Office of Medicaid
BOARD OF HEARINGS

Appellant Name and Address:

[redacted]

Appeal Decision:	Denied	**Appeal Number:**	2208894
Decision Date:	2/16/2023	**Hearing Date:**	12/30/2022
Hearing Officer:	Christine Therrien		

Appearance for Appellant:
Pro se
[redacted]

Appearance for MassHealth:
Phuong Luc, Clinical Pharmacist Consultant
Andrew Coelho, Clinical Pharmacist Consultant

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:**	DUR
Decision Date:	2/16/2023	**Hearing Date:**	12/30/2022
MassHealth's Rep.:	Phuong Luc, Clinical Pharmacist Consultant; Andrew Coelho, Clinical Pharmacist Consultant	**Appellant's Rep.:**	[redacted]
Hearing Location:	Telephonic		

Authority

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

Jurisdiction

Through notices dated 10/24/23 and 11/3/2022 MassHealth denied the appellant's request for prior authorization (PA) for EXONDYS-51. (130 CMR 450.204 and Exhibit 1 and 4). The appellant filed this appeal in a timely manner on 11/29/22. (130 CMR 610.015 and Exhibit 2). Denial of a request for prior authorization is valid grounds for appeal. (130 CMR 610.032).

Action Taken by MassHealth

MassHealth denied the appellant's request for PA for EXONDYS-51.

Issue

The issue on appeal is whether MassHealth was correct, pursuant to 130 CMR 450.204, in denying the appellant's PA for EXONDYS-51.

Summary of Evidence

The MassHealth Drug Utilization Review Program (DUR) pharmacist representative testified that the appellant's physician submitted a prior authorization request for EXONDYS-51. MassHealth denied

the request because "the information provided did not contain sufficient information to determine medical necessity." (Exhibit 1 and Exhibit 4 p. 46).

The appellant is an [redacted] with a diagnosis of Duchenne Muscular Dystrophy (DMD). DMD is a neuromuscular disorder caused by mutations in the dystrophin gene. The deletion of certain exons related to dystrophin production impacts how the genetic code is translated into protein. Patients with DMD have low levels of dystrophin protein in their muscle cells and suffer from progressive muscle deterioration, which eventually impacts their ambulation, respiration, and cardiac function. DMD ultimately causes most children to become reliant on a wheelchair by age 13 and reliant on a respirator to sleep by age 18. (Exhibit 4 and appellant's physician's testimony). Patients with DMD have an average life expectancy of approximately 30 years of age. (Exhibit 4, p. 3). EXONDYS-51 (eteplirsen) is the first medication targeted at the underlying cause of DMD. EXONDYS-51 treats DMD through "exon-skipping," which produces a truncated, but functional form of dystrophin protein and slows muscle degeneration. The FDA approved EXONDYS-51 for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. (Exhibit 4). "Studies have shown over long term follow up that eteplirsen-treated patients demonstrated a statistically significant advantage of 151m ($p<0.01$) on 6-minute walk test and experience a lower incidence of loss of ambulation." (Exhibit 1, pp. 3-4).[1] EXONDYS-51 slows or halts DMD by reducing muscle degeneration. (Exhibit 1, p.4). The appellant's physician testified that the appellant has been using EXONDYS-51 since 2015 through clinical research and in 2018 transitioned to commercial use when it became commercially available. The appellant remains able to walk at home independently and has been going for regular walks outdoors. The appellant uses a power scooter for long distances. (Exhibit 4 and Appellant's physician's testimony). The appellant wants to continue using EXONDYS-51 to maintain his independence and avoid confinement to a wheelchair. (Exhibit 4 and appellant's testimony). The appellant's physician testified that if the appellant discontinues using EXONDYS-51 he will be wheelchair bound very quickly.

The representative from the DUR testified that the use of EXONDYS-51 requires that a member must document and meet specific criteria for recertification of their prior authorization. The DUR representative testified that the MassHealth Drug List, Therapeutic Table 76: Neuromuscular Agents – Duchenne Muscular Dystrophy and Spinal Muscular Atrophy, outlines the recertification criteria.

> For recertification requests, documentation of all of the following is required:
> - member remains 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
> - 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); and
> - dosing remains appropriate; and
> - one of the following:
>   - member continues to utilize corticosteroids in combination with the requested

[1] Citing Mendell JR, Goemans N, Lowes LP, et al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. *Annals of Neurology*. 2016;79(2):257-271. doi:10.1002/ana.24555.

agent; or
    - contraindication to corticosteroids; and
- member has a stable or improving pattern of observed performance on at least two of the following five timed function tests as shown in medical records (all results for all tests must be included with the date of performance; 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).

(Exhibit 4, pp. 50-59).

