Clinical Policy Title:	risdiplam
Policy Number:	RxA.663
Drug(s) Applied:	Evrysdi®
Original Policy Date:	12/07/2020
Last Review Date:	10/19/2023
Line of Business Policy Applies to:	All lines of business (except Medicare)

Criteria

I. Initial Approval Criteria

A. Spinal Muscular Atrophy (must meet all):

1. Diagnosis of SMA;
2. Genetic testing confirms the presence of one of the following (a, b, or c):
   a. Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene);
   b. Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7);
   c. Compound heterozygous mutation in the SMN1 gene [e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)];
3. Documentation of one of the following baseline scores (a or b):
   a. For age < 2 years: Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorder (CHOP-INTEND) score or Hammersmith Infant Neurological Examination (HINE) Section 2 motor milestone score;
   b. For age ≥ 2 years: Hammersmith functional motor scale expanded (HFMSE) score, Revised Hammersmith Scale (RHS), Upper Limb Module (ULM), Revised Upper Limb Module (RULM), or 6-Minute Walk Test (6MWT);
4. Evrysdi® is prescribed by or in consultation with a neurologist specializing in neuromuscular disorders;
5. Patient does not require invasive ventilation or tracheostomy;
6. Patient is symptomatic at the time of request;
7. Patient is not receiving concurrent treatment with Spinraza® or Zolgensma®;
8. If the member is currently on Spinraza®, documentation of prescriber attestation of Spinraza® discontinuation upon initiation of Evrysdi®;
9. If the member has a history of treatment of Zolgensma®, provider must submit medical records (e.g., chart notes) documenting that there has been an inadequate response to gene therapy (e.g., sustained decrease in at least one motor test score over a period of 6 months);
10. Request meets one of the following (a, b, c, or d):
    a. If less than 2 months of age, dose does not exceed 0.15 mg/kg per day;
    b. If 2 months of age to less than 2 years of age, dose does not exceed 0.2 mg/kg per day;
    c. If 2 years of age and older, weighing less than 20 kg, dose does not exceed 0.25 mg/kg per day;
    d. If 2 years of age and older, weighing 20 kg or more, dose does not exceed 5 mg per day

Approval Duration

Commercial: 12 months

Medicaid: 12 months

II. Continued Therapy Approval

A. Spinal Muscular Atrophy (must meet all):

1. Member is currently receiving medication that has been authorized by RxAdvance or member has met initial approval criteria listed in this policy;
2. Member does not require tracheostomy or invasive ventilation;
3. Member is responding positively to therapy (i.e. clinically meaningful improvement in motor function or documentation of disease stabilization in normal motor decline);
4. Evrysdi® is not prescribed concurrently with Spinraza and/or Zolgensma;
5. Request meets one of the following (a, b, c, or d):
   a. If less than 2 months of age, dose does not exceed 0.15 mg/kg per day;
   b. If 2 months of age to less than 2 years of age, dose does not exceed 0.2 mg/kg per day;
   c. If 2 years of age and older, weighing less than 20 kg, dose does not exceed 0.25 mg/kg per day;
   d. If 2 years of age and older, weighing 20 kg or more, dose does not exceed 5 mg per day

Approval Duration

Commercial: 12 months

Medicaid: 12 months

References

1. Mercuri E, Baranello G, Kirschner J, et al. Update from SUNFISH Part 1: Safety, tolerability and PK/PD from the
2. Wang CH, Finkel RS, Bertini ES, et al. Consensus Statement for Standard of Care in Spinal Muscular Atrophy. Journal of Child Neurology. 2007; 22:1027-1049. 5. Cobben JM, de Visser M, Scheffer H, et al. Confirmation of clinical diagnosis in requests for prenatal prediction of SMA type I. J Neurol Neurosurg Psychiatry. 1993; 56: 319-21. Available at: https://smauk.org.uk/app/webroot/files/files/Publications%20and%20Leaflets/Consensus%20statement%20for%20SOC%20in%20SMA.pdf . Accessed September 5, 2022.
3. Dunaway Young S, Montes J, Kramer SS, et al. Six-minute walk test is reliable and valid in spinal muscular atrophy. Muscle and Nerve. 2016. 54: 836-842. Available at: https://pubmed.ncbi.nlm.nih.gov/27015431/. Accessed September 5, 2022.
4. ClinicalTrials.gov. A Study of Risdiplam in Infants with Genetically Diagnosed and Presymptomatic Spinal Muscular Atrophy (Rainbowfish). Available at: https://clinicaltrials.gov/ct2/show/NCT03779334 . Accessed September 5, 2022.

Review/Revision History	Review/Revised Date	P&T Approval Date
Policy established.	10/15/2020	12/07/2020
Policy was reviewed: 1. Continued Therapy Approval II.A.1 was rephrased to "Member is currently receiving medication that has been authorized by RxAdvance...". 2. References were reviewed and updated.	10/20/2021	12/07/2021

Policy was reviewed: 1. Initial Approval Criteria, I.A.2: Updated to remove prior age criteria. 2. Initial Approval Criteria, I.A.2: Updated diagnostic criteria from Patient is 2 years old or older to Genetic testing confirms the presence of one of the following (a, b, or c): a. Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene); b. Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7); c. Compound heterozygous mutation in the SMN1 gene [e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)]. 3. Initial Approval Criteria, I.A.8: Updated to include new documentation criteria If the member is currently on Spinraza®, documentation of prescriber attestation of Spinraza® discontinuation upon initiation of Evrysdi®. 4. Initial Approval Criteria, I.A.9: Updated to include new documentation criteria If the member has a history of treatment of Zolgensma®, provider must submit medical records (e.g., chart notes) documenting that there has been an inadequate response to gene therapy (e.g., sustained decrease in at least one motor test score over a period of 6 months). 5. Continued Therapy Approval Criteria, II.A.2: Updated to include new criteria pertaining to indication Spinal Muscular Atrophy , Member does not require tracheostomy or invasive ventilation. 6. Continued Therapy Approval Crtieria, II.A.4: Updated to include new combination therapy criteria Evrysdi is not prescribed concurrently with Spinraza and/or Zolgensma. 7. References were reviewed and updated.	09/05/2022	10/19/2022
Policy was reviewed.	10/19/2023	10/19/2023