Informational materials

Consensus on gene replacement therapy for the treatment for spinal muscular atrophy (version № 3)

ABSTRACT

The article represents an updated consensus of russian experts on the use of onasemnogene abeparvovec (Zolgensma®) gene replacement therapy in patients with spinal muscular atrophy 5q. Due to the introduction of expanded neonatal screening for SMA (spinal muscular atrophy) in Russia starting in 2023 and the accumulation of clinical experience in the use of gene replacement therapy, patient selection criteria and management algorithms have been revised. The main statements of the consensus include the extension of the indications for prescribing the drug regardless of the number of *SMN2* gene copies, algorithms for managing patients with an increased titer of AAV9 antibodies using bridge therapy, detailed recommendations for monitoring and correcting adverse events in the postinfusion period. Special attention is paid to the management of patients with hyperbilirubinemia and cytomegalovirus infection, the criteria for switching to gene replacement therapy from other types of pathogenetic therapy, as well as the possibilities of using an intrathecal form of gene replacement therapy in children over 6 months of age and adult patients. The consensus provides practical recommendations for clinicians to optimize SMA therapy in the context of modern treatment options.

Keywords: spinal muscular atrophy; gene replacement therapy; onasemnogene abeparvovec; bridge therapy; hyperbilirubinemia; cytomegalovirus infection.

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Introduction

Recently, there have been many positive changes in the diagnostics and treatment of patients with spinal muscular atrophy (SMA). Since January 2023, Russia has implemented an expanded neonatal screening program covering 36 diseases, including SMA [1]. Screening provided important information on the incidence of SMA. Thus, according to a recent publication summarizing data for 2023 and 2024, the screening identified, the prevalence of SMA is 1 case per 8439 newborns, which does not differ significantly from the data previously obtained in the pilot screening projects (1 case per 7953 newborns, $p > 0.05$) [2].

The availability of various options for pathogenetic therapy of SMA has considerably changed the natural course of SMA and resulted in improved survival and the acquisition of new or restoration of previously lost motor skills [3–5]. It should be noted that the existing clinical classification of SMA 5q, which includes 5 types based on the age at disease onset, does not reliably reflect the functional status over time, which is particulary relevant in the context of neonatal screening and prescription of pathogenetic therapy at the presymptomatic stage of the disease. In this regard, Russian and international experts proposed a new classification of SMA. According to the new classification, there are 3 functional classes of SMA: non-sitters, sitters and walkers [3–5].

Since the authorization of a single-dose gene replacement therapy, onasemnogene abeparvovec (OA) in the Russian Federation in 2021, an extensive clinical data on its use has been accumulated.

Consequently, leading SMA specialists concluded that it was necessary to systematize and update the consensus guidelines on patient management and treatment algorithms before and after infusion of gene replacement therapy, which is practically important.

OA is currently included in the Circle of Kindness Foundation Procurement List for the treatment of SMA patients. In 2025, due to the expansion of the therapeutic indications of OA approved by the Ministry of Health of the Russian Federation, the Circle of Kindness Foundation revised the categories of children for whom OA is recommended [6, 7].

1. Categories of children for whom onasemnogene abeparvovec (trade name: Zolgensma®), solution for infusion, is indicated:

1. A child with biallelic mutations in the *SMN1* gene.
2. A child belonging to one of the following categories:
   - 2.1. A child who has received a decision from a medical board involving no fewer than three federal medical centers (for children who have previously received other types of pathogenetic therapy), or
   - 2.2. A child with a documented recommendation from one of the four federal centers experienced in gene replacement therapy (National Medical Research Center for Children's Health of the Ministry of Health of Russia; Research Clinical Institute of Pediatrics and Pediatric Surgery named after Academician Yu.E. Veltishchev; Russian National Research Medical University named after N.I. Pirogov; National Medical Research Center named after V.A. Almazov of the Ministry of Health of the Russian Federation) (for children who have not previously received other types of pathogenetic treatment);

3. A child has a negative anti-AAV9 antibody test;
4. A written refusal of legally acceptable representatives from another type of pathogenetic therapy after the use of gene replacement therapy at the expense of the Foundation;
5. A child weighing no more than 21 kg.

Thus, the previously adopted criteria limiting the use of OA in patients with more than 3 *SMN2* gene copies are no longer relevant.

Consensus opinion

Due to the changes made to the Summary of Product Characteristics, onasemnogene abeparvovec can be recommended to a wide range of patients with a documented diagnosis of SMA, weighing no more than 21 kg, regardless of the *SMN2* gene copy number [7].

Background

The use of intravenous gene replacement therapy was studied in symptomatic and presymptomatic SMA patients in 6 clinical studies: Phase I (START) study [8], 3 Phase III STRIVE studies (STRIVE-US [9], STRIVE-EU [10], and STRIVE-AP [11-12]), SPR1NT and SMART studies [13–15]. Additionally, a long-term 15-year follow-up of patients who completed participation in clinical studies is ongoing, and a registry of patients who received gene replacement therapy in real-world clinical practice (RESTORE [16–18]) has been created. In contrast to the natural course of the disease, a single infusion of OA provides a rapid therapeutic effect that increases over time, improves motor function, and increases survival in SMA patients. To date, there are data on the preserved efficacy of single-dose gene replacement therapy for about 10 years [19]. In addition to these clinical studies, extensive experience has been accumulated in the use of gene replacement therapy in real-world settings, in both naïve patients and previously treated patients, including in the Russian SMA population [20–26]. The results indicate that after the infusion, the patient's motor functions improved orstabilized, the therapy was well tolerated, and the observed adverse events were transient.

2. Gene replacement therapy can be indicated for patients with biallelic *SMN1* gene mutations without clinical manifestations, including those identified during neonatal screening, regardless of the *SMN2* gene copy number, including patients with 1 and 5 copies of the *SMN2* gene.

