Best Practices for the Orthopaedic Care of Children with Spinal Muscular Atrophy: A Consensus Statement from the European Neuromuscular Centre Standard of Care Orthopaedic Working Group

Michael Vitale, MD, MPH1, a; Benjamin Roye, MD, MPH1; Zachary Bloom, MD2; Jennifer A. Kunes, BA1; Hiroko Matsumoto, PhD1, *; David Roye, MD1; David Farrington, MD3; Jack Flynn, MD4; Matthew Halanski, MD5; Carol Hasler, MD6; Lotfi Miladi, MD7; Susana Quijano-Roy, MD8; Christopher Reilly, MD9; Paul Sponsors, MD10; Muharrem Yazici, MD11; Brian Snyder, MD, PhD12, a


Correspondence to: Hiroko Matsumoto, PhD, Division of Pediatric Orthopaedic Surgery, Columbia University Medical Center, 3959 Broadway, 8 North, New York, NY 10032, E-mail: hm2174@cums.columbia.edu

Received: January 3, 2022; Accepted: January 3, 2022; Published: February 1, 2022

DOI: 10.55275/JPOSNA-2022-0006

Abstract:
Spinal muscular atrophy (SMA) is a genetic disease resulting in orthopaedic problems that warrant intervention. Recent genetic therapies appear to improve the natural course of this disease, prompting care providers to rethink treatment strategies. Insufficient high-quality evidence exists to guide optimal intervention in this new landscape. To address this, the European Neuromuscular Centre (ENMC) tasked an orthopaedic subgroup to update previous recommendations. A consensus statement on orthopaedic treatment of patients with SMA undergoing genetic therapy was sought via Delphi method. After review of the orthopaedic literature, three iterative surveys were administered from 2015–2016 to worldwide experts in orthopaedic care of children with SMA. Surveys identified important topics for optimal management, solicited responses regarding management and evaluation of identified topics, and clarified decision-making
strategies and indications for evaluation and management, respectively. In light of new evidence of the long-term effects of gene therapy on SMA patients, an additional review of literature was used to contextualize the consensus recommendations.

Thirteen experts on orthopaedic management of SMA identified treatment of spine deformity, hip instability, and joint contractures as issues of importance. Experts recommended instrumentation in spinal deformity for major curves greater than 50 degrees, while monitoring pain, respiratory function, kyphosis, pelvic obliquity, functional status, trunk imbalance, and rib deformity. Preferential use of magnetically controlled growth constructs and performance of final fusion with skipped levels to accommodate intrathecal access were also endorsed. Additionally, experts advocated for surgical correction of hip instability and/or contractures that cause pain or functional impairment.

**Level of Evidence:** Level IV

**Key Concepts:**
- Almost all patients with spinal muscular atrophy (SMA) develop scoliosis and most undergo scoliosis corrective surgery.
- Recent innovations in treatment for SMA necessitate re-evaluation of best practice guidelines based on the changing prognosis of these patients.
- A panel of worldwide experts in the orthopaedic care of children with SMA convened to develop a consensus on best practices in orthopaedic treatment of SMA patients in the era of gene therapy.

**Introduction**

Spinal muscular atrophy (SMA) is a genetic disorder that results in atrophy and progressive weakness of the truncal and peripheral musculature, occurring in 1 in 11,000 births.\(^1\)\(^,\)\(^2\) SMA is caused by loss-of-function variants of the survival motor neuron 1 (SMN1) gene, leading to under-expression of the survival motor neuron (SMN) protein and subsequent degeneration of motor neurons in the spinal cord and lower brainstem.\(^3\)\(^\text{-}^5\) In recent years, the intrathecally administered anti-sense genetic therapy Spinraza® (nusinersen: Biogen, Cambridge, MA, U.S.) has demonstrated the ability to slow the progression of symptoms.\(^6\)\(^\text{-}^9\) FDA trials demonstrated improvements in quality-of-life scores, progression and maintenance of motor milestones, and substantially fewer incidences of motor function regression versus control groups, which led to its accelerated approval as the first drug to treat SMA.\(^7\)\(^\text{-}^1\) The natural history of SMA is grim. The genetic etiology of SMA involves the survival motor neuron-1 (SMN1) gene; affected individuals have a homozygous SMN1 deletion, mutation, or combination of heterozygous deletion and mutation of the remaining SMN1 copy. The SMN2 gene also produces a smaller amount of SMN protein, and in individuals with SMA, the SMN2 copy-number influences phenotypic severity. Clinically, SMA is classified into four types by age of symptom onset and severity. SMA1 presents at age less than 6 months and results in severe muscle weakness, often resulting in inability to sit unsupported, and weak respiratory clearance leading to early mortality. SMA2 presents at ages between 7-18 months with delayed motor milestones and inability to walk independently. SMA3 presents later in age with loss of the ability to walk independently in childhood and adolescence.
SMA4 often presents later in life and results in mild motor impairment. Musculoskeletal ailments are very common, with recent surveys indicating that the majority of children with SMA experience at least intermittent back, hip, groin, and/or foot pain. The same survey found that a large proportion of these children have undergone scoliosis corrective surgery. The evidence supporting this procedure in patients with SMA is well documented. Hypotonic spinal curves continuously progress through childhood, and because conservative management is often unable to halt curve progression, spinal instrumentation is frequently indicated to preserve truncal balance, respiratory function, and quality of life.

