CLINICAL MEDICATION POLICY	
Policy Name:	Exondys 51™ (eteplirsen)
Policy Number:	MP-005-MC-ALL
Responsible Departments:	Medical Management, Medical Policy, Clinical Pharmacy
Provider Notice Date:	04/1/2017
Original Effective Date:	05/01/2017
Annual Approval Date:	03/15/2018
Revision Date:	N/A
Products:	Kentucky Medicare Assured
Application:	All participating and nonparticipating hospitals and providers
Page Number(s):	1 of 4

Disclaimer

Gateway Health℠ (Gateway) clinical medication policy is intended to serve only as a general reference resource regarding payment and coverage for the services described. This policy does not constitute medical advice and is not intended to govern or otherwise influence medical decisions.

POLICY STATEMENT

Gateway Health℠ may provide coverage under the medical or pharmacy benefits of the Company's Medicare products for medically necessary Exondys 51 (eteplirsen) intravenous administration in the treatment of Duchenne Muscular Dystrophy (DMD).

This policy is designed to address medical necessity guidelines that are appropriate for the majority of individuals with a particular disease, illness or condition. Each person's unique clinical circumstances warrant individual consideration, based upon review of applicable medical records.

DEFINITIONS

Duchenne Muscular Dystrophy (DMD) – A severe, progressive, X-chromosome-linked neuromuscular disorder with prematurely truncated, non-functional dystrophin, characterized by progressive muscle deterioration and weakness. The underlying cause of DMD is a mutation or error in the gene for dystrophin.

Dystrophin – An essential protein involved in muscle fiber function.

Exon 51 Skipping – Exon skipping is a potential treatment approach to correct for specific genetic mutations and restore production of dystrophin protein. Eteplirsen is designed to skip exon 51.

PROCEDURES

1. Exondys 51 is considered medically necessary as an intravenous infusion for the treatment of Duchenne Muscular Dystrophy (DMD) when the patient meets the following criteria:
   A. The patient must have a confirmed diagnosis of Duchenne Muscular Dystrophy (DMD) by submission of lab testing demonstrating mutation of the dystrophin gene amenable to exon 51 skipping; AND
   B. The patient must be on a stable dose of corticosteroids for at least 6 months prior to initiation of therapy; AND
   C. The patient must be an ambulating male between the ages of 7-13; AND
   D. The medication must be prescribed by, or in consultation with, a neurologist; AND
   E. Coverage will be provided for situations in which there is functional status that can be preserved; AND
   F. The patient must still either be able to walk 200m to 400m on the 6-minute walk test or alternatively must have some functional arm use; AND
   G. The dosing is within the following prescribing-supported parameter:
      1) Dose must not exceed 30 mg/kg of body weight once weekly; AND
   H. The administration of the medication must be done by a health care provider.

2. Contraindications
   There are no known contraindications to Exondys 51. DMD is largely a disease of children and young adults; therefore, there is no geriatric experience with Exondys 51.

3. When the Exondys 51 is not covered
   Exondys 51 is not covered for conditions other than those listed above because the scientific evidence has not been established.

   Coverage may be provided for any non-FDA labeled indication if it is determined that the use is a medically accepted indication supported by nationally recognized pharmacy compendia or peer-reviewed medical literature for treatment of the diagnosis(es) for which it is prescribed. These requests will be reviewed on a case-by-case basis to determine medical necessity.

When criteria are not met, the request will be forwarded to a Medical Director for review. The physician reviewer must override criteria when, in their professional judgment, the requested medication is medically necessary.

4. Post-Payment Audit Statement
   The medical record must include documentation that reflects the medical necessity criteria and is subject to audit by Gateway at any time pursuant to the terms of your provider agreement.

5. Place of Service
   The place of service for the administration of Exondys 51 is outpatient.

6. Coverage Determination
   Gateway Health℠ follows the coverage determinations made by CMS as outlined in either the national coverage determinations (NCD) or the state-specific local carrier determination (LCD).

   There is no specific NCD or North Carolina LCD for Exondys 51. For additional information, please see:
   http://www.palmettogba.com/palmetto/providers.nsf/docscat/Providers~JM%20Part%20B~Medical%20Policies~LCDs%20Coverage%20Articles%20NCDs

Governing Bodies Approval
On September 19, 2016, the FDA approved Exondys 51. 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. The drug received accelerated approval based on the surrogate endpoint of increase in dystrophin in skeletal muscle observed in some patients, which the FDA concluded was reasonable and likely to predict a clinical benefit, but is requiring further studies to confirm.

Exondys 51 (eteplirsen) is an intravenous infusion administered over 35 to 60 minutes once weekly. The most common side effects include balance disorder and vomiting.

CODING REQUIREMENTS

Procedure Codes

CPT/HCPCS Codes	Description
J3490/ J3590	Solution for Injection, eteplirsen , 100mg/2mL
J3490/ J3590	Solution for Injection, eteplirsen , 500mg/10mL

Diagnosis Codes

ICD-10 Code	Description
G71.0	Muscular Dystrophy (Duchenne Muscular Dystrophy)

REMBURSEMENT

Participating facilities will be reimbursed per their Gateway Health℠ contract.

POLICY SOURCE(S)

EXONDYS 51™ [package insert]. Sarepta Therapeutics, Inc., Cambridge, MA, September 2016. Available at: http://www.exondys51.com/.

Exondys 51™ In: Clinical Pharmacology [database online]. Gold Standard, Inc. Accessed on September 28, 2016.

McDonald CM, Henricson EK, Han JJ, et al. The 6-minute walk test as a new outcome measure in Duchenne muscular dystrophy. Muscle Nerve 2010; 41:500–510. Accessed on September 28, 2016, and abstract available at: http://onlinelibrary.wiley.com/doi/10.1002/mus.21544/abstract.

Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. Ann Neurol 2013; 74(5):637-647. Accessed on September 28, 2016, and abstract available at: http://onlinelibrary.wiley.com/doi/10.1002/ana.23982/abstract.

Policy History:

Date	Activity
N/A	LCD/NCD effective date
03/15/2017	QI/UM Committee approval
05/01/2017	Provider effective date