Evrysdi (risdiplam)

NOTICE

This policy contains information which is clinical in nature. The policy is not medical advice. The information in this policy is used by Wellmark to make determinations whether medical treatment is covered under the terms of a Wellmark member's health benefit plan. Physicians and other health care providers are responsible for medical advice and treatment. If you have specific health care needs, you should consult an appropriate health care professional. If you would like to request an accessible version of this document, please contact customer service at 800-524-9242.

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the Evrysdi drug policy is to ensure appropriate selection of patients for therapy based on product labeling, clinical guidelines and clinical studies in the treatment of spinal muscular atrophy (SMA).

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the patient has no exclusions to the prescribed therapy.

FDA-Approved Indications

Evrysdi is indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

POLICY

Required Documentation:

Submission of the following information is necessary to initiate the prior authorization review:

A. Initiation of therapy:
   1. Deletion or mutation at the SMN1 allele confirmed by genetic testing
   2. Medical records (e.g. chart notes, laboratory values) of the baseline assessment for at least one of the following assessment tools (based on patient age and motor ability) to establish baseline motor ability:
      i. Hammersmith Infant Neurological Exam Part 2 (HINE-2)
      ii. Hammersmith Functional Motor Scale Expanded (HFMSE)
      iii. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND)
      iv. Motor Function Measure 32 (MFM32)
      v. Bayley Scales of Infant and Toddler Development – Third Edition (BSID-III)
   3. Medical records documenting respiratory status and need for respiratory support
B. Continuation of therapy:

1. Medical records (e.g., chart notes, laboratory values) of the most recent (within 90 days prior to continuation request) assessment by at least one of the following assessments:
   i. HINE-2
   ii. HFMSE
   iii. CHOP-INTEND
   iv. MFM32
   v. BSID-III
   vi. For patients prescribed Evrysdi due to clinical worsening after receiving gene replacement therapy (e.g., Zolgensma): Documentation of the impact of Evrysdi therapy (e.g., impact on motor milestones)

Criteria for Initial Approval

A. Evrysdi (risdiplam) may be considered **medically necessary** for the treatment of spinal muscular atrophy (SMA) when the following criteria is met:
   1. Patient has a diagnosis of SMA confirmed by genetic testing showing deletion or mutation at the SMN1 allele (examples below)
      - Homozygous gene deletion or mutation (e.g., homozygous deletion of exon 7 at locus 5q13)1,5; or
      - Compound heterozygous mutation (e.g., deletion of SMN1 exon 7[allele 1] and mutation of SMN1 [allele 2])
   2. Patient has type 1, type 2, or type 3 SMA
   3. The medication must be prescribed by or in consultation with a neurologist or neuromuscular specialist with expertise in the treatment of SMA
   4. Patient is not dependent on either of the following:
      - Invasive ventilation or tracheostomy
      - Use of non-invasive ventilation beyond use for naps and nighttime sleep
   5. Member meets one of the following criteria:
      i. Patient has not previously received gene replacement therapy for SMA (e.g., Zolgensma), or
      ii. Patient has previously received gene replacement therapy for SMA (e.g., Zolgensma) and has experienced a worsening in clinical status since receiving gene replacement therapy that demonstrates a loss of efficacy of the gene therapy as demonstrated by a decline of minimally clinical important difference from highest score achieved or baseline on one of the following exams (based on patient age, motor ability, and specific exam)
         - HINE-2: Decline of at least 2 points on kicking and 1 point on any other milestone (excluding voluntary grasp)
         - HFMSE: Decline of at least 3 points
         - CHOP-INTEND: Decline of at least 4 points
         - MFM32: Decline from baseline
         - BSID-III: Inability to sit without support for more than 5 seconds per item 22 of test
   6. Patients will not use Evrysdi and Spinraza concomitantly

Approval will be for **12 months.**

B. Evrysdi (risdiplam) is considered **investigational** for the following:
   - Use of Evrysdi (risdiplam) in patients with type 0 and 4 spinal muscular atrophy (SMA)
   - Use of Evrysdi (risdiplam) in patients with type 1,2, and 3 spinal muscular atrophy (SMA) who require permanent/invasive ventilation
   - Concomitant use of Evrysdi (risdiplam) and gene replacement therapy
   - Concomitant use of Evrysdi (risdiplam) and Spinraza (nusinersen)

Continuation of Therapy

Note: Patients who were previously established on Evrysdi and subsequently administered gene therapy must meet all initial criteria prior to re-starting therapy on Evrysdi.

A. The continued treatment of Evrysdi (risdiplam) may be considered **medically necessary** for the treatment of spinal muscular atrophy (SMA) in patients who meet the initial criteria above AND the following criteria:
   1. Submission of medical records (e.g., chart notes, laboratory values) of the most recent (within 90 days prior to continuation request) assessment documenting a positive clinical response from pretreatment baseline to Evrysdi therapy, as demonstrated by at least one of the following assessments:
      i. HINE-2
         - One of the following:
            - Patient exhibited improvement or maintenance of previous improvement of at least a 2 point (or maximal score) increase in ability to kick; *or*
            - 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; *and*
         - One of the following:
            - Patient exhibited improvement or maintenance of previous improvement in more HINE-2 motor milestones than worsening (net positive improvement); *or*
            - Patient achieved and maintained any new motor milestones when they would otherwise be unexpected to do so (e.g., sit or stand unassisted, walk)
      ii. HFMSE
         - One of the following:
            - Patient exhibited improvement or maintenance of previous improvement of at least a 3-point increase in score; *or*
            - Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so
      iii. CHOP-INTEND
         - One of the following:
            - Patient exhibited improvement or maintenance of previous improvement of at least a 4-point increase in score; *or*
            - Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so
      iv. MFM32
         - Patient has experienced an increase in their MFM32 score from baseline and that increase correlates with a clinically significant functional improvement
      v. BSID-III
         - Member exhibited the ability to sit without support for at least 5 seconds after 12 months of treatment
      vi. Patient was prescribed Evrysdi due to clinical worsening after receiving gene replacement therapy (e.g., Zolgensma) and there is documentation of stabilization or improvement in clinical status with Evrysdi therapy (e.g., impact on motor milestones).
   2. Patient will not use Evrysdi and Spinraza concomitantly

Approval will be for **12 months.**

Evrysdi is considered **not medically necessary** for patients who do not meet the criteria set forth above.

Dosage and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

Quantity Limits

Medication Name	Quantity Limit	FDA-recommended dosing
Evrysdi (risdiplam) for oral solution 60 mg bottle	2 bottles (120 mg) per 24 days	• Less than 2 months of age: 0.15mg/kg once daily • 2 months to less than 2 years of age: 0.2 mg/kg once daily • 2 years of age and older weighing less than 20 kg: 0.25 mg/kg once daily • 2 years of age and older weighing 20 kg or more: 5 mg once daily

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- N/A

REFERENCES

- Evrysdi [package insert]. South San Francisco, CA: Genentech, Inc; May2022.

*Some content reprinted from CVSHealth

POLICY HISTORY

Policy #: 05.04.14
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