Though treatment of spinal deformity is well-established, operative management of hip instability has not generally been recommended based on the historically high probability of recurrent instability. While hip instability is common in this population, hip subluxation rarely causes pain, and surgically stabilized hips often re-dislocate. Thus, while historic data does not support surgical management of hip pathology, the changing presentation of SMA in light of new genetic treatment strategies calls into question whether more aggressive management is indicated to optimize function for patients with increased motor strength and stamina after genetic treatment.

Guidelines for treating contractures in patients with SMA have recommended incorporating both surgical and nonsurgical management. There is literature to support the use of orthotic treatments and physical therapy and acknowledgement of the efficacy of tenotomy for limb contractures. Like surgical treatments for hip instability, historic strategies must be re-examined in light of functional improvement of children with SMA treated with novel therapies like nusinersen.

Due to the rapidly evolving field of micromolecular treatment, modern health practitioners will likely witness a time in which these patients lead longer lives with better function. This will impact surgical decision-making in several important ways. First, some orthopaedic deformities that are initially asymptomatic, such as hip dysplasia, could ultimately become symptomatic with expanded life span or increased motor function and activity. Children with SMA who are treated with genetic therapies have a higher functional ability than previously seen, potentially worsening the observed and experienced effects of orthopaedic ailments on activities of daily life. Finally, patients receiving treatment may be able to better physiologically tolerate surgery, making them better surgical candidates. To address the changing landscape of treating children with SMA, the European Neuromuscular Centre (ENMC) initiated a multidisciplinary effort to improve care for children in this population. In response, a panel of experts was assembled to develop a consensus statement on orthopaedic management of patients with SMA in the era of genetic therapy. The following are the detailed findings of the orthopaedic working group subset of the ENMC, which reflect the recommendations of the entire working group.

**Materials and Methods**

**The ENMC Orthopaedic Standard of Care Working Group**

Experts were chosen to reflect diverse treatment regimens with a variety of each individual’s experience in treating children with SMA. Seventeen experts were included in the initial study distribution (15 orthopaedic surgeons and two pediatric neurologists) from seven countries in North America, Europe, and Asia (United States, Canada, France, Spain, Switzerland, Turkey and Japan). Participation was voluntary and without pay.

**Meeting at the Scoliosis Research Society (SRS)**

In September 2015, members of the expert panel met at the annual meeting of the Scoliosis Research Society (SRS) to brainstorm topics of interest for further investigation. As a result, 17 topics were proposed, of which 16 were used to guide the literature review (Table 1). One topic (“The role of physical and occupational therapy”) was deferred due to the presence of a separate Physical Therapy/Occupational Therapy working group.
Literature Review

An extensive review of the available literature was performed on the 16 topics proposed at SRS (Table 1). Each reviewer utilized PubMed with keywords consisting of combinations of “Spinal Muscular Atrophy” or “SMA” or “Neuromuscular” plus the topic of interest. Articles were excluded if a) less than 10% of the participants were children with SMA, b) it was not a medical intervention or review article, and c) if the article subject did not pertain to one of the 16 topics of interest. Articles were graded by Oxford Centre of Evidence-based Medicine-Levels of Evidence. 38 After consensus building, a second literature search was conducted to incorporate more recent evidence surrounding the implementation and effects of emerging therapies for SMA, including nusinersen. This second literature review utilized the same keywords and inclusion criteria described above for studies published during January 2016 through March 2021.

