Dear Friends,

On December 23, 2016, the FDA announced that it had approved Spinraza, the first-ever approved therapy for SMA. The months that have followed this groundbreaking announcement have been filled with celebration and hope for the future, but it has also been a time of reflection. Seventeen years ago, we had just two potential drugs in the beginning stages of preclinical discovery. Today we have an approved treatment, plus another 16 programs in development, including six in clinical trials. While these are momentous steps forward, it’s imperative our community press forward together, because our work is not done.

Thanks to the dedicated support of our community, we have invested nearly $70 million in research since 1984, including a planned $5 million investment in the coming fiscal year. There’s great promise in the research landscape, and so there’s also a pressing need for continued and growing investment, which is why Cure SMA is dedicating twice as much funding toward research and care in the next fiscal year. It will likely take a combination of therapies using different approaches and targets to achieve the greatest possible effect for SMA. We will continue to advance a comprehensive research program, funding the most promising projects to make the greatest impact on the future of SMA.

An SMA diagnosis can be overwhelming, which is why we attack SMA from all sides, including giving patients and families the information and resources they need to live active, engaged, and hopeful lives today. As we look ahead, we know the SMA landscape is evolving and the needs of our community are changing. This is why we’re currently working to develop a network of local care and treatment centers that will collaboratively collect patient care data to answer questions and deliver optimal clinical management of SMA. We are also working to implement support services on a local level to meet these changing needs of the SMA community.

When a small group of families founded this organization, then known as Families of SMA, in 1984, they did so with the hope that we would one day change the course of SMA for all those affected. We have now started to see this hope become real.

We extend our deepest gratitude to all of you who have been partners in this journey. The successes you will see highlighted in this report are a direct result of the dedication and generosity of our community.

Best,

Kenneth Hobby
President

Richard Rubenstein
Chairman of the Board
WHo W E A R E

VISION AND MISSION

Cure SMA leads the way to a world without spinal muscular atrophy, the number one genetic cause of death of infants. We fund and direct comprehensive research that drives breakthroughs in treatment and care, and we provide families the support they need for 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 spinal muscular atrophy. 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 affected by SMA 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.

HISTORY OF CURE SMA

In 1984, Audrey Lewis and a small group of families joined together so they could fund research toward a treatment and cure for SMA, and find new ways to support one another. For 30 years, that organization was known as Families of SMA. During that time, the community grew to well over 100,000 members and supporters.

In 2014, Families of SMA became Cure SMA, with the goal of continuing to expand the community. While families remain at the heart of our mission, the goal was to attract even more people to be part of our work, in order to sustain and even accelerate momentum toward our ultimate goal of a cure for SMA.
The 2017 Annual SMA Conference at Disney’s Contemporary Resort in Orlando, FL, was the largest in the history of our community. A record-breaking 2,073 attendees, including 448 researchers—also a record—came together for four days of workshops, meetings, symposiums, and fun.

Our annual conference is an invaluable resource for the entire SMA community, bringing together researchers, healthcare professionals, and families to network, learn, and collaborate. The conference is the largest in the world focused specifically on SMA, and it attracts the top scientists and companies in the field. Families have direct access to the latest information about research, and researchers have an opportunity to build personal connections with the patients who will benefit from their work. The conference is one of the most powerful examples of our community in action.

The conference kicked off with one of the most beloved events: our Researcher Relay Race. SMA researchers and children race to see who can cross the finish line first, with one rule: researchers have to race in a manual wheelchair. Conference attendees stand on the edge of the course cheering on the racers. Our 2017 crew of kid racers came through with another perfect record!

The 2017 conference also hosted record attendance to our annual newly diagnosed program. The newly diagnosed program provides education and community support to individuals and families during an often difficult time in their lives. Registration fees and hotel costs for newly diagnosed families are covered by Cure SMA.

Following the FDA approval of Spinraza, Cure SMA launched a newborn screening initiative, in partnership with the SMA Newborn Screening Coalition. Newborn screening would allow infants to begin receiving treatment even before showing symptoms of SMA, when data suggests it may be the most effective.

At the Annual SMA Conference, we announced the launch of a grassroots advocacy campaign to implement newborn screening in all 50 states. Shortly after the conference, Missouri Governor Eric Greitens signed Missouri S.B. 50, making Missouri the first state to institute newborn screening for SMA.
Each year, Cure SMA provides families affected by SMA with resources to help them live active, engaged and hopeful lives. Our 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 and that there is hope. Our family support programs also help build and maintain a strong and unified community.

The cornerstone of our family support programs is our newly diagnosed outreach. Because of increased awareness from the approval of Spinraza, a record number of families reached out to us for the first time last year. We’re committed to making sure that these families and all our families have the best, most accurate information about SMA and what it means for them, from day-to-day care to the changing landscape of research breakthroughs. In addition, individuals affected by SMA need specialized care and equipment, which can put enormous financial pressure on families. Cure SMA makes sure families understand all the options and resources available to them, and our equipment pool gives families access to specialized items like car beds, strollers and bathing systems at no cost. In the last year, 229 families were assisted by loans from our equipment pool.

