PHARMACY POLICY STATEMENT

Original Effective Date	Next Annual Review	Last Revision
11/28/2016	11/24/2017	11/29/2016
Policy Name		**Policy Number**
Exondys 51		SRx-0052
Policy Type		
Medical	Administrative	**PHARMACY** Reimbursement

Pharmacy Policy Statements prepared by CSMG Co. and its affiliates (including CareSource) are derived from literature based on and supported by clinical guidelines, nationally recognized utilization and technology assessment guidelines, other medical management industry standards, and published MCO clinical policy guidelines. Medically necessary services include, but are not limited to, those health care services or supplies that are proper and necessary for the diagnosis or treatment of disease, illness, or injury and without which the patient can be expected to suffer prolonged, increased or new morbidity, impairment of function, dysfunction of a body organ or part, or significant pain and discomfort. These services meet the standards of good medical practice in the local area, are the lowest cost alternative, and are not provided mainly for the convenience of the member or provider. Medically necessary services also include those services defined in any Evidence of Coverage documents, Pharmacy Policy Statements, Provider Manuals, Member Handbooks, and/or other policies and procedures.

Pharmacy Policy Statements prepared by CSMG Co. and its affiliates (including CareSource) do not ensure an authorization or payment of services. Please refer to the plan contract (often referred to as the Evidence of Coverage) for the service(s) referenced in the Pharmacy Policy Statement. If there is a conflict between the Pharmacy Policy Statement and the plan contract (i.e., Evidence of Coverage), then the plan contract (i.e., Evidence of Coverage) will be the controlling document used to make the determination

Contents of Policy

A. INTRODUCTION

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. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with EXONDYS 51.

The intent of CareSource Pharmacy Policy Statements is to encourage appropriate selection of patients for therapy according to product labeling, clinical guidelines, and/or clinical studies as well as to encourage use of preferred agents. The CareSource Pharmacy Policy Statement is a guideline for determining health care coverage for our patients with benefit plans covering prescription drugs. Pharmacy Policy Statements are written on selected prescription drugs requiring prior authorization or step therapy. The Pharmacy Policy Statement is used as a tool to be interpreted in conjunction with the member's specific benefit plan.

NOTE: The Introduction section is for your general knowledge and is not to be construed as policy coverage criteria. The rest of the policy uses specific words and concepts familiar to medical professionals and is intended for providers. A provider can be a person, such as a doctor, nurse, psychologist, or dentist. A provider can also be a place where medical care is given, like a hospital, clinic or lab. This policy informs providers about when a service may be covered.

B. DEFINITIONS

1. None applicable.

C. POLICY COVERAGE CRITERIA

1. Site of Service

Site of Service Administration	Coverage Criteria
Office, Outpatient, Home	**Preferred place of service is in the home.**

2. Coverage Criteria

CareSource will approve the use of Exondys 51 and consider its use medically necessary when the criteria have been met for each condition listed below. Prior authorization request should be submitted with chart notes and documentation supporting medical necessity.

Condition	Exondys 51 Coverage criteria:
Duchenne muscular dystrophy	1) A diagnosis of Duchenne Muscular Dystrophy with confirmed mutation of a DMD gene that is amenable to exon 51 skipping (*chart/lab notes required*) 2) Member is currently taking a corticosteroid or has contraindication to corticosteroids

All other uses of Exondys 51 are considered experimental/investigational; and therefore, will follow CareSource's off-label policy.

Please note that this policy is reviewed on an annual basis. New drugs and indications receiving FDA approval may not be reflected in this policy immediately.

Notes:

- Documented diagnosis must be confirmed by portions of the individual's medical record which need to be supplied with prior authorization request. These medical records may include, but are not limited to test reports, chart notes from provider's office, or hospital admission notes.
- Patient is required to have completed the trial(s) listed in the above criteria unless the patient is unable to tolerate or has a contraindication to trial medications. Documentation such as chart notes or pharmacy claims may be requested to verify trial(s), intolerance, or contraindication(s).
- Refer to the product package insert for dosing, administration and safety guidelines.

3. Dosage and Quantity Limits (listed if applicable)

Information for patients with renal or hepatic impairment is not included. See package insert for individual agents.

Condition	Dosage and Quantity Limit of Exondys 51
Duchenne Muscular Dystrophy	30 milligrams per kilogram of body weight once weekly

4. Authorization Period

Condition	Approval Period
Duchenne Muscular Dystrophy	The initial authorization of Exondys 51 is valid for 6 months. Continued treatment may be considered when patient continues to meet coverage criteria. A reauthorization after successful initiation period will be placed for 6 months. **ALL** authorizations are subject to continued eligibility. *Exondys 51 will only be covered on the medical benefit.*

5. Coding

HCPCS	
J3490	Unclassified drugs
J3590	Unclassified biologics

D. RELATED POLICIES

AD-0004: Medical Necessity - Off-Label, Approved Orphan and Compassionate Use Drugs

E. REVIEW/REVISION HISTORY

DATE	ACTION/DESCRIPTION
11/29/2016	Policy created

F. REFERENCES

1. Exondys 51 [Package Insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; September 2016.
2. Sarepta Therapeutics. An Open-Label, Multi-Center Study to Evaluate the Safety and Tolerability of Eteplirsen in Patients With Advanced Stage Duchenne Muscular Dystrophy. NLM Identifier: NCT02286947.
3. Sarepta Therapeutics. Confirmatory Study of Eteplirsen in DMD Patients (PROMOVI). NLM Identifier: NCT02255552.
4. Sarepta Therapeutics. An Open-Label, Multi-Center Study to Evaluate the Safety and Tolerability of Eteplirsen in Early Stage Duchenne Muscular Dystrophy. NLM Identifier: NCT02420379

The Pharmacy Policy detailed above has received due consideration and is approved.

Independent medical review – 12/12/2016