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

ZOLGENSMA® (onasemnogene abeparvovec-xioi) Letter of Appeal Guidance for a Self-Insured Employer Health Plan

How to Ask Your Employer to Reconsider a Denial

If your employer's health plan says it won't pay for ZOLGENSMA after your child's/dependent's doctor has prescribed it, you have the right to ask your employer to review that decision. This is called an "appeal." Appeals can take time, and starting treatment as early as possible is critical for the best outcomes. This guide provides useful tips to help you and your child's/dependent's doctor write a strong appeal to your employer or health plan administrator if treatment with ZOLGENSMA is denied.

You can find a sample appeal letter on page 2. It shows the kind of information your employer may need.
When preparing your appeal:

1. **Contact your employer's benefits manager** or the contact listed in your employee handbook to ensure the appeal letter is directed to the appropriate person
2. **Contact your child's/dependent's doctor** with any questions and to request the necessary clinical information for the appeal letter
3. **Importantly**, please remember that sending an appeal doesn't guarantee your employer will say yes to covering treatment for your child/dependent

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How Your Employer Can Help With Your Child's/Dependent's Appeal

Your employer helps provide your health insurance and may be able to support ZOLGENSMA coverage. Even if another company, called a third-party administrator (TPA), handles your insurance claims, your employer can still speak up for you and your child/dependent.

Here Are Some Ways Your Employer Can Help:

- **Review the coverage criteria for ZOLGENSMA** to better understand why your child/dependent was denied
- **Request an exception or approval** based on how serious and urgent your child's/dependent's spinal muscular atrophy (SMA) diagnosis is
- **Explore who else should be part of the conversation** about the denial and what they can do to help support access to treatment

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How to Complete Your Letter of Appeal

Your child's or dependent's doctor can help guide you through the appeal process, answer any questions, and provide the medical details needed to support your case.

What Your Letter of Appeal Should Include:

- A short summary of your child's/dependent's diagnosis and why it's the most appropriate treatment for your child/dependent (include your child's/dependent's medical history and the doctor's advice for treatment)
- Basic information about SMA, how ZOLGENSMA works, and why ZOLGENSMA is right for your child/dependent
- An explanation of why treatment is urgent
- A clear request for coverage, asking your employer to approve the treatment

You may need to contact your benefits manager to confirm the submission process for your appeal letter. The options might include email, interoffice mail, or traditional mail. Ask which method is preferred and whether any security requirements apply to protect your child's or dependent's personal health information.

Please see Indication and Important Safety Information on page 3 and click here for full Prescribing Information.

Letter of Appeal for a Self-Insured Employer Health Plan Template

[Date]
[Name of Employer]
ATTN: [HR Department/Employee Benefits Manager]
[PO Box or Street Address]
[City], [State] [ZIP Code]

Re: [Patient Name], [DOB], [Parent/Legal Guardian Name]
Insurance Card Information: Health Plan/Payor Number: [XXX]; Group Number: [XXX]; Member/Subscriber ID: [XXX]

Dear [HR representative/Employee benefit representative],

I am writing regarding the recent denial of coverage for ZOLGENSMA® (onasemnogene abeparvovec-xioi) for my [insert child/dependent, patient name], who has been diagnosed with spinal muscular atrophy (SMA). The letter I received from [insert name of third-party administrator (TPA) or health plan] stated that coverage was denied due to [include reason as stated in the denial letter]. As a parent/caregiver, this news has been [include details on personal impact (ie, incredibly difficult).] [Include details on how SMA has impacted your child/dependent (ie, SMA is a disease that makes it harder to move, eat, and do everyday activities. Every day my child/dependent goes without treatment, more abilities are potentially lost).] I'm asking for your consideration in helping us pursue an exception so that [insert patient name] can receive ZOLGENSMA. The information below outlines the importance of this treatment, the nature of SMA, and why swift action is essential. I would be deeply grateful for your consideration and support as we navigate this challenging situation.

About ZOLGENSMA

- ZOLGENSMA is the only one-time gene replacement therapy for SMA approved for children under 2 years old with SMA. The safety and efficacy of ZOLGENSMA has been established across multiple studies[1-6]
- ZOLGENSMA is designed to treat the genetic root cause of SMA, with over 5000 patients treated globally[7,*]
- ZOLGENSMA stops the progression of the disease and helps preserve critical motor functions with an expected lifetime cost that is less than or similar to the cost of lifelong, ongoing treatments and supporting medical costs[1,8]
- To learn more about ZOLGENSMA, visit https://www.zolgensma.com

What is SMA?

