BlueCross BlueShield Federal Employee Program

EXONDYS 51 (eteplirsen)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Exondys 51 (eteplirsen) is indicated for patients with a diagnosis of Duchenne muscular dystrophy (DMD) who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. DMD is a genetic disorder characterized by progressive muscle degeneration and weakness. DMD is caused by an exon mutation in a gene that codes for dystrophin, a protein that helps keep muscle intact. Exons are the sections of DNA that contain instructions for creating proteins; if an exon is mutated, a functional protein cannot be produced. Exondys 51 is designed to “skip over” a mutated exon and enable the synthesis of a shortened, functional form of dystrophin protein (1).

Regulatory Status

FDA-approved indication: Exondys 51 is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping (1).

Monitoring motor changes in patients with DMD requires functional evaluation along with measurement of muscle strength. The need for a reliable outcome measure in diseases of rapid deterioration such as DMD has led to the use of motor functional tests. In a large, multicenter, international clinical trial, the six minute walk test (6MWT) proved to be feasible and highly reliable. Also used are the Motor Function Measure (MFM) and North Star Ambulatory Assessment (NSAA) to help predict loss of ambulation 1 year before its occurrence in order to allow time to adapt rehabilitation, change the patient’s environment, and consider acquisition of assistive aids or the use of medications (2-4).

Summary

Exondys 51 (eteplirsen) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. Dystrophin levels should be measured at baseline to evaluate pretreatment dystrophin-positive fibers and sometime during therapy to evaluate the effect of Exondys 51 dose (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of

Exondys 51 while maintaining optimal therapeutic outcomes.

References

1. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; December 2024.
2. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Clinical Endpoints in Duchenne Muscular Dystrophy: Reliability, Concurrent Validity, and Minimal Clinically Important Differences from a Multicenter Study. Muscle Nerve. 2013 Sep; 48(3): 357–368.
3. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Endpoints in Duchenne Muscular Dystrophy: Longitudinal Natural History Observations Over 48 weeks from a Multicenter Study. Muscle Nerve. 2013 Sep; 48(3): 343–356.
4. Vuillerot C, Girardot F, et al. Monitoring changes and predicting loss of ambulation in Duchenne muscular dystrophy with the Motor Function Measure. *Developmental Medicine & Child Neurology* 2010, 52: 60–65.