ORIGNAL ARTICLE

From Genes to Function: Clinical Experience with the Effectiveness and Safety of Risdiplam and Nusinersen in Spinal Muscular Atrophy

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ABSTRACT

Objective: Spinal muscular atrophy (SMA) involves the survival motor neuron (SMN) 1 gene, leading to motor neuron degeneration. SMN2 is a homologous gene to SMN1, which can produce SMN protein at lower levels. The new gene-based drugs modify SMN2 pre-messenger RNA splicing, leading to production of functional SMN protein.
Materials & Methods: This study aimed to evaluate the effectiveness and safety of Risdiplam and Nusinersen in patients with SMA types I–III. Hammersmith Functional Motor Scale-Expanded (HFMSE) was used for motor evaluation.
Results: Results revealed that changes were significant after six months ($p< 0.001$). Improvements were compared between the two drugs, age groups, and SMA disease types, and no significant differences were found. No severe side effects were experienced and only a few patients reported headaches, and backaches following Nusinersen.
Conclusion: both Risdiplam and Nusinersen led to significant improvements in motor function; however, based on cost-effectiveness considerations, we recommend Risdiplam.

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Introduction

Spinal muscular atrophy (SMA) is an autosomal recessive disease, leading to degeneration of anterior horn cells (1). The pathology is based on mutations in the second allele of the survival motor neuron (SMN) 1 gene, causing motor neuron degeneration and deterioration of muscle force. Fortunately, A homologous gene to SMN1, called SMN2. SMN2 can produce SMN protein, but at lower levels than SMN1(2-4). SMA disease is divided into five types (0-4) based on the age at the onset of disease and the severity of impaired motor function. Genetic testing can help to determine the SMA disease type (1, 4). Currently, the main treatment approaches include gene-based disease-modifying drugs such as Nusinersen, Onasemnogene abeparvovec, and Risdiplam, which

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have been supported by several studies, as well as supportive care and therapies(5-8). Risdiplam and Nusinersen both modify SMN2 pre-messenger RNA splicing, leading to an increase in the production of functional full-length SMN protein (5, 7). The present study aimed to evaluate the effectiveness and safety of these two drugs in patients with SMA types I–III, specifically in the western region of Iran.

Materials & Methods

The current study was conducted from January 2023 to January 2025 over a period of two years at the Children's Hospital, a referral center for pediatric neurology in western Iran. Patients under 20 years of age with a confirmed diagnosis of SMA, based on genetic testing, from the Hamadan and Kermanshah provinces (western Iran) were enrolled in the study. One of two drugs was used for SMA patients: Risdiplam (Evrysdi®, Genentech, South San Francisco, CA, USA) or Nusinersen (Spinraza®, Biogen, Cambridge, MA, USA).

The medications were provided to patients through a national project by the Ministry of Health and Medical Education (Iran). The Ministry fully covered the cost of the medications as part of the established treatment plan, and neither patient funds nor the study budget were allocated for their purchase. Since two treatment options were available, the choice of drug for each patient was based on patient preference and consideration of individual contraindications. For example, patients with severe scoliosis could not receive Nusinersen via intrathecal injection, so Risdiplam (oral drug) was administered. The appropriate dosage for each patient was determined by a pediatric neurologist.

At the initial visit, informed consent was obtained from participants or their legal guardians prior to participation. Demographic data and clinical information, including the presence and severity of scoliosis, wheelchair dependency, swallowing ability, respiratory status, weight, and height were recorded in standardized checklists. Baseline laboratory evaluations, including Complete Blood Count (CBC), Blood Urea Nitrogen (BUN), Creatinine, Creatine Phosphokinase (CPK), Aspartate Aminotransferase (AST), Alanine Aminotransferase (ALT), Prothrombin Time (PT), International Normalized Ratio (INR), Partial Thromboplastin Time (PTT), and urinalysis for urine protein, were performed and documented in a dedicated laboratory checklist. These evaluations were repeated at one month and six months for follow-up.

