UnitedHealthcare Community Plan

Clinical Pharmacy Program Guidelines for Evrysdi

Program	Prior Authorization
Medication	Evrysdi™ (risdiplam)
Markets in Scope	Arizona, California, Colorado, Hawaii, Maryland, Nevada, New Jersey, New York, New York EPP, Pennsylvania- CHIP, Rhode Island, South Carolina
Issue Date	9/2020
Pharmacy and Therapeutics Approval Date	9/2020
Effective Date	11/2020

1. **Background:**
   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.

2. **Coverage Criteria:**

A. <u>Initial Authorization</u>

1. Evrysdi will be approved based on **<u>all</u>** 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 **<u>one</u>** 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 or tracheostomy
(2) Use of non-invasive ventilation beyond use for naps and nighttime sleep

-AND-

d. Physician attests that Evrysdi is not to be initiated in a patient less than 2 months of age

-AND-

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

-AND-

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

-AND-

g. 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 proactively 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) Upper Limb Module (ULM) Test
(5) Motor Function Measure 32 (MFM-32) Scale

-AND-

h. Prescribed by 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:

a. **One** of the following

(1) 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:

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

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

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

-OR-

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

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

ii. 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

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

iv. Patient has achieved and maintained any new motor milestones when they would otherwise be unexpected to do so

-OR-

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

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

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

-OR-

(d) ULM: **One** of the following:

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

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

-OR-

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

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

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

-AND-

e. Prescribed by a neurologist with expertise in the treatment of SMA

Authorization will be issued for 12 months.

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; August 2020.
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.
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, doi: https://doi.org/10.1016/j.ejpn.2020.07.001.

Program	Program type – Prior Authorization
Change Control	
9/2020	New program.