MEDICAL MUTUAL®

Drug Policy

Policy:	Evrysdi™ (risdiplam)	Initial Effective Date: 09/01/2020 Annual Review Date: 08/22/2024 Last Revised Date: 08/22/2024

Prior approval is required for some or all procedure codes listed in this Corporate Drug Policy.

OVERVIEW

The U.S. Food and Drug Administration approved Genentech's Evrysdi™ (risdiplam) for patients two months of age and older with spinal muscular atrophy (SMA) due to SMN protein deficiency resulting from mutations in chromosome 5q. It is in a new class of drugs – survivor of motor neuron 2 (SMN2) splicing modifiers. SMA is a rare genetic condition that causes increasing weakness in muscles. Evrysdi will be given as an oral solution once daily at a dose of 0.15mg/kg for children less than two months of age, 0.2mg/kg for children two months to less than 2 years of age, 0.25mg/kg for children 2 years of age and older weighing less than 20kg or 5mg for patients 2 years of age and older weighing 20kg or more.

POLICY STATEMENT

This policy involves the use of Evrysdi. Prior authorization is recommended for pharmacy benefit coverage of Evrysdi. Approval is recommended for those who meet the conditions of coverage in the **Initial Approval and Renewal Criteria, and Length of Authorization** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy.

I. Initial Approval Criteria

Coverage is provided in the following conditions:

Spinal Muscular Atrophy (SMA) †

- Patient must not have previously received treatment with SMA gene therapy (i.e., onasemnogene abeparvovec-xioi); **AND**
- Patient will not use in combination with other agents for SMA (e.g., onasemnogene abeparvovec, nusinersen, etc.); **AND**
- Patient does not have impaired hepatic function as defined by elevated laboratory values; **AND**

- Patient retains meaningful voluntary motor function (e.g. manipulate objects using upper extremities, ambulate, etc.); **AND**
- Patient must have a diagnosis of 5q spinal muscular atrophy confirmed by either homozygous deletion of the *SMN1* gene or dysfunctional mutation of the *SMN1* gene; **AND**
- Patient must have one of the following SMA phenotypes:
    - SMA I or II confirmed by genetic testing [documentation required]; **OR**
    - SMA III confirmed by genetic testing [documentation required] with symptomatic disease (i.e., impaired motor function and/or delayed motor milestones); **AND**
- Baseline documentation of one or more of the following:
    - Motor function/milestones, including but not limited to, the following validated scales: Hammersmith Infant Neurologic Exam (HINE), Hammersmith Functional Motor Scale Expanded (HFMSE), Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND), 6-minute walk test (6MWT), upper limb module (ULM), 32-Item Motor Function Measure (MFM32), Revised Upper Limb Module (RULM), etc.
    - Respiratory function tests [e.g., forced vital capacity (FVC), etc.]
    - Exacerbations necessitating hospitalization and/or antibiotic therapy for respiratory infection in the preceding year/timeframe
    - Patient weight (for patients without a gastrostomy tube)

† FDA-labeled indication(s)

II. Renewal Criteria

- Patient must not have previously received treatment with SMA gene therapy (i.e., onasemnogene abeparvovec-xioi); AND
- Absence of unacceptable toxicity which would preclude safe administration of the drug. Examples of unacceptable toxicity include the following: significant hepatic impairment **AND**
- Patient has responded to therapy compared to pretreatment baseline in one or more of the following:
    - Stability or improvement in net motor function/milestones, including but not limited to, the following validated scales: Hammersmith Infant Neurologic Exam (HINE), Hammersmith Functional Motor Scale Expanded (HFMSE), Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP

INTEND), 6-minute walk test (6MWT), upper limb module (ULM), 32-Item Motor Function Measure (MFM32), Revised Upper Limb Module (RULM), etc.

- Stability or improvement in respiratory function tests [e.g., forced vital capacity (FVC), etc.]
- Reduction in exacerbations necessitating hospitalization and/or antibiotic therapy for respiratory infection in the preceding year/timeframe
- Stable or increased patient weight (for patients without a gastrostomy tube)
- Slowed rate of decline in the aforementioned measures

III. Length of Authorization

Initial approval period: 6 months

Renewal approval period: 12 months

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Evrysdi has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. **Patient has Permanent Ventilator Dependence.** Data is needed to determine if this patient population with advanced spinal muscular atrophy would derive benefits from Evrysdi.

2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Evrysdi® oral solution [prescribing information]. South San Francisco, CA: Genentech/Roche; October 2023.
2. Arnold ES, Fischbeck KH. Spinal muscular atrophy. *Handb Clin Neurol*. 2018;148:591-601.
3. Prior TW, Leach ME, Finanger E. Spinal Muscular Atrophy. 2000 Feb 24 [Updated 2020 December 3]. In: Adam MP, Ardinger, HH, Pagon RA, et al, editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2020. Available at: https://www.ncbi.nlm.nih.gov/books/NBK1352/. Accessed on August 9, 2024.
4. Nicolau S, Waldrop MA, Connolly AM, et al. Spinal muscular atrophy. *Semin Pediatr Neurol*. 2021;37:100878.