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breakthrough.

# NEWBORN SCREENING FOR SMA:

TOOLS FOR ADVOCATES WORKING TO IMPROVE CHILDREN'S LIVES



# INTRODUCTION

## ABOUT THIS TOOLKIT

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In the last few years, there have been significant advancements in treating spinal muscular atrophy (SMA)—a debilitating and sometimes fatal genetic condition that affects muscle function—including the first approval of a drug to treat children and adults with the condition. Recent data also indicates early detection and treatment of SMA are critical, leading to SMA’s submission to the Federal Recommended Uniform Screening Panel (RUSP) for newborns.

The RUSP is intended as a guide for state-level newborn screening programs; states are not required to adopt the national recommendations. Furthermore, different states have different processes for how they handle newborn screening. However, with the help of people like you advocating to legislators on behalf of those living with SMA, **we can ensure SMA is included on each state’s newborn screening panel** to help newborns obtain treatment as early as possible to secure the best health outcomes possible.

**This toolkit is designed to provide information on SMA and the importance of early intervention as well as offer resources to help you advocate for the inclusion of SMA on your state’s required newborn screening panel. Specifically, this toolkit offers:**

- An overview of SMA
- How advocacy can help
- Understanding levels of government and legislation
- Making a difference in your community
- How to be an effective advocate
- Leave-behind resources, including:
  - An SMA fact sheet
  - A one-pager on the importance of newborn screening
  - A series of key talking points for policymakers

## SMA TYPES I - IV

**SMA Type I** (Werdnig-Hoffmann Disease) is the most severe and most common type. It is usually diagnosed during an infant’s first six months, and is often fatal early on in life. Babies with SMA type I face many physical challenges, including muscle weakness, as well as trouble breathing, coughing and swallowing.

**SMA Type II** is usually diagnosed between six months and two years of age. The first sign is often a delay or failure in meeting motor milestones. Those with type II can typically sit up without help but are unable to walk.

# ABOUT SMA

SMA is a genetic disorder that affects the motor nerve cells in the spinal cord and the leading genetic cause of death for infants. SMA is caused by a mutation in the survival motor neuron gene 1 (*SMN1*). In a healthy person, this gene produces a 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 takes away a person's ability to walk, eat, or even breathe.

There are four primary types of SMA—I, II, III, and IV—based on age of onset and highest physical milestone achieved. Type I is the most severe and most common, typically diagnosed during an infant's first six months. It is often fatal early on in life.

The earlier the age of onset, the greater the impact on motor function. Children who display symptoms in infancy typically have the lowest level of functioning (type I). SMA onset in children and teens (types II and III), or adults (type IV) generally correlates with increasingly higher levels of motor function.

Individuals with SMA have difficulty performing the basic functions of life; however, SMA does not affect a person's ability to think, learn, and build relationships with others.

## WHO IT IMPACTS

SMA affects approximately 1 in 11,000 babies, and can affect any race or gender.

About 1 in every 50 Americans is a genetic carrier for SMA. Because it is an autosomal recessive condition, generally both parents must be carriers for a child to inherit the condition.

## DIAGNOSTIC CHALLENGES

Timing of diagnosis is critical in SMA; however, research has also shown many families face significant diagnostic delays.

In addition to the physical and mental stress this can put on patients and caregivers, diagnostic delays can also involve expensive and, ultimately, unnecessary testing until patients arrive at a correct SMA diagnosis. More importantly, a delayed diagnosis may result in a missed opportunity for optimal early intervention for SMA.

## RESEARCH & TREATMENT

In December 2016, the FDA announced it had approved Spinraza – the first-ever approved therapy for SMA. Additional therapies are also in development for treating SMA. This includes treatments, like Spinraza, that address the underlying genetics of SMA, as well as therapies that work on the muscles, nerves, and other systems affected by SMA. Ultimately, these treatments may be used in combination with each other to treat all types, ages, and stages of SMA.

Ongoing research has shown beginning therapy as early as possible in the lifecycle of SMA is critical. Studies indicate therapy is most effective when it can begin in the first few months of life—before significant motor neuron loss occurs. This research reinforces the need for newborn screening, which would eliminate long and costly diagnostic delays, and allow pre-symptomatic therapy.

**SMA Type III** (Kugelberg-Welander Disease / Juvenile SMA) is usually diagnosed between 18 months and three years of age, but can be diagnosed as late as the teenage years. Those with SMA type III are initially able to walk, but have increasingly limited mobility as they grow.

