Mass General Brigham
Health Plan

Evrysdi (risdiplam)
Effective 05/01/2021

Plan	☐ MassHealth UPPL ☒Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☒ Pharmacy Benefit ☐ Medical Benefit		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	**Medical and Specialty Medications** All Plans | Phone: 877-519-1908 | Fax: 855-540-3693		
	Non-Specialty Medications All Plans | Phone: 800-711-4555 | Fax: 844-403-1029		
Exceptions	N/A		

Overview
Spinal muscular atrophy is a genetic disorder characterized by weakness and wasting (atrophy) in muscles used for movement (skeletal muscles). It is caused by a loss of specialized nerve cells, called motor neurons that control muscle movement. SMA is caused by an SMN1 gene that is missing or not working properly.

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

Coverage Guidelines
Authorization may be reviewed for members new to the plan who are currently receiving treatment with Evrysdi excluding when the product is obtained as samples or via manufacturer's patient assistance programs
OR
Authorization may be granted for members when ALL the following criteria are met, and documentation is provided:

1. Member has type 1, type 2, or type 3 SMA
2. The member has documented genetic testing confirming 5q SMA homozygous gene mutation, homozygous gene deletion, or compound heterozygote.
3. The member is at least 2 months of age but less than 25 years of age at the initiation of treatment
4. Member is not dependent on either of the following:
    a. Invasive ventilation or tracheostomy
    b. Use of non-invasive ventilation beyond naps and nighttime sleep
5. Member meets ONE of the following criteria:
    a. Member has not previously received gene therapy for SMA,
    b. Member has previously received gene therapy for SMA and has experienced a worsening in clinical status since receiving gene therapy as demonstrated by a decline of minimally clinical important difference from highest score achieved on one of the following exams (based on member age and motor ability)

i. HINE-2: Decline of at least 2 points on kicking and 1 point on any other milestone (excluding voluntary grasp)
ii. HFMSE: Decline of at least 3 points
iii. CHOP-INTEND: Decline of at least 4 points

6. Member will not use Evrysdi and Spinraza concomitantly
7. Member's daily dose will not exceed the following:
    a. Members 2 months to less than 2 years of age: 0.2 mg/kg
    b. Members 2 years of age and older weighing less than 20 kg: 0.25 mg/kg
    c. Members 2 years of age and older weighing 20 kg or more: 5 mg

Continuation of Therapy

Reauthorization may be granted when patient meets ALL initial criteria and following criteria:

1. Submission of medical records (e.g., chart notes, laboratory values) of the most recent (less than 1 month 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:
    a. HINE-2
        i. One of the following:
            1. Member exhibited improvement or maintenance of previous improvement of at least a 2 point (or maximal score) increase in ability to kick
            2. Member 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 of the following:
            1. Member exhibited improvement or maintenance of previous improvement in more HINE-2 motor milestones than worsening (net positive improvement)
            2. Member achieved and maintained any new motor milestones when they would otherwise be unexpected to do so (e.g., sit or stand unassisted, walk)
    b. HFMSE
        i. One of the following:
            1. Member exhibited improvement or maintenance of previous improvement of at least a 3-point increase in score
            2. Member has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so
    c. CHOP-INTEND
        i. One of the following:
            1. Member exhibited improvement or maintenance of previous improvement of at least a 4-point increase in score
            2. Member has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so
    d. MFM32
        i. Member has experienced an increase in their MFM32 score from baseline and that increase correlates with a clinically significant functional improvement

e. Member was prescribed Evrysdi due to clinical worsening after receiving gene therapy and there is documentation of stabilization or improvement in clinical status with Evrysdi therapy (e.g., impact on motor milestones).

Limitations

1. Initial approvals and reauthorizations will be for 12 months.
2. The following quantity limits apply:

Evrysdi 0.75mg/mL (80mL)	120mL per 30 days

References

1. Evrysdi [package insert]. South San Francisco, CA: Genentech, Inc; August 2020.

Review History

3/17/2021 – Created and Reviewed at March P&T. Effective 05/01/2021.