Office of Medicaid
BOARD OF HEARINGS

Appellant Name and Address:

[redacted]

Appeal Decision:	Denied	**Appeal Number:**	2301000
Decision Date:	5/3/2023	**Hearing Date:**	03/10/2023
Hearing Officer:	Casey Groff, Esq.		

Appearances for Appellant:

[redacted]

Appearances for MassHealth:

Meghan Serell, R.Ph., Pharm.D., Appeals Reviewer, Drug Utilization Review (DUR); Andrew Coelho, R.Ph., Pharm.D., Clinical Consultant Pharmacist, DUR

The Commonwealth of Massachusetts
Executive Office of Health and Human Services
Office of Medicaid
Board of Hearings
100 Hancock Street, Quincy, Massachusetts 02171

APPEAL DECISION

Appeal Decision:	Denied	**Issue:**	Prior Authorization; Drug
Decision Date:	5/3/2023	**Hearing Date:**	03/10/2023
MassHealth's Reps.:	Consultants of Drug Utilization Review Board	**Appellant's Reps.:**	*Pro se*; Providers of UMass Mem. Ctr DMD Program
Hearing Location:	Board of Hearings (Remote)	**Aid Pending:**	No

Authority

This hearing was conducted pursuant to Massachusetts General Laws Chapter 118E, Chapter 30A, and the rules and regulations promulgated thereunder.

Jurisdiction

On January 5, 2023, MassHealth denied Appellant's prior authorization (PA) request for Viltepso IV infusions (250 Mg/5 ml vial). See Exh. 2; see also Exh. 4, p. 54. Appellant filed a timely appeal with the Board of Hearings (BOH) on February 6, 2023. See Exhibit 1. Denial of a PA request is a valid basis for appeal. See 130 CMR 610.032.

Action Taken by MassHealth

MassHealth denied the appellant's PA request for Viltepso

Issue

The appeal issue is whether MassHealth erred in denying Appellant's PA request for Viltepso.

Summary of Evidence

MassHealth was represented at hearing by two pharmacists from the Drug Utilization Review (DUR) program. Dr. Serell, a DUR clinical pharmacist consultant, testified that Viltepso is a medication 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. See Exh. 4, p. 75. According to the drug's package insert, the drug's indication "is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with Viltepso … Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial." Id.[1]

MassHealth has established clinical criteria for prior authorization (PA) for Viltepso which MassHealth has published on the MassHealth Drug List, a publicly available document. Through this criteria, MassHealth requires, as a condition of coverage, that the member be "ambulatory" as defined by a current six-minute walk test (6MWT) of at least 200 meters, as well as evidence that the member has completed all five of the following timed function testis, with reported baseline measurements:

- timed ten-minute walk/run; and
- timed floor (supine) to stand; and
- timed four-step descend; and
- timed four-step climb; and
- timed sit to stand.

See Exhibit 4.

Additional PA criteria include requirements that the member has the appropriate diagnosis and genetic test results; that the drug is being prescribed by a neuromuscular neurologist and at an

[1] The drug packaging insert describes the study upon which approval was based, in relevant part, as follows:

> During the initial period (first 4 weeks) of Study 1, patients were randomized (double blind) to Viltepso or placebo. All patients then received 20 weeks of open-label Viltepso 40 mg/kg once weekly (0.5 times the recommended dosage) (N=8) or 80 mg/kg once weekly (N=8). Study 1 enrolled ambulatory male patients [redacted] of age (median age [redacted] years) on a stable corticosteroid regimen for at least 3 months….Efficacy was assessed based on change from baseline in dystrophin protein level …at Week 25. Muscle biopsies (left or right biceps brachii) were collected from patients at baseline and following 24 weeks of Viltepso treatment, …In patients who received Viltepso 80 mg/kg once weekly, mean dystrophin levels increased from 0.6% (SD 0.8) of normal at baseline to 5.9% (SD 4.5) of normal by Week 25, with a mean change in dystrophin of 5.3% (SD 4.5) of normal levels (p=0.01) as assessed by validated Western blot (normalized to myosin heavy chain); the median change from baseline was 3.8%. All patients demonstrated an increase in dystrophin levels over their baseline values. As assessed by mass spectrometry (normalized to filamin C), mean dystrophin levels increased from 0.6% (SD 0.2) of normal at baseline to 4.2% (SD 3.7) of normal by Week 25, with a mean change in dystrophin of 3.7% (SD 3.8) of normal levels (nominal p=0.03, not adjusted for multiple comparisons); the median change from baseline was 1.9%. See Exh. 4, p. 77.

appropriate weekly dosage; and evidence that the member has received, and will continue to use, corticosteroids in combination with the requested agent. Id. Each criterion must be supported through documentation submitted in the PA request for MassHealth to find medical necessity for the drug has been met.

