

## How were the recommendations developed?

A workgroup of healthcare professionals from the U.S. and Western Europe convened to review how the approved SMA treatments have evolved since the 2018 SMA care recommendations. Additionally, a group of SMA community members shared their experiences and insights on treatments, resources, and information needed for informed shared decision-making. The findings from both groups resulted in updated recommendations, which were developed and published for the SMA community.

### Core Recommendation

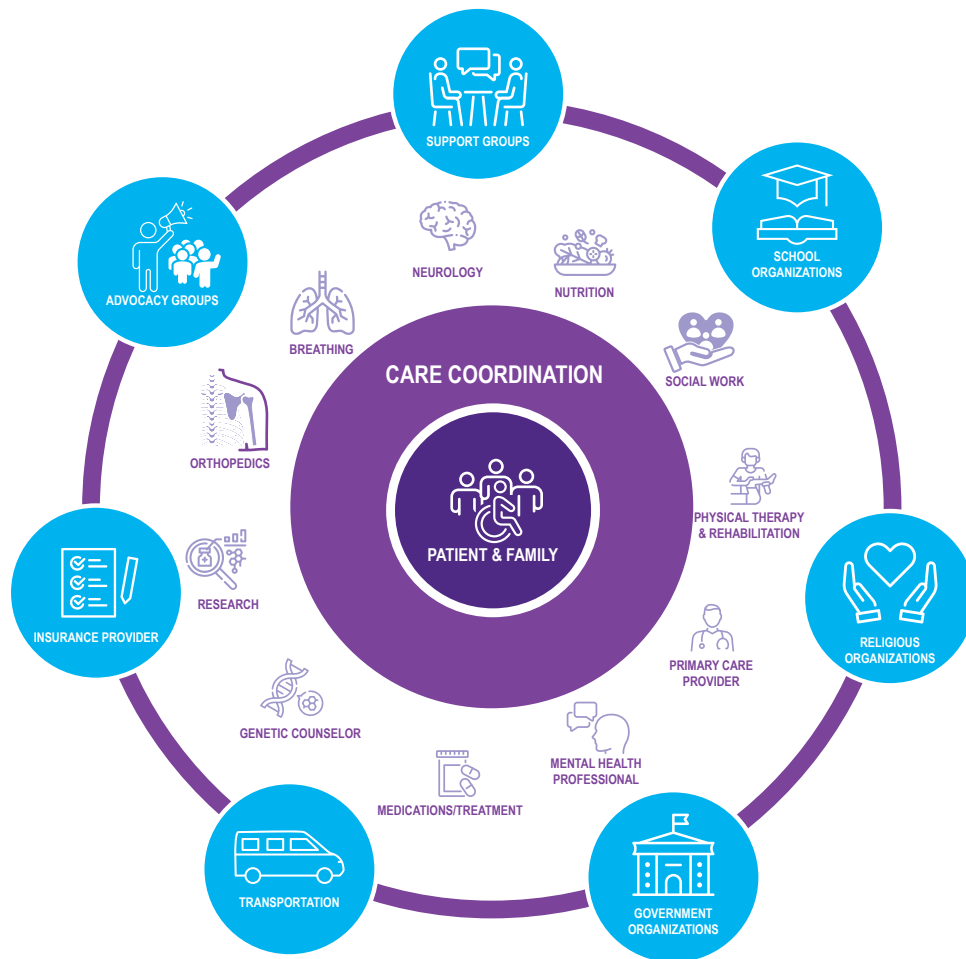
Patient and family perspectives, and treatment safety and side effects, are essential considerations when making decisions about starting, changing or discontinuing an SMA treatment.

Patients and families are encouraged to work with their healthcare provider team to have their questions answered about SMA and discuss available treatments, such as how the medications work, how they are given and frequency, safety concerns, potential side effects and necessary monitoring, and timeline expectations for treatment response. Treatment information is available from multiple additional sources including [www.curesma.org](http://www.curesma.org). Having an open discussion about your capacity to comply with what may be required after receiving a treatment is essential and may help determine which treatment may be best. Questions may include how often medical visits will be and the travel required, and what ongoing tests will be performed to monitor treatment response and side effects. Every treatment requires ongoing clinic follow-ups and evaluations. In general, the treatments provide improvement, however, none of the current treatments are a cure for SMA.