**SMA Type IV** is very rare. It usually surfaces in adulthood and leads to mild motor impairment. While symptoms can begin as early as age 18, they usually begin after age 35.

# WHAT ADVOCACY CAN DO TO HELP

## What is advocacy and why should individuals committed to SMA be involved in advocacy?

Officially, an advocate is a person who supports, defends, and argues for a cause. To advocate is to act in support of a particular issue or cause. Advocates may be individuals, nonprofit groups, independent agencies, or other organizations. Being an SMA advocate allows you to be a voice for the SMA community, calling on the public and policymakers to support federal and state newborn screening laws.

Although it may be hard to believe, you, as the local constituent, possess persuasive ability to influence a member of Congress than a lobbyist. Why? Because **you vote. Your opinion and concerns matter.**

Engaging with your elected officials helps bring attention to an issue they likely haven't considered before.

## Understanding Advocacy

To be an effective advocate, it's important to understand the different levels of government along with the state and federal legislative processes. While you can engage in advocacy at any time, there are points in the legislative process when your argument will be the most impactful.

Oftentimes, a lawmaker gets an idea for a policy proposal from constituents or interest groups in his/her district. Legislative proposals might address an unmet need, make a change in an existing law to strengthen the law or create a new program. **As a first step, legislators—at the state and federal levels—will usually bring up a policy proposal in a committee. The process will then begin in the committee.**

Once a bill becomes law, Congress will vote for its reauthorization or make appropriations, as necessary. For example, the Newborn Screening Saves Lives Reauthorization Act of 2014 amended the Public Health Service Act to revise and extend through Fiscal Year 2019 a grant program for screening, counseling, and other services related to heritable disorders that can be detected in newborns. Your Senator or Representative may be able to impact in the appropriations process each year. **Updates related to that bill can be found at:** [www.congress.gov/bill/113th-congress/house-bill/1281](http://www.congress.gov/bill/113th-congress/house-bill/1281)

“Never doubt that a small group of thoughtful, committed citizens can change the world. Indeed, it is the only thing that ever has.”

—Margaret Mead, American cultural anthropologist



## Quick Action:

### How to advocate for newborn screening appropriations

- Visit the webpage of the House and Senate Committees Appropriations website to determine if anyone from your state is assigned to the committees
- Conduct phone calls to the officers of the Chairman and Ranking Member
- Write a concise letter and send it to each committee member
- Leverage social media channels (Twitter, Facebook) to post comments on members' profiles, urging them to support your cause.

# THE FEDERAL LEGISLATIVE PROCESS



<b>HOUSE</b>	<p>A bill is introduced in the House or the Senate and referred to the appropriate committee(s) that deals with that issue. For example, a bill regarding children's health may be referred to the Senate HELP or House Energy &amp; Commerce Committee.</p>	<b>SENATE</b>
<b>House Subcommittee Action</b>	<p>Many times, the bill will be referred to a subcommittee. For example, a children's health bill pertaining to newborn screenings may be sent to the House Energy &amp; Commerce Subcommittee on Health. If the subcommittee alters or approves the bill, it will move to the full committee.</p>	<b>Senate Subcommittee Action</b>
<b>House Committee Action</b>	<p>Typically, the full committee then holds hearings and makes revisions to the bill. If the full committee approves the bill, it is "reported" to the full House. If the committee takes no action on the bill, the bill "dies."</p>	<b>Senate Committee Action</b>
<b>House Rules Action Committee</b>	<p>House bills must be considered by the Rules Committee, which is responsible for scheduling action. The committee debates on the pending bill as well as allows amendments to the bill.</p>	<b>(Senate Leaders typically schedule Senate floor action of the pending bill)</b>
<b>House Floor Action</b>	<p>The bill is debated on the House floor and can be amended from its original form. The full House votes on the bill, and—if it passes—it proceeds to the Senate chamber to be considered by the Senate. If the bill has already been passed in the Senate, both versions of the bill go to a conference committee to be "reconciled."</p>	<b>Senate Floor Action</b>

## Conference Committee Action

The Senate and House versions of the legislation are debated and a compromise is arranged. The compromise version then goes back to the House and Senate, where each chamber must approve the compromise. Once the bill has been approved by both chambers in identical form, the bill goes to the president for signing.



## State Advocacy

The legislative process varies from state to state. For example, in Washington state, the legislature adjourns in late April, whereas the Wisconsin legislature will generally remain in session into December.