SMA is a rare, devastating, genetic disease that affects the motor nerve cells in the spinal cord and impacts the muscles used for breathing, eating, crawling, and walking.[3,9-12]

Urgency to treat SMA

- Neuromuscular experts generally recommend starting care as soon as possible, especially for the most common and severest form of SMA, which can be detected through newborn screening, because the disease can progress quickly and cause lasting damage to the nerves that control movement[9,13-15]
- Children with certain types of SMA, such as type 1 (infantile-onset) or type 2, can experience early and rapid onset of symptoms starting in infancy[9,11]
- Without timely treatment, SMA can lead to serious complications, including difficulty breathing and the need for permanent ventilatory support[16]
- The disease is characterized by severe muscle weakness; a decline in respiratory, eating, and swallowing functions; and failure to achieve important motor milestones, such as sitting without support[11,12]

In summary, I am requesting a reconsideration of our health plan policy to allow coverage for ZOLGENSMA for the treatment of [insert child/dependent, patient name], to offer [include details on why your child/dependent should be treated with ZOLGENSMA (ie, him/her the best chance for a healthy future. ZOLGENSMA would offer him/her a significantly improved prognosis over the course of the disease) specify the specific motor milestones you expect your child/dependent to achieve (eg, avoidance of ventilation, ability to sit, feed, stand, crawl, walk)].

Sincerely,

[Insert your name]

This letter is provided as an example and is meant for educational purposes only. Novartis cannot guarantee insurance coverage or reimbursement. Coverage and reimbursement may vary significantly by employer, payor, plan, patient, and setting of care.
*Including clinical trials, commercially, and through managed access programs as of July 2025.

Please see Indication and Important Safety Information on page 3 and click here for full Prescribing Information.

Indication and Important Safety Information for ZOLGENSMA® (onasemnogene abeparvovec-xioi) suspension for intravenous infusion

INDICATION
What is ZOLGENSMA?

ZOLGENSMA is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA). ZOLGENSMA is given as a one-time infusion into a vein. ZOLGENSMA was not evaluated in patients with advanced SMA.

IMPORTANT SAFETY INFORMATION
What is the most important information I should know about ZOLGENSMA?

- ZOLGENSMA can increase liver enzyme levels and cause acute serious liver injury or acute liver failure which could result in death.
- Patients will receive an oral corticosteroid before and after infusion with ZOLGENSMA and will undergo regular blood tests to monitor liver function.
- Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish, if the patient misses a dose of corticosteroid or vomits it up, or if the patient experiences a decrease in alertness.

What should I watch for before, during, and after infusion with ZOLGENSMA?

- Infections before or after ZOLGENSMA infusion can lead to more serious complications. Caregivers and close contacts with the patient should follow infection prevention procedures. Contact the patient's doctor immediately if the patient experiences any signs of a possible infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.
- Decreased platelet counts could occur following infusion with ZOLGENSMA. Seek immediate medical attention if the patient experiences unexpected bleeding or bruising.
- Thrombotic microangiopathy (TMA) has been reported to generally occur within the first two weeks after ZOLGENSMA infusion. Seek immediate medical attention if the patient experiences any signs or symptoms of TMA, such as unexpected bruising or bleeding, seizures, or decreased urine output.
- There is a theoretical risk of tumor development with gene therapies such as ZOLGENSMA. Contact the patient's doctor and Novartis Gene Therapies, Inc. at 1-833-828-3947 if a tumor develops.
- Infusion-related reactions may occur during and after ZOLGENSMA infusion. Seek immediate medical evaluation if signs and symptoms of infusion-related reaction occur which may include rash, hives, vomiting, shortness of breath, respiratory symptoms, and/or changes in heart rate and blood pressure.

What do I need to know about vaccinations and ZOLGENSMA?

- Talk with the patient's doctor to decide if adjustments to the vaccination schedule are needed to accommodate treatment with a corticosteroid.
- Protection against influenza and respiratory syncytial virus (RSV) is recommended and vaccination status should be up-to-date prior to ZOLGENSMA administration. Please consult the patient's doctor.

Do I need to take precautions with the patient's bodily waste?

Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool. Use good hand hygiene when coming into direct contact with patient body waste for one month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out with regular trash.

What are the possible or likely side effects of ZOLGENSMA?

The most common side effects that occurred in patients treated with ZOLGENSMA were elevated liver enzymes and vomiting.

The safety information provided here is not comprehensive. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.

You are encouraged to report suspected side effects by contacting the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch, or Novartis Gene Therapies, Inc. at 1-833-828-3947.

Please click here for full Prescribing Information.

References: 1. ZOLGENSMA. Prescribing information. Novartis Gene Therapies, Inc. **2.** 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. **3.** 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. **4.** 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. **5.** Strauss KA, Farrar MA,

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