Next, Dr. Serell reviewed the contents of the PA request at issue, which was received by MassHealth's DUR program on January 4, 2023 and signed by [redacted] the Appellant's treating neurologist. According to PA request, [redacted] sought approval of Viltepso (viltolarsen), a weekly IV infusion on behalf of Appellant, a MassHealth member over the age of [redacted], for the treatment of DMD. See Exh. 4, p. 3. The requested dosage was 4400 mg weekly, which is supplied as 250mg/5ml vials Id. at 10-11. The PA request confirmed that Appellant currently takes Prednisone, a corticosteroid, which he has been on since 2007. Id. at 4.

Next, the PA form, which is designed specifically for "neuromuscular agents," listed a series of questions regarding the member's ambulatory status and results of timed function tests. [redacted] checked "No" in response to the question of whether Appellant was ambulatory as defined by a current 6MWT of at least 200 meters, and repeatedly answered "n/a patient is non ambulatory" in response to each section requesting results of previously observed 6MWTs, as well as the five timed function tests (listed above). Id.

Dr. Serell testified that [redacted] wrote a letter of medical necessity dated 1/3/23, in support of the requested treatment, which was included in the PA request. In the letter [redacted] provided a summary of Appellant's medical history and an overview of DMD and its clinical progression. In describing her "rational for treatment," [redacted] wrote, in part, the following:

> DMD is caused by mutations in the DMD gene on the X chromosome that results in little or no production of dystrophin, a protein that supports muscle health. Exon skipping is a treatment strategy in which sections of genetic code are "skipped" (spliced out or left out) during the protein manufacturing process. This treatment strategy allows cells to create a shortened dystrophin protein that contains essential functional portions. The FDA-approved label presents the opportunity to increase dystrophin in patients with mutations amenable to skipping exon 53 **regardless of age or ambulation status.** In addition, data from published literature strongly supports the idea that low-level increases in dystrophin production are reasonably likely to predict clinical benefit… [2]
>
> …
>
> In the best medical judgement of our team at the UMass DMD Program, it is our opinion that [Appellant] needs weekly infusions of Viltepso to treat his DMD. Without weekly infusions of Viltepso and the dystrophin the treatment has been proven to produce based on clinical trial data, we know that his muscles will continue to weaken and atrophy, resulting in patient morbidity and eventually mortality. As previously stated, DMD is a progressive disease, with effects on

[2] [redacted] cited to, and described, the underlying studies that support this position.

> muscle loss that are irreversible. DMD has already deprived [Appellant] of his ability to perform activities of daily living, including walking, dressing, toileting and preparing meals without assistance. However, [Appellant's] condition is currently stable with no significant loss of cardiopulmonary function or upper extremity function in the last l9 months. Therefore, it is my expert medical opinion that treatment with Viltepso must be initiated in order to further slow the loss of [Appellant's] ability to perform additional and important activities of daily living as well as to maintain his pulmonary function.

See Exh. 4 at 7-9 (citations omitted).

[redacted] also pointed out that the FDA's accelerated approval of Viltespo is not a partial or interim approval, but a full FDA approval, meeting the safety and efficacy standards outlined in FDASIA. Id.

Dr. Serell next reviewed medical documentation included in the PA submission showing Appellant had neurology encounters on May 18, 2022 and November 9, 2022 with [redacted], and Karen Courtney Shellenbarger, N.P. See id. at 12 - 52. Progress notes from both visits indicated that Appellant was overall in good health with his neuromuscular function remaining "stable" over the past year. According to the notes, Appellant was non-ambulatory and unable to stand independently; he operates a joystick power wheelchair which he obtained in May of 2019; he is able to feed himself and drink on his own; he requires partial assistance with toileting, and full assistance with dressing and turning in bed. Id. at 12-18. Under the section for clinical "impression," the provider reported that Appellant "again demonstrates stable non-ambulatory function as evidenced by a stable PUL score of 12/42, Gr 2 proximal and Grade 3-4 distal UE strength and continued independent feeding skills." Id. at 18-38. Additionally, the documentation reflected that from July 2020 through June of 2021, Appellant received infusions of Vyondys – a different exon skipping agent indicated for treatment of DMD. However, he discontinued this medication on the basis that there had been no perceived clinical benefit. Id. at 20.