Background

Newborn screening for SMA allows detection of the disease before the onset of symptoms, within one week after birth. In this case, pathogenetic therapy, including gene replacement therapy, can be indicated before the first symptoms of the disease appear and provide the greatest protective effect [27].

The SPR1NT study confirmed the favorable safety profile and high tolerability of gene replacement therapy in patients with 2 and 3 *SMN2* gene copies [13, 14].

There are also cases of patients with 1 *SMN2* gene copy who were identified as part of neonatal screening and received gene replacement therapy at the pre-symptomatic stage, which allowed them to reach age-appropriate development by the age of 30 months [28]. At the same time, patients with 1 *SMN2* gene copy have the most severe course of the disease [29], and therefore therapy should be prescribed as soon as possible. Onasemnogene abeparvovec is preferable in such patients [30]; if its immediate use is impossible, bridge therapy with other pathogenetic agents should be considered before gene replacement therapy prescription.

According to pilot neonatal screening projects in different countries, 6%–38% of SMA patients have 4 or more copies of the *SMN2* gene [31]. In the article, Tizzano EF et al. studied the use of onasemnogene abeparvovec in patients with ⩾4 *SMN2 gene copies* identified at the pre-symptomatic stage during neonatal screening [32]: patients tolerated the therapy well and acquired new motor skills. Literature data also indicate that symptomatic SMA patients with 5 *SMN2* gene copies are more likely to develop SMA type III, but SMA types I and II may also occur [29, 33].

Furthermore, a recently published pharmacoeconomic study demonstrated the benefits of pre-symptomatic initiation of SMA therapy in a cohort of patients born with 4 copies of the *SMN2* gene. A pharmacoeconomic effect was demonstrated for all drugs used in the treatment of patients with SMA, but the use of OA was characterized by the greatest cost reduction compared to other drugs [34].

Consensus opinion

The mechanism of action of gene replacement therapy is based on the introduction of a functional *SMN1* gene copy into transduced cells. Therefore, the number of *SMN2* gene copies is not a crucial factor in the use of onasemnogene abeparvovec. The mechanism of action and a single-dose administration of onasemnogene abeparvovec make it possible to use it in patients regardless of the *SMN2* gene copy number. Patients with SMA and 1 *SMN2* gene copy should be treated immediately, preferably with gene replacement therapy. Bridge therapy with the immediate use of splicing modifiers may be considered.

The management of children with 5 *SMN2* gene copies should be as follows: symptomatic children should be treated immediately; pre-symptomatic patients should undergo a comprehensive examination with mandatory ENMG (M-response amplitude of the ulnar nerve) and assessment of motor skills using scales. If necessary, the issue of the prescription of pathogenetic therapy should be settled.

3. All patients with planned gene replacement therapy should be tested for antibodies to adeno-associated viral vector serotype 9 (AAV9). Patients with a baseline anti-AAV9 antibody titer of more than 1:50 should be retested in 2–4 weeks or an alternative pathogenetic treatment should be considered. If the titer decreases below 1:50, gene replacement therapy can be prescribed.

Background

It is necessary to determine the level of anti-AAV9 antibodies titers in patients before OA infusion [7]. Anti-AAV9 antibody formation can take place after natural exposure to the virus. Several studies on the prevalence of anti-AAV9 antibodies in the overall population showed low rates of prior exposure to AAV9 in the pediatric population [7]. Nevertheless, it can reach 14% in newborns in the first month of life, which is most often associated with transplacental transmission of antibodies from the mother [35,36]. The literature data show that the half-life of transplacentally transmitted antibodies is approximately 6 weeks [37]. During newborn screening in patients with planned gene replacement therapy, testing for anti-AAV9 antibodies can be performed simultaneously with confirmatory diagnosis. Retesting may be performed if anti-AAV9 antibody titers are reported as above 1:50. The retesting period depends on the anti-AAV9 antibody titers and is determined by the attending physician. Depending on the baseline level of antibodies, antibody testing is recommended once every 2–4 weeks [36–37]. The efficacy and safety of OA in patients with anti-AAV9 antibody titers above 1:50 are unknown [7]. No dose adjustment is required in patients with baseline anti-AAV9 antibody titers exceeding 1:50 [7]. One of the approaches that can be used in routine clinical practice with increased anti-AAV9 antibody titers is bridge therapy (for more details, see section 4).

4. Bridge therapy.

If increased anti-AAV9 antibody titers (more than 1:50) are detected in patients with planned gene replacement therapy, another short-term (up to 4 months) pathogenetic therapy is recommended to control the disease before the infusion of gene replacement therapy.

Background

The use of bridge therapy (short-term therapy with other pathogenetic agents for up to 4 months inclusive) before gene replacement therapy may be relevant for patients with SMA and a transient increase in the antibody titer to adeno-associated virus serotype 9 (AAV9), since studies confirm the short-term nature of the transient increase in the anti-AAV9 antibody titer [36–38]. In patients with SMA detected during neonatal screening, an increase in the anti-AAV9 antibody titer is due to transplacental transfer of anti-IgG antibodies; the average anti-AAV9 antibody titer normalizes to ⩽1:50 within 6 weeks and depends on the degree of baseline increase [36, 37]. Depending on the baseline level of antibodies, determination of the level of antibodies is recommended once every 2–4 weeks [36, 37, 39]. If the level of anti-AAV9 antibodies is normalized to at least 1:50, gene replacement therapy is recommended.

5. Prior to infusion of gene replacement therapy in patients with SMA, the following are required in addition to anti-AAV9 antibody testing: a clinical blood count and biochemical blood test (laboratory tests of liver and kidney function). Hyperbilirubinemia is not an absolute contraindication to the gene replacement therapy. In case of hyperbilirubinemia, it is preferable to reduce its level to 2×ULN (upper limit of normal). In the case of acute or chronic uncontrolled active infections, including cytomegalovirus infection (CMV), treatment should be delayed until clinical and laboratory recovery/ remission occurs. In severe cases of SMA requiring immediate pathogenetic therapy, the issue of patient management should be considered individually at a consilium of multidisciplinary team specialists.

