Guidance for
Appealing a Denial

zolgensma®
(onasemnogene abeparvovec-xioi)
suspension for intravenous infusion

If treatment with ZOLGENSMA® (onasemnogene abeparvovec-xioi) is denied by your patient's health plan, you may appeal this decision by phone or letter. You may also request a peer-to-peer review with an appropriate specialist. The following suggestions may assist you with initiating an appeal:

1. **Call your Novartis Gene Therapies Regional Account Associate Director (RAAD) to discuss specific aspects of the published health plan policy. They can assist you in crafting a tailored and more effective response based on the reason stated in the denial letter.**
   - Some health plans may have a designated contact for ZOLGENSMA treatment requests. Your Novartis Gene Therapies RAAD can provide you with the appropriate contact person or department, if applicable

2. **Gather the necessary information:**
   - Patient-specific denial letter
   - Health plan policy as published or communicated
   - Patient's clinical chart data, including lab test results, diagnosis confirmation, documentation of pulmonary and motor function, disease progression, and nutritional status, and swallowing evaluation
   - ZOLGENSMA product information and relevant supporting clinical literature

3. **Include the following key elements in your appeals call or letter:**
   - Confirm if reason for denial is based on a health plan decision that ZOLGENSMA is not medically necessary, or due to lack of health plan approval processes for gene therapies
   - Reiterate the request for treatment, and carefully highlight the key reasons for denial and address them with specific clinical rationale. Be as detailed as possible when addressing each concern stated in the denial letter
   - Communicate the importance of treating early to prevent future motor neuron loss. If relevant, describe the natural history of spinal muscular atrophy (SMA) Type 1
   - Request a decision be made within 72 hours due to clinical urgency
   - Request a peer-to-peer review with a specialist, such as a neuromuscular or pediatric neurologist familiar with SMA, for further discussion and clarification

4. **If appealing by letter, attach a copy of the denial letter to the appeal letter before sending. You may wish to include a list of supporting clinical literature and any additional resources you believe would be appropriate.**

5. **For additional suggestions, you may wish to refer to *Guidance for Writing a Letter of Medical Necessity* in this Reimbursement Resource Binder.**

*Your **Novartis Gene Therapies RAAD** is available to assist with any questions you may have about the denial process and initiating an appeal. You may also contact the OneGene Program® at 855-441-GENE (4363) to assist you in evaluating your patient's benefits.*

Indication and Important Safety Information

zolgensma®
(onasemnogene abeparvovec-xioi)
suspension for intravenous infusion

Indication

ZOLGENSMA is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene.

Limitations of Use

The safety and effectiveness of repeat administration or the use in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) has not been evaluated with ZOLGENSMA.

Important Safety Information

BOXED WARNING: Acute Serious Liver Injury and Acute Liver Failure

Acute serious liver injury, acute liver failure, and elevated aminotransferases can occur with ZOLGENSMA. Patients with preexisting liver impairment may be at higher risk. Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing (e.g., hepatic aminotransferases [aspartate aminotransferase (AST) and alanine aminotransferase (ALT)], total bilirubin, and prothrombin time). Administer a systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion.

WARNINGS AND PRECAUTIONS

Thrombocytopenia
Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first two weeks after ZOLGENSMA infusion. Monitor platelet counts before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards.

Thrombotic Microangiopathy
Cases of thrombotic microangiopathy (TMA) were reported approximately 1 week after ZOLGENSMA infusion. Obtain baseline creatinine and complete blood count before ZOLGENSMA infusion. Following infusion, monitor for thrombocytopenia as well as other signs and symptoms of TMA. Consult a pediatric hematologist and/or pediatric nephrologist immediately to manage if clinically indicated.

Elevated Troponin-I
Increases in cardiac troponin-I levels were observed following ZOLGENSMA infusion. Monitor troponin-I before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards.

ADVERSE REACTIONS

The most commonly observed adverse reactions (incidence ≥5%) in clinical studies were elevated aminotransferases and vomiting.

Please see accompanying Full Prescribing Information.

NOVARTIS

Bannockburn, IL 60015

11/2021 US-AVXS101-19-0052 V3