Many state governments mirror the structure of the Congress. States like North Carolina and Pennsylvania have a bicameral legislature, consisting of the Senate and the House of Representatives. Like Congress, the Senate chamber is viewed to be more impactful, while more members sit in the House of Representatives. However, the Nebraska state legislature is a unicameral body, meaning—instead of a Senate and House of Representatives—there is just one legislative house.

### There are two key variances between Congress and state legislatures:

#### 1. Generally, state legislative sessions are not as long.

While Congress convenes, throughout the year, state legislative sessions vary in length and timing.

Forty-six states hold annual legislative sessions. Today, state legislatures in just four states meet on a biannual basis: Montana, Nevada, North Dakota and Texas. For example, the Texas Legislature holds 140-day legislative sessions biannually unless the governor calls a special session to tackle a specific issue.

In contrast, legislative sessions in California, Massachusetts, Michigan, New Jersey and Wisconsin span eight months or longer each year.

Legislative sessions in 18 states are shorter than three months long: Arkansas, Florida, Georgia, Idaho, Kansas, Kentucky, Louisiana, Maryland, Mississippi, Montana, North Dakota, Oklahoma, Tennessee, Utah, Virginia, Washington, West Virginia and Wyoming.

Still, legislative sessions often go into overtime through special sessions. In 2015, the Washington State Legislature spent 176 days in session this year, well beyond the prescribed 90 days—the most in that state's history.

#### 2. Being a state legislator isn't always a full time job.

Job descriptions for state senators and representatives vary at legislatures across the country. Being a legislator is a full-time job in a handful of states, but elected officials in most states balance both public and private careers.

The National Conference of State Legislatures (NCSL) has grouped the 50 state legislatures into three major categories: Green, Gray and Gold to explain how it all works.

#### Green Legislatures (Full-time)

Green legislatures require the most time of legislators, usually 80 percent or more of a full-time job.

##### Key characteristics include:

- Large-sized staffs
- Paid enough to make a living without requiring outside income
- More similar to Congress than other state legislatures
- Most of the nation's largest population states fall in this category

Note: Because there are marked differences within the category, NCSL has subdivided the Green states. Those in Green generally spend more time on the job because their sessions are longer and their districts larger than those in Green Lite. As a result, they tend to have more staff and are compensated at a higher rate.

#### Gray Legislatures (Hybrid)

Legislatures in the Gray category are hybrids. Legislatures in these states typically say that they spend more than two-thirds of a full time job being legislators. **Key characteristics include:**

- Intermediate-sized staff
- Income from legislative work is greater than in the Gold states (below), but it's usually not enough to allow them to make a living without having other sources of income
- States in the middle of the population range tend to have these legislatures

**TIP:** Unlike federal advocacy, legislation usually moves quicker at the state level. Legislative sessions can be much shorter and, if you live in a state without political gridlock, it can be easy to pass legislation through. Your direct impact can be significant.

## Gold Legislatures (Part-time)

Gold Legislatures require less time from legislators, usually about half of the time spent in Green Legislatures and/or a full-time job. **Key characteristics include:**

- Small staff
- Often called “traditional” or “citizen” legislatures
- Most often found in the smallest population, more rural states

