

Agamree (vamorolone)
Effective 07/01/2025

Plan	<input type="checkbox"/> MassHealth UPPL <input checked="" type="checkbox"/> Commercial/Exchange	Program Type	<input checked="" type="checkbox"/> Prior Authorization <input type="checkbox"/> Quantity Limit <input type="checkbox"/> Step Therapy
Benefit	<input checked="" type="checkbox"/> Pharmacy Benefit <input type="checkbox"/> Medical Benefit		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	Medical Benefit Pharmacy Benefit	Phone: 833-895-2611 Phone: 800-711-4555	Fax: 888-656-6671 Fax: 844-403-1029
Exceptions	N/A		

Overview

Agamree (vamorolone) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

Coverage Guidelines

Authorization may be reviewed for members new to the plan within the last 90 days who are currently receiving treatment with the requested medication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

OR

Authorization may be granted for members when ALL the following criteria are met:

1. Member has a diagnosis of Duchenne muscular dystrophy (DMD) that has been confirmed by genetic testing demonstrating a mutation in the DMD gene. Laboratory confirmation of genetic testing is required.
2. Member is 2 years of age or older.
3. Member meets one of the following:
 - a. Member had a clinically significant adverse reaction to treatment with prednisone or prednisolone (e.g., clinically significant weight gain, Cushingoid appearance, psychiatric/behavioral issues persisted beyond the first 6 weeks of therapy, etc)
 - b. Treatment with prednisone or prednisolone is clinically inappropriate for the member
4. Requested medication is prescribed by or in consultation with a specialist with experience treating DMD

Continuation of Therapy

Requests for reauthorization will be approved when the following criteria are met:

1. Member meets initial criteria
2. Documentation the member has responded to treatment with vamorolone. Examples include motor function tests (e.g., 6 minute walk test [6MWT], time to stand test [TTSDT], time to walk/run [TTWR]) or pulmonary function tests.

Limitations

1. Initial approvals will be granted for 6 months.
2. Reauthorization approvals will be granted for 12 months.
3. The following quantity limitations apply:

Drug Name and Dosage Form	Quantity Limit
Agamree oral suspension	7.5 mL per day

References

1. Agamree [package insert], Coral Gables, FL: Catalyst Pharmaceuticals, Inc.; June 2024.
2. Angelini C, Pegoraro E, Turella E, Intino MT, Pini A, Costa C. Deflazacort in Duchenne dystrophy: study of long-term effect. *Muscle Nerve*. 1994;17(4):386-91.
3. Birnkrant DJ, Bushby K, Bann CM, et al for the DMD Care Considerations Working Group. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *Lancet Neurol*. 2018[a]; 17(3):251-267. doi: 10.1016/S1474-4422(18)30025-3.
4. Birnkrant DJ, Bushby K, Bann CM, et al for the DMD Care Considerations Working Group. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurol*. 2018[b]; 17(4):347-361. doi: 10.1016/S1474-4422(18)30025-5.
5. Birnkrant DJ, Bushby K, Bann CM, et al for the DMD Care Considerations Working Group. Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency management, psychosocial care, and transitions of care across the lifespan. *Lancet Neurol*. 2018[c]; 17(5):251-267. doi: 10.1016/S1474-4422(18)30026-7.
6. Bonifati MD, Ruzza G, Bonometto P, et al. A multicenter, double-blind, randomized trial of deflazacort versus prednisone in Duchenne muscular dystrophy. *Muscle Nerve*. 2000;23(9):1344-7.
7. Catalyst Pharmaceuticals. Press release: Catalyst Pharmaceuticals reports FDA approval of Agamree (vamorolone) for Duchenne muscular dystrophy granted to Santhera Pharmaceuticals. Catalyst Pharmaceuticals Web site. October 26, 2023. <https://ir.catalystpharma.com/news-releases/news-release-details/catalyst-pharmaceuticals-reports-fda-approval-agamreer>. Accessed February 15, 2024.
8. Conklin LS, Damsker JM, Hoffman EP, et al. Phase IIa trial in Duchenne muscular dystrophy shows vamorolone is a first-in-class dissociative steroid anti-inflammatory drug. *Pharmacol Res*. 2018; 136:140-150. doi: 10.1016/j.phrs.2048.09.007.
9. Cowen L, Mancini M, Martin A, et al. Variability and trends in corticosteroid use by male United States participants with Duchenne muscular dystrophy in the Duchenne Registry. *BMC Neurol*. 2019;19(1):84. doi: 10.1186/s12883-019-1304-8.
10. Dang UJ, Damsker JM, Guglieri M, et al. Efficacy and safety of vamorolone over 48 weeks in boys with Duchenne muscular dystrophy: a randomized controlled trial. *Neurology*. 2024;102(5):e208112. doi: 10.1212/WNL.00000000000208112.
11. Darras BT. Duchenne and Becker muscular dystrophy: clinical features and diagnosis. UpToDate Web site. Updated June 22, 2022. <http://www.uptodate.com>. Accessed February 7, 2024.
12. Darras BT. Duchenne and Becker muscular dystrophy: genetics and pathogenesis. UpToDate Web site. Updated January 27, 2023[a]. <http://www.uptodate.com>. Accessed February 7, 2024.
13. Darras BT. Duchenne and Becker muscular dystrophy: glucocorticoid and disease-modifying treatment. UpToDate Web site. Updated January 17, 2024. <http://www.uptodate.com>. Accessed February 7, 2024.
14. Darras BT. Duchenne and Becker muscular dystrophy: management and prognosis. UpToDate Web site. Updated October 12, 2023[b]. <http://www.uptodate.com>. Accessed February 7, 2024.
15. Gloss D, Moxley RT 3rd, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology*. 2016;86(5):465-72.



16. Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. *Neurology*. 2016;87(20):2123-2131.
17. Gronseth GS, Cox J, Gloss D, et al. American Academy of Neurology clinical practice guideline process manual, 2017 edition. AAN Web Site. <https://www.aan.com/practice/what-are-clinical-practice-guidelines>. Accessed February 8, 2024.
18. Grounds MD, Lloyd EM. Considering the promise of vamorolone for treating Duchenne muscular dystrophy. *J Neuromuscul Dis*. 2023;10(6):10013-1030. doi: 10.3233/JND-230161.
19. Guglieri M, Bushby K, McDermott MP, et al. Effect of different corticosteroid dosing regimens on clinical outcomes in boys with Duchenne muscular dystrophy: a randomized clinical trial. *JAMA*. 2022[a];327(15):1456-1468. doi: 10.1001/jama.2022.4315.
20. Guglieri M, Clemens PR, Perlman SJ, et al. Efficacy and safety of vamorolone vs placebo and prednisone among boys with Duchenne muscular dystrophy: a randomized clinical trial. *JAMA Neurol*. 2022[b]; 79(10):1005-1014. doi: 10.1001/jamaneurol.2022.2480.
21. Hoffman EP, Schwartz BD, Mengle-Gaw LJ, et al. Vamorolone trial in Duchenne muscular dystrophy shows dose-related improvement of muscle function. *Neurology*. 2019;93(13):e1312-1323. doi: 10.1212/WNL.0000000000008168.
22. Institute for Clinical and Economic Review (ICER). The effectiveness and value of deflazacort and exon-skipping therapies for the management of Duchenne muscular dystrophy. ICER Web site. Updated August https://icer.org/wp-content/uploads/2020/10/ICER_DMD-Final-Report_081519-2-1.pdf. Accessed February 21, 2024.
23. Joseph S, Wang C, Bushby K, et al for the UK NorthStar Clinical Network. Fractures and linear growth in a nationwide cohort of boys with Duchenne muscular dystrophy with and without glucocorticoid treatment: results from the UK NorthStar database. *JAMA Neurol*. 2109;76(6):701-709. doi: 10.1001/jamaneurol.2019.0242.
24. Kourakis S, Timpani CA, Campbell DG, et al. Standard of care versus new-wave corticosteroids in the treatment of Duchenne muscular dystrophy: can we do better? *Ophanet J Rare Dis*. 2021;16(1):117. doi: 10.1186/s13023-021-01758-9.
25. Lamb MM, West NA, Ouyang L, et al. Corticosteroid treatment and growth patterns in ambulatory males with Duchenne muscular dystrophy. *J Pediatr*. 2016;173:207-2013.e3. doi: 10.1016/j.peds.2016.02.067.
26. Leigh F, Ferlini A, Biggar D, et al. Neurology care, diagnostics, and emerging therapies of the patient with Duchenne muscular dystrophy. *Pediatrics*. 2018;142(Suppl 2):S5-S16. doi: 10.1542/peds.208-0333C.
27. Mah JK, Clemens PR, Guglieri M, et al. Efficacy and safety of vamorolone in Duchenne muscular dystrophy: a 30-month nonrandomized controlled open-label extension trial. *JAMA Netw Open*. 2022;5(1):e2144178. doi: 10/1001/jamanetworkopen.2021.44178.
28. Marden JR, Freimark J, Yao Z, et al. Real-world outcomes of long-term prednisone and deflazacort use in patients with Duchenne muscular dystrophy: experience at a single, large care center. *J Comp Eff Res*. 2020;9(3):177-189. Doi: 10.2217/cer-2019-0170.
29. Matthews E, Brassington R, Kuntzer T, Jichi F, Manzur AY. Corticosteroids for the treatment of Duchenne muscular dystrophy. *Cochrane Database Syst Rev*. 2016;(5):CD003725.
30. McDonald CM, Henricson EK, Abresch RT, et al for the CINRG Investigators. Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study. *Lancet*. 2018;391(10119):451-461. doi: 10.1016/S0140-6736(17)32160-8.
31. Mesa LE, Dubrovsky AL, Corderi J, Marco P, Flores D. Steroids in Duchenne muscular dystrophy--deflazacort trial. *Neuromuscul Disord*. 1991;1(4):261-6.



32. North Star Clinical Network. North Star Ambulatory Assessment. Updated September 30, 2020. https://www.musculardystrophyuk.org/static/s3fs-public/2021-08/NSAA%20_Manual_%2015102020.pdf?VersionId=BaPGDWk5TxA3rtF2DDipAVYIOJ5Eoumo. Accessed February 21, 2024.
33. Quinlivan R, Messer B, Murphy P, et al on behalf of the ANSN. Adult North Star Network (ANSN): consensus guideline for the standard of care of adults with Duchenne muscular dystrophy. *J Neuromuscul Dis.* 2021;8(6):899-926. doi: 10.3233/JND-200609.
34. Reitter B. Deflazacort vs. prednisone in Duchenne muscular dystrophy: trends of an ongoing study. *Brain Dev.* 1995;17 Suppl:39-43.
35. Smith EC, Conklin LS, Hoffman EP, et al. Efficacy and safety of vamorolone in Duchenne muscular dystrophy: an 18-month interim analysis of a non-randomized open-label extension study. *PLoS Med.* 2020;17(9):e1003222. doi: 10.1371/journal.pmed.1003222.

Review History

09/11/2024 – Reviewed at September P&T. Effective 11/01/2024.

06/11/2025 – Reviewed at June P&T. Removed deflazacort step. Effective 07/01/2025.

