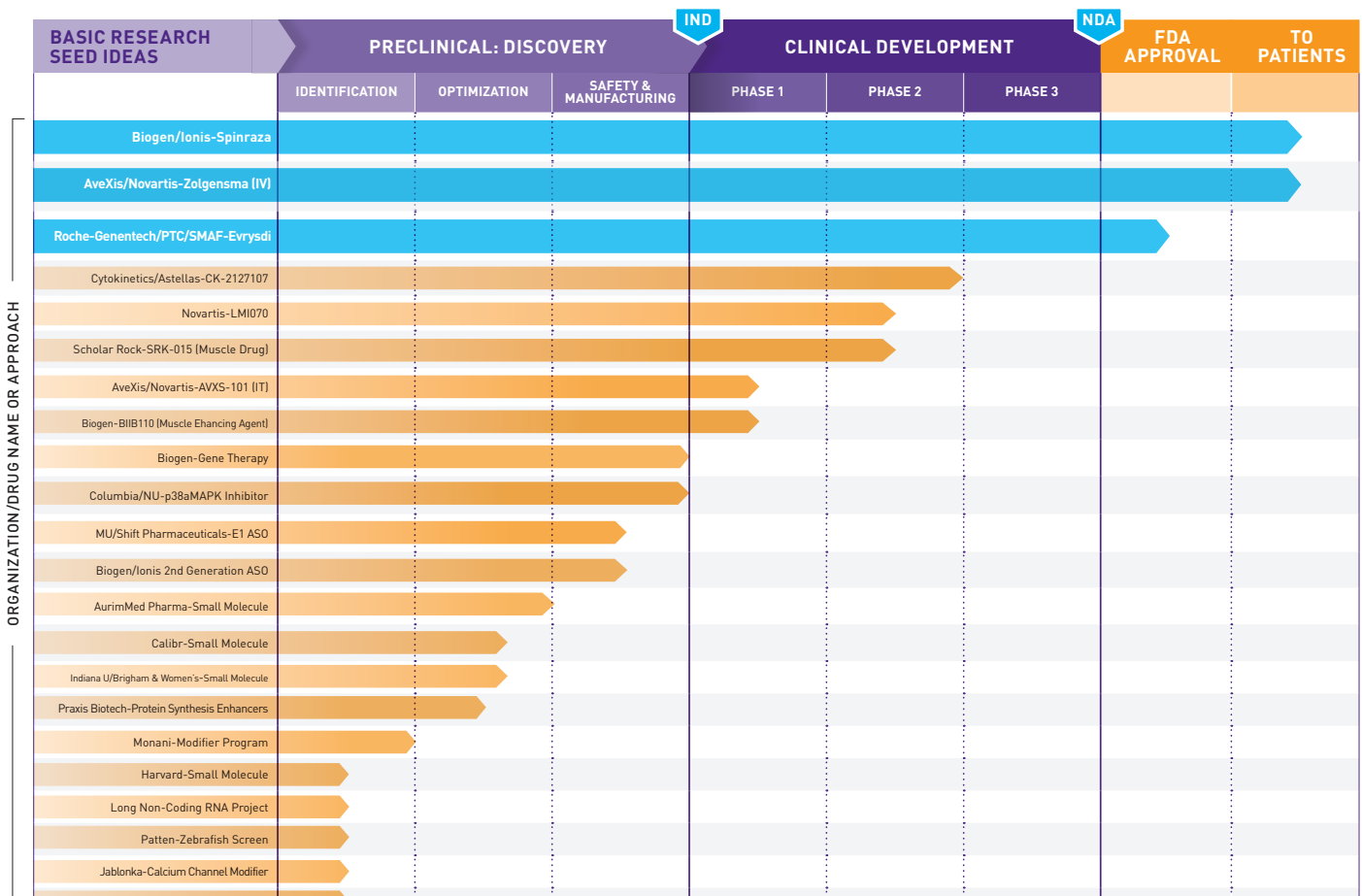




Cure SMA Drug Pipeline

Cure SMA has directed and invested in comprehensive research that has shaped the scientific community’s understanding of SMA, led to breakthroughs in treatment and care, and provided individuals and families the support they need today.

As our thinking around treatment needs for SMA evolves, new research strategies will lead to symptomatic and muscle-targeting therapies that will work in combination with approved disease-modifying genetic treatments.



