Mass General Brigham
Health Plan

Vyondys 53 (golodirsen)
Effective 12/01/2020

Plan	☐ MassHealth UPPL ☒Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☐ Pharmacy Benefit ☒ Medical Benefit		
Specialty Limitations	N/A		
Contact Information	**Medical and Specialty Medications** All Plans | Phone: 877-519-1908 | Fax: 855-540-3693		
	Non-Specialty Medications All Plans | Phone: 800-711-4555 | Fax: 844-403-1029		
Exceptions	N/A		

Overview
Golodirsen 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 53 skipping.

Coverage Guidelines
Authorization may be granted for members who are currently receiving treatment with Vyondys 53 excluding when the product is obtained as samples or via manufacturer's patient assistance program, AND the member meets all the reauthorization criteria
OR
Authorization may be approved when all of the following criteria are met, and documentation has been provided:

1. The member has a diagnosis of DMD with medical records confirming a mutation of the DMD gene that is amenable to exon 53 skipping
2. The prescribing physician is a neurologist or a provider who specializes in the treatment of DMD
3. The member is ambulatory as defined by a current six-minute walk test (6MWT - distance walked in six minutes in meters) of ≥ 200 meters (test must have been observed or completed by the treating provider, or ordered by the treating provider and completed by a qualified medical practitioner)
4. Member has been receiving a stable dose of corticosteroids for a period of at least 6 months **OR** the member has a contraindication to corticosteroids
5. The member is not on concomitant therapy with other DMD-directed antisense oligonucleotides

Reauthorizations:
Reauthorizations may be approved for 12 months when **ALL** the following is met:

1. The member remains ambulatory as defined by a current 6MWT of ≥ 200 meters (test must have been observed or completed by the treating provider, or ordered by the treating provider and completed by a qualified medical practitioner)

2. The member has a stable or improving pattern of 6MWTs as shown in medical records with results of a pretreatment baseline and all interim results (all previous 6MWTs results must be included)
3. The member continues to utilize corticosteroids in combination with the requested agent **OR** the member has a contraindication to corticosteroids

Limitations

1. Approvals will be granted for 6 months.

References

1. Vyondys 53 (golodirsen) [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; March 2020
2. Frank DE, Schnell FJ, Akana C, et al. Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy. Neurology 2020; 94:e2270
3. FDA grants accelerated approval to first targeted treatment for rare Duchenne muscular dystrophy mutation. https://www.fda.gov/news-events/press-announcements/fda-grants-accelerated-approval-first-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation (Accessed on December 17, 2019).

Review History

09/16/2020: Created and Reviewed at Sept P&T Meeting. Effective 12/01/2020.