At the baseline visit and at follow-up assessments conducted at one, three, and six months, patients' motor function was evaluated using the Hammersmith Functional Motor Scale–Expanded (HFMSE) checklist (9-11). The tool comprises 33 items that evaluate various aspects of motor performance across diverse muscle groups. These items assess functional domains, including postural control, seated stability, fine motor tasks, mobility (e.g., rolling, crawling, and ambulation), and weight-bearing activities, as well as complex movements such as squatting and stair navigation. Each item is scored 0, 1, or 2, and the total score is calculated as the sum of all item scores.

For patients with type I SMA, the Hammersmith Infant Neurological Examination (HINE)- Motor Milestones checklist was also completed at each visit (12, 13). This checklist assesses eight items: Head control, sitting, voluntary grasp, ability to kick, rolling, crawling, standing, and walking. Each item is scored from 0 to 4, and the total score is obtained by summing the individual item scores.

At each follow-up visit (one, three, and six months after initiating treatment), patients were asked about potential drug-related side effects. These included anaphylactic reactions, fever, rash, gastrointestinal symptoms, respiratory symptoms, and neurological symptoms, such as headache, dizziness, vertigo, backache, and post-lumbar puncture syndrome. Neurological symptoms were specifically assessed in patients receiving intrathecal Nusinersen. All data were recorded in a designated checklist.

Finally, all data collected through the checklists, including demographic information, laboratory results, motor function assessments, and reported side effects were entered into IBM SPSS Statistics for Windows (Version 22.0; Armonk, NY: IBM Corp.) for analysis. A paired t-test was used to compare means before and after drug administration. For comparisons between two groups, an independent t-test was applied, and for comparisons among three groups, the Kruskal–Wallis test was used. A significance level of 0.05 was considered.

Results

In this study, 16 cases were enrolled. One infant with SMA type I was enrolled at the age of 4 months and, after about three months, died due to severe progressive pneumonia. Therefore, 15 cases were finally evaluated in this study. The cases were followed for six months after initiating Risdiplam or Nusinersen, and their motor function was assessed during this period. Among these 15 cases, one had SMA type I, three had SMA type II, and 11 had SMA type III. Six girls and nine boys were evaluated. Five were under ten years old, and ten were older than ten years.

On the initial physical exam, scoliosis was evaluated. One patient (SMA type II, older than 10 years) had a history of surgery due to severe scoliosis.

Two cases had severe scoliosis, seven patients had mild scoliosis, and five patients did not have scoliosis. No patient had a history of any disease other than SMA. The patient with SMA type I and all three patients with SMA type II had a history of several severe respiratory infections leading to hospitalization. Patients with SMA type III had no history of hospitalization due to respiratory infections, and only one of them had a history of hospitalization due to an upper limb fracture. Three cases (one SMA type II and two SMA type III) were wheelchair-dependent, three cases (one SMA type I and two SMA type II) were carried by their parents, and the others were able to stand and walk independently. While all patients demonstrated unimpaired swallowing, one patient with SMA type I required a percutaneous gastrostomy for feeding support, despite possessing functional swallowing ability. Additionally, all patients had normal breathing ability except the SMA type I patient, who required supportive oxygen or Continuous Positive Airway Pressure (CPAP) during some episodes. For all cases, dependency on a wheelchair did not change during the follow-up period. Additionally, the patients' swallowing ability and respiratory function did not deteriorate during the study.

Regarding laboratory findings, ten cases had elevated CPK at the initial visit, and CPK levels in these patients remained elevated. However, no patient experienced a rise in CPK levels after drug initiation. All other laboratory evaluations, including CBC, AST, ALT, coagulation tests, and urinalysis, were normal both initially and on follow-up tests. No patient reported any severe drug reactions or side effects from Risdiplam or Nusinersen. Only four patients who received intrathecal Nusinersen reported headaches, and two patients reported backaches during the first two days after injection.

Changes in HFMSE scores between the first and last visits (baseline and after six months) ranged from -2 to 9, with a mean value of 3.73 ± 2.9 and a median of 3 (Figure 1). Mean and median values of score changes in different groups based on SMA disease types, drug types, and age groups are presented in Table 1. HFMSE scores for each patient at different visits (baseline, and after 1, 3, and 6 months) and their variations are shown in Figures 2 and 3.

