Potential Health Plan Prior Authorization Criteria Guide

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

ZOLGENSMA® (onasemnogene abeparvovec-xioi) is covered by most health plans. However, coverage may vary from patient to patient. Obtaining approval for ZOLGENSMA can be a complex and lengthy process. Below is a list of prior authorization (PA) criteria to consider when obtaining approval for ZOLGENSMA. This guide is intended to help prevent coverage delays and ensure your patients get started on ZOLGENSMA as quickly as possible.

Coverage criteria for some health plans may be less restrictive than others. However, specific criteria apply for all health plans. To help you navigate the PA process, be sure to review the PA guidelines on the insurer's website or contact Novartis Patient Support™ at **1-855-441-GENE (4363), Monday-Friday (8 AM to 8 PM ET)**, to help guide you based on your patient's individual health plan and its benefit structure.

Important PA requirements to consider when obtaining ZOLGENSMA:

☑ Confirmation of spinal muscular atrophy (SMA) diagnosis via genetic testing

- Before ZOLGENSMA can be given, genetic testing is required to confirm a diagnosis of SMA. Lab testing must be inclusive of all requirements and all lab work must be submitted together. Coverage may be denied if confirmatory testing is inconclusive

☑ *Survival motor neuron 2* (*SMN2*) gene copy number documentation

- Depending upon the health plan's policy for ZOLGENSMA, initial approval may be limited to patients with a certain number of copies of the *SMN2* gene. However, the number of copies of the *SMN2* gene is not always indicative of SMA type or the severity of the disease
- Prior to submitting the PA, it is essential to determine the accurate and definite *SMN2* copy number

☑ Pre-screening results from an Anti–Adeno-Associated Virus 9 (AAV9) Antibody Test

- Ensure that your patients' anti-AAV9 antibody levels are within the normal range after they have been tested*
- Consider retesting anti-AAV9 antibody levels if the Anti-AAV9 Antibody Test results report indicates your patient has elevated results. You may retest at any time by reaching out to gtx.labkitrequest@novartis.com to request a Novartis Laboratory Testing Program Specimen Collection Kit

☑ Documented patient weight

- Determine patient weight in kilograms, as ZOLGENSMA dosing is weight-based and needs to be recorded on the ZOLGENSMA order form

☑ Patient history

- Documentation of onset of clinical signs and symptoms of SMA, results of motor function testing using established neuromuscular functioning tests such as the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND), and swallowing evaluation are all required by most health plans and are essential to getting your patients approved for ZOLGENSMA

*Levels may vary between lab partners.

To prevent coverage delays, ensure that you have all the necessary and up-to-date information required by the health plan before you submit a PA.

*Please contact Novartis Patient Support at **1-855-441-GENE (4363), Monday-Friday (8 AM to 8 PM ET)**, for any plan-specific PA criteria questions*

Please see Indication and Important Safety Information on the next page and click here for Full Prescribing Information, including Boxed WARNING for Serious Liver Injury and Acute Liver Failure.

Indication and Important Safety Information

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

INDICATION

ZOLGENSMA is an adeno-associated virus (AAV) 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 (eg, complete paralysis of limbs, permanent ventilator dependence) has not been evaluated with ZOLGENSMA.

IMPORTANT SAFETY INFORMATION

BOXED WARNING: Serious Liver Injury and Acute Liver Failure

Cases of acute liver failure with fatal outcomes have been reported. Acute serious liver injury, acute liver failure, and elevated aminotransferases can also 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. Administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion, and at other times as clinically indicated. If acute serious liver injury or acute liver failure is suspected, promptly consult a pediatric gastroenterologist or hepatologist.

WARNINGS AND PRECAUTIONS

Systemic Immune Response

Patients with underlying active infection, either acute or chronic uncontrolled, could be at an increased risk of serious systemic immune response. Administer ZOLGENSMA to patients who are clinically stable in their overall health status (eg, hydration and nutritional status, absence of infection). Postpone ZOLGENSMA in patients with infections until the infection has resolved and the patient is clinically stable.

Thrombocytopenia

Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first 2 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 to occur generally within the first 2 weeks after ZOLGENSMA infusion. TMA can result in life-threatening or fatal outcomes. Obtain baseline creatinine and complete blood count before ZOLGENSMA infusion. Following infusion, monitor platelet counts closely as well as other signs and symptoms of TMA. Consult a pediatric hematologist and/or pediatric nephrologist immediately to manage as clinically indicated.

Elevated Troponin I

Increases in cardiac troponin I levels have occurred following ZOLGENSMA infusion. Consider cardiac evaluation after ZOLGENSMA infusion and consult a cardiologist as needed.

AAV Vector Integration and Risk of Tumorigenicity

There is a theoretical risk of tumorigenicity due to integration of AAV vector DNA into the genome. Cases of tumor have been reported in patients who received ZOLGENSMA post-approval; a causal relationship has not been established based on tumor analysis. In some cases, limited information was available. Report cases of tumor development in patients who received ZOLGENSMA to Novartis Gene Therapies, Inc. at 1-833-828-3947.

Infusion-Related Reactions

Infusion-related reactions, including hypersensitivity reactions and anaphylaxis, have occurred with ZOLGENSMA infusion. Signs and symptoms may include rash, urticaria, vomiting, dyspnea, respiratory symptoms, and/or alterations in heart rate and blood pressure. Monitor patients during and after treatment with ZOLGENSMA. If an infusion-related reaction occurs, interrupt ZOLGENSMA infusion and administer supportive treatment to manage the infusion-related reaction as appropriate. Infusion of ZOLGENSMA may be resumed based on clinical assessment.

ADVERSE REACTIONS

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

Please click here for Full Prescribing Information.

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Novartis Pharmaceuticals Corporation
East Hanover, New Jersey 07936-1080