In total, 1,535 families received family support services from Cure SMA last year, thanks to the generosity of our donors. Many of these families also choose to participate in grassroots fundraising events across the country to raise support for others affected by SMA. From walk-n-rolls to galas to marathons to bowl-a-thons, and everything in between, over 260 events were held this year.
The developments of this past year showed why there’s such great reason for hope in our community. Thanks to the dedication of our supporters and the ingenuity of our researchers, we now have the first-ever approved treatment that targets the underlying genetics of SMA. On December 23, 2016, the FDA announced that it had approved Spinraza, a treatment developed by Biogen and Ionis, making it the first-ever approved therapy for SMA. Cure SMA provided the very first research funding for this program beginning in 2003.

But our work is not done. We know what we need to do to develop and deliver effective therapies. And we’re on the verge of further breakthroughs that will continue to change the course of SMA for everyone affected—from infants to adults—and eventually lead to a cure. The approval of Spinraza is just the leading edge of a robust drug pipeline, with a breadth and depth that reflects our goal of treatments for all ages and types. Through conversations with independent SMA experts, our scientific advisory groups, and the Science and Medicine Committee in our Board of Directors, Cure SMA created a new strategic research plan to guide us into this next phase of SMA research. This plan identifies the areas of greatest need and where we are best positioned to make a significant difference.

Guided by this plan, Cure SMA funded over $2.5 million in research this past fiscal year, reflecting the broad needs of our community. This funding included over $1 million for 10 new basic research grants to uncover ideas that will generate new approaches in SMA drug development, plus ongoing funding for 13 additional grants covering everything from the development of new clinical trial outcome measures to drug discovery for combination therapies.

This funding also included an externally led Patient Focused Drug Development (PFDD) Meeting with the FDA, at which over 400 of our families gathered in person and via webcast to share with the FDA the impact of SMA, and their hopes and expectations for future treatments. This was only the second externally led PFDD meeting to be granted by the FDA.
**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 for everyone affected, and eventually lead to a cure.

### SMA DRUG PIPELINE

<table>
<thead>
<tr>
<th>ORGANIZATION/DRUG NAME OR APPROACH</th>
<th>IND</th>
<th>FDA APPROVAL TO PATIENTS</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>BASIC RESEARCH SEED IDEAS</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Biogen/Ionis-Spinraza</td>
<td></td>
<td></td>
</tr>
<tr>
<td>AveXis – AXS-101 (systemic)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Roche-Genentech/PTC/SMAF-RG7916</td>
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<td></td>
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<tr>
<td>Roche-Genentech-Olesoxime</td>
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<tr>
<td>Cytokinetics/Astellas-CK-2127107</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Novartis-LMI070</td>
<td></td>
<td></td>
</tr>
<tr>
<td>AveXis – AXS-101 (CNS-delivered)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Scholar Rock – SRK-015 (muscle drug)</td>
<td></td>
<td></td>
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<tr>
<td>Genzyme/Voyager Therapeutics – CHS Gene Therapy</td>
<td></td>
<td></td>
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<tr>
<td>AurimMed Pharma/Nemours - Small Molecule</td>
<td></td>
<td></td>
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<tr>
<td>Denenthen-Gene Therapy</td>
<td></td>
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<tr>
<td>Calibr-Small Molecule</td>
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<tr>
<td>MIU Shift Pharmaceuticals-81 ASD</td>
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</tr>
<tr>
<td>Spotlight Innovation U – STL-182</td>
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</tr>
<tr>
<td>Indiana U/Brightham &amp; Women’s - Small Molecule</td>
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<tr>
<td>Harvard-Small Molecule</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Columbia/NJ-p38aDMAPK Inhibitor</td>
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</tr>
</tbody>
</table>

**IND** = Investigational New Drug  
**NDA** = New Drug Application  

Last updated: January 2018
## STATEMENT OF FINANCIAL POSITION
### YEARS ENDED JUNE 30, 2017 AND 2016

### ASSETS

<table>
<thead>
<tr>
<th></th>
<th>2017</th>
<th>2016</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash and cash Equivalents</td>
<td>$3,176,246</td>
<td>$2,674,491</td>
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<tr>
<td>Contribution Receivables</td>
<td>536,684</td>
<td>166,327</td>
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<tr>
<td>Endowment Fund</td>
<td>1,032,313</td>
<td>854,008</td>
</tr>
<tr>
<td>Other Assets</td>
<td>239,299</td>
<td>221,974</td>
</tr>
<tr>
<td><strong>Total Assets</strong></td>
<td><strong>$4,984,542</strong></td>
<td><strong>$3,916,800</strong></td>
</tr>
</tbody>
</table>

