UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2026 P 2217-8
Program	Prior Authorization/Medical Necessity
Medication	Evrysdi® (risdiplam)
P&T Approval Date	9/2020, 9/2021, 7/2022, 8/2023, 7/2024, 12/2024, 4/2025, 4/2026
Effective Date	7/1/2026

1. Background:

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

2. Coverage Criteria[a]:

A. Initial Authorization

1. **Evrysdi** will be approved based on **all** of the following criteria:

   a. Diagnosis of spinal muscular atrophy (SMA)

   -AND-

   b. Submission of medical records (e.g., chart notes, laboratory values) confirming the mutation or deletion of genes in chromosome 5q resulting in **one** of the following:

      (1) Homozygous gene deletion or mutation of SMN1 gene (e.g., homozygous deletion of exon 7 at locus 5q13)

      -OR-

      (2) Compound heterozygous mutation of SMN1 gene (e.g., deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2])

   -AND-

   c. Patient is not dependent on either of the following:

      (1) Invasive ventilation
      (2) Use of non-invasive ventilation beyond use for naps and nighttime sleep

   -AND-

   d. Patient is not receiving concomitant chronic survival motor neuron (SMN) modifying therapy [e.g., Spinraza (nusinersen)]

   -AND-

   e. **One** of the following:

© 2026 UnitedHealthcare Services, Inc.

(1) Patient has not previously received gene replacement therapy for the treatment of SMA [e.g., Itvisma (onasemnogene abeparvovec-brve), Zolgensma (onasemnogene abeparvovec-xioi)]

-OR-

(2) **Both** of the following:

(a) Patient has previously received gene replacement therapy [e.g., Itvisma (onasemnogene abeparvovec-brve), Zolgensma (onasemnogene abeparvovec-xioi)] for the treatment of SMA

-AND-

(b) Submission of medical records (e.g., chart notes, laboratory values) documenting a clinically meaningful functional decline (e.g., loss of motor milestone) since receiving gene replacement therapy [e.g., Itvisma (onasemnogene abeparvovec-brve), Zolgensma (onasemnogene abeparvovec-xioi)]

-AND-

f. Submission of medical records (e.g., chart notes, laboratory values) documenting the baseline assessment of at least **one** of the following exams (based on patient age and motor ability) to establish baseline motor ability (baseline motor function analysis could include assessments evaluated prior to receipt of previous chronic SMN modifying therapy if transitioning therapy)*:

* Baseline assessments for patients less than 2 months of age requesting Evrysdi are not necessary in order to not delay access to initial therapy in recently diagnosed infants. Initial assessments shortly post-therapy can serve as baseline with respect to efficacy reauthorization assessment.

(1) Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)
(2) Hammersmith Infant Neurological Exam Part 2 (HINE-2)
(3) Hammersmith Functional Motor Scale Expanded (HFMSE)
(4) Revised Upper Limb Module (RULM) Test
(5) Motor Function Measure 32 (MFM-32) Scale

-AND-

g. Prescribed by or in consultation with a neurologist with expertise in the treatment of SMA

Authorization will be issued for 12 months.

B. Reauthorization

1. **Evrysdi** will be approved based on **all** of the following criteria:

© 2026 UnitedHealthcare Services, Inc.

a. Submission of medical records (e.g., chart notes, laboratory values) with the most recent results documenting a positive clinical response to Evrysdi compared to pretreatment baseline status (inclusive of baseline assessments prior to receipt of previous chronic SMN modifying therapy) as demonstrated by at least **one** of the following exams:

(1) CHOP INTEND: **One** of the following:

(a) Improvement or maintenance of previous improvement of at least a 4 point increase in score from pretreatment baseline

(b) Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so

-OR-

(2) HINE-2: **One** of the following:

(a) Improvement or maintenance of previous improvement of at least 2 point (or maximal score) increase in ability to kick

(b) Improvement or maintenance of previous improvement of at least 1 point increase in any other HINE-2 milestone (e.g., head control, rolling, sitting, crawling, etc.), excluding voluntary grasp

(c) The patient exhibited improvement, or maintenance of previous improvement in more HINE motor milestones than worsening, from pretreatment baseline (net positive improvement)

(d) Patient has achieved and maintained any new motor milestones when they would otherwise be unexpected to do so

-OR-

(3) HFMSE: **One** of the following:

(a) Improvement or maintenance of previous improvement of at least a 3 point increase in score from pretreatment baseline

(b) Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so

-OR-

(4) RULM: **One** of the following:

(a) Improvement or maintenance of previous improvement of at least a 2 point increase in score from pretreatment baseline

© 2026 UnitedHealthcare Services, Inc.

(b) Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so

-OR-

(5) MFM-32: **One** of the following:

(a) Improvement or maintenance of previous improvement of at least a 3 point increase in score from pretreatment baseline

(b) Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so

-AND-

b. Patient is not dependent on either of the following:

(1) Invasive ventilation
(2) Use of non-invasive ventilation beyond use for naps and nighttime sleep

-AND-

c. Patient is not receiving concomitant chronic survival motor neuron (SMN) modifying therapy [e.g., Spinraza (nusinersen)]

-AND-

d. Prescribed by or in consultation with a neurologist with expertise in the treatment of SMA

Authorization will be issued for 12 months.