IND = Investigational New Drug NDA = New Drug Application Last updated: September 2020

The SMA Drug Pipeline reflects the SMA community’s progress in gaining access to Evrysdi, Spinraza, and Zolgensma, as well as the progress of additional research programs currently in development. We continue to identify and explore 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.

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RICHARD RUBENSTEIN

We would like to extend our gratitude and congratulations to Richard Rubenstein for his time and efforts dedicated to Cure SMA since 2009. He became Chairman of the Board in 2013 and has now completed his term in this role this year.

During that time, he advanced the organization's key strategic initiatives—pushing forward with new support programs for the adult community and local patient care for all. His dedication has helped bring SMA to where it is today, with widespread newborn screening and three FDA-approved treatments that have forever changed the diagnosis, treatment, and care for SMA.

We are pleased that he will be staying with our Board as a member emeritus in the years ahead.

Thank you, Richard for your years of dedication, support, and leadership to the mission to Cure SMA!



Kenneth Hobby
President



Nick Farrell
Chairman of the Board



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FINANCIAL BREAKDOWN

In 2020, as the world was met with the challenges of the global COVID-19 pandemic, Cure SMA was able to transition much of our programming to continue supporting the SMA community virtually while minimizing the financial impact for our ongoing research and care initiatives.

STATEMENT OF FINANCIAL POSITION FOR THE YEAR ENDED JUNE 30, 2020 AND 2019

ASSETS	2020	2019
Cash, cash equivalents, and investments	\$7,871,329	\$5,584,223
Receivables and other assets	1,374,739	3,834,150
Fixed assets, net	713,654	844,177
Other assets	192,252	113,736
Total Assets	\$10,151,974	\$10,376,286
LIABILITIES	2020	2019
Accounts payable and accrued expenses	\$1,491,995	\$2,215,545
Research and grants payable	2,582,049	3,055,935
Total Liabilities	4,074,044	5,271,480
NET ASSETS	2020	2019
Unrestricted	\$1,463,867	\$497,975
Temporarily restricted	3,371,596	3,440,835
Permanently restricted	1,242,467	1,165,996
Total Net Assets	6,077,930	5,104,806
Total Liabilities and Net Assets	\$10,151,974	\$10,376,286

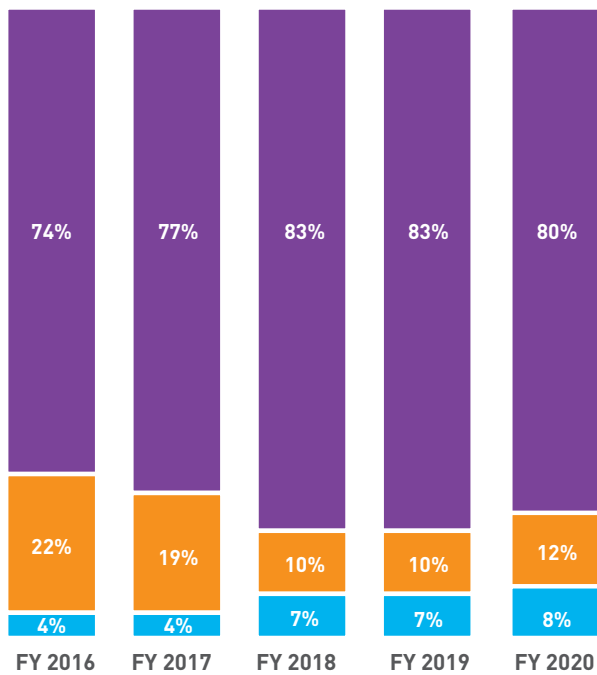
STATEMENT OF ACTIVITIES FOR THE YEAR ENDED JUNE 30, 2020 AND 2019

SUPPORT AND REVENUE	2020	2019
Contributions:		
Contributions and grants	\$6,991,682	\$8,470,475
Chapter events, net	3,395,622	3,680,607
Conference	1,640,500	2,007,413
Other revenue	75,412	119,505
Total Revenue	12,103,216	14,278,000
EXPENSES	2020	2019
Research	\$3,808,347	\$5,155,498
Patient services	2,262,997	3,313,869
Family support	2,100,125	2,462,687
Awareness	731,290	763,974
Management & general	858,845	1,010,788
Fundraising	1,368,488	1,422,300
Total Expenses	11,130,092	14,129,116
Change in Net Assets from Operations	\$973,124	\$148,884

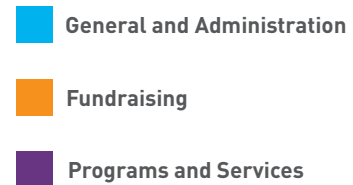
Financial information presented has been summarized from financial statements audited by Warady & Davis LLP dated June 30, 2020 and 2019.



