Potential Health Plan
Prior Authorization (PA) Criteria

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

Most plans are expected to have a coverage policy in place for ZOLGENSMA® (onasemnogene abeparvovec-xioi), or are expected to cover treatment by medical necessity only. Some health plans may designate a specific contact for ZOLGENSMA treatment requests. Your Novartis Gene Therapies Regional Account Associate Director (RAAD) can provide you with the appropriate contact person or department, if applicable.

Coverage criteria for some plans may be less restrictive than others. However, specific criteria apply for all plans. **Your Novartis Gene Therapies RAAD will help you determine how to access ZOLGENSMA based on your patient's individual health plan and its benefit structure.**

The following PA requirements apply for most health plans:

- ☑ **Genetic confirmation of spinal muscular atrophy (SMA)**
  - Diagnostic testing must confirm missing or mutated survival motor neuron 1 (*SMN1*) gene
- ☑ **Documented patient weight**
  - Weight must be recorded on the ZOLGENSMA Prescription Form
- ☑ ***SMN2* copy number**
  - Approval of ZOLGENSMA may be limited to patients with a lower number of copies of the *SMN2* gene. Additional documentation of medical necessity may be required for patients with more than 2 copies of *SMN2*
- ☑ **Prescreening results from adeno-associated virus 9 (AAV9) Antibody Test**
- ☑ **Documentation that the prescriber is a specialist (ie, pediatric neurologist, neuromuscular specialist or neurologist)**

In addition to the above criteria, some health plans may also require the following criteria:

- 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

*Please contact your **Novartis Gene Therapies RAAD** for any plan-specific PA criteria questions.*

Please see Indication and Important Safety Information, and accompanying Full Prescribing Information including Boxed Warning for Acute 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 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

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