Office of Medicaid
BOARD OF HEARINGS

Appellant Name and Address:

[redacted]

Appeal Decision:	Approved	**Appeal Number:**	2413926
Decision Date:	1/16/2025	**Hearing Date:**	10/15/2024
Deputy Director/Hearing Officer:	Paul C. Moore	**Record Closed:**	12/20/2024

Appellant Representatives:

[redacted]

Tufts Health Plan Representatives:
John Shinn, Esq., Sherin and Lodgen, LLP; David Dohan, M.D., and Thomas Amoroso, M.D., both medical directors from Tufts Health Plan (via Microsoft Teams)
MassHealth Representative: Michaele Freeman, Contract Manager (observing only)

APPEAL DECISION

Appeal Decision:	Approved	**Issue:**	Prior Authorization, MassHealth Drug List, EPSDT
Decision Date:	1/16/2025	**Hearing Date:**	10/15/2024
Tufts Health Plan Reps.:	Attorney Shinn; Dr. Dohan; Dr. Amoroso	**Appellant Reps.:**	[redacted]
Hearing Location:	Board of Hearings (via Microsoft Teams)		

Authority

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

Jurisdiction

Through a notice dated July 17, 2024, Tufts Health Plan, MassHealth's agent ("Tufts"), denied the appellant's internal appeal of a prior authorization (PA) request for Elevidys gene therapy infusion (Exh. 1). The appellant filed this external appeal with the Board of Hearings (BOH) in a timely manner on July 18, 2024 (130 CMR 610.015(B)(1); Exh. 2). Denial of PA request is a valid ground for appeal (130 CMR 610.032).

A virtual hearing was held on October 15, 2024. At the close of the hearing, the Deputy Director/Hearing Officer held the hearing record open until November 20, 2024, for the appellant to file a post-hearing legal memorandum, and until December 20, 2024, for Tufts to file a responsive post-hearing legal memorandum.

Action Taken by Tufts Health Plan

Tufts denied the appellant's first level appeal of a denial of a PA request for prescribed Elevidys gene therapy infusion.

Issue

Was Tufts, MassHealth's agent, correct in denying the appellant's PA request for Elevidys?

Summary of Evidence

A. Documentary evidence and testimony

The appellant, a [redacted] year-old boy, was represented at a virtual hearing by an attorney, his mother, and his grandmother.[1] Tufts Health Plan was represented by an attorney, and two of its medical directors.

According to written records submitted by Tufts prior to the hearing, the appellant is enrolled in MassHealth Standard and in Tufts Health Together ("Tufts"), a Medicaid managed care plan.[2] He was diagnosed with Duchenne muscular dystrophy ("DMD") on June 11, 2024, just after his [redacted] birthday. The appellant's pediatric neurologist at [redacted] submitted a prior authorization request to Tufts on July 10, 2024, on the appellant's behalf, seeking coverage of Elevidys gene therapy infusion, a drug recently approved by the federal Food and Drug Administration ("FDA") for the treatment of DMD. By letter dated July 12, 2024, Tufts denied the PA request; the denial letter stated in relevant part:

> [Tufts] utilizes the MassHealth United Formulary. MassHealth's coverage guideline entitled [DMD] Disease Modifying Agents for the coverage of Elevidys (delandistrogene moxeparvovec-rokl) require that you are at least [redacted] years of age and less than [redacted] years of age at the time of administration. . . .

(Exh. 5, p. 95)

Thereafter, the appellant filed an expedited internal appeal for the denial decision with Tufts on July 16, 2024 (*Id.*, p. 108). Prior to deciding on the expedited internal appeal, Tufts referred the case to a consulting organization, [redacted] A pediatric neurologist there, [redacted] had a peer-to-peer discussion with [redacted] a second pediatric neurologist at [redacted] familiar with the appellant's case. After this peer-to-peer discussion with [redacted] recommended upholding the denial of the PA request (*Id.*, at pp. 122-125).