Background

Due to the increased risk of serious systemic immune response, it is recommended that patients are clinically stable in their overall health status, with no infectious diseases present and without laboratory abnormalities prior to onasemnogene abeparvovec infusion.

Before using gene replacement therapy, the following laboratory tests are required [7]:

- determination of anti-AAV9 antibody titers;
- liver function test: alanine aminotransferase (ALT), aspartate aminotransferase (AST), total bilirubin, albumin, prothrombin time, activated partial thromboplastin time (APTT), and international normalized ratio (INR);
- complete blood count (including hemoglobin level and platelet count);
- creatinine.

In case of acute or chronic uncontrolled active infections, treatment should be postponed until the infection is resolved or the patient is clinically stable.

Recently, there has been an increase in the incidence of neonatal jaundice, congenital or acquired CMV infection cases in SMA patients before the use of gene replacement therapy.

Based on the above, an urgent task is to develop a unified algorithm for the management of such patients.

According to the 2017 Russian guidelines for the management of full-term and premature newborns with indirect hyperbilirubinemia, physiological neonatal jaundice is recorded in 60%–80% of healthy full-term newborns, appears within 24 hours after birth, and resolves by Day 10 [40]. In the biochemical blood test, the maximum concentration of total bilirubin does not exceed 255 μmol/L, and no direct hyperbilirubinemia is observed. No anemia or polycythemia is noted. The first alarming signs are the persistence of jaundice for more than 10 days and an increase in the serum bilirubin above 255 μmol/L. In most cases, pathological hyperbilirubinemia presenting in the first week of life is characterized by a predominance of the indirect (unconjugated) bilirubin fraction [40].

In all cases of unspecified pathological jaundice, additional examination is required [40]:

- Collection of medical history (family history of pathological jaundice and/or Gilbert's syndrome);
- Physical examination: severity of jaundice; liver and spleen size; color and frequency of stool and urine; type of feeding; changes over time in the body weight; regurgitation and/or vomiting;
- Functional study methods, if necessary: ultrasound of the brain and abdominal organs; MRI, if indicated;
- Additional laboratory tests: clinical blood count and biochemical blood test (bilirubin and its fractions, transaminase activity [alanine aminotransferase, ALT, and aspartate aminotransferase, AST] and gamma-glutamyl transferase [GBT]), blood test for thyroid hormones (free thyroxine [T4] and thyroid-stimulating hormone [TSH]), PCR test for CMV DNA in the serum, urine, and saliva (determining the DNA titer) with quantification of viral DNA load.

Consensus opinion

A decrease in bilirubin is achieved through the induction of microsomal liver enzymes, normalization of fluid balance, infusion therapy, and/or phototherapy. Based on the management of patients with hyperbilirubinemia, a reduction in parameters to acceptable values occurs on average within 5 days. In case of cholestasis, ursodeoxycholic acid can be prescribed. In case of a combined increase in the activity of transaminases and bilirubin, a blood test for intrauterine infections is recommended. The choice of treatment strategy for cytomegalovirus infection during SMA therapy is influenced by the severity of the disease and the *SMN2* gene copy number. Gene replacement therapy is contraindicated in cases of severe cytomegalovirus disease. If there are signs of acute cytomegalovirus infection (laboratory changes in the complete blood count: anemia, thrombocytopenia, leukopenia, neutropenia, elevated transaminases, direct hyperbilirubinemia), pathological indices in the cerebrospinal fluid (increased protein, pleocytosis), diagnostic titer of CMV DNA determined in any biological material, clinical signs such as jaundice, petechial rash, hepato- and splenomegaly, and other signs [41]) in patients with 3 or more *SMN2* gene copies before administration of onasemnogene abeparvovec, specific antiviral therapy with oral valganciclovir or intravenous ganciclovir (in case of decreased tolerance to enteric load) is required until the CMV DNA titer decreases below the diagnostic value. It is also necessary to examine the breast milk for the presence of a diagnostic titer of CMV DNA and, if it is detected, decide on the need to treat the mother and temporarily stop breastfeeding. Onasemnogene abeparvovec should be infused no earlier than 10 days after a negative PCR test. If there are signs of acute CMV infection in patients with 2 *SMN2* gene copies, bridge therapy or alternative pathogenetic therapy should be considered unless gene replacement therapy is possible.

6. Laboratory parameters and possible adverse events should be closely monitored in the post-infusion period in all patients who have received gene replacement therapy (OA). If the patient's clinical condition worsens due to an adverse event or negative changes in laboratory findings during the post-infusion period following gene replacement therapy, it is necessary to decide on the need for hospitalization in a multidisciplinary healthcare facility.

Background

In the post-infusion period following gene replacement therapy, glucocorticosteroid therapy is required due to the immune response to the capsid proteins of the AAV9-based vector [7]. This can lead to elevated transaminase activity or decreased platelet count. Immunomodulation with glucocorticosteroids is indicated to suppress the immune response; the dose can be adjusted in case of adverse events and changes in laboratory findings.

The most common post-infusion adverse reactions (ARs) were elevated liver enzymes (24.2%), hepatotoxicity (9.1%), vomiting (8.1%), thrombocytopenia (6.1%), elevated troponin (5.1%), and pyrexia (5.1%) [7].

Prednisolone at a dose of 1 mg/kg/day *per os* is recommended for all patients 24 hours before the OA infusion. In case of intolerance to prednisolone *per os*, parenteral administration may be considered. Following gene replacement therapy, the glucocorticosteroid therapy regimen may need to be adjusted, such as using glucocorticosteroids for a longer period or increasing the dose. In some cases, pulse therapy or a slower dose tapering may be required [7].