Table 1. Median, Mean, and Standard Deviation of Score Changes Across Groups by SMA Disease Type, Drug Type, and Age Group, with Analytic Test Results for Group Comparisons.

		Median	Mean	Standard Deviation	P-value
SMA disease type	1	3	3	-	0.365
	2	2	2	1	
	3	4	4.27	3.3	
Age Group	< 10 years old	3	3.50	2.5	0.863
	≥ 10 years old	3	3.82	3.3	
Drug	Risdiplam	2.5	3.76	3.6	0.990
	Nusinersen	3	3.72	2.9	

A comparison of mean patient scores was conducted between baseline and at three- and six-months post-drug initiation. The mean score at baseline was 30.73 ± 18.6, increasing to 32.00 ± 19.4 at the three-month mark and further to 34.47 ± 19.6 at six months. The differences in mean scores for all cases were evaluated between visits (before and after drug initiation). The change was insignificant between baseline and the 3-month visit ($p = 0.157$), but it was significant over six months ($p < 0.001$). Additionally, score changes over six months were compared between recipients of the two drugs, age groups, and SMA disease types, and no significant differences were found. Details and values are presented in Table 1. If this study considers an increase in score of three as a positive response, according to two previous similar studies (14, 15), ten cases (66.7%) showed a positive response after six months of treatment.

Detailed case descriptions

SMA type I case

Only one SMA type I patient was completely evaluated in this study, who was receiving Risdiplam. This patient was diagnosed at the age of six months and had been undergoing physiotherapy and occupational therapy from nine months of age. This treatment plan was continued regularly, even during the period when she received Risdiplam. Following the initiation of Risdiplam at three years of age, the patient underwent a six-month follow-up assessment. She had previously received Risdiplam for approximately 3.5 months at the age of two. However, since the drug was discontinued for several months, the baseline and follow-up visits were considered from the time of treatment initiation at three years of age. At the baseline visit, the patient could swallow but had a Percutaneous Endoscopic Gastrostomy (PEG) tube for

feeding support. She could breathe normally but required supplementary oxygen and CPAP during some episodes, specifically during sleep. For this case, the HINE checklist was also completed at each visit in addition to the HFMSE. The HINE checklist score increased from 5 to 8 over six months of treatment. The patient demonstrated enhancements in several HINE sub-scores, namely stable sitting, voluntary grasp, and the ability to perform kicking movements. Moreover, the parents reported that after receiving the drug, swallowing ability, chest expansion, and limb movements improved. During the treatment course, she was admitted once to the Pediatric Intensive Care Unit (PICU) due to a respiratory infection. During follow-up, the patient did not experience any complications or motor function deterioration.

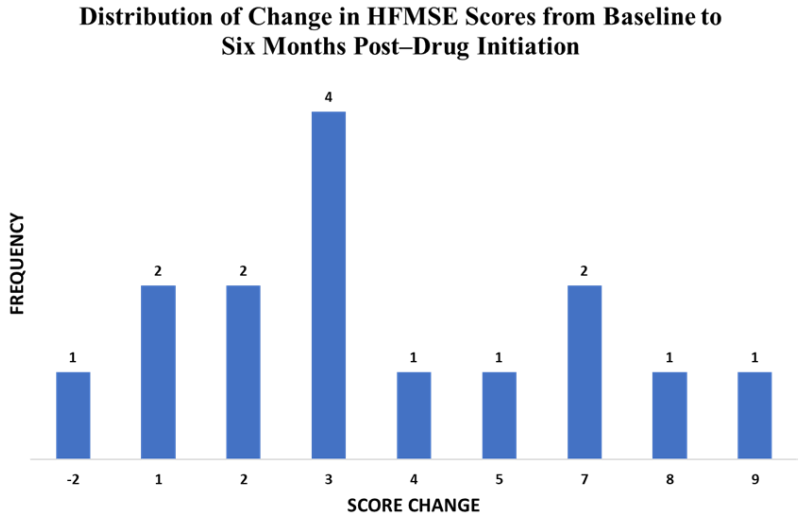

Figure 1. Distribution of Change in HFMSE Scores from Baseline to 6 Months Post–Drug Initiation

SMA type II cases

All three SMA type II cases had a history of hospital admission due to pneumonia. Nevertheless, after three months of drug initiation up to the follow-up period, none experienced severe infections or required hospitalization. One of the SMA type II patients receiving Risdiplam reported improvements in the ability to swallow and in the rotational movement of her arms and legs. Additionally, her voice became clearer. Another SMA type II patient receiving Risdiplam also reported improvements in her ability to hold objects with her hands and to sit stably without support. The persistent occasional cough she had before decreased, and her defecation became easier. The third case reported no subjective changes apart from improved motor function on the checklist.