Dr. Serell testified that the PA request lacked documentation to establish Appellant was "ambulatory" or that he had baseline measurements of the required five timed function tests, which are required to demonstrate medical necessity for Viltepso. Thus, through a notice dated January 5, 2023, MassHealth denied Appellant's PA request for Viltepso 250 mg/5ml based on the following rational: "[i]nformation provided did not contain sufficient information to determine medical necessity…." Id. at 54. [3]

[3] Following Appellant's request to appeal the decision, MassHealth notified Appellant, through a letter dated February 21, 2023, that resolution could be achieved upon providing evidence demonstrating Appellant is ambulatory, as well as documentation of his baseline measurements for the five timed function tests. Id. at 56. On March 3, 2023, DUR received documentation from Appellant's provider; however, it contained previously submitted information, and did not establish the requisite components for approval of the drug.

Appellant appeared at the hearing, along with his treating neurologist and nurse practitioner, both of whom are from the DMD Program at UMASS Memorial Children's Medical Center.[4] First, Appellant provided background on his condition indicating that his is [redacted] and suffers from DMD – a fatal genetic disorder characterized by the progressive loss of muscle. It is a multi-systemic condition and effects many parts of the body and results in deterioration of the skeletal, cardiac heart, and pulmonary lung muscles. Appellant explained that although he is unable walk, he still retains the ability to manage aspects of his daily life including management of his medical care; engaging in interests such as using the computer for gaming and chatting with friends; and the basic functions of being able to breathe, eat, use the bathroom, operate his power wheelchair, and the small things like scratching an itch. Appellant noted that as he gets older, he is losing the ability to do these tasks independently and this will progress. DMD does not just take away the ability to walk or use arms, but also it will ultimately take away his ability to use all of his upper body, use his wheelchair, breathe without a ventilator, and ultimately his heart's ability to function, costing him his life.

Appellant asserted that he feels MassHealth is discriminating against him by denying coverage of this drug. If he were able to gain access to Viltepso – a drug made specifically for his genetic mutation - he would not continue down the path his body is headed, and this could possibly save his life. Appellant explained that as someone with DMD, walking is overrated; rather it is stability that is desired, and he wishes to retain a comfortable life.

Appellant expressed frustration with the PA process, noting that the rational for denial was that his PA did not contain "sufficient information to establish medical necessity" and instructed him to resubmit the PA with complete clinical information for reconsideration. The actual reason for the denial, however, was not based on medical necessity, but through a restricted policy requiring that the member be able to walk over 200 meters under 6-minutes. This policy discriminates against people with this disease who are no longer walking. Further it suggests that once a patient loses the ability to walk, their life is no longer important.

Appellant argued that nowhere on the FDA label for Viltepso does it require the patient walk. The sole goal is to stabilize treatment to slow the progression of muscle loss. Appellant argued that his case meets the definition of medical necessity under 130 CMR § 450.204 which he read in-full into the record. Viltepso, Appellant explained, was approved on the FDA accelerated approval pathway, which allows a surrogate endpoint to evaluate the safety and efficacy of therapies for serious conditions that have an unmet need. Here, the surrogate endpoint is production of dystrophin, a protein found in skeletal muscles. DMD patients lack this protein causing muscle degeneration. Therefore, Appellant argued, this medication is reasonably likely to result in clinical benefit, consistent with the first component of the medical necessity definition. Additionally, Viltepso treats a small subset of DMD patients. There is only one other drug in its class approved for DMD treatment, which has less robust data than Viltepso, and is

[4] Appellant's neurologist is also the Director of the DMD Program at UMass Medical Center and a professor of pediatric neurology.

more costly. Prednisone is not a comparable medication, as it is a steroid and does not produce dystrophin. As there is no "comparable" drug less costly to the agency, the second component of the medical necessity definition is met.

Finally, Appellant explained that Medicaid, through the drug rebate program, approves coverage of drugs of their medically accepted indication. Here, Viltepso's clinically approved indication is for any DMD patient with gene mutation amendable to exon 53 skipping regardless of age, ambulatory status, or any other paramotor. Appellant asserted that his doctor has prescribed this medication for its FDA approved medically accepted indication for on label use and by law the state must cover the drug. To withhold coverage because of an inability to walk is unlawful and discriminatory. Accordingly, Appellant requested BOH overturn the MassHealth decision.