[1] The appellant did not attend the hearing.

[2] Pursuant to 130 CMR 508.001(A), "MassHealth Member Participation in Managed Care:" "MassHealth members who are younger than [redacted] years old must enroll in a MassHealth managed care provider available for their coverage type. Members described in 130 CMR 508.001(B) or who are excluded from participation in a MassHealth managed care provider pursuant to 130 CMR 508.002(A) are not required to enroll with a MassHealth managed care provider."

On July 17, 2024, Tufts notified the appellant by letter that it was upholding the original denial because "the reviewer's findings indicate that the [MassHealth criteria] have not been met as the [appellant] is outside the age range of [redacted] at the time of administration" (*Id*. at 127). The appellant subsequently filed this external appeal with the Board of Hearings.

According to [redacted] one of the Tufts medical directors, DMD results from a genetic mutation in the gene that makes dystrophin, a vital protein in the human body. This protein is crucial for the development and function of muscle tissue. DMD severely impairs the production of dystrophin, leading to the destruction of muscle tissue, eventually including heart and lung muscle. Patients are typically boys. Over time, the loss of dystrophin leads to an inability to walk, and a loss of cardiac and lung function. According to [redacted] the occurrence of DMD is associated with an early death. Elevidys is the only gene therapy approved for DMD, using a virus that introduces a gene into the body to facilitate the correct production of dystrophin (Testimony).[3]

[redacted] another medical director at Tufts, testified that, after various studies were conducted by Sarepta Therapeutics, the company which produces the drug, Elevidys received accelerated approval by the FDA for treatment of DMD in June 2023; specifically, the drug was approved for treatment in ambulatory [redacted] In June 2024, based on additional study results, the FDA expanded Elevidys approval to all ambulatory and non-ambulatory children with DMD [redacted] and over (FDA press release, June 20, 2024, Exh. 7). According to [redacted] this expanded approval was "controversial;" the FDA scientific panel that examined the clinical trial results in children over age [redacted] did not find significant clinical improvement in any age group which received the drug. Despite this fact, the FDA commissioner overrode the opinion of this panel, according to [redacted] and approved the use of the drug in all ambulatory and non-ambulatory children aged [redacted] and above (Testimony).[4]

The MassHealth Drug List, a published, publicly available document which enumerates the requirements for all drugs approved for MassHealth members, states the following about the approval of Elevidys:

- Documentation of all of the following is required:
    - appropriate diagnosis; **and**
    - member is [redacted] at the time of administration; **and**
    - prescriber is a neuromuscular specialist; **and**
    - copy of genetic test with a confirmed mutation in the DMD gene; **and**
    - member does not have any deletion in exon 8 or exon 9 of the DMD gene; **and**

[3] [redacted] testified that DMD patients are also typically maintained on doses of corticosteroids.

[4] The FDA wrote in its June 20, 2024, press release: "While the large, randomized study of Elevidys failed to meet its statistical primary endpoint of improvement versus placebo in the North Star Ambulatory Assessment, the FDA found the observations regarding the secondary endpoints and exploratory endpoints to be compelling and to indicate clinical benefit compared to placebo. These endpoints include improvements in time to rise from the floor, 10-meter walk/run, time to ascend four steps and creatine kinase levels" (Exh. 7).

- copy of baseline anti-AAVrh74 total binding antibody titers < 1:400; **and**
- member has a baseline measurement for both of the following:
  - North Star Ambulatory Assessment, including scores and times on individual items (within the past three months); **and**
  - Six-minute walk test (within the past three months); **and**
- member is ambulatory as defined by a current six-minute walk test (6MWT – distance walked in six minutes in meters) ≥ 200 meters; **and**
- one of the following:
  - member is on a stable dose of corticosteroid; **or**
  - attestation that the member will continue to utilize chronic corticosteroids after Elevidys infusion; **or**
  - demonstrated contraindication to corticosteroids; **and**
- member has not previously received treatment with a gene therapy for DMD; **and**
- infusion will take place in a qualified treatment facility; **and**
- member is not currently utilizing antisense oligonucleotides; **and**
- appropriate dosing.