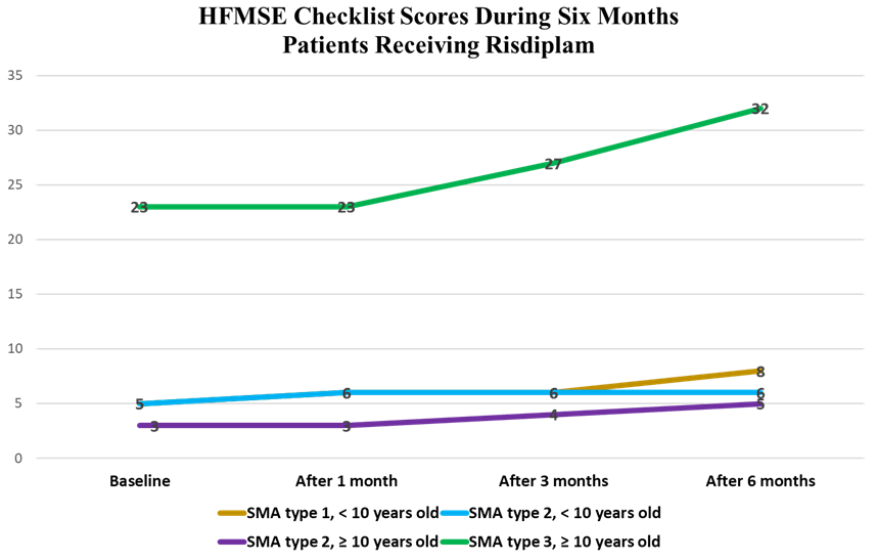

Figure 2. HFMSE Scores of Patients Receiving Risdiplam at Baseline and After 1, 3, and 6 Months, and Their Changes Over Time

SMA type III cases

Among these cases, seven patients reported qualitative functional improvements beyond those captured by the HFMSE scale. These included reduced fatigability during physical exertion, increased exercise tolerance, and enhanced postural balance, which facilitated ambulation. Furthermore, these patients noted improved gait ease and a concurrent reduction in the frequency of falls."

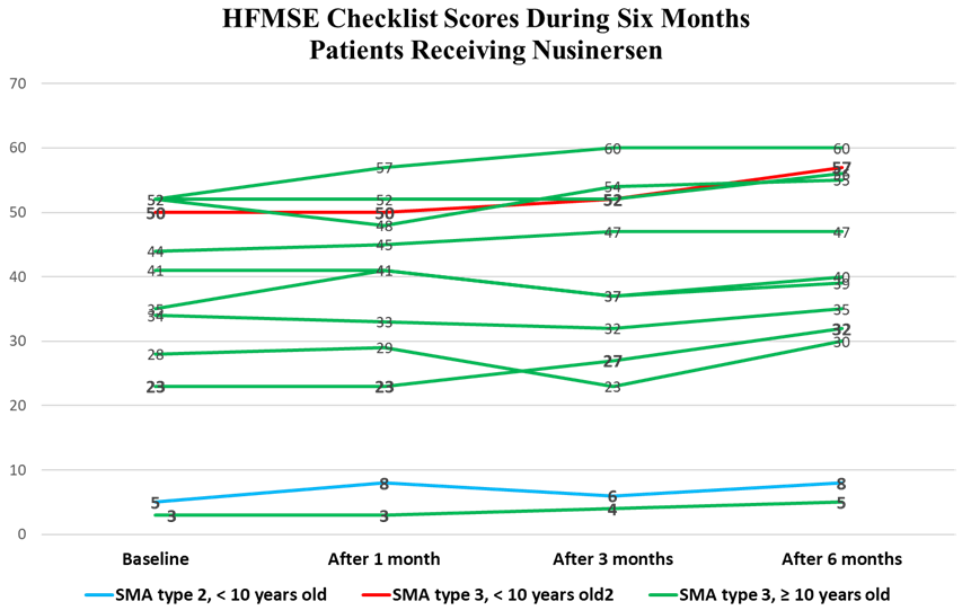

Figure 3. HFMSE Scores of Patients Receiving Nusinersen at Baseline and After 1, 3, and 6 Months, and Their Changes Over Time