Next [redacted] testified that in her medical opinion, Viltepso is medically necessary to treat Appellant and maintain his clinical stability. Based on the medical literature, [redacted] explained, there is strong evidentiary support that low levels of increased dystrophin production will result in a clinical benefit. The underlying study of Viltepso showed that, over a course of treatment, the drug resulted in a 5.9 percent increase of dystrophin production. By improving the amount of dystrophin, research has shown improvements, and the expectation is for better outcomes. Dr Wong acknowledged that it is too soon to tell if Viltepso is beneficial for non-ambulatory patients; however, given that it is proven to replenish dystrophin, it is expected to have beneficial impact not just on muscles in the legs for walking, but in the arm and respiratory muscles as well.

Next, [redacted] agreed with Appellant's assertion that walking or ambulation is not a good criterion for establishing medical necessity for this drug. Rather, in young adult DMD patients, the goal of treatment is to achieve stable pulmonary function - a key factor in maintaining health. Appellant's last measured pulmonary function test (PFT) was at 41% and that has remained stable for the last year. This percentage is way above PFT levels of patients on a full-time ventilator. This differential impacts not only one's quality of life, but additionally the level of care is significantly greater when one becomes a ventilated patient, including the cost of full-time nursing care. Overall, the young adult criteria for Viltepso should be focused on maintaining stable pulmonary function rather than ambulation.

In response to Appellant's testimony, the DUR representative explained that MassHealth creates PA requirements based on a review of the clinical literature, medical studies, and in consultation with licensed physicians and pharmacists. As indicated on the package insert, Viltepso's approval was based on a study consisting of ambulatory boys ages [redacted]. While the study showed an increased dystrophin production, it has yet to establish a clinical benefit. In its review of the medical literature and clinical trial evidence, MassHealth determined that Viltepso lacks the data to show reasonable expectation that it will improve or stabilize a condition or prevent worsening for members who are no longer ambulatory; nor is there available evidence to show Viltepso has any impact on respiratory or cardiac outcomes. As such, MassHealth chose to limit coverage to members that had the same criteria as were studied in the clinical trial.

When asked about potential alternative treatment options, Dr. Andrew Coehlo, a representative

from DUR, explained that traditionally corticosteroids have been the go-to therapy for DMD treatment. Another therapy is Vyondys 53, which is in the same class of neuromuscular agents as Viltepso. However, after trialing the drug for about a year, Appellant discontinued it due to lack of perceived efficacy. The two drugs work similarly, and Dr. Coehlo agreed that there is slightly better data for Viltepso than Vyondys; however clinical data is extremely lacking for both agents.[5]

Findings of Fact

Based on a preponderance of the evidence, I find the following:

1. Appellant is [redacted] and suffers from Duchenne muscular dystrophy (DMD); he has a confirmed genetic mutation of DMD gene amenable to exon 53 skipping.

2. DMD is caused by mutations in the DMD gene on the X chromosome that results in little or no production of dystrophin, a protein that supports muscle health.

3. Because DMD patients lack adequate dystrophin, their muscles (skeletal, cardiac, and pulmonary) continue to weaken and atrophy, resulting in progressive irreversible muscle loss, patient morbidity, and eventually mortality.

4. There is no cure for DMD, but the traditional go-to therapy has been corticosteroids, which is in a different therapeutic class than "exon-skipping" drugs aimed to replenish dystrophin in DMD patients.

5. Appellant is non-ambulatory and unable to stand independently; he operates a joystick power wheelchair which he obtained in May of 2019; he still retains the ability to feed himself, drink independently; engage in interests, such as using the computer for gaming and chatting with friends; he requires partial assistance with toileting, and full assistance with dressing and turning in bed.

6. On January 4, 2023, MassHealth's DUR program received a PA request on behalf of Appellant and signed by Brenda Wong, M.D., Appellant's treating neurologist, seeking approval of Viltepso (viltolarsen), a weekly IV infusion 250mg/5ml vial.

7. Viltepso is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

8. The PA form noted that Appellant is non-ambulatory and did not have current measured 6MWT or baseline measurements for any of the five timed function tests.

9. Appellant takes Prednisone, a corticosteroid, which he has been on since 2007.

[5] In response, Appellant reiterated his argument that steroids are not an "alternative" treatment, as they are not "exon-skipping," and do not have any role in producing dystrophin.