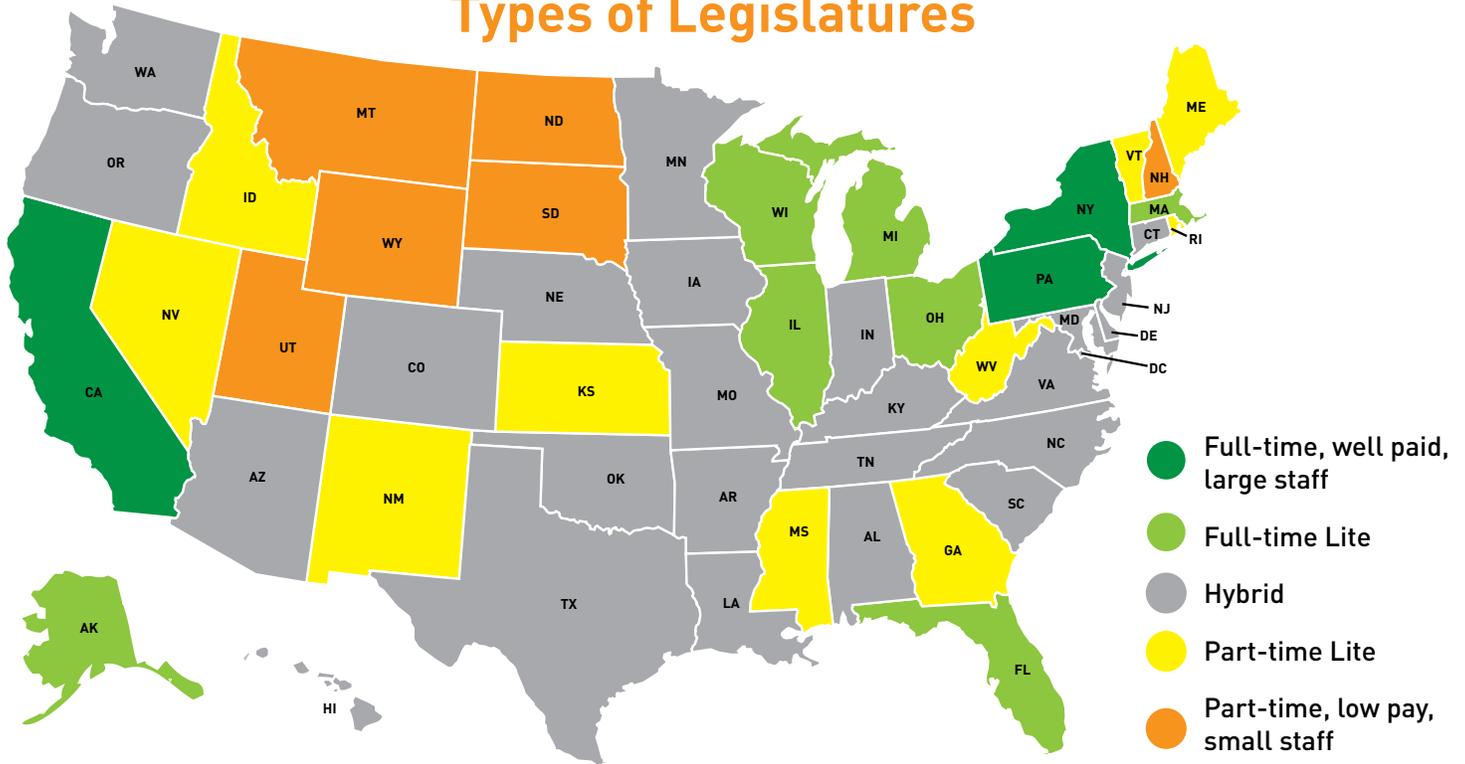
Note: Because there are marked differences within the category, NCSL has subdivided the Gold states. The legislatures in Gold are the most traditional or citizen legislatures. The legislatures in Gold Lite are slightly less traditional.

Regardless of what legislative structure you have in your state or how long the session is, it’s important to remember that you can engage with your lawmakers at any time.

Additionally, each state government has committees that oversee issues related to children’s health. The main webpage for your state legislature will list the committees and provide a brief discussion of the issues they oversee.

As a first step, [visit www.openstates.org/find\\_your\\_legislator/](http://www.openstates.org/find_your_legislator/) to find who represents you on the state level and what committees he or she sits on.

## Types of Legislatures



Green	Green Lite	Gray			Gold Lite	Gold
California New York Pennsylvania	Alaska Florida Illinois Massachusetts Michigan Ohio Wisconsin	Alabama Arizona Arkansas Colorado Connecticut Delaware Hawaii	Indiana Iowa Kentucky Louisiana Maryland Minnesota New Jersey Missouri	Nebraska North Carolina Oklahoma Oregon South Carolina Tennessee Texas Virginia Washington	Georgia Idaho Kansas Maine Mississippi Nevada New Mexico Rhode Island Vermont West Virginia	Montana New Hampshire North Dakota South Dakota Utah Wyoming



## How SMA is added to State Newborn Screening Laws

Typically, newborn screening requirements for SMA will be made into law by one of the following paths:

- SMA condition is automatically added to state screening requirements once its included in the federal RUSP
- Legislative action or bill requiring screening for SMA
- A governor unilaterally makes the decision for SMA screening

As part of the process, many states also have special committees that advise the legislature or governor regarding newborn screening decisions. Many of these committees have opportunities for open comment when individuals from that state can give input on their recommendations. The talking points and information in this toolkit can also be used to help shape public testimony before these advisory committees, if you have that opportunity.

### How to Engage

Engaging with a legislator make seem like a daunting task. However, it's important to remember that they work in the best interest of their constituents – that means ensuring healthier, safer communities. Newborn screening is an apolitical topic and generally has bipartisan support. With the right approach, it will be easy to build support and help condition the legislation environment.

Outlined below are general tips for contacting your elected official, along with specific recommendations for in-person visits, phone outreach, and writing letters. Key talking points for policymakers specific to SMA and newborn screening can be found in this toolkit.