Discussion

SMA is a genetic disease characterized by degeneration of motor neurons in the anterior horn of the spinal cord and the motor nuclei in the lower brainstem, while cognitive abilities are preserved. For many years, the disease was managed only with supportive care, but with the introduction of gene expression–modifying drugs, the prospects for patients with SMA have changed. Although several studies have been published worldwide on the effectiveness of these drugs, there remains a shortage of clinical experience in implementing them across different SMA patient groups, nationalities, and regions. This study used the HFMSE checklist, evaluating various aspects of motor function and is commonly applied in studies assessing motor function in patients with SMA.

In this study, HFMSE scores increased significantly after six months of treatment; however, the changes were not significant after three months. A study by Ashrafi et al. evaluated 73 children treated with Risdiplam and 52 children treated with Nusinersen over six months, and the changes in HFMSE scores were significant after both three and six months (14). A systematic review by Daghriri et al. of 10 studies reported significant improvements in motor function among patients with SMA types I, II, and III, using different scales such as the HFMSE, the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND), and the 6-Minute Walk Test (6MWT) (16). Considering a three-point increase in the HFMSE score as a positive response, 66.7% of patients in the present study achieved this outcome. Similarly, in the study by Ashrafi et al., 76% of patients achieved this response (14). In a small cohort study of six adults, the HFMSE did not demonstrate significant changes after treatment with Risdiplam; however, this may be attributed to the small sample size. Moreover, the study evaluated only adults, whereas children may respond better to the treatment (17).

Differences in score improvements were not significant between the groups that received Nusinersen and Risdiplam in this study, consistent with the results of the study by Ashrafi et al. (14). On the other hand, a study of the long-term comparative efficacy of Risdiplam and Nusinersen in children with SMA type I, showed that a 45% higher rate of achieving a HINE (Module 2) motor mile stone response was obtained among patients treated with

Risdiplam. Furthermore, Risdiplam receivers had lower rates of death, needing for permanent ventilation (18). In addition, a systematic review comparing Risdiplam with other treatments for SMA types I–III reported that indirect comparisons suggest Risdiplam is more effective than Nusinersen for type I SMA. (19).

Consistent with the findings of Brakmeier et al., the obtained results indicate improvements in swallowing function among several patients. This aligns with their report, observing that subjective swallowing quality significantly improved following 12 months of Risdiplam therapy." (20). Overall, results regarding improvements in respiratory and nutritional function are contradictory. Two systematic reviews reported no significant improvement in respiratory function following treatment with disease-modifying drugs (21, 22). One of them concluded no decline in respiratory function among patients, and Nusinersen can stabilize the function of respiratory muscles (21). Another study evaluating Nusinersen over three years reported that changes in respiratory function was variable with some patients without any change in respiratory function, some patients improving, and some patients deteriorating (23).

In the present investigation, no patient experienced any deterioration in their function during the treatment, including respiratory, swallowing, walking, and motor functions, valuable even in the absence of significant improvements. In addition, this study did not observe any side effects from Risdiplam. The only side effects from Nusinersen were headache and backache after injection, which have also been reported by two systematic reviews in this field (16, 22).

Although the present study tried to gather and report the best possible data, limitations in conducting this study exist. The crucial limitation is the small population of this disease, as well as the diversity of cases regarding SMA type, prescribed drug, age, sex, and other biological factors, affecting outcomes and make comparisons challenging. Besides, assessing motor function using checklists like HFMSE can be influenced by patients' energy levels, fatigue, and environmental factors, so scores may not always reflect patients' true abilities. However, this study used the most comprehensive checklist available to cover different aspects. Although occupational and physiotherapy contribute to motor improvement, quantifying their specific impact remains challenging. Consequently, these therapies may act as confounding variables that were not fully accounted for in the studies included in this review The last challenging issue regarding SMA disease-modifying drugs, which was not the primary goal of this study, is their cost-effectiveness. A study in China, the country with the highest number of SMA cases, reported that Risdiplam and Nusinersen have improved Quality-Adjusted Life Years (QALYs), and Risdiplam was a cost-effective treatment compared to Nusinersen (24).

