Dear Friends,

This has been another milestone year for the spinal muscular atrophy community. The pace of our progress means the landscape of SMA is changing rapidly. Cure SMA continues to drive these new developments, and establish the initiatives necessary to meet the promise of those developments.

The first FDA approval of a therapy, and the continued development of several additional therapies, have forever changed the experience of SMA. As these treatments extend lifespan, we will need more doctors and healthcare providers trained in SMA care, particularly for the teen and adult SMA communities.

With these needs in mind, this year we launched our SMA Care Center Network. The goal of the SMA Care Center Network is to develop an evidence-based standard of care, expand access to approved SMA treatments, and help accelerate and enhance insurance coverage and reimbursement.

In addition, we are increasingly focused on locally offered programs and services. We’re committed to making sure that families have access to the support they need, right in the communities where they live, attend school or work, and receive SMA treatment and care. This year, we launched our Summit of Strength program, a series of 16 local one-day symposiums, uniting our community around support, advocacy, and care.

This year was highlighted by the approval of the federal recommendation that SMA newborn screening be implemented nationwide. At least eight states have adopted this recommendation, and at least a dozen more are currently in the process.

Research continues to be an area of focus. This year, we have more ongoing clinical trials for SMA therapies than ever before. The research community’s interest in and drive to treat SMA remains high. We’ll continue working with our basic and industry research partners, until we have a combination of treatments that will allow each individual to receive therapies suited to their age, stage, and type of disease.

Our family support programs—including our Annual SMA Conference—remain the vital and thriving bedrock of our community. We welcomed nearly 1,800 families, researchers and clinicians to our 2018 Annual SMA Conference in Dallas, TX, an event that included our first-ever clinical care conference.

Your support and dedication has gotten us this far, and your continued support will be necessary as we continue to move closer to our ultimate goal: Cure SMA.

Gratefully,

Kenneth Hobby
President

Richard Rubenstein
Chairman of the Board
WHAT WE’VE ACHieved TOGETHER THIS YEAR

450 NEWLY DIAGNOSED FAMILIES REACHED

$5 MILLION INVESTED IN RESEARCH THIS YEAR

1800 FAMILIES AND RESEARCHER AT THE 2018 ANNUAL SMA CONFERENCE

1640 FAMILY SUPPORT ITEMS SENT

14 LOCAL TREATMENT ACCESS GRANTS

19 DRUG PROGRAMS CURRENTLY IN DEVELOPMENT

1 APPROVED THERAPY

$6 MILLION INCREASE IN FEDERAL NEWBORN SCREENING

16 SUMMIT OF STRENGTH LOCAL SYMPOSIUMS

2018 CLINICAL CARE

CARE CENTER NETWORK

This year, we launched our SMA Care Center Network. The goal of the SMA Care Center Network is to develop an evidence-based standard of care that will improve the lives of all those affected by SMA. The network will also expand access to approved SMA treatments, increase the number of sites for SMA clinical trials, and help accelerate and enhance insurance coverage and reimbursement.

Through the SMA Care Center Network, we will collaboratively collect patient care data. This data will help answer questions about the impact of SMA, and develop strategies for optimal care for those affected by SMA.

Over the course of the next three years, Cure SMA will invest $9.1 million in the development of the SMA Care Center Network. As the SMA Care Center Network continues to grow, an unprecedented level of support will be required to maintain and expand the network, until individuals affected by SMA across the US all have access to the best possible care.
The 2018 Annual SMA Conference, held at the Hilton Anatole Hotel in Dallas, Texas, was an impactful weekend of community, research and fun.

Nearly 1,800 families, researchers and clinicians gathered for our largest Midwestern conference to date! It was an honor to welcome SMA families, clinicians and researchers attend the conference from 30 countries across the globe. The Conference began on Thursday with the Newly Diagnosed Program, followed by the Meet & Greet and Researcher Relay Race.

The Meet & Greet is a much-loved tradition at the Annual SMA Conference, during which new families are welcomed into the community, and returning families, clinicians, and researchers join together in a fun, relaxed setting before the start of conference workshops.

Family workshops kicked off on Friday, June 15, along with the Family Friendly Researcher Poster Session and the Family & Researcher Dance Party.

