



Eteplirsen (Exondys 51™)

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Number: MG.MM.PH.30

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Definitions

Duchenne Muscular Dystrophy (DMD) is an inherited disorder that results in a deficiency of dystrophin causing a loss of muscle function and weakness. DMD primarily affects males and is the most common, and severe, form of muscular dystrophy in children. Symptom onset usually occurs between the ages of 3 and 5. It is one of more than thirty forms of muscular dystrophy.

The DMD gene provides instructions for making the protein dystrophin. Dystrophin, a protein that protects muscles from deterioration, is located primarily in skeletal and heart muscle.

Eteplirsen (Exondys 51) is an antisense oligonucleotide, administered via intravenous infusion, designed to bind to exon 51 of dystrophin pre-mRNA. This results in the exclusion of exon 51 during mRNA processing in those members with genetic mutations that are amenable to exon 51 skipping. The exon skipping which occurs as a result of eteplirsen binding is intended to allow for the production of internally truncated dystrophin proteins.

Guideline

Eteplirsen is considered medically necessary for the treatment of DMD in members who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

Initial coverage will be provided for 4 weeks when **all** of the following criteria have been met:

- I. Member is ≥ 7 years of age
- II. Member is diagnosed with DMD and with confirmed mutation of the DMD gene that is amenable to exon 51 skipping

- III. Member is able to independently walk a mean distance of ≥ 300 meters in the 6-minute walk test
- IV. Member is ambulatory without assistance or devices (e.g. cane, walker, wheelchair)
- V. Member is currently receiving treatment with glucocorticoids and **one** of the following conditions has been met and documented (**a or b**):
 - a. Member has received glucocorticoids for at least 24 weeks AND, according to the prescribing physician, the member has experienced **at least one** of the following significant intolerable adverse effects (i, ii, iii, or iv)
 - i. Cushingoid appearance
 - ii. Central (truncal) obesity
 - iii. Undesirable weight gain (defined as $\geq 10\%$ of body weight gain increase over a 6-month period)
 - iv. Diabetes and/or hypertension that is difficult to manage according to the prescribing physician
 - b. According to the prescribing physician, the member has experienced a severe behavioral adverse effect while on glucocorticoid therapy that has (or would) require a dose reduction
- VI. Member has stable pulmonary function and cardiac function
- VII. Member has a FVC of $\geq 30\%$ or Brooke Score of ≤ 5
- VIII. Documentation of baseline blood urea nitrogen (BUN)/serum creatinine (SCR) ratio demonstrating normal kidney function
- IX. Documentation of baseline urinalysis demonstrating absence of proteinuria
- X. Member has had an adequate trial of Emflaza (deflazacort)
- XI. Medication is prescribed by a pediatric neurologist specializing in DMD treatment
- XII. Medication is prescribed at its FDA-approved dosing of 30 mg/kg once per week

Renewal Guideline

Coverage will be provided for 24 weeks when **all** of the following criteria are met:

- I. Medication is prescribed by a pediatric neurologist specializing in DMD treatment
- II. Medication is prescribed at its FDA-approved dosing of 30 mg/kg once per week.
- III. Member remains ambulatory without assistance or devices (e.g. cane, walker, wheelchair) (documentation required)
- IV. Member's pulmonary and cardiac function has remained stable
- V. Member continues to receive treatment with glucocorticoids

Limitations/Exclusions

The use of Eteplirsen is considered experimental or investigational for all other uses.

Revision History

9/8/17

Added Eteplirsen coverage for members diagnosed with DMD who meet criteria above.

Applicable Procedure Codes

J1428	Injection, eteplirsen, 10 mg (Eff. 01/01/2018)
J3490	Unclassified drugs (prior to 1/1/2018)
J3590	Unclassified biologics (prior to 1/1/2018)

Applicable Diagnosis Codes

G71.0	Muscular dystrophy
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