In Conclusion

Disease-modifying drugs, including Risdiplam and Nusinersen, caused significant improvements in motor function scores after six months, although changes were not significant after three months. This study did not detect considerable variation between drugs and SMA types, but side effects were more frequent with Nusinersen. Considering the cost-effectiveness studies, we recommend Risdiplam for SMA treatment, consistent with previous studies. Additionally, measuring levels of physical activity or therapy as potential confounders in further studies with larger sample sizes are recommend.

Acknowledgment

Ethics committee of Hamadan University of Medical Sciences has approved this project. (IR.UMSHA.REC.1401.1046)

Authors' Contribution

Study design: Parinaz Sedighi, Afshin Fayyazi, Seyyed Mohammad Mahdi Hosseiny

Data gathering and Project conduction

Parinaz Sedighi, Afshin Fayyazi, Firozeh Hosseini, Kiana Karimi, Hossein Esfahani , Seyyed Mohammad Mahdi Hosseiny Statistical analysis: Parinaz Sedighi, Kiana Karimi, Writing article draft: Parinaz Sedighi, Seyyed Mohammad Mahdi Hosseiny, Article Edition and appraisal: Parinaz Sedighi, Afshin Fayyazi, Firozeh Hosseini, Kiana Karimi, Hossein Esfahani, Seyyed Mohammad Mahdi Hosseiny

Conflict of Interest

The authors declared no conflicts of interests regarding the publication of this article.

References

1. Prior TW, Leach ME, Finanger EL. Spinal Muscular Atrophy. Seattle, WA: University of Washington; 2024.
2. Verhaart IEC, Robertson A, Wilson IJ, Aartsma-Rus A, Cameron S, Jones CC, et al. Prevalence, incidence and carrier frequency of 5q-linked spinal muscular atrophy - a literature review. Orphanet J Rare Dis. 2017;12(1):124.
3. Lefebvre S, Bürglen L, Reboullet S, Clermont O, Burlet P, Viollet L, et al. Identification and characterization of a spinal muscular atrophy-determining gene. Cell. 1995;80(1):155–65.

4. Chongmelaxme B, Yodsurang V, Vichayachaipat P, Srimatimanon T, Sanmaneechai O. Gene-based therapy for the treatment of spinal muscular atrophy types 1 and 2: a systematic review and meta-analysis. GENE THERAPY. 2025;32(4):301–30.

5. Finkel RS, Mercuri E, Darras BT, Connolly AM, Kuntz NL, Kirschner J, et al. Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy. N Engl J Med. 2017;377(18):1723–32.

6. Day JW, Finkel RS, Chiriboga CA, Connolly AM, Crawford TO, Darras BT, et al. Onasemnogene abeparvovec gene therapy for symptomatic infantile-onset spinal muscular atrophy in patients with two copies of SMN2 (STR1VE): an open-label, single-arm, multicentre, phase 3 trial. Lancet Neurol. 2021;20(4):284–93.

7. Baranello G, Darras BT, Day JW, Deconinck N, Klein A, Masson R, et al. Risdiplam in Type 1 Spinal Muscular Atrophy. N Engl J Med. 2021;384(10):915–23.

8. Walter MC, Chiriboga C, Duong T, Goemans N, Mayhew A, Ouillade L, et al. Improving Care and Empowering Adults Living with SMA: A Call to Action in the New Treatment Era. J Neuromuscul Dis. 2021;8(4):543–51.

9. O'Hagen JM, Glanzman AM, McDermott MP, Ryan PA, Flickinger J, Quigley J, et al. An expanded version of the Hammersmith Functional Motor Scale for SMA II and III patients. Neuromuscul Disord. 2007;17(9-10):693–7.