Then, for 30 days after the OA infusion (including the day of infusion), oral prednisolone (1 mg/kg/day) should

be continued. Over the next 28 days, the dose of systemic glucocorticosteroid should be tapered gradually; therapy with these drugs should not be abruptly discontinued. For patients with insignificant changes (clinical findings are within the normal range; total bilirubin, ALT, and AST are below 2×ULN (upper limit of normal) at the end of the 30-day period), a gradual reduction in the dose of prednisolone (or an equivalent dose of another glucocorticosteroid) is recommended, e.g., 0.5 mg/kg/day for 2 weeks, then 0.25 mg/kg/day for 2 weeks [7].

Liver function should be monitored by assessing ALT, AST, and total bilirubin for at least 3 months after OA infusion or as clinically indicated. Patients with deteriorated liver function tests and/or signs or symptoms of an acute disease should be promptly examined and closely monitored [7]. In the absence of an adequate response to therapy with a glucocorticosteroid at a dose equivalent to an oral prednisolone dose of 1 mg/kg/day, the patient should be immediately consulted by a pediatric gastroenterologist or hepatologist. In case of intolerance to glucocorticosteroids, intravenous administration may be considered.

Platelet count should be measured prior to OA infusion and carefully monitored for a significant decrease within 2 weeks after the infusion and regularly thereafter: at least once a week in the first month and once every 2 weeks in the second and third months, until the platelet count returns to baseline [7].

Cases of thrombotic microangiopathy (TMA) have been reported to occur in the post-marketing period. Typically, TMA was reported within the first 2 weeks after OA administration. TMA is characterized by thrombocytopenia and microangiopathic hemolytic anemia with high LDH levels, low haptoglobin levels, and the presence of schistocytes in the peripheral blood smear [7, 42]. Acute kidney injury has also been observed. In some cases, concurrent immune system activation (e.g., infections, vaccinations) has been recognized as a contributing factor.

Close attention to the signs and symptoms of TMA is recommended; renal function and urine output should be closely monitored, as TMA can result in life-threatening complications or death [7, 43]. In some cases, predisposing genetic factors (such as congenital complement system disorders) are detected in patients with TMA developed following gene replacement therapy, which requires additional exclusion of such causes [43].

Thrombocytopenia is a key sign of TMA. Therefore, the platelet count should be carefully monitored for a significant decrease during the first 2 weeks after the infusion and regularly thereafter, along with other signs and symptoms, such as arterial hypertension, skin and subcutaneous hemorrhages, convulsions, or decreased urine output. If these signs and symptoms occur together with thrombocytopenia, further diagnostic evaluation is required to detect hemolytic anemia and impaired renal function [7].

In case of clinical signs, symptoms, and/or laboratory findings of TMA, the patient should be immediately consulted by a pediatric hematologist and pediatric nephrologist to select TMA therapy as clinically indicated [7]. If necessary, in case of acute kidney injury and severe condition of a patient with progression of TMA signs, it is necessary to decide on the need for plasmapheresis, hemodialysis, and eculizumab therapy to reduce the manifestations of the disease and mitigate the risk of adverse outcomes [42].

In animal studies, toxicity manifested ascardiac complications was reported [7]. The clinical significance of these observations is unknown. There were cases of increased cardiac troponin I following infusion with Zolgensma. In completed clinical studies, after administration of Zolgensma, no cardiological changes of concern were noted. If clinically indicated, a cardiac test should be considered, and, if needed, a cardiologist should be consulted.

Thus, in the post-infusion period after gene replacement therapy, patients should be followed up in a local healthcare facility or at the center that performed gene replacement therapy to monitor laboratory findings and the patient's clinical condition. In the event of clinically significant adverse events following the gene replacement therapy, it is necessary to decide on the need for emergency hospitalization in a local multidisciplinary healthcare facility with the availability of multidisciplinary medical care or in the federal center that performed the gene replacement therapy infusion. The timing of medical care in case of severe ARs can be critical for the prognosis of the patient's condition.

7. It is recommended to consider switching to gene replacement therapy in patients with individual intolerance and/or adverse events to the prescribed lifelong pathogenetic therapy, severe medical conditions that make it impossible to conduct regular intrathecal injections or oral drug administrations, lack of efficacy assessed for 12 months from the start of therapy, or inability to ensure regular administration of drugs as indicated in the instruction for use (e.g., due to low adherence to the prescribed treatment).

Background

Management of SMA patients, as well as approaches to prescribing and selecting the best drug therapy, are complicated. To assess the efficacy of pathogenetic therapy, a dynamic evaluation of motor skills using various scales is required [30]. To ensure uniformity of results and misinterpretation of complex clinical cases, assessments

should be regularly performed in specialized healthcare facilities (in some cases, federal healthcare facilities) that have extensive experience in evaluating patients with various types of SMA.

Therapy switching has been studied in several articles [26, 44–46].

There are data indicating a reduction in adherence to lifelong pathogenetic therapy over time and a decrease in efficacy at the end of the interdose period with regular intrathecal injections, which can be manifested by a deterioration in motor function when assessed using various motor scales (e.g., HFMSE) [47-49]. The data demonstrate that patients with medical indications for a therapy switch may receive additional clinical benefits. However, when choosing a therapy switch strategy, the benefit-risk balance should always be carefully assessed. In the absence of objective advantages in favor of a therapy switch, this approach is not advisable.

The criteria of suboptimal response to therapy (insufficient treatment efficacy) in SMA patients are as follows [50]:

1) overall worsening of the score confirmed by two consecutive measurements using any of the following 3 scales:
   - the patient loses >2 points on the horizontal push or 1 point on other HINE scales, excluding conscious grasp;
   - the patient loses >4 points on the CHOP INTEND scale;
   - the patient loses >3 points on the HFMSE scale.
2) and/or deterioration of pulmonary function:
   - an increase in the need for respiratory support during the day and/or an increase, atypical for the patient, in the number of respiratory infections requiring inpatient treatment that cannot be explained by aspiration or lung disease.

