Evrysdi (risdiplam)

Override(s)	Approval Duration
Prior Authorization Quantity Limit	Initial and continuation requests: 6 months

Medications	Quantity Limit
Evrysdi (risdiplam)	May be subject to quantity limit

APPROVAL CRITERIA

Initial requests for Evrysdi (risdiplam) may be approved if the following criteria are met:

I. Individual has a diagnosis of spinal muscular atrophy (SMA) by documentation of *either*:
   A. Spinal Muscular Atrophy (SMA) diagnostic test results confirming 0 copies of SMN1; **OR**
   B. Molecular genetic testing of 5q SMA for any of the following:
      1. Homozygous gene deletion; **OR**
      2. Homozygous conversion mutation; **OR**
      3. Compound heterozygote;

AND

II. Individual has documentation of SMA-associated signs and symptoms;

AND

III. Documentation is provided that individual has baseline motor ability assessments that support diagnosis based on age and motor ability (baseline motor ability assessments include but are not limited to the following: Hammersmith Infant Neurological Examination, The Children's Hospital of Philadelphia Infant Test of Neurological Disorders (CHOP INTEND), Hammersmith Function Motor Scale – Expanded (HFMSE), 6-Minute Walk Test (6MWT), Revised Upper Limb Module (RULM);

AND

IV. Requested medication has been prescribed by or in consultation with a neurologist who specializes in spinal muscular atrophy;

AND

V. Individual does not require use of invasive ventilation or tracheostomy as a result of advanced SMA disease.

Initial requests for Evrysdi (risdiplam) following treatment with Zolgensma (onasemnogene abeparvovec-xioi) may be approved if the following criteria are met:

I. When risdiplam therapy is determined to meet the above criteria; **AND**

II. Documentation is provided that individual has experienced a decline in clinical status (for example, loss of motor milestone) since receipt of gene therapy.

Continuation requests for Evrysdi (risdiplam) may be approved if the following criteria are met:

I. When initial therapy was determined to meet the above criteria; **AND**
II. Documentation is provided that individual has motor ability assessments that support improvement or stabilization compared baseline motor ability assessments (baseline motor ability assessments include but are not limited to the following: Hammersmith Infant Neurological Examination, The Children's Hospital of Philadelphia Infant Test of Neurological Disorders (CHOP INTEND), Hammersmith Function Motor Scale – Expanded (HFMSE), 6-Minute Walk Test (6MWT), Revised Upper Limb Module (RULM); **AND**
III. Individual does not require use of invasive ventilation or tracheostomy as a result of advanced SMA disease.

Requests for Evrysdi (risdiplam) may not be approved for the following:

I. When used in combination with Spinraza (nusinersen).

Key References:

1. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm.
2. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
3. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
4. Chiriboga CA, Bruno C, Duong T, et al. Risdiplam in Patients Previously Treated with Other Therapies for Spinal Muscular Atrophy: An Interim Analysis from the JEWELFISH Study [published correction appears in Neurol Ther. 2023 Oct;12(5):1799-1801. doi: 10.1007/s40120-023-00503-7]. *Neurol Ther*. 2023;12(2):543-557. doi:10.1007/s40120-023-00444-1.
5. Baranello G, Darras BT, Day JW, et al; FIREFISH Working Group. Risdiplam in Type 1 Spinal Muscular Atrophy. N Engl J Med. 2021 Mar 11;384(10):915-923. doi: 10.1056/NEJMoa2009965. Epub 2021 Feb 24.
6. Darras BT, Masson R, Mazurkiewicz-Bełdzińska M, et al; FIREFISH Working Group. Risdiplam-Treated Infants with Type 1 Spinal Muscular Atrophy versus Historical Controls. N Engl J Med. 2021 Jul 29;385(5):427-435. doi: 10.1056/NEJMoa2102047.
7. Clinicaltrials.gov [Internet]. Bethesda, MD: National Library of Medicine (US) 2000 Feb 29- . Identifier NCT02908685. A two part seamless, multi-center randomized, placebo-controlled, double-blind study to investigate the safety, tolerability, pharmacokinetics, pharmacodynamics and efficacy of RO7034067 in Type 2 and 3 spinal muscular atrophy patients: 2016 Sept 21 [cited 2020 Jan 29]. Available from: https://clinicaltrials.gov/ct2/show/study/NCT02908685.
8. Mercuri E, Baranello G, Boespflug-Tanguy O, et al. Risdiplam in types 2 and 3 spinal muscular atrophy: A randomised, placebo-controlled, dose-finding trial followed by 24 months of treatment. *Eur J Neurol*. 2023;30(7):1945-1956. doi:10.1111/ene.15499.
9. Oskoui M, Day JW, Deconinck N, et al. Two-year efficacy and safety of risdiplam in patients with type 2 or non-ambulant type 3 spinal muscular atrophy (SMA) [published correction appears in J Neurol. 2023 May;270(5):2547-2549. doi: 10.1007/s00415-023-11658-6]. *J Neurol*. 2023;270(5):2531-2546. doi:10.1007/s00415-023-11560-1.
10. 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):103-115. doi:10.1016/j.nmd.2017.11.005.

11. Pierzchlewicz K, Kępa I, Podogrodzki J, Kotulska K. Spinal Muscular Atrophy: The Use of Functional Motor Scales in the Era of Disease-Modifying Treatment. *Child Neurol Open*. 2021;8:2329048X211008725. Published 2021 Apr 27. doi:10.1177/2329048X211008725.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.