BlueCross BlueShield
Federal Employee Program

EVRYSDI
(risdiplam)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Evrysdi (risdiplam) is a survival of motor neuron 2 (SMN2) splicing modifier designed to treat patients with spinal muscular atrophy (SMA) caused by mutations in chromosome 5q that lead to SMN protein deficiency. Evrysdi was shown to increase exon 7 inclusion in SMN2 messenger ribonucleic acid (mRNA) transcripts and production of full-length SMN protein in the brain (1).

Regulatory Status

FDA-approved indication: 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 (1).

In the clinical studies done for Evrysdi, the patients in these studies had Type I, II, or III SMA. The clinical studies did not include Types 0 and IV (1).

Evrysdi powder must be constituted to the oral solution by a pharmacist prior to dispensing to the patient. The constituted oral solution must be kept in the original amber bottle to protect from light and stored in a refrigerator. Any unused portion should be discarded 64 days after constitution (1).

Evrysdi may cause embryofetal harm when administered to a pregnant woman. Female patients of reproductive potential should undergo pregnancy testing prior to treatment and be advised to use effective contraception during treatment with Evrysdi and for at least 1 month after the last dose (1).

Multiple tools have been developed in order to determine a baseline motor milestone score for patients with SMA. These assessments can also be utilized to measure improvement and include: Hammersmith Infant Neurologic Exam (HINE), Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND), Upper Limb Module (ULM), and the Hammersmith Functional Motor Scale (HFMS) / Hammersmith Functional Motor Scale - Expanded (HFMSE) (2-3).

The safety and effectiveness of Evrysdi in pediatric patients have been established (1).

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Summary

Evrysdi (risdiplam) is a survival of motor neuron 2 (SMN2) splicing modifier designed to treat patients with spinal muscular atrophy (SMA) caused by mutations in chromosome 5q that lead to SMN protein deficiency. Evrysdi was shown to increase exon 7 inclusion in SMN2 messenger ribonucleic acid (mRNA) transcripts and production of full-length SMN protein in the brain. The safety and effectiveness of Evrysdi in pediatric patients have been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Evrysdi while maintaining optimal therapeutic outcomes.

References

1. Evrysdi [package insert]. South San Francisco, CA: Genentech, Inc.; February 2025.
2. Mazzone E, Bianco F, et al. Assessing upper limb function in nonambulant SMA patients: Development of a new module. *Neuromuscular Disorders* 21 (2011) pg: 406-412.
3. De Sanctis, Roberto, et. al. Developmental milestones in type I spinal muscular atrophy. *Neuromuscular Disorders* 26 (2016) pg: 754-759.