The assessment should be performed 12 months after the initiation of therapy compared to baseline. In this case, the patient's general condition and motor status at baseline are important. Changes in the patient's condition over time should be fully reflected in the medical records.

For switching, the following time intervals are recommended: gene replacement therapy can be initiated 3–5 days after the last dose of the previous therapy with risdiplam or at least 30 days after the last dose of the previous therapy with nusinersen, based on the pharmacokinetics of these drugs.

It should be kept in mind that switching from another pathogenetic therapy to gene replacement therapy may increase the risk of adverse events, according to real-world clinical practice data.

In any case, the need to switch from another pathogenetic therapy to gene replacement therapy should be decided individually based on a collective opinion of a team of experts from several healthcare facilities, considering the benefit-risk assessment.

8. Add-on therapy of SMA currently has limitations in real-world clinical practice.

Background

Add-on therapy means use of pathogenetic therapy (risdiplam or nusinersen) after a single-dose gene replacement therapy, the effect of which, presumably, persists throughout the patient's life [51]. There is no convincing evidence of the superior efficacy and safety of add-on therapy approach for SMA compared to OA monotherapy. Therefore, add-on therapy should not be used in routine clinical practice, and specialists should adhere to the strategy of choosing monotherapy with an effective drug. There is an expert opinion that the outcomes of controlled clinical studies directly comparing the monotherapy and add-on therapy approach can be considered the gold standard for answering this unresolved issue [38].

Although some clinical studies and publications evaluated the benefits of add-on therapy approach (such as nusinersen or risdiplam after the use of onasemnogene abeparvovec), they provided no convincing evidence that the combination is superior to any individual treatment due to the lack of an adequate control group [52, 53]. All three approved drugs primarily exert their effect by increasing the production of the SMN protein, but the issue of the additional benefit of targeting motor neurons remains unresolved [38, 54]. In addition, the significant cost of treatment with disease-modifying drugs casts doubts on the cost efficiency and robustness of this strategy, especially when the cost of the drug is added to the cost of traditional medical care [55].

It should be noted that irreversible degeneration of motor neurons and muscle tissue in patients with severe symptoms is probably the most important factor for the lack of expected efficacy or phenotypic recovery, regardless of the amount of SMN protein produced, which is observed with any treatment strategy [38, 56].

9. Vaccination.

It is recommended to pay special attention to the prevention, monitoring, and treatment of infectious diseases before and after OA infusion.

Timely seasonal prevention of infections caused by respiratory syncytial virus (RSV) is recommended. If possible, the patient's immunization schedule should be adjusted due to the use of glucocorticosteroids in the pre- and post-infusion period of OA.

Background

During neonatal screening, BCG vaccination in presymptomatic SMA patients is recommended in the

first days of life, according to the national immunization schedule [57]. Gene replacement therapy, followed by corticosteroid therapy, can be performed no earlier than 2 weeks after BCG vaccination [58].

If possible, the patient's immunization schedule should be adjusted due to the use of glucocorticosteroids before and after OA infusion [7, 59]. Seasonal vaccination against RSV is recommended [7]. Patients receiving glucocorticosteroids in immunosuppressive doses (e.g., prednisolone 20 mg or 2 mg/kg body weight or another glucocorticosteroid at an equivalent dose daily for ⩾2 weeks) should not receive live vaccines, such as measles-mumps-rubella vaccine and varicella vaccine [7].

The experts recommend mandatory vaccination against RSV in children with symptomatic SMA; the timing of preventive therapy should not depend on the timing of gene replacement therapy. In presymptomatic SMA children for whom the gene replacement therapy is planned, the prevention of RSV infection is optional.

10. Intrathecal gene replacement therapy.

The efficacy and safety of intrathecal gene replacement therapy were studied in 3 clinical studies: STRONG, STEER and STRENGTH [60–62]. It was found that in both the naïve SMA population and previously treated SMA patients, this type of gene replacement therapy showed improvement/stabilization of motor skills with good tolerability. The AR incidence was low and corresponded to the expected one.

The drug is intrathecally injected, which provides a higher availability of targeted motor neurons and allows for more efficient delivery of the target SMN gene, whereas the dose administered is universal and does not depend on the patient's age or body weight.

Intrathecal gene replacement therapy expands clinical treatment options for patients with SMA, particularly school-aged patients and adults, by providing a single-dose regimen for patient groups, who previously lacked access to this type of treatment.

Consensus opinion

Based on the data from clinical studies, single-dose administration, and targeted use in the nervous tissue, intrathecal gene replacement therapy has the potential to be used in the following SMA populations with different baseline motor statuses (non-sitters, sitters, walkers):

- symptomatic and presymptomatic SMA children (over 6 months of age) and adults who have not previously received pathogenetic treatment;
- children (over 6 months of age) and adults with SMA and adverse events/intolerance to nusinersen or risdiplam;
- children (over 6 months of age) and adults with SMA and reduced efficacy or low adherence to nusinersen or risdiplam;
- children (over 6 months of age) and adults with severe progressive scoliosis limiting regular intrathecal administation of nusinersen;
- children (over 6 months of age) and adults with post-lumbar puncture syndrome, which precludes the administration of regular intrathecal injections of nusinersen;
- SMA adults taking risdiplam and planning to conceive and give birth to children.