10. As of Appellant's most recent neurology visits on May 18, 2022 and November 9, 2022 he was found to demonstrate stable non-ambulatory function, with no significant loss of cardiopulmonary function or upper extremity function in the last l9 months, as evidenced by a stable PUL score of 12/42, Gr 2 proximal and Grade 3-4 distal UE strength and continued independent feeding skills.

11. From July 2020 through June of 2021, Appellant received infusions of Vyondys – a different exon skipping agent indicated for treatment of DMD; but ultimately discontinued this medication due to lack of efficacy.

12. MassHealth established the following clinical criteria for approval of Viltepso:

    a. Documentation that the member is "ambulatory" as defined by a current 6MWT of at least 200 meters, as well as evidence that the member has completed all five of the following timed function tests, with reported baseline measurements: timed ten-minute walk/run; and timed floor (supine) to stand; and timed four-step descend; and timed four-step climb; and timed sit to stand.

    b. Documentation that the member has the appropriate diagnosis and genetic test results; that the drug is being prescribed by a neuromuscular neurologist and at an appropriate weekly dosage; and evidence that the member has received, and will continue to use, corticosteroids in combination with the requested agent.

13. The PA criteria for Viltepso is published in the MassHealth Drug List.

14. In determining PA criteria for drugs in the MDL, MassHealth, in consultation with licensed physicians, pharmacists, and other specialists review the clinical literature and medical studies relevant to the specific drug.

15. Viltepso received FDA accelerated approval based on a study in which ambulatory boys, ages 4-10 with the specific DMD gene amenable to exon 53 skipping, who trialed the drug over a 25 week treatment period, showed a 5.9% increase in dystrophin production in skeletal muscle.

16. The increased production of dystrophin served as a "surrogate endpoint" for FDA accelerated approval of Viltepso and pending results that the drug offers a "clinical benefit" in a confirmatory trial, as indicated in the drug package insert.

17. To date, there has been no clinical study showing the that Viltepso offers a clinical benefit to non-ambulatory patients.

18. In its review of the drug's medical literature and clinical trial evidence, MassHealth concluded that Viltepso lacked the data to show reasonable expectation that it will improve or stabilize a condition or prevent worsening for members who are no longer ambulatory; nor is there available evidence to show Viltepso has any impact on respiratory or cardiac outcomes.

19. Based on its review, MassHealth opted to limit coverage of Viltepso to members that met the same clinical criteria as those studied in the trial, including the requirement the member be ambulatory.

20. MassHealth denied Appellant's PA request on January 5, 2023 because there was no evidence to indicate Appellant was ambulatory, a required condition of coverage.

Analysis and Conclusions of Law

This appeal concerns whether MassHealth erred in denying Appellant's request for coverage of Viltepso. Viltepso is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients, like Appellant, who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. MassHealth's denial of the drug was based on its determination that Appellant did not satisfy medical necessity criteria for drug approval.

Pursuant to agency regulation, MassHealth does not cover a medical service unless it is "medically necessary." The threshold considerations for determining whether a service is medically necessary are set forth under 130 CMR 450.204, which states, in full:
450.204: Medical Necessity

(A) A service is medically necessary if

(1) it is reasonably calculated to prevent, diagnose, prevent the worsening of, alleviate, correct, or cure conditions in the member that endanger life, cause suffering or pain, cause physical deformity or malfunction, threaten to cause or to aggravate a handicap, or result in illness or infirmity; and

(2) there is no other medical service or site of service, comparable in effect, available, and suitable for the member requesting the service, that is more conservative or less costly to the MassHealth agency. Services that are less costly to the MassHealth agency include, but are not limited to, health care reasonably known by the provider, or identified by the MassHealth agency pursuant to a prior-authorization request, to be available to the member through sources described in 130 CMR 450.317(C), 503.007, or 517.007.

(B) Medically necessary services must be of a quality that meets professionally recognized standards of health care, and must be substantiated by records including evidence of such medical necessity and quality. …

(C) A provider's opinion or clinical determination that a service is not medically necessary does not constitute an action by the MassHealth agency.

(D) Additional requirements about the medical necessity of MassHealth services are contained in other MassHealth regulations and medical necessity and coverage guidelines.

(emphasis added).

As subsection (D) indicates, MassHealth establishes additional medical necessity criteria throughout its regulations and publications governing specific health-related service-types. The authority to implement these "additional" requirements is derived from federal law governing state medical assistance programs, such as MassHealth. Specifically, state agencies are required to specify the "amount, duration, and scope of each services that it provides for [its members]." 42 C.F.R. § 440.230. Although it may not "arbitrarily reduce or deny services" based on a member's diagnosis or medical condition, the agency is permitted to "place appropriate limits on a service based on such criteria as medical necessity or utilization control procedures." See id.