On Saturday, families enjoyed more workshops, including “Top Tips on Traveling Around the World” presented by Cure SMA Committee Member, Srin Madipalli, in addition to the Researcher Q&A, PJ Party & Movie Night, and Roll-a-Palooza sponsored by Adyn’s Dream.

On Sunday, the conference concluded with the special session, It’s a Wonderful Life. A panel of SMA adults took the stage to answer questions from the audience about their experiences, and shared encouraging messages of perseverance, joy, hardships and growth.

The Annual SMA Conference is the centerpiece of Cure SMA’s national support programs, which also include our newly diagnosed information packets, care packages, equipment pool, and more. Over the course of this year, more than 1,640 family support items were distributed, and 450 families reached out to Cure SMA for the first time.
As the largest organization dedicated to the spinal muscularatrophy community, Cure SMA is uniquely positioned. We’re able to leverage our collective strength for a national impact. And, through our 34 chapters, we’re also able to offer personalized, local support to each individual and family affected by SMA.

With an FDA approved treatment for SMA, it is more critical than ever that those affected by SMA have access to the support and resources they need, right in the communities where they live and attend school or work. The experience of SMA is changing, and this requires us to expand capacity and expertise in both treatment and care.

We believe that all those affected by SMA should be able to access approved treatments, enroll in clinical trials, and receive top-notch care, within their own community. As treatments extend lifespan, and more individuals with SMA live longer, this need will continue to increase—and we want to be sure we are positioned to address that need.

Earlier this year, Cure SMA funded $650,000 in grants to increase capacity at SMA treatment sites across the US. Fourteen sites received a grant to help reduce practical barriers that create delays for our community in accessing treatments and care. These barriers include pre-authorization and insurance approvals, scheduling, and clinic support. In addition, the grants will help to increase the number of affected individuals that sites can follow, treat and evaluate.

Finally, this year Cure SMA launched the Summit of Strength Program. The Summit of Strength is a one-day conference that brings the local SMA community together to share experiences, advocate for one another, and learn about the latest advances in research and care. The Summit of Strength was held in 16 locations across 15 states.
The first FDA approval of a therapy for SMA has created an opportunity for our community to move forward on another of our long-term priorities: newborn screening. SMA is the leading genetic cause of death for infants, but we have an historic opportunity to change that through newborn screening and early treatment.

Currently, the average infant with SMA type I is not diagnosed until five months of age. This means that these babies are not receiving treatment during the critical early weeks and months of life, when research suggests it may be most effective.

In February, the federal Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) recommended that newborn screening for SMA be implemented nationwide. This recommendation was subsequently approved by Health and Human Services Secretary Alex Azar. Cure SMA was there at every step in the process, submitting the nomination and supporting it from acceptance to vote.

This is an important first victory as we work toward our goal of having every baby born in the United States screened for SMA, but much remains to be done. Now, each state must decide individually whether to implement SMA newborn screening.

At least eight states have already adopted this recommendation. Four have begun pilot screening, and at least a dozen more are currently considering action.

**NEWBORN SCREENING STATE STATUS**

- **Adopted and implemented**
- **Adopted and not implemented**
- **Pilots**

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**NEWBORN SCREENING**

**2018**

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Feb. 2017 – Cure SMA submitted nomination to the ACHDNC

May 2017 – Nomination moved to evidence review

Feb. 2018 – ACHDNC recommends SMA be added to the RUSP

Mar. 2018 – Recommendation submitted to Secretary Azar

Jul. 2018 – SMA officially added to the RUSP
The complexities of newborn screening require a sophisticated, multifaceted approach. With this in mind, Cure SMA has been working on newborn screening through several different avenues, bringing our full resources to ensure that babies born with SMA have the opportunity to receive the most effective treatment.

ADVOCACY

We are working with family advocates, clinicians, legislators and administrators, to encourage speedy implementation of SMA newborn screening. This process requires resources in all 50 states.

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. This year, thanks to our advocacy, funding for the Health Resources and Services Administration (HRSA) Heritable Disorders Program increased by more than $2 million, and funding for the CDC’s newborn screening quality assurance program increased by almost $4 million.

FAMILY SUPPORT

Families that receive an SMA diagnosis through newborn screening are another example of how rapidly the experience of SMA is changing. While they will face many of the same issues as families that receive a symptomatic diagnosis, they will also have their own unique experiences and decisions to make.