REFERENCES

1. Decree of the Government of the Russian Federation dated June 9, 2022 No. 1510-r *"On the Purchase and Distribution of the Federal Center for Planning and Organization of Drug Provision for Citizens of the Ministry of Health of the Russian Federation of medical Equipment for extended Neonatal Screening"*. (In Russ.) Available at: http://static.government.ru/media/files/S09lzM6CAgjxKuh8hpy0FCRV1NeMgQUu.pdf
2. Akhkiamova MA, Marakhonov AV, Zabnenkova VV, et al. Genotype structure alterations in 5q SMA patients as a result of the newborn screening program implementation in the Russian Federation. *Int J Mol Sci.* 2025;26(16):7891. doi: 10.3390/ijms26167891
3. Bieniaszewska A, Sobieska M, Gajewska E. Functional and structural changes in patients with spinal muscular atrophy treated in poland during 12-month follow-up: a prospective cohort study. *J Clin Med.* 2024;13(14):4232. doi: 10.3390/jcm13144232
4. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard of care in spinal muscular atrophy. *J Child Neurol.* 2007;22(8):1027–1049. doi: 10.1177/0883073807305788
5. Papina YO, Melnik EA, Belousova ED, et al. Functional class criteria identification in patients with spinal muscular atrophy 5q. *Neuromuscular diseases*. 2024;14(4):58–70. doi: 10.17650/2222-8721-2024-14-4-58-70 EDN: EIZVHG
6. The Circle of Goodness Foundation. (Internet). (In Russ.) Available at: https://фондкругдобра.рф/перечни/перечень-категорий-детей/
7. *General characteristics of the drug Zolgensma (onasemnogen abeparvovek)*. (In Russ.) Available at: https://www.novartis.com/ru-ru/sites/novartis_ru/files/2025-10-31_131-02001-Zolgensma%20SmPC_0019_v.7.0_RUr.pdf
8. Mendell JR, Al-Zaidy S, Shell R, et al. Single-dose gene-replacement therapy for spinal muscular atrophy. *N Engl J Med.* 2017;377(18):1713–1722. doi: 10.1056/nejmoa1706198
9. Day JW, Finkel RS, Chiriboga CA, 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):284–293. doi: 10.1016/s1474-4422(21)00001-6
10. Mercuri E, Muntoni F, Baranello G, et al. Onasemnogene abeparvovec gene therapy for symptomatic infantile-onset spinal muscular atrophy type 1 (STR1VE-EU): an open-label, single-arm, multicentre, phase 3 trial. *Lancet Neurol.* 2021;20(10):832–841. doi: 10.1016/S1474-4422(21)00251-9
11. Mercuri E, Baranello G, Day JW, et al. Onasemnogene abeparvovec gene-replacement therapy (GRT) for spinal muscular atrophy type 1 (SMA1): Global pivotal phase 3 study program (STR1VE-US, STR1VE-EU, STR1VE-AP). *J Neurological Sci.* 2019;405:277–278. doi: 10.1016/j.jns.2019.10.1338
12. ClinicalTrials.gov. [Internet]. *Single-dose gene replacement therapy using for patients with spinal muscular atrophy type 1 with one or two*