The federal Medicaid statute's provisions for coverage of outpatient prescription drugs (also referred to herein as "Medicaid-eligible drugs") are set forth in 42 U.S.C. § 1396r–8. The statute requires, generally, that state medical assistance programs provide coverage for "any covered outpatient drug."[6] However, the statute further permits states to exclude or otherwise restrict coverage (i.e. deny reimbursement) of a covered outpatient drug under any one of the following four circumstances:

> (i) the prescribed use is not for a medically accepted indication (as defined in subsection (k)(6));[7]
> (ii) the drug is contained in the list referred to in paragraph (2);[8]
> (iii) the drug is subject to such restrictions pursuant to an agreement between a manufacturer and a State authorized by the Secretary under subsection (a)(1) or in effect pursuant to subsection (a)(4); or
> ***(iv) the State has excluded coverage of the drug from its formulary established in accordance with paragraph (4).***

See 42 U.S.C. § 1396r-8(d)(1)(B)(emphasis added).

The restriction on Viltepso, at issue in this case, is a result of the permitted exclusion in section (iv) above, and the provisions incorporated therein by reference. Under subsection (d)(4) of the federal Medicaid statute, states may establish a "formulary" if it meets the following requirements:

> (A) The formulary is developed by a committee consisting of physicians, pharmacists, and other appropriate individuals … [or the State's DUR Board].

[6] A "covered outpatient drug" is a drug which may be dispensed only upon prescription and which is approved for safety and effectiveness as a prescription drug under the federal Food, Drug, and Cosmetic Act. § 1396r–8(k)(2)(A).
[7] The term "medically accepted indication" means "any use for a covered outpatient drug which is approved under the Federal Food, Drug, and Cosmetic Act…***or*** the use of which is supported by one or more citations included or approved for inclusion in any of the compendia described in subsection (g)(1)(B)(i)." See 42 USC § 1396r-8(k)(6).
[8] The listed drugs in subsection (2) include drugs used for cosmetic purposes, or found by HHS to be subject to clinical abuse or inappropriate use.

(B) Except as provided in subparagraph (C), the formulary includes the [Medicaid-eligible drugs]… (other than any drug excluded from coverage or otherwise restricted under paragraph (2)).
(C) A covered outpatient drug may be excluded with respect to the treatment of a specific disease or condition for an identified population (if any) only if, based on the drug's labeling…***the excluded drug does not have a significant, clinically meaningful therapeutic advantage in terms of safety, effectiveness, or clinical outcome of such treatment for such population*** over other drugs included in the formulary and there is a written explanation (available to the public) of the basis for the exclusion.
(D) ***The State plan permits coverage of a drug excluded from the formulary…pursuant to a prior authorization program*** that is consistent with paragraph (5).

….

See 42 USC §1396r-8(d)(4) (emphasis added).

In other words, the federal Medicaid statute permits a state (through its formulary committee) to remove a Medicaid-eligible drug from the formulary, with the result that the drug will no longer be covered for reimbursement, if the drug does not have the requisite clinical evidence of efficacy described in subsection (C), above. Additionally, subsection (D) above, incorporates by reference, a provision governing state agency "prior authorization programs," the requirements of which, are applicable to all states, regardless of whether it has established a formulary. Under this provision, state Medicaid agencies "may require, as a condition of coverage or payment for a [Medicaid-eligible drug], the approval of the drug before its dispensing for any medically accepted indication…."[9] See 42 USC §1396r-8(d)(5).

Consistent with these directives, MassHealth established the MassHealth Drug List (MDL) – a "formulary" within the meaning of §1396r-8(d)(4) which is published online at www.mass.gov/druglist. The MDL identifies the drugs that MassHealth covers, designates which drugs are subject to prior approval, and establishes specific medical necessity criteria for drugs requiring PA. See 130 CMR 406.422; see also 130 CMR 450.303. According to the MDL website, the "criteria [which are used to determine medical necessity] are based upon generally accepted standards of practice, review of the medical literature, federal and state policies, as well as laws applicable to the Massachusetts Medicaid Program."[10] Further, the criteria reflects MassHealth's policy as described in its pharmacy regulations and the reviews conducted by the agency and the DUR board.[11]

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[9] One significant difference is that states with formularies, such as Massachusetts, may impose PA restrictions consistent with formulary exclusion criteria; whereas states without formularies cannot impose such restrictions, and must ultimately approve the drug if it is being prescribed for a medically accepted indication, and it does not fall into a separate restricted category. See Edmonds v. Levine, 417 F.Supp.2d 1323, 1329 (2006). Additionally, the only requirements for the "general" prior authorization program are that the state respond to the prescriber within 24 hours of the request, and to make available, a 72-hour supply of the drug in emergency situations. See 42 USC §1396r-8(d)(5)(A)-(B).