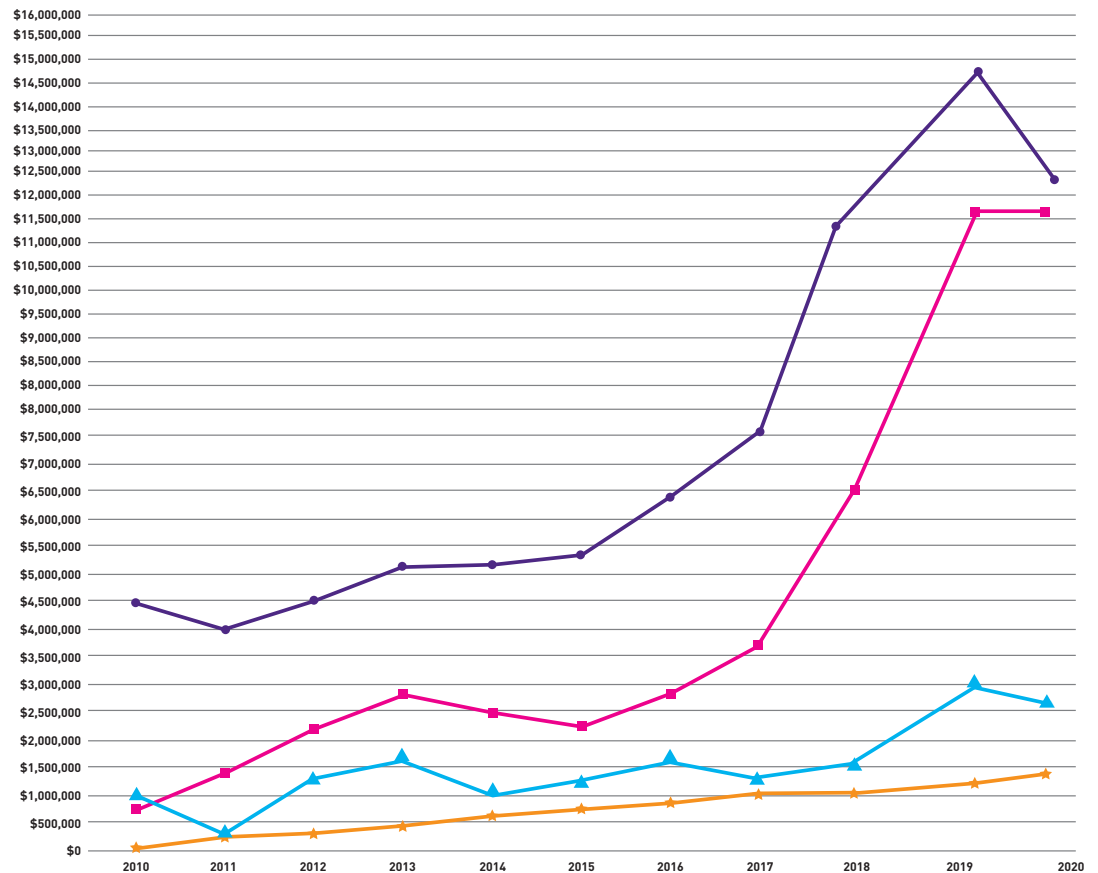
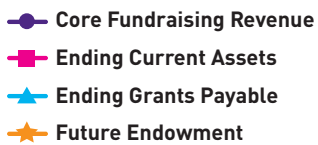
WHERE YOUR CONTRIBUTIONS GO

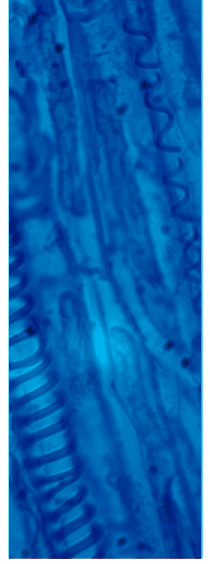


80 percent of every dollar spent in 2020 funded research, patient services, family support, and awareness.



Historical Trends





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