Harvard Pilgrim Health Care | TUFTS Health Plan

Pharmacy Medical Necessity Guidelines: **Evrysdi® (risdiplam)**

Effective: March 10, 2026

Guideline Type	☒ Prior Authorization ☐ Non-Formulary ☐ Step-Therapy ☐ Administrative

Applies to:

Commercial Products

☒ Harvard Pilgrim Health Care Commercial products; Fax: 617-673-0988

☒ Tufts Health Plan Commercial products; Fax: 617-673-0988

Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax: 617-673-0988

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

Overview

Food and Drug Administration – Approved Indications

Evrysdi (risdiplam) is a survival of motor neuron 2 (SMN2) splicing modifier indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

Clinical Guideline Coverage Criteria

The plan may authorize coverage of Evrysdi for Members when **ALL** of the following criteria are met:

Initial Authorization Criteria

1. Documented diagnosis of spinal muscular atrophy type 1, 2, or 3 confirmed by molecular genetic testing of any of the following: SMN1 homozygous gene deletion, homozygous conversion mutation (i.e., SMN1 gene conversion to SMN2 gene), or compound heterozygote mutation

AND

2. Prescribed by or in consultation with a board-certified neurologist with special qualification in child neurology and treatment of spinal muscular atrophy

AND

3. Documentation of baseline (pre-treatment) motor function skills as measured by one (1) of the following:
    a. Hammersmith Infant Neurological Exam Part 2 (HINE-2)
    b. Hammersmith Functional Motor Scale Expanded (HFMSE)
    c. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND)

AND

4. Documentation the patient is not using Evrysdi in combination with Spinraza

AND

5. Documentation of **one (1)** of the following:
    a. The patient has not previously received gene replacement therapy for the treatment of spinal muscular atrophy
    b. Both of the following:
        i. The patient has previously received gene replacement therapy for the treatment of spinal muscular atrophy
        ii. The patient has experienced a decline in clinical status since receipt of gene replacement therapy

AND

6. Documentation the patient is not ventilation dependent (defined as using a ventilator 16 hours or more a day)

Reauthorization Criteria

1. Documented diagnosis of spinal muscular atrophy type 1, 2, or 3 confirmed by molecular genetic testing

AND

2. Prescribed by or in consultation with a board-certified neurologist with special qualification in child neurology and treatment

of spinal muscular atrophy

AND

3. Documentation the patient is not using Evrysdi in combination with Spinraza

AND

4. Documentation of **one (1)** of the following:
    a. The patient has not previously received gene replacement therapy for the treatment of spinal muscular atrophy
    b. Both of the following:
        i. The patient has previously received gene replacement therapy for the treatment of spinal muscular atrophy
        ii. The patient has experienced a decline in clinical status since receipt of gene replacement therapy

AND

5. Documentation the patient is not ventilation dependent (defined as using a ventilator 16 hours or more a day)

AND

6. Documentation of disease stabilization or clinical improvement of spinal muscular atrophy symptoms as demonstrated by at least **one (1)** of the following assessments:
    a. **Both** of the following with respect to HINE-2:
        i. **One (1)** of the following:
            1. Patient exhibited improvement or maintenance of previous improvement of at least a 2-point (or maximal score) increase in ability to kick
            2. Patient exhibited improvement or maintenance of previous improvement of at least a 1-point (or maximal score) increase in any other HINE-2 milestone (e.g., head control, rolling, sitting, crawling, standing, or walking) excluding voluntary grasp
        ii. **One (1)** of the following:
            1. Patient exhibited improvement or maintenance of previous improvement in more HINE-2 motor milestones than worsening (net positive improvement)
            2. Patient achieved and maintained any new motor milestones when they would otherwise be unexpected to do so (e.g., sit or stand unassisted, walk)
    b. **One (1)** of the following with respect to HFMSE:
        i. Patient exhibited improvement or maintenance of previous improvement of at least a 3-point increase in score
        ii. Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so
    c. **One (1)** of the following with respect to CHOP-INTEND
        i. Patient exhibited improvement or maintenance of previous improvement of at least a 4-point increase in score
        ii. Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so

Limitations

1. Coverage of Evrysdi (risdiplam) for SMA type 4 will not be authorized.
2. Authorizations will be provided for 12 months.
3. Members new to the plan stable on Evrysdi should be reviewed against Initial Authorization Criteria. Members who have already initiated the requested medication using samples, free goods, similar offerings, or self-paying for the medication directly do not qualify for an established clinical response and should be reviewed against initial approval criteria.
   For treatment-experienced Members, Providers must submit documentation of a physical assessment, motor function-based testing, and need for medical intervention related to SMA symptoms, relative to baseline.
4. If gene therapy is subsequently administered, Evrysdi authorization will be terminated, and the Member must reapply for coverage.
5. For a non-formulary medication request, please refer to the Pharmacy Medical Necessity Guidelines for Formulary Exceptions and submit a formulary exception request to the plan as indicated.

Codes

None

References

1. Evrysdi (risdiplam) [prescribing information]. South San Francisco, CA: Genentech, Inc.; February 2026.
2. Baranello G, et al. Risdiplam in type 1 spinal muscular atrophy. N Engl J Med. 2021;384:915-23.
3. Chiriboga C et al. Risdiplam in patients previously treated with other therapies for spinal muscular atrophy: an interim analysis from the JEWELFISH study. Neurol Ther. 2023 Apr;12(2):543-557.

4. High KA, MD, Roncarolo, MG, MD. Gene Therapy. N Engl J Med 2019;381:455-64.
5. Markowitz JA et al. Spinal Muscular Atrophy: A clinical and research update. Pediatric Neurology. 2012;46:1-12.
6. Masson R, et al. Safety and efficacy of risdiplam in patients with type 1 spinal muscular atrophy (FIREFISH part 2): secondary analyses from an open-label trial. Lancet Neurol. 2022 Dec;21(12):1110-1119.
7. Mendell JR, Al-Zaidy S, Shell R, et al. Single-dose gene-replacement therapy for spinal muscular atrophy. N Engl J Med. 2017; 377:1713-22.
8. Mercuri E, et al. Safety and efficacy of once-daily risdiplam in type 2 and non-ambulant type 3 spinal muscular atrophy (SUNFISH part 2): a phase 3, double-blind, randomised, placebo-controlled trial. Lancet Neurol. 2022 Jan;21(1):42-52.
9. Mercuri E, Finkel RS, Muntoni F et al. Diagnosis and management of spinal muscular atrophy: Part 1: Recommendations for diagnosis, rehabilitation, orthopedic and nutritional care. Neuromuscul Disord. 2018; 28(2): 103-15.
10. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard of care in spinal muscular atrophy. J Child Neurol. Aug 2007;22(8):1027-49.

Approval And Revision History

September 13, 2022: Reviewed by the Pharmacy & Therapeutics Committee.

- May 9, 2023: No changes.
- May 14, 2024: Provided specific assessments to use for pre-treatment (baseline) motor function skills. Added specific requirements to establish disease stabilization or clinical improvement in Reauthorization Criteria (eff 8/1/24).
- March 11, 2025: No changes (eff 3/11/25)
- March 10, 2026: Administrative update to the limitation statement to include members initiated on the requested medication by self-paying for the medication directly do not qualify for established clinical response and should be reviewed against initial approval criteria. Administrative update to remove CareLink from Medical Necessity Guideline template.

Background, Product and Disclaimer Information

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be 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 the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy 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 Pharmacy 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 policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.