Delphi Process

The working group developed their statement by means of the Delphi technique, an iterative process used to identify clustered responses and generate consensus opinion.39,40 This process has been used in generating guidelines for treatment of SMA in the past by members of the larger ENMC group, and due to past successes, was recommended for all working groups.14 All questionnaires were created and distributed via an online questionnaire tool to be completed within 2–4 weeks. Responses were anonymized and results were uploaded to a secure database. All questions were analyzed and categorized to consensus (≥70% agreement) and equipoise (30%–70% agreement) based on the standard from the ENMC.

First Questionnaire (November 2015): Literature review results and topics of interest (Table 1) were distributed to the expert panel who were asked whether or not they “were confident in providing a best practice guideline” for each of the topics based on available literature and their knowledge of orthopaedic management of SMA.

Second Questionnaire (December 2015): Topics in which ≥70% of the experts were confident providing best practice guidelines were included in the second questionnaire. Each topic contained three to five open-ended questions on surgical decision-making, pertinent signs and symptoms, appropriate diagnostic tests, surgical and nonsurgical interventions, and postoperative management per the ENMC format. All questions were proposed for non-sitters (SMA1), sitters (SMA2), and walkers (SMA3/4). Thus, 16 unique questions were posed for each of the three ambulatory categories, resulting in 48 total questions. Open-ended responses were collected and analyzed for the final questionnaire.

Final Questionnaire (January 2016): Based on consensus responses to the previous questionnaire, the working group leaders formulated a final questionnaire. The final questionnaire consisted of 12 questions on spine deformity, three questions on hip instability, and two questions on contracture management, decided by
the iterative process. Questions were multiple choice and experts were instructed to choose the best response(s) for each question.

**Results**

**Literature Review**

In the initial literature review, 81 articles were found, and 35 were included. Many of the recovered studies included outcomes related to multiple topics of interest. In the subsequent literature review, 381 studies were found and 109 met inclusion criteria.

**Effects of Gene Therapy on Disease Progression**

Overall, nusinersen treatment in SMA is associated with significant improvement in motor function. These benefits exist in age groups ranging from infancy to adulthood, although younger age, better baseline function, and early intervention relative to symptom onset are predictive of improvements on motor function scales.\(^{37,41-72}\) Improvements also differ based on SMN2 copy number.\(^{73}\) Despite functional improvement, there has been no prevention of scoliosis progression with nusinersen use alone.\(^{65}\) While the overall effect of nusinersen treatment on life expectancy in less severe SMA subtypes is still unknown, increased likelihood of survival over 13 months has been demonstrated with nusinersen treatment in SMA I.\(^{5,74}\) Patient and caregiver satisfaction with nusinersen treatment is primarily hindered by treatment administration and time commitment.\(^{75}\) The most common adverse effects of nusinersen treatment are attributed to lumbar puncture, such as headache, back pain, and post-lumbar puncture syndrome.\(^{11}\)

**Nutritional Evaluation and Bone Quality**

**Before 2016.** Four studies were included. Evidence suggests that nutritional analysis of children with SMA should include nutritional intake, nutritional needs, underlying medical problems, physical examination, anthropometric measurements, body composition, and biochemical markers.\(^{14,76,77}\) As a result of their higher risk for osteopenia, children with SMA have an increased risk of fragility fractures.\(^{78}\) No studies evaluated surgical risk based on nutritional status or bone quality.

**During or After 2016:** Thirteen studies were included: eight on nutrition challenges, three on effects of nusinersen on nutrition, and two on bone quality. Feeding difficulties and nutrient deficiencies (such as deficiency in fatty acids, vitamin A, vitamin D, vitamin K, folate, calcium, iron and magnesium) remain common in children with SMA.\(^{79-82}\) Increasing evidence shows aberrant fat distribution, fatty acid metabolism, and energy expenditure in these patients.\(^{81,83-85}\) As a result, individual assessment and nutrition planning are recommended.\(^{86}\)

Nusinersen has been shown to decrease the frequency of severe malnutrition in SMA I patients.\(^{87}\) Increased bulbar function (e.g., bite force) after treatment with nusinersen may contribute to improved nutritional outcomes.\(^{88}\) It is important to note, however, that despite initial improvement following administration of nusinersen, symptoms of feeding fatigue and unsafe swallowing subsequently re-appeared in SMA I patients.\(^{89}\)

Low bone mineral density and femur fractures are highly prevalent in all subtypes of SMA.\(^{90}\) Intravenous bisphosphonate has been proposed to improve bone mineral density and decrease fractures in SMA, however evidence is limited.\(^{91}\)

