JOHNS HOPKINS MEDICINE / JOHNS HOPKINS HEALTHCARE	Johns Hopkins HealthCare LLC **Pharmacy Public** **Medical Management Drug Policies**	*Policy Number*	MMDP033
		Effective Date	05/08/2020
		Review Date	05/08/2020
	Subject **Vyondys 53**	*Revision Date*	11/10/2021
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This document applies to the following Participating Organizations:

US Family Health Plan

Keywords: Vyondys 53

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I. POLICY

Vyondys 53 (golodirsen) will require prior authorization to ensure appropriate use. The process for initiating a prior authorization request can be found in policy PHARM 20.

II. POLICY CRITERIA

A. **Vyondys 53** may be approved for patients meeting ALL the following:
   1. Patient is at least 6 years of age
   2. Documented diagnosis of Duchenne Muscular Dystrophy (DMD)
   3. Documentation showing the patient has the mutation of the DMD gene that is amenable to exon 53 skipping
   4. Documentation showing the patient is ambulatory supported by:
      a. An independent 6 -minute walk test distance of greater than or equal to 250 meters, and one of the following:
         I. North Star Ambulatory Assessment score of greater than 17
         II. Gower's test (time to rise from the floor) of less than 7 seconds
   5. Documentation showing the patient has stable pulmonary and cardiac function, evidenced as:
      a. Left ventricular ejection fraction greater than or equal to 50%
      b. Percent predicted forced vital capacity of at least 50% and no requirement for nocturnal ventilation
   6. Documentation showing baseline glomerular filtration rate (GFR)
   7. Patient has been on a stable, optimized dose of corticosteroids for at least 6 months
   8. Vyondys 53 has been prescribed by, or in consultation with, a neurologist with expertise in treating DMD

III. AUTHORIZATION PERIOD/LIMITATIONS

A. Initial therapy may be approved for 3 months
B. Continuation of therapy may be approved in 6 months intervals with documentation showing ALL the following:
   1. Patient continues to be ambulatory without assistance
   2. Patient has had stabilization, improvement, or reduced disease progression, supported by a 6-minute walk test result of 250 meters or greater
   3. Patient continues to have stable cardiac and pulmonary function

4. Patient is being monitored monthly for signs of renal toxicity, supported by recent renal function tests
5. Patient is compliant with all scheduled medication administrations
6. Vyondys 53 dosing does not exceed 30mg/kg once weekly
7. Patient is not receiving any other exon skipping therapy

IV. EXCLUSIONS

A. Vyondys 53 will not be approved for any of the follow:
1. Concurrent use with other exon skipping therapies
2. Dosages greater than 30mg/kg infused once weekly
3. Other muscular dystrophy disorders
4. Patients who have lost ambulation. There is no literature evidence supporting a benefit in patients with advanced muscle deterioration and disease. Vyondys 53 has only been investigated in ambulatory patients.
5. Any indications or usages that are not FDA-approved, or guideline-supported

V. RECOMMENDED DOSAGE

Please refer to the FDA-approved prescribing information for indication-specific dosing details.

VI. CODES

Note: The following CPT/HCPCS codes are included below for informational purposes. Inclusion or exclusion of a CPT/ HCPCS code(s) below does not signify or imply member coverage or provider reimbursement. The member's specific benefit plan determines coverage.

Medication	HCPCS/CPT Code
Vyondys 53 100mg/2mL Solution Injection, golodirsen, 10mg	J1429

VII. REFERENCES

1. Vyondys 53 [Prescribing Information]. Cambridge, MA: Sarepta Therapeutics Inc; December 2019
2. Frank DE, Schnell FJ, Akana C, et.al. Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy. Neurology. 2020 Mar 5. Available at: https://n.neurology.org/content/neurology/early/2020/03/04/WNL.0000000000009233.full.pdf. Accessed on 4/22/2020.
3. Gloss D, Moxley RT, Ashwal S, et. al. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. 2016 Feb 2;86(5):465-72.

VIII. APPROVALS

Signature on file at JHHC

DATE OF REVISION	SUMMARY OF CHANGE
05/04/2020	Policy Creation
11/10/2021	Removed Priority Partners and EHP as applicable LOBs

Review Date: 05/08/2020

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