We’re already working to address this, developing educational materials that will help families who receive a diagnosis through newborn screening understand their options, and make the decisions that are best for their child and their family.

STATE IMPLEMENTATION

Once states begin screening for SMA, they will need to quickly connect families with treatment options near them, connect them to centers experienced in SMA care, educate families on next steps, and follow up to make sure babies are reaching the best outcomes. That’s why we’re building partnerships with states as they prepare to screen, educating them about our family support programs and our SMA Care Center Network.

By working with states, we’ll help make sure families get timely treatment and care, connect with our community, and receive the resources they need at every step of the way. In addition, Cure SMA made grants to four states to help cover start-up costs for SMA screening, thus avoiding a common barrier to speedy implementation of screening.

RESEARCH

Clinicians who care for babies identified through newborn screening must venture into uncharted waters. With that in mind, Cure SMA convened a working group of SMA clinicians to set a standard for how babies with SMA will be guided toward appropriate treatment. This work was published in the Journal of Neuromuscular Diseases in April.

Cure SMA is also building a newborn screening data portal. This portal will be used to collect information on outcomes for these babies, building an evidence-based standard of care for those identified through newborn screening.
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.

As the SMA research landscape has developed and the drug pipeline has grown to include the first-ever FDA approved treatment, new pathways for research have opened up. The FDA-approved therapy, as well as several other therapies currently in development, work by increasing the amount of survival motor neuron (SMN) protein, the protein that is lacking in SMA.

But there are other systems, pathways and processes that are affected in SMA. And approaches that work on these areas, or on the muscles and nerves, could be used in combination with approaches that increase SMN protein. This important work will allow us to develop maximally effective treatments for all types, ages and stages of SMA.

This year, we announced 10 basic research grants, totaling $1.3 million – our largest contribution to date. We understand that our commitment to the treatment and cure of SMA is not about just seeking solutions, but about helping to create them.

In addition to these grants, this year we also 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 and will benefit the broader SMA community.

The collaboration is comprised of three primary topics groups, who are working on expanding access to clinical trials; educating parents and physicians about SMA, to reduce time to diagnosis; and engaging with regulators to help them understand the unmet needs in SMA, and the economic burden of the disease.

Finally, in 2018 we also hosted the 22nd Annual SMA Researcher Meeting, our largest ever with 500 attendees. The goal of the meeting is to create open communication of early, unpublished scientific data, accelerating the pace of research. The meeting also furthers research by building productive collaborations—including cross-disciplinary dialogue, partnerships, integration of new researchers and drug companies, and educational opportunities for junior researchers.
### STATEMENT OF FINANCIAL POSITION
#### YEAR ENDED JUNE 30, 2018

#### ASSETS

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<thead>
<tr>
<th>Description</th>
<th>2018</th>
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</thead>
<tbody>
<tr>
<td>Cash, cash equivalents and investments</td>
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<tr>
<td>Receivables and other assets</td>
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<td>Fixed assets, net</td>
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<td>Other assets</td>
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<td><strong>Total Assets</strong></td>
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#### LIABILITIES

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<td>Accounts payable and accrued expenses</td>
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<td>Research and grants payable</td>
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<td><strong>Total Liabilities</strong></td>
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#### NET ASSETS

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<th>Description</th>
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<tr>
<td>Unrestricted</td>
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<td>Temporarily restricted</td>
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<td>Permanently restricted</td>
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#### Support and Revenue

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<tr>
<td>Contributions:</td>
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<tr>
<td>Contributions and Grants</td>
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<td>Chapter events, net</td>
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<td>Conference</td>
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<td>Other Revenue</td>
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<td><strong>Total Revenue</strong></td>
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#### EXPENSES

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<td>Research</td>
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<td>Patient Services</td>
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<td>Family Support</td>
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<td>Awareness</td>
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<td>Fundraising</td>
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<td><strong>Total Expenses</strong></td>
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**Total Net Income**

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<tr>
<th>Description</th>
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</thead>
<tbody>
<tr>
<td><strong>Total Net Income</strong></td>
<td><strong>$1,806,742</strong></td>
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Financial information presented has been summarized from financial statements audited by Warady & Davis LLP dated June 30, 2018.
83% of every dollar spent in 2018 funded research, patient services, family support and awareness.
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