[a] State mandates may apply. Any federal regulatory requirements and the member specific benefit plan coverage may also impact coverage criteria. Other policies and utilization management programs may apply.

3. Additional Clinical Rules:

- Notwithstanding Coverage Criteria, UnitedHealthcare may approve initial and re-authorization based solely on previous claim/medication history, diagnosis codes (ICD-10) and/or claim logic. Use of automated approval and re-approval processes varies by program and/or therapeutic class.
- Supply limits may be in place.

4. References:

1. Evrysdi [package insert]. South San Francisco, CA: Genentech, Inc; January 2026.
2. Mercuri E, Darras BT, Chiriboga CA, et al. Nusinersen versus Sham Control in Later-Onset Spinal Muscular Atrophy. N Engl J Med. 2018 Feb 15;378(7):625-635.
3. Finkel RS, Mercuri E, Darras BT, et al. Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy. N Engl J Med. 2017 Nov 2;377(18):1723-1732.

© 2026 UnitedHealthcare Services, Inc.

4. Markowitz JA, Singh P, Darras BT. Spinal Muscular Atrophy: A Clinical and Research Update. Pediatric Neurology 46 (2012) 1-12.
5. Mendell JR, Al-Zaidy S, Shell R, et al. Single-dose gene-replacement therapy for spinal muscular atrophy. N Engl J Med. 2017;377:1713-22.
6. Chiriboga C, Mercuri E, Fischer D, et al. JEWELFISH: Risdiplam (RG7916) increased survival of motor neuron (SMN) protein levels in non-naïve patients with spinal muscular atrophy (SMA). Presented at the 6th International Congress of Myology in Bordeux, France; March 25-28, 2019. Poster.
7. Chiriboga C, Bruno C, Duong T, et al. JEWELFISH: Safety and pharmacodynamic data in non-naïve patients with spinal muscular atrophy receiving treatment with risdiplam. Presented at the 2020 Virtual SMA Research & Clinical Care Meeting. June 12, 2020.
8. Day JW, Annoussamy M, Baranello G, et al. SUNFISH Part 1: 24-month safety and exploratory outcomes of risdiplam (RG7916) treatment in patients with Type 2 or 3 spinal muscular atrophy (SMA). Presented at the 2020 Virtual SMA Research & Clinical Care Meeting. June 12, 2020.
9. Servais L, Baranello G, Masson R, et al. FIREFISH Part 2: Efficacy and safety of risdiplam (RG7916) in infants with Type 1 spinal muscular atrophy (SMA). Presented at the 2020 Virtual SMA Research & Clinical Care Meeting. June 12, 2020.
10. Kirschner J, Butoianu N, Goemans N, et al. European ad-hoc consensus statement on gene replacement therapy for spinal muscular atrophy. European Journal of Paediatric Neurology. 2020.
11. 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 Jul 3;:]. Neurol Ther. 2023;12(2):543-557.
12. Pera MC, Coratti G, Mazzone ES, Montes J, Scoto M, De Sanctis R, Main M, Mayhew A, Muni Lofra R, Dunaway Young S, Glanzman AM, Duong T, Pasternak A, Ramsey D, Darras B, Day JW, Finkel RS, De Vivo DC, Sormani MP, Bovis F, Straub V, Muntoni F, Pane M, Mercuri E; iSMAC Consortium Group. Revised upper limb module for spinal muscular atrophy: 12 month changes. Muscle Nerve. 2019 Apr;59(4):426-430. Epub 2019 Feb 7. PMID: 30677148.
13. Stolte B, Bois JM, Bolz S, Kizina K, Totzeck A, Schlag M, Kleinschnitz C, Hagenacker T. Minimal clinically important differences in functional motor scores in adults with spinal muscular atrophy. Eur J Neurol. 2020 Dec;27(12):2586-2594. Epub 2020 Sep 6. PMID: 32781490.
14. Kirschner J, Bernert G, Butoianu N, et al. 2024 update: European consensus statement on gene therapy for spinal muscular atrophy. Eur J Paediatr Neurol. 2024;51:73-78.

Program	Prior Authorization/Medical Necessity – Evrysdi (risdiplam)
Change Control	
9/2020	New program
9/2021	Annual review with no changes to clinical coverage criteria. Updated reference.
7/2022	Updated criteria to align with new labeled indication in patients of all ages. Updated reference.
8/2023	Annual review. Updated references.
7/2024	Annual review. Revised prescriber requirement and updated Upper Limb Module to Revised Upper Limb Module test. Updated references.
12/2024	Added criteria for patients that have documented decline from pretreatment baseline status following administration of gene replacement therapy. Updated references.

© 2026 UnitedHealthcare Services, Inc.

4/2025	Revised criteria for patients that have documented decline from pretreatment baseline status following administration of gene replacement therapy.
4/2026	Annual review. Added Itvisma as an example of gene therapy. Updated references.

© 2026 UnitedHealthcare Services, Inc.