### LIABILITIES AND NET ASSETS

<table>
<thead>
<tr>
<th></th>
<th>2017</th>
<th>2016</th>
</tr>
</thead>
<tbody>
<tr>
<td>Accounts Payable</td>
<td>$1,299,429</td>
<td>$622,416</td>
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<tr>
<td>Other Liabilities</td>
<td>990,000</td>
<td>978,750</td>
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<tr>
<td><strong>Total Liabilities</strong></td>
<td><strong>2,289,429</strong></td>
<td><strong>1,601,166</strong></td>
</tr>
<tr>
<td>Net Assets</td>
<td>2,695,113</td>
<td>2,315,634</td>
</tr>
<tr>
<td><strong>Total Liabilities and Assets</strong></td>
<td><strong>$4,984,542</strong></td>
<td><strong>$3,916,800</strong></td>
</tr>
</tbody>
</table>

Financial information presented has been summarized from financial statements audited by Kaufman, Rossin & Co., P.A dated June 30, 2017.
# STATEMENT OF ACTIVITIES
YEARS ENDED JUNE 30, 2017 AND 2016

## REVENUES

<table>
<thead>
<tr>
<th></th>
<th>2017</th>
<th>2016</th>
</tr>
</thead>
<tbody>
<tr>
<td>Support from the public:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Contributions and Chapter Events</td>
<td>$5,536,748</td>
<td>$4,787,739</td>
</tr>
<tr>
<td>Annual Campaigns</td>
<td>695,372</td>
<td>749,024</td>
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<tr>
<td>Industry Collaboration</td>
<td>320,298</td>
<td>103,831</td>
</tr>
<tr>
<td>Conference</td>
<td>1,061,543</td>
<td>706,089</td>
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<tr>
<td><strong>Total support from public</strong></td>
<td><strong>7,613,961</strong></td>
<td><strong>6,346,683</strong></td>
</tr>
<tr>
<td>Other Revenue:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Interest Income</td>
<td>87,854</td>
<td>534</td>
</tr>
<tr>
<td>Miscellaneous Income</td>
<td>756,918</td>
<td>183,494</td>
</tr>
<tr>
<td><strong>Total Other Revenue</strong></td>
<td><strong>844,772</strong></td>
<td><strong>184,028</strong></td>
</tr>
<tr>
<td><strong>Total Revenue</strong></td>
<td><strong>$8,458,733</strong></td>
<td><strong>$6,530,711</strong></td>
</tr>
</tbody>
</table>

## EXPENSES

<table>
<thead>
<tr>
<th></th>
<th>2017</th>
<th>2016</th>
</tr>
</thead>
<tbody>
<tr>
<td>Program Services:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Research</td>
<td>$3,099,625</td>
<td>$2,112,946</td>
</tr>
<tr>
<td>Patient Services</td>
<td>949,094</td>
<td>662,833</td>
</tr>
<tr>
<td>Family Support</td>
<td>1,414,116</td>
<td>1,192,837</td>
</tr>
<tr>
<td>Awareness</td>
<td>767,242</td>
<td>678,031</td>
</tr>
<tr>
<td><strong>Total Program Services</strong></td>
<td><strong>6,230,077</strong></td>
<td><strong>4,646,647</strong></td>
</tr>
<tr>
<td>Support Services:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>General and administrative</td>
<td>329,533</td>
<td>277,608</td>
</tr>
<tr>
<td>Fundraising and Event Related</td>
<td>1,519,644</td>
<td>1,352,035</td>
</tr>
<tr>
<td><strong>Total Support Services</strong></td>
<td><strong>1,849,177</strong></td>
<td><strong>1,629,643</strong></td>
</tr>
<tr>
<td><strong>Total Expenses</strong></td>
<td><strong>$8,079,254</strong></td>
<td><strong>$6,276,290</strong></td>
</tr>
<tr>
<td>Change in Net Assets</td>
<td>379,479</td>
<td>254,421</td>
</tr>
<tr>
<td>Net Assets, Beginning of Year</td>
<td>2,315,634</td>
<td>2,061,213</td>
</tr>
<tr>
<td><strong>Net Assets, End of Year</strong></td>
<td><strong>$2,695,113</strong></td>
<td><strong>$2,315,634</strong></td>
</tr>
</tbody>
</table>
FINANCIAL HISTORICAL TREND

Over the past decade, Cure SMA’s focus has been growth in revenue streams from various sources as well as growth in total assets and endowments. These diverse indicators reflect the organization’s overall financial stability. Cure SMA is supported through a balance of families, researchers, clinicians and corporate partners that recognize the importance of our work. These funds have been strategically leveraged to accelerate world-wide research projects and have provided local programs for families with SMA. We are extremely proud of the work being done which wouldn’t be possible without our generous donors and volunteers.

WHERE THE MONEY GOES

Based on fiscal year audited financial statements.

$0.77 of every dollar spent in 2017 funded research, patient services, family support, and awareness.
HISTORICAL TRENDS

[Graph showing historical trends for Core Fundraising Revenue, Research Expenses, Ending Current Assets, and Future Endowment from 2010 to 2017.]
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