10. Glanzman AM, O'Hagen JM, McDermott MP, Martens WB, Flickinger J, Riley S, et al. Validation of the Expanded Hammersmith Functional Motor Scale in spinal muscular atrophy type II and III. J Child Neurol. 2011;26(12):1499–507.

11. Biogen. Hammersmith Functional Motor Scale (HFMS) – Slovenian version.

12. Haataja L, Mercuri E, Regev R, Cowan F, Rutherford M, Dubowitz V, et al. Optimality score for the neurologic examination of the infant at 12 and 18 months of age. The Journal of Pediatrics. 1999;135(2):153–61.

13. Biogen. Hammersmith Infant Neurological Examination (HINE) – Slovenian version.

14. Ashrafi MR, Babaee M, Hashemi Nazari SS, Barzegar M, Ghazavi M, Beiraghi Toosi M, et al. Comparative efficacy of risdiplam and nusinersen in Type 2 and 3 spinal muscular atrophy patients: A cohort study using real-world data. J Neuromuscul Dis. 2024;11(6):1190–9.

15. Erdos J, Wild C. Mid- and long-term (at least 12 months) follow-up of patients with spinal muscular atrophy (SMA) treated with nusinersen, onasemnogene abeparvovec, risdiplam or combination therapies: A systematic review of real-world study data. Eur J Paediatr Neurol. 2022;39:1–10.

16. Daghriri S, Alorfi YA, Alayed RS, Altaf WF, Alharbi GE, Alsulami H, et al. The efficacy and safety of Nusinersen for spinal muscular atrophy types 1, 2, 3: a systematic review of the current evidence. The Egyptian Journal of Neurology, Psychiatry and Neurosurgery. 2025;61(1):30.

17. Severa G, Alfaro MdC, Alimi Ichola C, Shoaito H, Souvannanorath S, Authier F-J, et al. Risdiplam: therapeutic effects and tolerability in a small cohort of 6 adult type 2 and type 3 SMA patients. Orphanet Journal of Rare Diseases. 2024;19(1):430.

18. Kokaliaris C, Evans R, Hawkins N, Mahajan A, Scott DA, Sutherland CS, et al. Long-Term Comparative Efficacy and Safety of Risdiplam and Nusinersen in Children with Type 1 Spinal Muscular Atrophy. Advances in Therapy. 2024;41(6):2414–34.

19. Ribero VA, Daigl M, Martí Y, Gorni K, Evans R, Scott DA, et al. How does risdiplam compare with other treatments for Types 1-3 spinal muscular atrophy: a systematic literature review and indirect treatment comparison. J Comp Eff Res. 2022;11(5):347–70.

20. Brakemeier S, Lipka J, Schlag M, Kleinschnitz C, Hagenacker T. Risdiplam improves subjective swallowing quality in non-ambulatory adult patients with 5q-spinal muscular atrophy despite advanced motor impairment. JOURNAL OF NEUROLOGY. 2024;271(5):2649–57.

21. Kant-Smits K, Bartels B, van der Heiden L, Veldhoen ES, van der Ent K, van der Pol WL, et al. The Effect of Disease-Modifying Therapies on Lung Function and Respiratory Muscle Strength in Spinal Muscular Atrophy: Systematic Review and Meta-Analysis. RESPIRATORY CARE. 2025;70(3):337–48.

22. Giess D, Erdos J, Wild C. An updated systematic review on spinal muscular atrophy patients treated with nusinersen, onasemnogene abeparvovec (at least 24 months), risdiplam (at least 12 months) or combination therapies. European Journal of Paediatric Neurology. 2024;51:84–92.

23. Scheijmans FEV, Cuppen I, van Eijk RPA, Wijngaarde CA, Schoenmakers MAGC, van der Woude DR, et al. Population-based assessment of nusinersen efficacy in children with spinal muscular atrophy: a 3-year follow-up study. Brain Communications. 2022;4(6):fcac269.

24. Huang YY, Yuan HM, Huang Z. Cost-effectiveness analysis of Risdiplam and Nusinersen for the treatment of Chinese patients with spinal muscular atrophy. Expert Review of Pharmacoeconomics & Outcomes Research. 2025;25(6):943–53.