

# Cure SMA Awareness & Advocacy Kit

## About Cure SMA

Cure SMA is dedicated to the treatment and cure of spinal muscular atrophy (SMA)—a disease that takes away a person’s ability to walk, eat, or breathe. It is the number one genetic cause of death for infants.

Since 1984, we’ve directed and invested in comprehensive research that has shaped the scientific community’s understanding of SMA. We are currently on the verge of breakthroughs in treatment that will strengthen our children’s bodies, extend life, and lead to a cure.

We have deep expertise in every aspect of SMA—from the day-to-day realities to the nuances of care options—and until we have a cure, we’ll do everything we can to support children and families affected by the disease.

Learn more about how you can help us reach a treatment and cure at [www.cureSMA.org](http://www.cureSMA.org).

## Background

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 each other. 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 treatment and cure for SMA.



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Have a general inquiry or not sure who to contact? Send an email to [fundraising@curesma.org](mailto:fundraising@curesma.org) and we'll make sure it gets to the right person.



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breakthrough.**

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## Facts and Stats

Spinal muscular atrophy (SMA) is a disease that robs people of physical strength by affecting the motor nerve cells in the spinal cord, taking away the ability to walk, eat, or breathe. It is the **number one genetic cause of death** for infants.

SMA is caused by a **mutation in the survival motor neuron gene 1 (*SMN2*)**. In a healthy person, this gene produces a protein—called survival motor neuron protein or SMN protein—that is critical to the function of the nerves that control our muscles. Without it, those nerve cells cannot properly function and eventually die, leading to debilitating and often fatal muscle weakness.

SMA affects approximately **1 in 10,000 babies**, and about **1 in 50 Americans** (over 6 million in total) is a genetic carrier. SMA can affect any race or gender.

There's great reason for hope. We know what causes SMA and what we need to do to develop effective therapies, and **we're on the verge of major breakthroughs** that will strengthen our children's bodies, extend life, and eventually lead to a cure.

In the last 15 years, we've gone from one potential drug in the beginning stages of preclinical discovery, to **18 ongoing drug programs today**.

Cure SMA has led and invested in the research that has made today's breakthroughs possible, with **\$60 million in funding for research**. Cure SMA has funded half of the ongoing drug programs for SMA.

**Six of these drug programs are now in clinical trials**, including one in Phase 3 clinical trials. This means we're getting closer and closer to an FDA-approved therapy for people affected by SMA.

Cure SMA also works to improve quality of life for children and families affected by the disease today, through newly diagnosed care packages, an equipment pool, care series booklets, and more. We reach **over 3,600 families every year** through our direct support efforts.

With 34 chapters around the country, we have **110,000 families and supporters** working with us.

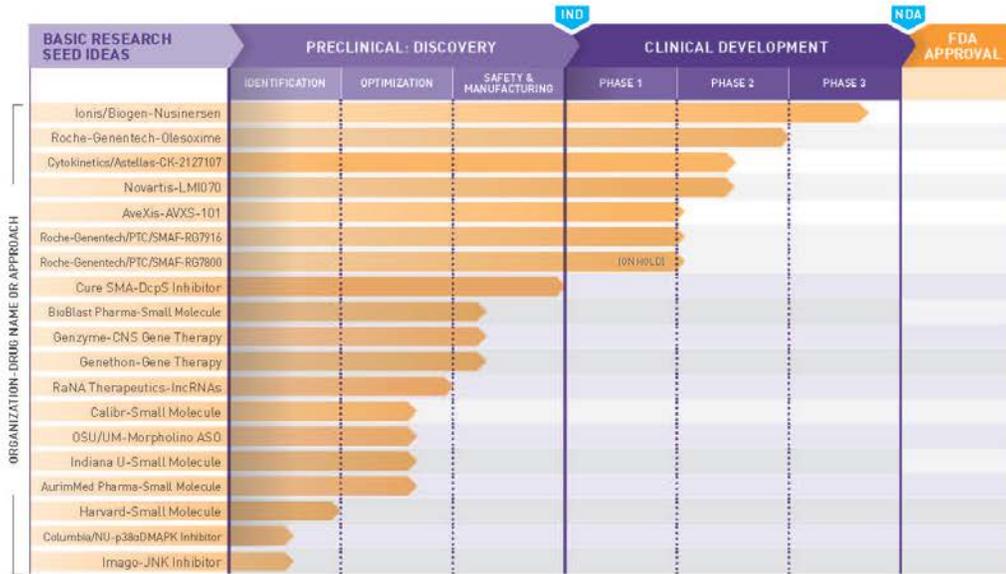
Our Annual SMA Conference, which includes the SMA Researcher Meeting, is the **largest event of its kind in the world**, bringing together families, researchers, and clinicians to network, learn and collaborate.