**Spine**

**Before 2016:** Thirteen studies were included on the management of spinal deformity. Scoliosis is highly prevalent in children with SMA, with an incidence of 60%–90%. Initial presentation and management begins in early childhood, with high risk for progression.\(^{14,19,24,26,92}\) As a result, orthotic management is recommended to slow progression until the patient is of age to undergo spinal instrumentation.\(^{25,93}\) Growth-friendly instrumentation (GF) and posterior spinal fusion (PSF) have been employed for surgical treatment in skeletally immature and mature patients, respectively.\(^{14,17-19,21,32,94}\)
One study on magnetically controlled growing rods (MCGRs) in two patients with SMA showed improved Cobb major curve angle at 2-years of follow-up and improved postoperative pulmonary function. No studies were found on management of cervical spine pathology in patients with SMA or on how to best accommodate intrathecal access in patients needing spinal instrumentation, though multi-segmental spine constructs have been proposed.

During or After 2016: Twenty-nine studies were included on management of spinal deformity: two on spinal instrumentation, one on management of cervical spine pathology, and 26 on intrathecal access in complex spinal deformity or instrumentation.

MCGRs and VEPTRs continue to improve Cobb major curve angle at 2-3 years of follow-up in SMA patients with spinal deformity. In the setting of cervical spine deformity, strong instrumentation and perioperative control of abnormal involuntary movements (via botulinum toxin injection, physical therapy, muscle division technique, or intrathecal baclofen pump) have been suggested.

Intrathecal access in patients with severe scoliosis or spine instrumentation has become increasingly relevant in the age of gene therapy. Conventional approaches to intrathecal administration are hindered by complex spinal anatomy. Therefore, SMA patients with complicated spines often require fluoroscopic, ultrasound, or CT guidance for intrathecal administration. Reservoir placement may be effective but is also associated with complications such as infection and catheter separation. Rib-based and spine-based growing instrumentation has been used to slow progression of thoracic wall collapse but has poor efficacy in increasing thoracic volume in these children. Poor pulmonary function should not be an absolute contraindication for spinal deformity surgery; noninvasive pulmonary support can improve pulmonary outcomes following surgery on patients with SMA.

During or After 2016: Twenty studies on pulmonary health in SMA were included: 13 on the effect of spinal surgery on pulmonary health and seven on pulmonary outcomes of nusinersen treatment. History of pneumonia is associated with postoperative respiratory complications in neuromuscular spine deformity.

Chest Deformity, Thoracic Insufficiency, and Pulmonary Health

Before 2016: Thirteen studies were included. Children with SMA have increased incidence of thoracic insufficiency as a result of hypotonic scoliosis and poor muscular support. As curves worsen, the deleterious effect of spine deformity on pulmonary function and lung expansion magnifies, and children with SMA have a high propensity to develop parasol rib deformities. Rib-based and spine-based growing instrumentation has been used to slow progression of thoracic wall collapse but has poor efficacy in increasing thoracic volume in these children. Poor pulmonary function should not be an absolute contraindication for spinal deformity surgery; noninvasive pulmonary support can improve pulmonary outcomes following surgery on patients with SMA.

During or After 2016: Twenty studies on pulmonary health in SMA were included: 13 on the effect of spinal surgery on pulmonary health and seven on pulmonary outcomes of nusinersen treatment. History of pneumonia is associated with postoperative respiratory complications in neuromuscular spine deformity.

Figure 1. “Skip” construct in SMA patient requiring intrathecal therapy. L1-L2 levels not exposed (no fusion) to allow for drug delivery.
surgery. Still, positive outcomes are seen with scoliosis surgery even in patients with FVC of below 30% predicted. The effect on pulmonary function metrics after spine surgery is variable; postoperative decrease in pulmonary function (e.g., from 61.9%–63.9% predicted FVC preoperatively to 56.3%–57.6% postoperatively), increase, and no change have all been reported. Postoperative reduction in the rate of decline of pulmonary function (e.g., from FVC decline of 5.31% per year preoperatively to 1.77% per year postoperatively) has also been described. Even with variable FVC outcomes, health-related quality of life pulmonary function subscores improve after growth-friendly instrumentation (example in Figure 2).

While nusinersen is associated with improved motor function in pediatric patients, evidence regarding the effect on pulmonary function is mixed. Nusinersen treatment has been associated with both improvement and no effect on lung function in various studies measured via FVC and need for assisted ventilation. Responses to nusinersen likely differ between SMN type and timing of intervention relative to symptom onset.