[10] See https://mhdl.pharmacy.services.conduent.com/MHDL/

[11] The website further states that "MassHealth determines the PA status of drugs on the List on the basis of the following: MassHealth program requirements; and ongoing evaluation of the drugs' utilization, therapeutic efficacy, safety, and cost….Evaluation of a drug includes a thorough review by physicians and pharmacists using medical

Pursuant to this process, MassHealth imposed the following conditions of coverage for approval of Viltepso:

- Documentation of all of the following is required:
  - appropriate diagnosis; **and**
  - confirmed out-of-frame deletion in the DMD gene that is amenable to exon 53 skipping; **and**
  - prescriber is a neuromuscular neurologist or consult notes from a neuromuscular neurology office are provided; **and**
  - *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);* **and**
  - appropriate dosing (80 mg/kg intravenously every week); **and**
  - one of the following:
    - member has received a corticosteroid for at least three months prior and member will continue to use a corticosteroid in combination with the requested agent; **or**
    - contraindication to corticosteroids; **and**
  - *member has at least a baseline measurement for each of the following timed function tests as shown in medical records (tests must have been observed or completed by the treating provider, or ordered by the treating provider and completed by a qualified medical practitioner):*
    - *timed ten-meter walk/run (time in seconds);* ***and***
    - *timed floor (supine) to stand (time in seconds);* ***and***
    - *timed four-step descend (time in seconds);* ***and***
    - *timed four-step climb (time in seconds);* ***and***
    - *timed sit to stand (time in seconds).*

See Exh. 4, pp. 69 (emphasis added); see also www.mass.gov/druglist.

It is undisputed that Appellant is no longer "ambulatory" as defined by completion of a 6MWT. This fact was reflected in the PA request itself, in the clinical records, and through Appellant's testimony at hearing. As there was no documentation included within the PA request to demonstrate Appellant was ambulatory or had baseline measurements for the timed function tests, MassHealth denied coverage of Viltepso.

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literature and consulting with specialists, other physicians, or both. References used may include AHFS Drug Information; Drug Facts and Comparisons, Micromedex; National Comprehensive Cancer Network (NCCN); literature from peer-reviewed medical journals; Drug Topics Red Book, Approved Drug Products with Therapeutic Equivalence Evaluations (also known as the "Orange Book"); the Massachusetts List of Interchangeable Drug Products, and manufacturers' product information." See id.

Appellant and his provider presented compelling testimony and arguments in opposition to the MassHealth action. Specifically, Appellant asserted that Viltepso is FDA approved for the indication of treating DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping - the very condition he suffers from. Appellant argued that MassHealth's PA criteria not only limits accessibility of the drug to the very population the drug was intended to help, but also, is discriminatory against non-ambulatory individuals. Additionally, [redacted] opined that because the clinical trial revealed the drug's positive effect on the production of dystrophin– the essential protein lacking in DMD patients – there is a strong expectation that Viltespo will bring clinical benefit to all muscle areas in the body. Finally, both Appellant and [redacted] articulated that the more critical aspect of DMD treatment is to maintain stability, regardless of ambulation status, and in particular, stability in one's pulmonary function – an aspect of Appellant's health which he has not yet lost.

Appellant correctly asserts that the PA criteria limits MassHealth coverage of Viltespo to a subset of the DMD population that would otherwise be covered under the drug's FDA approved indication. However, federal law permits state Medicaid agencies with established formularies, such as MassHealth, to exclude otherwise covered drugs from a population when it determines a drug "***does not have a significant, clinically meaningful therapeutic advantage in terms of safety, effectiveness, or clinical outcome of such treatment for such population..***" See 42 USC §1396r-8(d)(4). The evidence indicates that MassHealth developed the PA criteria upon a deliberate review of the medical literature and in consultation with licensed physicians and pharmacists. As both parties acknowledged, there are no studies to date to verify that Viltepso offers a clinical benefit in non-ambulatory patients. Rather, Viltepso was released through the FDA's accelerated approval process after a clinical study demonstrated that the subjects who trialed the drug - all of whom where ambulatory (walking) boys ages 4 to 10 - had an increase in dystrophin production in skeletal muscle when taken with a corticosteroid. While accelerated approval is indeed a full FDA approval, as Appellant asserts, its very purpose is to expedite the approval process for drugs that fill an unmet need by establishing a "surrogate endpoint," that is, a measure of predicting clinical benefit, but is not itself a measure of clinical benefit.[12] As Viltepso's drug packaging states, "continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials."[13] See Exh. 4, p. 75. In accordance with

