Pharmacologic Treatment of Duchenne Muscular Dystrophy

Description:

Amondys 45® (casimersen), Exondys 51® (eteplirsen), Viltepso™ (vitolarsen), and Vyondys 53® (golodirsen) are drugs used for Duchenne Muscular Dystrophy. Duchenne muscular dystrophy (DMD) is a rare genetic condition characterized by progressive muscle deterioration and weakness of skeletal and heart muscles. DMD is the most common childhood onset form of muscular dystrophy which primary affects males, though in rare cases may affect females. It is caused by an absence of a functional dystrophin, which is a protein that helps keep muscle cells intact. Dystrophin gene is thought to be defective when its structure contains one or more exon deletions due to a genetic mutation. This disease primarily affects boys. Symptom onset is usually in early childhood, between ages 3 and 5. Muscle weakness can begin as early as age 3, first affecting the muscles of the hips, pelvic area, thighs and shoulders, and later the skeletal (voluntary) muscles in the arms, legs, and trunk. By the early teens, the heart and respiratory muscles also get affected, often requiring the use of assistive devices. Tests used to diagnose DMD vary from a blood test (measuring creatine kinase) to the muscle biopsy (measuring dystrophin protein levels), to the genetic testing (looking for the defective dystrophin gene).

Standard of therapy is aimed at slowing the loss of muscle strength to maximize the quality of life, and involves physical therapy and medications, such as steroids: prednisone and deflazacort. Assistive devices for breathing difficulties may also be used later in the stages of disease progression. Exon skipping treatments are a novel approach used in the management of this disease.

Policy:

The intent of this policy is to define clinical characteristics to identify patients who qualify for AMONDYS 45™ (casimersen), EXONDYS 51™ (eteplirsen), VYONDYS 53™ (golodirsen), and VILTEPSO™ (viltolarsen). These drugs require a prior authorization and will be covered when the criteria have been met.

Prior Authorization Criteria:

1. Initial Request: Approve when the member meets ALL of the following:
    a. Member must have a diagnosis of DMD;
    b. Documentation of genetic testing must confirm the DMD gene mutation of the patient is amenable to exon 45, 51, or 53 skipping;

c. Documentation must confirm a stable dose of corticosteroids prior to starting therapy or a documented reason not to be on corticosteroids;
d. Documentation indicates kidney function testing prior to starting therapy (except for eteplirsen); and
e. Patient is not concurrently being treated with another exon skipping therapy for DMD.

References:

1. Muscular Dystrophy UK. Online database. Available at: http://www.musculardystrophyuk.org/progress-in-research/background-information/what-is-exon-skipping-and-howdoes-it-work. Accessed on May 2022.
2. Summary of Practice Guidelines for Patients and their Families: Duchenne Muscular Dystrophy: Treatment with Corticosteroids. Available at: https://www.aan.com/Guidelines/home/GetGuidelineContent/733. Accessed on May 2022.
3. The Muscular Dystrophy Association (MDA): For Strength, Independence & Life. Available at: https://www.mda.org/disease/duchenne-muscular-dystrophy/causes-inheritance. Accessed on May 2022.
4. Amondys-45 Prescribing Information. Sarepta Therapeutics, Cambridge, MA. February 2021.
5. Vyondys-53 Prescribing Information. Sarepta Therapeutics, Cambridge, MA. February 2021.
6. Viltepso Prescribing Information. NS Pharma, Inc., Paramus, NJ. March 2021.
7. Vyondys 53® Prescribing Information. Sarepta Therapeutics, Cambridge, MA. February 2021.