Hip
Six studies published before 2016 were included on hip instability, a common occurrence in patients with SMA. Most studies recommend against surgery, as surgically repaired hips tend to progressively and continuously re-sublux, and hip pathology has rarely been shown to cause pain. Scoliosis and hip subluxation most often present concurrently; scoliosis may develop before hip subluxation, but hip subluxation rarely precedes scoliosis.

Contractures
Before 2016: Seven studies were included on management of contractures. Contractures are common in patients with SMA as a result of decreased range of motion, prolonged static positioning, and agonist-antagonist muscle imbalance. They most notably develop at the knee, elbow, hip, and ankle.
Functionally and symptomatically, contractures can lead to pain and inhibit function in patients with SMA.\textsuperscript{36,148-152}

During or After 2016: One study was included, demonstrating that even minimal hip and knee joint contractures are associated with diminished motor ability.\textsuperscript{153} No studies were found on the effect of gene therapy on joint contractures.

Delphi Process

The ENMC Orthopaedic Standard of Care Working Group

Thirteen experts chose to participate (12 orthopaedic surgeons, one pediatric neurologist). The 13 responding experts practice in six countries in North America and Europe (United States, Canada, France, Spain, Switzerland and Turkey).

First Questionnaire Results: There were 12 expert responses—seven from North America and five from Europe. Four topics out of the 16 proposed were selected by consensus decision (>70%) (Tables 1 and 2).

This survey posed 17 questions that group members then rated based on whether or not they felt that a) the question was important and b) they were confident in providing a best practice guideline in response. As a result of the first round of the process, six questions were selected:

1. Timing of spinal instrumentation
2. Management and prevention of chest wall deformity
3. Timing and rationale for intervention for hip pathology
4. Role of hip osteotomy
5. Management of contractures of upper and lower extremity
6. Surgical planning and implantation devices

Second Questionnaire Results: There were seven expert responses—four from North America and three from Europe. Responses informed the creation of the final questionnaire.

Table 2. Topics with Consensus Support for Development of an Expert Consensus Statement

| 1. Timing of interventions for scoliosis |
| 2. Management of chest wall deformity |
| 3. Timing and rationale of intervention for hip instability |
| 4. Contracture management |

Final Questionnaire Results: There were 11 expert responses—seven from North America and four from Europe. The responses from experts from the United States were not statistically significantly different from the responses from experts from Europe. The expert consensus statement was created from the answers supplied to this questionnaire (Tables 3 and 4, Figure 3).

Discussion

In light of the scientific breakthroughs in treating SMA with gene therapy, management strategies must adapt to appropriately address the changing approach for the orthopaedic care of this population. Previous studies have recommended aggressive bracing and surgical treatment of scoliosis for children with SMA.\textsuperscript{17-20,24-28,32,36,95,129,154} The expert consensus statement developed in this study adds to existing literature by recommending specific treatment strategies stratified by age and major curve, while also providing recommendations on interpreting accompanying characteristics such as curve progression, pain, functional changes and hyperkyphosis among other symptoms. Importantly, as genetic therapies become a mainstay of care in this population, orthopaedic treatments are recommended to accommodate intrathecal access by leaving lumbar vertebral levels un-instrumented when performing spinal surgery. In the setting of spine instrumentation or severe scoliosis, fluoroscopic, ultrasound, or CT guidance may be used for intrathecal access. Without “skip” level constructs, repeated intrathecal injection (every 4 months for the duration of life) may result in a significant cumulative radiation dose for patients and thus may present a health risk.\textsuperscript{104} In addition, promising results from early studies\textsuperscript{155-158} and the professional experience...
of the committee members informs the recommendation for use of magnetically controlled growing rods for this population.

While nascent literature explains the historic futility in treating hip subluxation, experts overwhelmingly believe that the new therapies will decrease the incidence of both hip pathology and recurrence after repair via improved muscle tone.\(^{32,35}\) As a result, our consensus recommendation reflects this and advises aggressive treatment of hip instability to capitalize on the treatment’s effects and allow for maximal mobility. In the same vein, previous studies on contracture management have recommended both conservative treatment such as orthoses, physiotherapy, and occupational therapy as well as interventional operations such as tendon lengthening and/or tenotomy in this population.\(^{24,36}\) Historic surgical contracture correction was recommended only to facilitate sitting, allow for comfortable footwear,
and prevent pressure ulcers. In light of the changes in muscle tone as a result of new treatment options, the experts in this study recommended surgery for contractures in cases of decreased function and to correct painful contractures of the lower extremity.

