BlueCross BlueShield
Federal Employee Program

VILTEPSO
(viltolarsen)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Viltepso (viltolarsen) is an antisense oligonucleotide designed to bind to exon 53 on dystrophin pre-mRNA resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon 53 skipping is intended to allow for production of an internally truncated dystrophin protein in patients with genetic mutations that are amenable to exon 53 skipping (1).

Regulatory Status

FDA-approved indication: Viltepso 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 53 skipping (1).

Renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting Viltepso. Glomerular filtration rate using an exogenous filtration marker may also be measured before starting Viltepso. During treatment, urine dipstick should be monitored every month, and serum cystatin C and urine protein-to-creatinine ratio should be monitored every three months (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).

Viltepso is indicated for patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping, including pediatric patients. DMD is largely a disease of children and young adults (1).

BlueCross BlueShield
Federal Employee Program

VILTEPSO
(viltolarsen)

Summary

Viltepso (viltolarsen) 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 53 skipping. Renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. DMD is largely a disease of children and young adults (1).

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

References

1. Viltepso [Package Insert]. Paramus, NJ: NS Pharma, Inc.; March 2021.
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.