(MassHealth Drug List)

Elevidys was added to the MassHealth Drug List effective March 4, 2024, for ambulatory children with DMD ages [redacted] and the eligibility criteria have not been updated since that time (Testimony, Exh. 11).

[redacted] testified that Tufts, as an agent of MassHealth, is required to follow the MassHealth published formulary, or MassHealth Drug List, when deciding whether to approve a prior authorization request for a drug prescribed for a MassHealth member. Here, where the appellant is age [redacted] the MassHealth Drug List does not permit Tufts to authorize coverage of this drug for the appellant (Testimony).

Among the documents compiled by Tufts and submitted to the Board of Hearings prior to hearing, a letter of medical necessity ("LOMN") for Elevidys dated July 2, 2024, from [redacted] a pediatric neurologist at [redacted] is included. The LOMN states in relevant part:

> I had the pleasure of seeing [the appellant] in our neurology clinic for evaluation of DMD. History is provided by his mother, grandmother and grandfather. . . .
>
> Started prednisone and is doing well.

Initial history: [The appellant] has a history of gross motor delays. Mom has noticed that [the appellant] has difficulty using his leg properly since very young age especially when running, jumping or using the stairs. He crawled at the age of [redacted] and walked at the age of [redacted] His (*sic*) has a waddling gait and unable to lift his feet of (*sic*) the ground when running. Given no improvement of his symptoms and evidence of 'Gower maneuver,' he was referred to the neuromuscular clinic for concern of muscular dystrophy. Mom has read about this condition and is concern (*sic*) about the prognosis. There is also concern for instability and lack of good balance. There is (*sic*) no associated paresthesias, ptosis, dysarthria, dysphagia, or neck weakness. Mom believes subtle motor delay progression but no significant improvement over the years. He gets IEP PT/OT and speech therapy at school with some improvement of his gross motor skills. He has being (*sic*) involved in a hybrid program, part time in a sub separate classroom for more individualized instruction and part time in the general education classroom with the same aged peers. He is not taking any medications.

His energy level is generally good and his level of cognition is not a concern. There is no daytime sleepiness or morning headaches. No history of significant falls or fractures, saddle anesthesia or vertigo. There is no concern of bulbar or respiratory symptoms such as shortness of breath, choking or coughing with eating. Family history significant for maternal second degree male with suspected muscular dystrophy. No other family history of neurological conditions.

On his initial exam he had + Gowers, calf hypertrophy, waddling and lordotic gait/run. Invitae NM panel confirmed at (*sic*) diagnosis of DMD.

Assessment: [The appellant] is a [redacted] with newly diagnosed DMD, confirmed by genetic testing showing a deletion of exons 46-51 in DMD. Clinically, he has proximal muscle weakness, calf hypertrophy, and markedly elevated creatine kinase.

Since our last visit, Elevidys gene therapy has been approved for DMD boys [redacted] and older. We had a follow-up discussion today regarding gene therapy. We reviewed the . . . consent form, discussed specific logistics around eligibility – [the appellant's] AAVrh74 antibody is negative, alternative therapies including exon skipping, Givinostat, and continuing care with prednisone alone. While therapies such as exon skipping and Givinostat may medically and conceptually be given concurrently to Elevidys, I cannot guarantee that insurance would approve such combinations. We explicitly discussed the limited efficacy data and limited long-term safety data for Elevidys. The family confirmed that they are interested in moving forward with treatment.

(Exh. 5, pp. 26-27)

The appellant's attorney indicated that [redacted] submitted a letter of appeal for the appellant dated August 16, 2024, following the internal appeal denial, which states in pertinent part:

> Of note, the appellant is relatively strong for a boy his age with DMD. His North Star Ambulatory Assessment (NSAA), a comprehensive measure of strength and motor function, score is 25. By comparison, the mean NSAA for [redacted] the children that showed the most benefit in study one (see below) was 20. Based on our experience with neurodegenerative diseases and gene therapy, treating early before muscle deterioration is likely why the younger boys showed more improvement. [The appellant's] relatively preserved strength (similar or even better than a typical [redacted] DMD boy) makes him an excellent candidate for gene therapy and increases the likelihood of a clinically meaningful benefit.