We encountered some limitations in this endeavor. First, the final expert panel in this study consisted of 13 members, despite the initial 17 invitations. Though literature demonstrates that the Delphi Process can be employed with as few as 10 to 15 members, it functions optimally with 15 to 20 members. Second, our next questionnaire had suboptimal involvement with seven responses. As a result, fewer choices were available to include in the final questionnaire, leading to the

### Table 4. Agreement with Items Describing the Treatment of SMA Reaching Equipoise (30%–70% in Favor or Against)

<table>
<thead>
<tr>
<th>Items Describing Treatment of SMA in Spine Deformity</th>
<th>n/N (%) in Agreement</th>
</tr>
</thead>
<tbody>
<tr>
<td>GF instrumentation may be used in skeletally immature patients with major curve angle of 40–50°.</td>
<td>5/11 (45%)</td>
</tr>
<tr>
<td>Rib-based constructs may be used to control parasol rib deformity vs. spine-based constructs.</td>
<td>6/11 (55%)</td>
</tr>
<tr>
<td>The decision to implement spinal instrumentation may or may not be encouraged by: back pain or independent ambulation.</td>
<td>5/11 (45%) 4/11 (36%)</td>
</tr>
<tr>
<td>2-year-old children with large progressive curves may receive GF instrumentation.</td>
<td>6/11 (55%)</td>
</tr>
</tbody>
</table>

**Items Describing Treatment of SMA in Hip Instability**

<table>
<thead>
<tr>
<th>Items Describing Treatment of SMA in Hip Instability</th>
<th>n/N (%) in Agreement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hip osteotomy technique and approach may depend on pathology and imaging.</td>
<td>7/11 (64%)</td>
</tr>
</tbody>
</table>

**Items Describing Treatment of SMA in Contractures**

<table>
<thead>
<tr>
<th>Items Describing Treatment of SMA in Contractures</th>
<th>n/N (%) in Agreement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Contractures of the upper extremity may be surgically managed when they cause pain.</td>
<td>6/11 (55%)</td>
</tr>
</tbody>
</table>

**Figure 3.** Expert panel recommendations regarding spine surgery in the setting of large, progressive curves in patients of varying ages.

Assuming a large, progressive curve, what approach is appropriate for each given age?

---

Copyright © 2022 JPOSNA®
possibility that essential viewpoints were omitted from further discussion. Third, physicians for this venture were selected from North America, Europe, and Asia, but ultimately only included members from North America and Europe. Future processes should involve more expert physicians from diverse backgrounds and practices across the globe.

Orthopaedic treatment for patients with SMA is perpetually changing. Contemporary analysis of patients with SMA is warranted to understand the incidence of musculoskeletal manifestations of SMA, their impact on quality of life and function, and the impact of treatments. Additionally, critical analysis of methodology is necessary to identify the best ways to select appropriate endpoints. Topics of further investigation should include the effect of gene therapy on decreasing the incidence and severity of contractures, fragility fractures, hip instability, and spinal deformity in order to fully understand the effects of this revolutionary therapy on this population. Because gene therapies continue to change the trajectory of this disease and the perception of its prognosis, we may need to revisit the Delphi process to accommodate for the many anticipated innovations in treatment for SMA in the coming years, such as the oral genetic therapy Evrysdi (risdiplam) that was approved by the FDA in August 2020.

Additional Links

- The SMA Foundation: “Since its inception in 2003, the SMA Foundation has spent around $150M on basic, translational, and clinical research to accelerate progress towards finding a treatment for SMA.” https://smafoundation.org/research/

- The Pediatric Spine Foundation: “The Pediatric Spine Foundation is a community dedicated to improving the quality of care and the outcome of treatment for patients and families dealing with chest wall and spine deformities.” https://pediatricspinefoundation.org/

- POSNA Academy Webinar “The Orthopedic Management of Spinal Muscular Atrophy: New Treatments…Next Steps,” presented by Jill E. Larson, MD; John Grayhack, MD; Brian Snyder, MD, PhD*; Amanda T. Whitaker, MD; Michael Vitale, MD https://www.posnacademy.org/media/The+Orthopedic+Management+Spinal+Muscular+Atrophy+A+New+Treatments%E2%80%A6Next+Steps/1_0d4j9im6

References