## TREATMENT CONSIDERATIONS BASED ON PATIENT CHARACTERISTICS

	NEWLY DIAGNOSED (NEWBORN SCREENING OR DUE TO SYMPTOMS)	NOT NEWLY DIAGNOSED	ADOLESCENT & ADULT
Factors Influencing Treatment Choice	<p>Starting treatment</p> <ul style="list-style-type: none"> <li>SMN2 copy number</li> <li>Age at diagnosis</li> <li>Age at start of SMA symptoms</li> <li>Screening laboratory test results</li> </ul> <p>Start any SMN-enhancing treatment as soon as possible for best outcomes</p>	<p>Starting, changing or adding treatment:</p> <ul style="list-style-type: none"> <li>Current clinical status and other medical issues, for example, complex spine anatomy, or liver disease</li> <li>Function loss after being stable on treatment (may be due to slowed loss of function due to being on treatment but not preventing further decline)</li> </ul>	<ul style="list-style-type: none"> <li>Treatment intolerance</li> <li>Quality of life</li> <li>Benefit vs. Burden</li> <li>Treatment side effects</li> <li>Loss of function</li> <li>Reproductive concerns</li> <li>Pregnancy</li> <li>Disease progression despite treatment</li> <li>Patient perspective</li> </ul>
Treatment Plan (a shared effort to identify the patient's goals for treatment and what will be provided to treat or manage SMA)	<p>Monitor for 6-12 months UNLESS:</p> <ul style="list-style-type: none"> <li>Side effects or intolerance to medication not acceptable to patient or healthcare provider</li> <li>Not tolerating how medication is given, e.g., intrathecal, oral or intravenous</li> </ul>	<ul style="list-style-type: none"> <li>Significant disease progression as determined by the healthcare provider and patient/caregiver</li> <li>Loss of motor milestones in an infant or young child:</li> </ul>	<ul style="list-style-type: none"> <li>Abnormal side effect monitoring laboratory test results</li> <li>Pregnancy</li> </ul>
Anticipated Outcomes	<ul style="list-style-type: none"> <li>Improved motor function and survival compared to untreated SMA</li> </ul>	<ul style="list-style-type: none"> <li>Variable and may include slowing progression of SMA disease, maintaining current motor function, or restoring or increasing some function to perform activities of daily living, and optimize independence</li> </ul>	
Additional Care Recommendations	<p>Supportive Multidisciplinary Care:</p> <ul style="list-style-type: none"> <li>Motor development and strength</li> <li>Spine and hips</li> </ul>	<ul style="list-style-type: none"> <li>Breathing</li> <li>Nutrition</li> </ul>	<ul style="list-style-type: none"> <li>Care coordination</li> <li>Mental and emotional health</li> </ul>

# SMA Care Coordination: Connecting Patient, Family, Providers & Community



## Who can help navigate care?

Access to care coordination and interdisciplinary/ multidisciplinary care are essential to the success of providing SMN-enhancing treatment to individuals living with SMA. Continuing multidisciplinary medical health care is equally important to receiving treatments for SMA. Care coordination is essential to ensure that families and healthcare teams work together to track progress and address any medical concerns that may arise. Supportive multidisciplinary medical care is necessary to optimize the outcomes of treatment and includes care such as physical therapy, occupational therapy, speech therapy, breathing evaluations and support, bone health and nutrition support. In addition, staying up to date with all vaccinations is important to staying healthy.

## Further Resources

Have you or someone you know been diagnosed with SMA? Please email [infopack@curesma.org](mailto:infopack@curesma.org) and provide your name and mailing address so we can send an unbiased, comprehensive information packet on SMA right to your door. The information packet includes our SMA Care Series Booklets that cover a range of topics and information needed to make decisions on treatment and care. They also cover genetics and testing, current research updates, managing daily life, and more. Upon emailing a member of our team will reach out to provide you with resources and support as you navigate the diagnosis.

For more information, please check out these additional resources.

[Resources Guide Request Form](#)

[Understanding SMA](#)

[Update in Best Practices Summary Reports](#)

[Information about specific SMA treatments](#)

**To review the published manuscript on Treatment recommendations:**

[Spinal Muscular Atrophy Update in Best Practices: Recommendations for Treatment Considerations](#)