SMN2 copies. Available at: https://clinicaltrials.gov/ct2/show/NCT03837184?term=onasemnogene&draw=2&rank=3
13. Strauss KA, Farrar MA, Muntoni F, et al. Onasemnogene abeparvovec for presymptomatic infants with two copies of SMN2 at risk for spinal muscular atrophy type 1: the phase III SPR1NT trial. *Nat Med*. 2022;28(7):1381–1389. doi: 10.1038/s41591-022-01866-4
14. Strauss KA, Farrar MA, Muntoni F, et al. Onasemnogene abeparvovec for presymptomatic infants with three copies of SMN2 at risk for spinal muscular atrophy: the phase III SPR1NT trial. *Nat Med*. 2022;28(7):1390–1397. doi: 10.1038/s41591-022-01867-3
15. McMillan HJ, Baranello G, Farrar MA, et al.; SMART Study Group. Safety and efficacy of IV onasemnogene abeparvovec for pediatric patients with spinal muscular atrophy: the phase 3b SMART study. *Neurology*. 2025;104(2):e210268. doi: 10.1212/WNL.0000000000210268
16. Finkel RS, Day JW, De Vivo DC, et al. RESTORE: a prospective multinational registry of patients with genetically confirmed spinal muscular atrophy: rationale and study design. *J Neuromuscul Dis*. 2020;7(2):145–52. doi: 10.3233/JND-190451
17. ClinicalTrials.gov. [Internet]. *Long-term follow-up study for patients from AVXS-101-CL-101 (START)*. Available at: https://clinicaltrials.gov/study/NCT03421977?term=LT-001&rank=5
18. ClinicalTrials.gov. [Internet]. Long-term follow-up study of patients receiving onasemnogene abeparvovec-xioi. Available at: https://clinicaltrials.gov/study/NCT04042025?term=LT-002&rank=12
19. Waldrop MA. Long-term follow-up of onasemnogene abeparvovec gene therapy for patient with spinal muscular atrophy type 1 from the START trial. Poster P278 presented at Muscular Dystrophy Association (MDA) Clinical and Scientific Conference; 2025. Available at: https://www.mdaconference.org/abstract-library/long-term-follow-up-of-onasemnogene-abeparvovec-gene-therapy-for-patients-with-spinal-muscular-atrophy-type-1-from-the-start-trial/
20. Fisenko DA, Kurenkov AL, Kuzenkova LM, et al. The efficacy of gene therapy with onasemnogene abeparvovec in spinal muscular atrophy in young patients. *L.O. Badalyan Neurological Journal*. 2025;6(1):13–25. doi: 10.46563/2686-8997-2025-6-1-13-25 EDN: YYENRS
21. Shchugareva LM, Koroleva VD, Poteshkina OV. Experience of using onasemnogen abeparvovek in children with spinal muscular atrophy 5q with 2 or 3 copies of the SMN2 gene in a multidisciplinary center in Saint Petersburg. *Neuromuscular diseases*. 2025;15(2):10–19. doi: 10.17650/2222-8721-2025-15-2-10-19 EDN: RIKVEA
22. Artemyeva SB, Papina YO, Shidlovskaya OA, et al. Experience of using gene replacement therapy with Zolgensma® (onasemnogene abeparvovec) in real clinical practice in Russia. *Neuromuscular diseases*. 2022;12(1):29–38. doi: 10.17650/2222-8721-2022-12-1-29-38 EDN: PWQXHK
23. Nevmerzhitskaya KS, Sapego EYu, Morozova DA. Short-term safety and efficacy of onasemnogene abeparvovec in 10 patients with spinal muscular atrophy: cohort study. *Current pediatrics*. 2021;20(6s):589–594. doi: 10.15690/vsp.v20i6S.2367 EDN: FILUPQ
24. Weiß C, Becker LL, Friese J, et al. Efficacy and safety of gene therapy with onasemnogene abeparvovec in children with spinal muscular atrophy in the D-A-CH-region: a population-based observational study. *Lancet Reg Health Eur*. 2024;47:101092. doi: 10.1016/j.lanepe.2024.101092
25. Stettner GM, Hasselmann O, Tscherter A, et al. Treatment of spinal muscular atrophy with onasemnogene abeparvovec in Switzerland: a prospective observational case series study. *BMC Neurol*. 2023;23(1):88. doi: 10.1186/s12883-023-03133-6
26. Pane M, Berti B, Capasso A, et al. Onasemnogene abeparvovec in spinal muscular atrophy: predictors of efficacy and safety in naïve patients with spinal muscular atrophy and following switch from other therapies. *EClinicalMedicine*. 2023;59:101997. doi: 10.1016/j.eclinm.2023.101997
27. Cooper K, Nalbant G, Sutton A, et al. Systematic review of presymptomatic treatment for spinal muscular atrophy. *Int J Neonatal Screen*. 2024;10(3):56. doi: 10.3390/ijns10030056
28. Brown SM, Ajjarapu AS, Ramachandra D, et al. Onasemnogene-abeparvovec administration to premature infants with spinal muscular atrophy. *Ann Clin Transl Neurol*. 2024;11(11):3042–3046. doi: 10.1002/acn3.52213
29. Calucho M, Bernal S, Alías L, et al. Correlation between SMA type and SMN2 copy number revisited: an analysis of 625 unrelated Spanish patients and a compilation of 2834 reported cases. *Neuromuscul Disord*. 2018;28(3):208–215. doi: 10.1016/j.nmd.2018.01.003
30. Ministry of Health of the Russian Federation. (Internet). Clinical recommendations. G12.0, G12.1. *Proximal spinal muscular atrophy 5q*. Age group: children. 2023. Available at: https://cr.minzdrav.gov.ru/preview-cr/593_3?ysclid=md3jh81tz8807577555
31. Aragon-Gawinska K, Mouraux C, Dangouloff T, Servais L. Spinal muscular atrophy treatment in patients identified by newborn screening: a systematic review. *Genes (Basel)*. 2023;14(7):1377. doi: 10.3390/genes14071377
32. Tizzano EF, Quijano-Roy S, Servais L, et al.; RESTORE Study Group. Outcomes for patients in the RESTORE registry with spinal muscular atrophy and four or more SMN2 gene copies treated with onasemnogene abeparvovec. *Eur J Paediatr Neurol*. 2024;53:18–24. doi: 10.1016/j.ejpn.2024.08.006
33. Lamadrid-González J, Castellar-Leones S, Contreras-Velásquez JC, Bermúdez V. SMN2 copy number association with spinal muscular atrophy severity: insights from colombian patients. *J Clin Med*. 2024;13(21):6402. doi: 10.3390/jcm13216402
34. Avxentyev NA, Makarov AS, Germanenko OYu, et al. Pharmacoeconomic evaluation of spinal muscular atrophy therapy in patients with four SMN2 copies diagnosed through newborn screening. *S.S. Korsakov Journal of Neurology and Psychiatry*. 2025;125(12):188–199. doi: 10.17116/jnevro2025125121188 EDN: ARLQSA
35. Seroprevalence and half-life of pre-existing anti-adeno-associated virus serotype 9 (AAV9) antibodies in neonates. Poster N 45 presented at MDA 2023 Congress. Available at: https://www.mdaconference.org/abstract-library/seroprevalence-and-half-life-of-pre-existing-anti-adeno-associated-virus-serotype-9-aav9-antibodies-in-neonates/
36. Van Olden RW, Lo Bianco C, Dilly KW, et al. Adeno-associated virus serotype 9 antibodies in neonates and young children: seroprevalence and kinetics. *Mol Ther Methods Clin Dev*. 2024;32(4):101344, ISSN 2329-0501. doi: 10.1016/j.omtm.2024.101344
37. Day JW, Finkel RS, Mercuri E, et al. Adeno-associated virus serotype 9 antibodies in patients screened for treatment with onasemnogene abeparvovec. *Mol Ther Methods Clin Dev*. 2021;21:76–82. doi: 10.1016/j.omtm.2021.02.014
38. Kirschner J, Bernert G, Butoianu N, et al. 2024 update: European consensus statement on gene therapy for spinal muscular atrophy. *Eur J Paediatr Neurol*. 2024;51:73–78. doi: 10.1016/j.ejpn.2024.06.001
39. Kichula EA, Proud CM, Farrar MA, et al. Expert recommendations and clinical considerations in the use of onasemnogene abeparvovec gene therapy for spinal muscular atrophy. *Muscle Nerve*. 2021;64(4):413–427. doi: 10.1002/mus.27363
40. Volodin NN, Degtyarev DN, Degtyareva AV, et al. Management tactics of full-term and premature newborns with indirect hyperbilirubinemia (guideline). *Neonatology: news, opinions, training*. 2017;(2):113–126. (In Russ.). EDN: ZFQUWV
41. Balashova EN, Vasiliev VV, Weinstein NP, et al. Congenital cytomegalovirus infection (guideline). *Neonatology: news, opinions, training*. 2023;11(4):68–87. doi: 10.33029/2308-2402-2023-11-4-68-87 EDN: SMQCPI
42. Chand D, Zaidman C, Arya K, et al. Thrombotic microangiopathy following onasemnogene abeparvovec for spinal muscular atrophy: a case series. *J Pediatr*. 2021;231:265–268. doi: 10.1016/j.jpeds.2020.11.054
43. Servais L, Horton R, Saade D, et al. 261st ENMC International Workshop: management of safety issues arising following AAV gene therapy. 17th-19th June 2022, Hoofddorp, The Netherlands. *Neuromuscul Disord*. 2023;33(11):884–896. doi: 10.1016/j.nmd.2023.09.008
44. Belančić A, Strbad T, Štiglić MK, et al. Switching from nusinersen to risdiplam: a croatian real-world experience on effectiveness and safety. *J Pers Med*. 2024;14:244. doi: 10.3390/jpm14030244

