Trillium Community Health Plan

Golodirsen (Vyondys 53)

Prior Authorization Form/Prescription

PLEASE COMPLETE ALL SECTIONS FOR A TIMELY REVIEW

Date: _____ Date Medication Required: _____
Ship to: ❍ Physician ❍ Patient's Home ❍ Other: _____

Patient Information

Last Name:	First Name:	Middle:	DOB: ____/____/_____
Address:	City:	State:	Zip:
Daytime Phone:	Evening Phone:	Sex: ☐ Male ☐ Female	

Insurance Information (*Attach copies of cards*)

Primary Insurance:		Secondary Insurance:	
ID #	Group #	ID #	Group #
City:	State:	City:	State:

Physician Information

Name:	Specialty:	NPI:	
Address:	City:	State:	Zip:
Phone #:	Secure Fax #:	Office Contact:	

Primary Diagnosis

ICD-10 Code: _____

☐ Duchenne muscular dystrophy (DMD) ☐ Other: _____

Prescription Information

MEDICATION	STRENGTH	DIRECTIONS	QUANTITY	REFILLS
Vyondys 53 (Golodirsen)				

Clinical Information

***** *Please submit supporting clinical documentation* *****

☐ **INITIAL THERAPY** ☐ **CONTINUATION OF THERAPY;** Therapy start date: _____

1. Has patient had a positive response to the prescribed therapy? ☐ Yes ***Please submit documentation*** ☐ No ☐ Not applicable

Complete this section ONLY if the patient is <u>initiating</u> therapy OR if the patient is <u>new</u> to this health plan:

1. Has patient had an inadequate response to corticosteroid (e.g., prednisone, deflazacort) therapy? ☐ Yes ☐ No
2. Is Vyondys 53 medically necessary* for the patient? ☐ Yes ***Please submit documentation*** ☐ No

*Please note that **Vyondys 53 is considered not medically necessary for DMD in patients who have a confirmed mutation of the DMD gene that is amenable to skipping based on the following**:

a. Golodirsen does not have proven efficacy in the treatment of DMD
   i. **Vyondys 53 was approved based on an observed increase in dystrophin in skeletal muscle, but it is unknown if that increase is clinically significant.** Currently there is no clear threshold for the amount of dystrophin increase required to produce clinical benefit. Previous research has suggested dystrophin levels of at least 20-29% of normal are needed to avoid muscular dystrophy, and levels of at least 10% of normal can produce a more mild form of dystrophy. At week 180 of Vyondys 53's pivotal study (Study 1, a 24-week randomized controlled trial, and Study 2, a 212-week open-label extension trial; N=12), Golodirsen-treated patients had mean dystrophin levels that were only 0.93% of normal per Western blot analysis. In addition, a third study (Study 3, a 48-week open-label trial; N=13) found that the mean change in dystrophin from baseline after 48 weeks of treatment was 0.28% of normal per Western blot analysis; the median increase in dystrophin was 0.1%.
   ii. **The pivotal study for approval is not reliable.** The observed increase in dystrophin was primarily measured as percentage of dystrophin-positive fibers, which does not reflect the actual quantity of dystrophin present. The reliability of the pivotal study for approval (Study 1 and Study 2) has been questioned by FDA Office of Drug Evaluation director Ellis Unger, MD, and FDA chief scientist Luciana Borio, MD, who both called for retraction of the study.
   iii. **True clinical benefit has not been established.** There was no statistically significant difference in change in the 6-minute walk test (6MWT) distance, a clinical outcome measure used to assess disease progression, between Golodirsen-treated patients and placebo-treated patients. Of note, half of the patients receiving Golodirsen 30 mg/kg/week (n/N=2/4) lost the ability to ambulate. One of these patients continued to decline in ambulatory function despite a consistent increase in dystrophin-positive fibers.

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Furthermore, although the results of an external control comparison suggest Golodirsen may slow decline of ambulation as evidenced by improvements in the 6MWT, these observations are considered insufficient evidence to support clinical benefit of Golodirsen given the small sample size, variability in the DMD disease course, and known limitations with using historical control groups.

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b. **There is an alternative treatment option** (corticosteroids) with well-established efficacy in slowing decline of muscle strength and function (including motor, respiratory, and cardiac).

Complete this section ONLY for indications <u>other</u> than DMD:

1. Has patient tried and failed, or is contraindicated to, accepted standards of care? ☐Yes ☐No
   If yes, submit documentation and answer the following:
   a. Please list all previous therapies: _____
   b. Was patient adherent to previously tried therapies? ☐Yes ☐No ☐No, patient intolerant to drug

Physician's Signature: _____ **Date:** _____ ☐DAW

INFORMATION BELOW IS TO BE COMPLETE BY THE HEALTH PLAN/EPS PA STAFF

Authorization Information

Authorization number:	**Decision Due Date:**
J-Code:	**Coverage:** ❑ State excludes ❑ COB (secondary)
Line of Business: ❑ Commercial ❑ Health Insurance Marketplace ❑ Medicaid ❑ Medicare	**Benefit:** ❑ Medical ❑ Pharmacy

Criteria:
❑ Centene Policy
Date Policy last reviewed/approved by plan (we want to be sure we are using the version approved by your plan): _____

❑ State Specific (please include policy)

Medicare only criteria for CY2019 and CY2020:
❑ PART B use LCD or NCD ❑ PART D use the Medicare Part D Vyondys 53 specific criteria