[12] The FDA website explains its Accelerated Approval Program as follows:

> The FDA instituted its Accelerated Approval Program "to allow for earlier approval of drugs that treat serious conditions, and fill an unmet medical need based on a surrogate endpoint. A surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign or other measure that is thought to predict clinical benefit but is not itself a measure of clinical benefit. The use of a surrogate endpoint can considerably shorten the time required prior to receiving FDA approval. Drug companies are still required to conduct studies to confirm the anticipated clinical benefit. If the confirmatory trial shows that the drug actually provides a clinical benefit, then the FDA grants traditional approval for the drug. If the confirmatory trial does not show that the drug provides clinical benefit, FDA has regulatory procedures in place that could lead to removing the drug from the market.

See https://www.fda.gov/drugs/nda-and-bla-approvals/accelerated-approval-program.

[13] Through its website, the FDA indicates Viltepso's approval is subject to an ongoing study to verify the drug's clinical benefit, namely "'A Phase 3 Randomized, Double-blind, Placebo controlled, Multi-center Study to Assess

its review, MassHealth concluded that the drug is not expected to improve or stabilize a condition or prevent worsening for members who are no longer ambulatory, nor was there sufficient clinical evidence to show Viltepso has any impact on respiratory or cardiac outcomes. MassHealth did not exceed its authority by opting to limit coverage of Viltepso to members that met the same clinical criteria as those studied in the trial. It is undisputed that Appellant does not meet MassHealth's PA criteria for Viltepso. Accordingly, MassHealth did not err in denying Appellant's PA request.

Appellant's remaining arguments constitute challenges to the legality of the PA criteria, as incorporated into MassHealth regulation, and therefore cannot be adjudicated in this hearing decision. MassHealth Fair Hearing Rules address the authority of the hearing officer as follows:

> ***The hearing officer must not render a decision regarding the legality of federal or state law including, but not limited to, the MassHealth regulations. If*** the legality of such law or regulations is raised by the appellant, ***the hearing officer must render a decision based on the applicable law or regulation as interpreted by the MassHealth agency***. Such decision must include a statement that the hearing officer cannot rule on the legality of such law or regulation and must be subject to judicial review in accordance with 130 CMR 610.092.

See 130 CMR 610.082(C)(2) (emphasis added); see also 130 CMR 450.244.

The appeal issue is therefore limited to whether MassHealth incorrectly denied the drug in accordance with the facts of this case and based on the applicable law "as interpreted by the MassHealth agency." Id. As discussed above, Appellant did not establish, by a preponderance of the evidence, that MassHealth erred in denying his PA request. As such, this appeal is DENIED.

Any challenge to MassHealth's interpretation of the law, or whether its policy is unlawful or discriminatory, may be addressed via judicial review in accordance with M.G.L. c. 30A.

Order for MassHealth

None.

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the Efficacy and Safety of Viltolarsen in Ambulant Boys with Duchenne Muscular Dystrophy.' The study [which has a projected completion date of 12/31/2024] will assess treatment with viltolarsen 80 mg/kg over 48 weeks. The primary endpoint will be Time to Stand." See https://www.fda.gov/drugs/accelerated-approval-program/ongoing-non-malignant-hematology-neurological-disorders-and-other-indications-accelerated-approvals#footnote2_7e9hi55

Notification of Your Right to Appeal to Court

If you disagree with this decision, you have the right to appeal to Court in accordance with Chapter 30A of the Massachusetts General Laws. To appeal, you must file a complaint with the Superior Court for the county where you reside, or Suffolk County Superior Court, within 30 days of your receipt of this decision.

Casey Groff, Esq.
Hearing Officer
Board of Hearings

cc:
MassHealth Representative: UMMS Drug Utilization Review, Commonwealth Medicine, 333 South Street, Shrewsbury, MA 01545

[redacted]