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## 2016 SMA DRUG PIPELINE

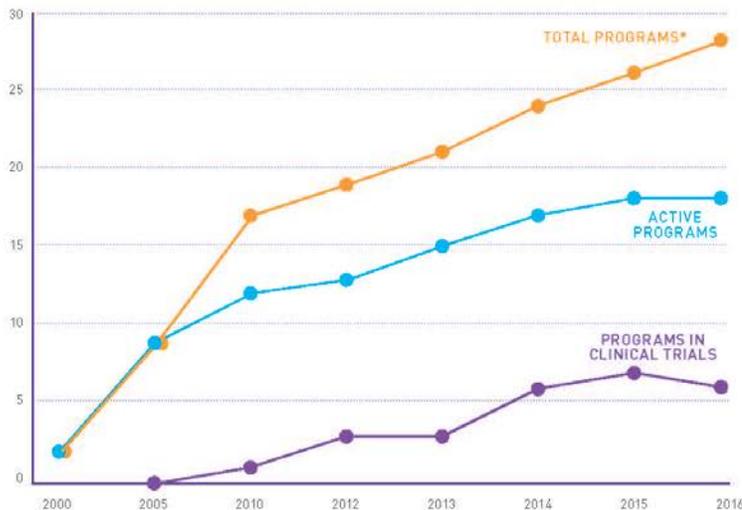
Our approach to research is focused on continually expanding the pipeline of potential drugs and therapies for SMA, cultivating new approaches and advancing promising ideas. The drug candidates shown below represent all four of our therapeutic approaches. Every year, the pipeline gets broader and deeper. We've seen enormous growth over the past decade, and we expect that growth to continue in the coming years as more projects approach FDA approval.



IND = Investigational New Drug    NDA = New Drug Application

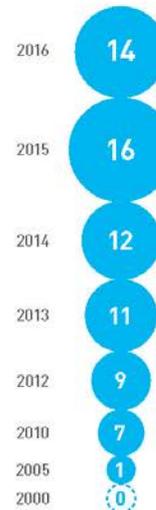
Last updated: June 2016.

### HOW THE PIPELINE HAS GROWN



\*Includes failures

### NUMBER OF COMPANIES INVESTING IN SMA DRUG PROGRAMS



Make today a breakthrough.

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## We can Cure SMA. How can you help?

**Join or start chapter.** We have 34 chapters all around the United States, and we can put you in touch with your chapter. Or, if there isn't a chapter near you, we'd love to talk to you about starting one.

**Host an event.** We have over 300 events each year, most organized by chapters or by families, in memory or in honor of a loved one. Our team will help you plan a successful and meaningful event.

**Attend an event.** You don't have to organize an event to help raise funds and awareness for SMA. Select "Event Calendar" under the Get Involved menu on our website, and find an event near you.

**Set up an individual fundraising campaign.** Use individual fundraising pages to ask for donations in lieu of gifts for a special event or achievement: birthdays, anniversaries, graduations, and more.

**Donate.** You can contribute to our research and programs through a direct, tax deductible donation.

**Reach out to local or national media.** Everyone affected by SMA has a unique and compelling story, and sharing your story is one of the best ways to motivate people to action. We can provide you with a media kit or help you craft a story pitch.

**Reach out to celebrities and other influencers.** Celebrities often respond to a simple, personal ask—and their visibility can educate the wider public about SMA. We can provide you with advice, support, bracelets and other promotional materials, and more.

**Participate in advocacy efforts.** Cure SMA is at the forefront of important issues such as FDA approvals, newborn screening, medical reimbursement and more. The specific actions needed change often. Please see the last page of this packet for more information.

**Advance medical professional awareness.** As treatments are developed for SMA, we need more medical professionals who are able to provide top-notch care for SMA. Contact us to request a medical professional awareness kit or to find other ways to get involved.

**Join us for SMA Awareness Month.** August is SMA Awareness Month. In addition to any of the above activities, you can request a proclamation from your state or local government, organize a candlelighting, or plan an SMA Awareness Day with your local baseball team.

**Use social media.** Like us on Facebook ([www.facebook.com/cureSMA](https://www.facebook.com/cureSMA)), and follow us on Twitter ([@cureSMA](https://twitter.com/cureSMA)). We'll keep you updated on all these and more ways to get involved. You can also like and share our posts, or create your own posts, in order to educate your family and friends about SMA.



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## Advocacy can make a difference in the lives of all those affected by SMA

The policies that Congress debates and the laws it passes affect many aspects of life for those affected by spinal muscular atrophy (SMA). Though there is currently no approved treatment for SMA, there's great reason for hope. We know what causes SMA and what we need to do to develop effective therapies, and we're on the verge of major breakthroughs that will strengthen our children's bodies, extend life, and eventually lead to a cure.

### Together we can ask our lawmakers to:

- ▶ **Expedite the drug-approval process for SMA therapies**
- ▶ **Implement Newborn Screening for SMA**
- ▶ **Secure federal funding for SMA research**
- ▶ **Make the patient voice heard in the drug-approval process for SMA therapies**

### Ways to get involved: Take Action! Your story makes a difference!

#### Visit [www.cureSMA.org](http://www.cureSMA.org) to Sign Up for our Email List!

Advocacy opportunities will come directly to your inbox.

#### **Send an Email or Make a Call**

Let your elected officials know you want to work with them on SMA.

#### **Send an Invitation**

Invite your elected official to participate in a Chapter event in your town.

#### **Schedule a Meeting**

Every elected official has a local office. Reach out to your elected officials on your home turf!

#### **Submit a Letter to the Editor**

Submit a letter that should be in the news! A letter to the editor is an effective way to raise awareness in your local community about SMA.

#### **Use Social Media to Spread the Word @cureSMA**

Use social media to advance awareness about SMA. Send a Twitter message or post a "call to action" about SMA on your Facebook page. Social media is a great way to thank elected officials for their support.

**Questions? Want More Information? Contact [advocacy@curesma.org](mailto:advocacy@curesma.org)**