The DUR representative testified that the appellant did not provide documentation showing he was able to complete the six-minute walk test (6MWT) at regular intervals. The appellant's 6MWT results over the prior two years are 2/25/21: 205M, 8/19/21: 175M, 2/10/22: 175M, 10/11/22: 173M. (Exhibit 4, p. 4). The DUR representative testified that MassHealth requires 6MWT to be completed at regular intervals which she said were quarterly to compare to the same quarter in the prior year. (Exhibit 4, p. 48). The DUR representative testified that in 2015 the appellant's pre-treatment 6MWT was 395M and over time he has declined but it was still greater than 200M until August of 2021.[2] The DUR representative testified that based on the majority of clinical trials, ambulatory is defined as a 6MWT of >200M.

MassHealth does not define regular intervals in any regulation nor does the evaluation criteria for EXONDYS-51 list regular intervals as a requirement. (Exhibit 4, pp. 50-59). The appellant's physician testified that quarterly testing is unnecessary, expensive, and very difficult for these patients and their families. The appellant's physician testified that it was hard to do evaluations during the pandemic. The appellant's physician testified that the appellant is [redacted] and still walking which shows he is stable. The appellant's physician testified that timed tests are irrelevant and whoever came up with these guidelines does not understand DMD. The appellant's physician testified that while the appellant has not walked more than 200M during the previous three 6MWT he has remained stable which is what EXONDYS-51 is designed to accomplish. The appellant's physician testified that DMD is an incurable and progressive disease and the muscle loss is irreversible. The appellant's physician stated that EXONDYS-51 will never improve the appellant's condition, the drug is designed to make the patient stable which means a slower decline. The appellant's physician testified that the drug is meant to prolong the ambulatory phase of the patient and help them walk longer than to the age of 12 or 13 years. The appellant's physician testified that the fact that the appellant is [redacted] and still walking means the drug is working. The appellant's physician testified that patient's with DMD do decline but with this drug they decline more slowly. (Exhibit 4, p. 3). The appellant's physician disagreed with the DUR definition of ambulatory and testified that in the field of neurology ambulatory is defined as the ability to walk 10M. The appellant's "Ambulatory Documentation" submitted with the prior authorization indicates his continued stability. (Exhibit 4).

[2] The appellant was [redacted] old in 2015.

2/25/21 ASSESSMENT: "Motor function testing today reveals improved walking abilities on 6MWT compared to September 2020. [Appellant's] stability of motor function is different than the expected trajectory in UNTREATED DMD, which involves worsening of motor function in the preteen and teenage years."

8/19/21 ASSESSMENT: "Motor function testing today reveals stable gross motor abilities compared to February of this year, and 6MWT slightly reduced compared to 2/2021 but may be attributed to recent fall with groin injury. [Appellant's] overall stability of motor function is different than the expected trajectory in untreated DMD which involves worsening of motor function in the preteen and teenage years."

2/10/22 ASSESSMENT: "Motor function testing today reveals stable gross motor abilities overall with stable 6MWT. [Appellant's] overall stability of motor function is different than the expected trajectory in untreated DMD, which involves worsening of motor function in the preteen and teenage years."

10/11/22 ASSESSMENT: "Motor function testing today reveals stable 6MWT test distance overall. This is different than the expected trajectory and untreated DMD, which involves worsening of motor function in the preteen and teenage years."

The appellant stated that the DUR decision is unjustified and that a choice is being made for him by people who do not live with DMD.

Findings of Fact

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

1. A prior authorization request for EXONDYS-51 was submitted on behalf of the appellant. (Exhibit 1).

2. MassHealth denied the request because "the information provided did not contain sufficient information to determine medical necessity." (Exhibit 1 and Exhibit 4 p. 46).

3. The appellant is an [redacted] with a diagnosis of Duchenne Muscular Dystrophy (DMD). (Testimony).

4. DMD is a neuromuscular disorder caused by mutations in the dystrophin gene. The deletion of certain exons related to dystrophin production impacts how the genetic code is translated into protein. Patients with DMD have low levels of dystrophin protein in their muscle cells and suffer from progressive muscle deterioration, which eventually impacts their ambulation, respiration, and cardiac function. DMD ultimately causes most children to become reliant on a wheelchair by age 13 and reliant on a respirator to sleep by age 18 with an average life expectancy of 30 years. (Exhibit 4 and testimony).

5. EXONDYS-51 (eteplirsen) is the first medication targeted at the underlying cause of DMD. EXONDYS-51 treats DMD through "exon-skipping," which produces a truncated, but

functional form of dystrophin protein and slows muscle degeneration. The FDA approved EXONDYS-51 for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. (Exhibit 4).

