Medical Necessity Guidelines: Zolgensma (onasemnogene abeparvovec) Gene Therapy for Treatment of Spinal Muscular Atrophy (SMA) for Tufts Health Together, Tufts Health RITogether and Tufts Health Unify

Effective: January 1, 2021

Prior Authorization Required If REQUIRED, submit supporting clinical documentation pertinent to service request.	**Yes ☒ No ☐**

Applies to:
COMMERCIAL Products
☐Tufts Health Plan Commercial products; Fax: 617.972.9409
☐Tufts Health Freedom Plan products; Fax: 617.972.9409
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

TUFTS HEALTH PUBLIC PLANS Products
☐Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax:888.415.9055
☒Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans; Fax: 888.415.9055
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax: 857.304.6404
☒Tufts Health Unify* – OneCare Plan (a dual-eligible product); Fax: 857.304.6304
*****The MNG applies to Tufts Health Unify members unless a less restrictive LCD or NCD exists.

SENIOR Products
- Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product) **–** Refer to the Tufts Health Plan SCO Prior Authorization List
- Tufts Medicare Preferred HMO, (a Medicare Advantage product) – Refer to the Tufts Medicare Preferred HMO Prior Authorization and Inpatient Notification List

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to make sure that prior authorization has been obtained.

OVERVIEW

Zolgensma (onasemnogene abeparvovec) is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene. SMN1 gene mutation can be confirmed with newborn screening or genetic testing of symptomatic infants. Zolgensma (onasemnogene abeparvovec) is a one-time-only dose given intravenously (IV) over the course of 60 minutes. Per prescribing information, baseline clinical evaluation and testing of liver function and testing of platelet counts and troponin-I levels should be performed prior to Zolgensma (onasemnogene abeparvovec) infusion and monitored for at least 3 months following infusion.

Zolgensma (onasemnogene abeparvovec) is a Preferred product for SMA for Tufts Health Together

NOTE: Authorization will be valid for the earlier of 6 months from the date of approval or up to the date of the Member's second birthday. All criteria below must be met at time of infusion.

CLINICAL COVERAGE CRITERIA

Tufts Health Plan will cover **one** dose of Zolgensma (onasemnogene abeparvovec) **per lifetime** for the treatment of presymptomatic and symptomatic spinal muscular atrophy (SMA) for Members when prescribed by a neuromuscular specialist **and** when required documentation supports **ALL** the following criteria are met:

1. Member has -appropriate diagnosis (Type 1, 2 or 3 SMA)
2. Member is less than two years of age
3. Genetic testing confirms bi-allelic mutation in the survival motor neuron 1 (SMN1) gene (e.g. SMN1 homozygous gene deletion or mutation or compound heterozygous mutation)

4. Genetic testing confirms the member has two or three copies of the survival motor neuron 2 (SMN 2) gene
5. Pre-treatment testing confirms baseline anti-adeno-associated virus serotype 9 (anti-AAV9) antibody titers ≤ 1:50
6. Member does not have evidence of complete paralysis of limbs
7. Member does not have evidence of permanent ventilator dependence (defined as any of the following: member has an endotracheal tube, member has a tracheotomy tube, member had at least 14 days of continuous respiratory assistance for at least 16 hours per day) at the time the requested agent is to be administered

LIMITATIONS

Tufts Health Plan will not cover:

- Repeat infusion of Zolgensma (onasemnogene abeparvovec) as the safety and effectiveness of repeat administration has not been evaluated and FDA approval has not been given. This includes previous infusion(s) administered as part of a clinical trial or administered when Member was covered under a different health plan
- Members with SMA Type 4. SMA type 4 is typically considered late-onset or adult-onset SMA. Members less than two years of age should not be diagnosed with SMA type 4.

CODES

The following CPT code(s) require prior authorization when requested for Zolgensma (onasemnogene abeparvovec):

Table 1: CPT Codes

CPT Code	Description
J3399	Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5x10

REFERENCES

1. Table 76: Neuromuscular Agents – Duchenne Muscular Dystrophy and Spinal Muscular Atrophy accessed on 6/3/20 @ masshealthdruglist.ehs.state.ma.us/MHDL/pubtheradetail.do?id=1387&drugId=7273
2. U.S. Food and Drug Administration. FDA approves innovative gene therapy to treat pediatric patients with spinal muscular atrophy, a rare disease and leading genetic cause of infant mortality. May 2019. Available at fda.gov/news-events/press announcements/fda approves-innovative-gene-therapy-treat-pediatric-patients-spinal-muscular-atrophy-rare-disease. Accessed June 11, 2020.
3. United States Food and Drug Administration. Package Insert-ZOLGENSMA® (onasemnogene abeparvovec-xioi). Available at fda.gov. Accessed June 10, 2020.
4. Prior TW, Snyder PJ, Rink BD, et al. Newborn and carrier screening for spinal muscular atrophy. Am J Med Genet A. 2010 Jul;152A (7):1608-16.
5. Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type 1. Available at clinicaltrials.gov/ct2/show/NCT02122952?term=zolgensma&rank=8 Accessed June 13, 2020.
6. Long-Term Follow-up Study for Patients From AVXS-101-CL-101 (START). Available at clinicaltrials.gov/ct2/show/NCT03421977?term=zolgensma&rank=3 Accessed June, 13, 2020.

APPROVAL HISTORY

July 15, 2020: Reviewed by the Integrated Medical Policy Advisory Committee (IMPAC)

Subsequent endorsement date(s) and changes made:

- December 16, 2020: Reviewed at IMPAC. Coverage criteria and initial authorization period updated to align with MassHealth Evaluation Criteria for Approval of Zolgensma, effective January 1, 2021.
- February 24, 2021: Overview language added. Zolgensma is a Preferred product for Together members.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Medical Necessity Guidelines are developed to determine coverage for benefits, and are published to provide a better understanding of the basis upon which coverage decisions are made. We make coverage decisions using these guidelines, along with the Member's benefit document, and in coordination with

the Member's physician(s) on a case-by-case basis considering the individual Member's health care needs.

Medical Necessity Guidelines are developed for selected therapeutic or diagnostic services found to be safe and proven effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in our service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guideline is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.