45. Weiß C, Ziegler A, Becker LL, et al. Gene replacement therapy with onasemnogene abeparvovec in children with spinal muscular atrophy aged 24 months or younger and bodyweight up to 15 kg: an observational cohort study. *Lancet Child Adolesc Health*. 2022;6(1):17–27. doi: 10.1016/S2352-4642(21)00287-X
46. Powell JC, Meiling JB, Cartwright MS. A case series evaluating patient perceptions after switching from nusinersen to risdiplam for spinal muscular atrophy. *Muscle Nerve*. 2024;69(2):179–184. doi: 10.1002/mus.28015
47. Osmanovic A, Schreiber-Katz O, Petri S. Nusinersen wearing-off in adult 5q-spinal muscular atrophy patients. *Brain Sci*. 2021;11:367. doi: 10.3390/11030367
48. Fox D, To TM, Seetasith A, et al. Adherence and persistence to nusinersen for spinal muscular atrophy: a US claims-based analysis. *Adv Ther*. 2023;40(3):903–919. doi: 10.1007/s12325-022-02376-y
49. Patel A, Toro W, Yang M, et al. Risdiplam utilization, adherence, and associated health care costs for patients with spinal muscular atrophy: a United States retrospective claims database analysis. *Orphanet J Rare Dis*. 2024;19(1):494. doi: 10.1186/s13023-024-03399-0
50. Consensus on gene replacement therapy for the treatment for spinal muscular atrophy (release № 2). *L.O. Badalyan Neurological Journal*. 2023;4(2):64–73. doi: 10.46563/2686-8997-2023-4-2-64-73 EDN: CGPZYN
51. Proud CM, Mercuri E, Finkel RS, et al. Combination disease-modifying treatment in spinal muscular atrophy: a proposed classification. *Ann Clin Transl Neurol*. 2023;10(11):2155–2160. doi: 10.1002/acn3.51889
52. Chiriboga CA, Bruno C, Duong T, et al. Risdiplam in patients previously treated with other therapies for spinal muscular atrophy: an interim analysis from the JEWELFISH study. *Neurol Ther*. 2023;12(2):543–557. doi: 10.1007/s40120-023-00444-1
53. Harada Y, Rao VK, Arya K, et al., Combination molecular therapies for type 1 spinal muscular atrophy. *Muscle Nerve*. 2020;62(4):550–554. doi: 10.1002/mus.27034
54. Schorling DC, Pechmann A, Kirschner J. Advances in treatment of spinal muscular atrophy: new phenotypes, new challenges, new implications for care. *J Neuromuscul Dis*. 2020;7(1):1–13. doi: 10.3233/JND-190424
55. Dangouloff T, Hiligsmann M, Deconinck N, et al. Financial cost and quality of life of patients with spinal muscular atrophy identified by symptoms or newborn screening. *Dev Med Child Neurol*. 2023;65(1):67–77. doi: 10.1111/dmcn.15286
56. Kirschner J, Butoianu N, Goemans N, et al. European ad-hoc consensus statement on gene replacement therapy for spinal muscular atrophy. *Eur J Paediatr Neurol*. 2020;28:38–43. doi: 10.1016/j.ejpn.2020.07.001
57. Order of the Ministry of Health of the Russian Federation No. 1122n *"On approval of the national calendar of preventive vaccinations, the calendar of preventive vaccinations for epidemic indications and the procedure for preventive vaccinations"*. (In Russ.). Available at: http://publication.pravo.gov.ru/Document/View/0001202112200070
58. Kotulska K, Jozwiak S, Jedrzejowska M, et al. Newborn screening and gene therapy in SMA: challenges related to vaccinations. *Front Neurol*. 2022;13:890860. doi: 10.3389/fneur.2022.890860
59. Skorikova MS, Vlodavets DV. General principles of vaccination of patients with neuromuscular diseases. *Neuromuscular diseases*. 2023;13(3):40–47. doi: 10.17650/2222-8721-2023-13-3-40-47 EDN: GSPGDZ
60. Finkel RS, Darras BT, Mendell JR, et al. Intrathecal onasemnogene abeparvovec for sitting, nonambulatory patients with spinal muscular atrophy: phase I ascending-dose study (STRONG). *J Neuromuscul Dis*. 2023;10(3):389–404. doi: 10.3233/JND-221560
61. Proud CM, Vũ DC, Wilmshurst JM, et al.; STEER Study Group. Intrathecal onasemnogene abeparvovec in treatment-naive patients with spinal muscular atrophy: a phase 3, randomized controlled trial. *Nat Med*. 2026;32(2):481–487. doi: 10.1038/s41591-025-04103-w
62. Kwon JM, Munell F, Le Goff L, et al. Intrathecal onasemnogene abeparvovec for treatment-experienced patients with spinal muscular atrophy: a phase 3b, open-label trial. *Nat Med*. 2026;32(2):488–493. doi: 10.1038/s41591-025-04119-2

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