6. "Studies have shown over long term follow up that eteplirsen-treated patients demonstrated a statistically significant advantage of 151m ($p<0.01$) on 6-minute walk test and experience a lower incidence of loss of ambulation." (Exhibit 1, pp. 3-4).

7. EXONDYS-51 slows or halts DMD by reducing muscle degeneration. (Exhibit 1, p. 4).

8. The appellant has been using EXONDYS-51 since 2015 through clinical research and in 2018 transitioned to commercial use when it became commercially available. (Testimony).

9. The appellant remains able to walk at home independently and has been going for regular walks outdoors. The appellant uses a power scooter for long distances. (Exhibit 4 and Testimony).

10. The use of EXONDYS-51 requires that a member must document and meet specific criteria for recertification of their prior authorization. The MassHealth Drug List, Therapeutic Table 76: Neuromuscular Agents – Duchenne Muscular Dystrophy and Spinal Muscular Atrophy, outlines the recertification criteria.

    For recertification requests, documentation of all of the following is required:
    - member remains 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
    - 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); and
    - dosing remains appropriate; and
    - one of the following:
    - member continues to utilize corticosteroids in combination with the requested agent; or
    - contraindication to corticosteroids; and
    - member has a stable or improving pattern of observed performance on at least two of the following five timed function tests as shown in medical records (all results for all tests must be included with the date of performance; 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).

    (Exhibit 4, pp. 50-59).

11. The documentation the appellant provided showed his 6MWT results over the prior two years

were 2/25/21: 205M, 8/19/21: 175M, 2/10/22: 175M, 10/11/22: 173M. (Exhibit 4, p. 4)

12. In 2015 the appellant's pre-treatment 6MWT was 395M and over time he has declined but it was greater than 200M until August of 2021.

13. MassHealth does not define regular intervals in any regulation nor does the evaluation criteria for EXONDYS-51 list regular intervals as a requirement. (Exhibit 4, pp. 50-59).

14. DMD is an incurable and progressive disease and the muscle loss is irreversible. EXONDYS-51 will never improve the appellant's condition, the drug is designed to make the patient stable which means a slower decline. (Testimony and Exhibit 4, p. 3).

15. "Ambulatory Documentation" submitted with the prior authorization indicates the appellant's continued stability. (Exhibit 4).

    2/25/21 ASSESSMENT: "Motor function testing Today reveals improved walking abilities on 6MWT compared to September 2020. [Appellant's] stability of motor function is different than the expected trajectory in UNTREATED DMD, which involves worsening of motor function in the preteen and teenage years."

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Analysis and Conclusions of Law

Pursuant to 130 CMR 450.204, MassHealth will not pay a provider for services that are not medically necessary; and may impose sanctions on a provider for providing or prescribing a service or for admitting a member to an inpatient facility where such service or admission is not medically necessary.

(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 Division. Services that are less costly to the Division include, but are not limited to, health care reasonably known by the provider, or identified by the Division 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.

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

MassHealth denied the appellant's request for coverage of EXONDYS-51 on the basis that it had insufficient information to determine medical necessity. The DUR representative testified that EXONDYS-51 recertification evaluation criteria includes documenting that the "member remains ambulatory as defined by a current six-minute walk test (6MWT – distance walked in six minutes in meters) of ≥ 200 meters." (Exhibit 4, p. 59). The DUR representative maintained that based on a majority of clinical trials ambulatory is defined as being able to complete a 200-meter walk in six minutes. The appellant's physician testified that in the field of neurology ambulatory is defined as the ability to walk 10 meters. Neither party provided a resource to support their definition of ambulatory. The recertification evaluation criteria further state that documentation must be provided to show a "member has a stable or improving pattern of 6MWTs…" (Exhibit 4, p. 59). The appellant's physician and his medical records both state the appellant's condition is stable despite his inability to walk 200 meters within 6 minutes.

The Introduction to the MassHealth Drug List states that "[t]he criteria for prior authorization identify the clinical information MassHealth considers when determining medical necessity for selected medications. The criteria 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."[3] 130 CMR 450.204(D) states that there are additional medical necessity requirements laid out in the coverage guidelines and in this case those criteria are listed in the MassHealth Drug List, Therapeutic Table 76: Neuromuscular Agents – Duchenne Muscular Dystrophy and Spinal Muscular Atrophy. Since the appellant's medical records indicate he cannot complete the 6MWT as listed in Therapeutic Table 76 for EXONDYS-51 he does not meet the DUR medical necessity requirements. This appeal is DENIED.

Order for MassHealth

None.

Notification of Your Right to Appeal to Court

[3] https://mhdl.pharmacy.services.conduent.com/MHDL/pubintro.do?category=Introduction+to+MassHealth+Drug+List

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.

Christine Therrien
Hearing Officer
Board of Hearings

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