#### General Tips for Contacting Your Elected Official

- Contact your legislative office when an issue is being heard in committee, especially if your legislator serves on the committee discussing the issue.
  - If the legislator is unavailable, ask if there is a staff person who oversees health and/or public safety issues.
- Be brief and specific, and get to the point. Have talking points prepared ahead of time, if possible, to help articulate your points quickly.
  - If asked, be prepared to give your name, address, and the organization you represent (if any).

- If you live in the representative's district, tell them.
- If you are reaching out about an existing newborn screening law or bill, give the bill number and subject. State your position on the bill and a brief reason for that position.
- Tell your legislator what you want them to do.
  - Thank them if they agree with you.
  - If they disagree with you or aren't sure, tell them how a bill will affect your family, friends, community, business, or job. Include one or two specific examples.
  - Be prepared with statistics to support you. Data around the importance of newborn screening for children living with SMA are important to include. ***The Importance of Newborn Screening for SMA*** one-pager in this toolkit can help.
  - Offer to be a resource for issues related to disability advocacy.
- Thank them for their time.

## Tips for Personal Visits

In-person meetings can go a long way in advocacy. A personal visit is generally the most effective means of communicating with a legislator as it provides you with the opportunity to build a relationship.

- Make an appointment with their scheduler or office coordinator. Let them know you would like 15 to 30 minutes to discuss newborn screening. Tell the scheduler what issue you want to discuss so the right staff person is made aware of your visit.
  - Accept an appointment with the appropriate staff member if the legislator is unavailable.
  - If you do not have an appointment, you may not be able to speak with anyone or you may only have a few minutes.
- Make the most of the time you have with your legislator. Practice beforehand what you want to say. Allow time for questions.
  - If you don't know the answer to a question, say so and follow up later.
  - If possible, leave a one-page fact sheet with the most important information about your issue. Include your contact information. ***The Importance of Newborn Screening for SMA*** one-pager in this toolkit might be a good leave-behind.
- Take your camera to take photos with your representative. If they are agreeable to a photo, share it your social media pages and within the SMA community.
- Follow up with a thank-you note or letter, reminding your legislator (or staff) what you talked about and the actions you asked them to take. Include the answers to any questions that you were unable to answer during your visit. Send them your picture.

## Tips for Letters or Emails

- Identify the issue upfront and how it personally impacts you. **(If there is an existing newborn screening law or legislation in your state, include the bill number and what the bill is about at the beginning of your letter or email).**
- Include your address and telephone number.
- If you want to comment on more than one issue, send a separate message for each one.

- Invite the legislator to contact you to talk about your position on the bill or issue.
- Ask your legislator for their position on the issue and request a reply.

## Tips for Phone Calls

- Before you place a call, write down notes about the points you want to make. That way, you won't forget anything.
- Ask for the staff person who covers health. Legislators are often too busy to take phone calls and rely on their staff to keep them informed.
- If you request a return call, you will usually get one from the staff if you are a constituent. Ask if there is a time to speak with the legislator directly.

# MAXIMIZING YOUR IMPACT

**Remember:** Multiple calls, emails, letters, or in-person meetings on an issue have a greater impact than a single communication. Be persistent with your correspondence and encourage others to join the cause. And, no matter how you reach out to your elected officials and their staff, the key is to show them your passion and why you care. Together, we can ensure all newborns across every state are screened for SMA.

# FOR MORE INFORMATION

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Cure SMA has provided this toolkit as a resource to our community. The approval of Spinraza and continued progress of the drug pipeline make this a critical time to push for newborn screening for SMA across the entire US. Because states have their own processes for adding SMA to newborn screening, we need SMA advocates in every state.

We welcome your comments, suggestions and ideas as we move forward on this important initiative. Please reach out to us at any time for information, resources or support in your advocacy activities.



Visit: [www.cureSMA.org/newbornscreening](http://www.cureSMA.org/newbornscreening)



Email: [advocacy@curesma.org](mailto:advocacy@curesma.org) or [info@curesma.org](mailto:info@curesma.org)



Call: 800.886.1762.



Or, reach out to the Cure SMA territory representative who covers your area of the country, and they will assist you or put you in touch with the right resources

# OUR VALUES

We have reached a number of milestones together, by maintaining a strong, unified community. This newborn screening initiative provides an opportunity to further expand that community, welcoming in legislators, governors, committee members and more. As you advocate for newborn screening, we ask you to keep in mind the values that define our community.

## 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.

# 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, adults and families affected by the disease.

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

## Cure SMA



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