(Exh. 9, Attachment One)

[redacted] testified that, when deciding whether to authorize Elevidys for the appellant, Tufts did not consider the results of the appellant's NSAA, including the results of a recent 6-minute walk test (6MWT) completed by the appellant. Instead, Tufts simply compared the appellant's age with the age requirements for Elevidys set forth in the MassHealth Drug List (Testimony).[5]

Medical documentation in the record (Exh. 5) reflects that, as of July 2, 2024, the appellant's anti-AAVrh74 total binding antibody titers were less than 1:400 (considered a negative result) (Exh. 5, p. 39), and that he has no deletion in exon 8 and/or exon 9 in the DMD gene (*Id*. at 55).[6] In addition, the appellant has not previously been treated with gene therapy for DMD, he is on a corticosteroid, and he is currently not utilizing antisense oligonucleotide medications such as Amondys 45 or Exondys 51 (*Id*.).

The Tufts Health Together Member Handbook for 2024 reflects the following;

> Pharmacy Program:
>
> . . . Our pharmacy program doesn't cover all drugs, medical devices and prescriptions. Some drugs and medical devices must meet certain clinical guidelines before we can cover them. Your Provider must ask us for Prior Authorization before we'll cover one of these drugs or medical devices. . . .

[5] The MassHealth Drug List requires, *inter alia*, [redacted] children with DMD to be "ambulatory" in order to be eligible to receive Elevidys, while the FDA, in its June 20, 2024, press release authorizing Elevidys for children [redacted] and above, makes no distinction between ambulatory and non-ambulatory children (Exh. 7).

[6] [redacted] testified that documentation initially submitted to Tufts by [redacted] the appellant's neurologist, incorrectly reported that the appellant had a deletion in exon 8 and/or exon 9 in the DMD gene; this was subsequently corrected by [redacted] to reflect that he does not. Having a deletion in exon 8 and/or exon 9 in the DMD gene is a contraindication for treatment with Elevidys, per the medical literature and the MassHealth Drug List criteria.

Prior Authorization:

Some drugs and medical devices require Prior Authorization, which means your Provider must ask us for approval before we'll cover the drug. One of our clinicians will review this request. We'll cover the drug or medical device according to our clinical guidelines if

● There is a medical reason you need the particular drug or medical device.
● Depending on the drug or medical device, other drugs or medical devices on the MassHealth Drug List have not worked.

We allow for one emergency 72-hour supply of your prescription to be filled at the pharmacy while your doctor submits a request to us. . . .

(Exh. 6, p. 20). There was no evidence presented by the appellant, nor by Tufts, about the potential cost of this drug treatment.

The appellant's attorney argued that, under 130 CMR 450.140 *et seq*. – the MassHealth regulations incorporating the requirements of Early and Periodic Screening, Diagnostic and Testing (EPSDT) services -- MassHealth or its agent must conduct an individualized assessment of all children under age [redacted] who are enrolled in MassHealth Standard or CommonHealth.[7] These regulations also state that EPSDT diagnosis and treatment services consist of all medically necessary services listed in § 1905 of the Social Security Act that are needed to correct or ameliorate physical and mental illnesses and conditions discovered by a screening, whether or not such services are covered under the Medicaid State Plan, and are payable for MassHealth Standard and MassHealth CommonHealth members younger than [redacted] if the service is determined by the MassHealth agency to be medically necessary.[8] He asserted that here, Tufts, as MassHealth's agent, failed to conduct the required individualized assessment of the appellant, including reviewing the results of the appellant's NSAA. Had they done so, the attorney argued, Tufts would have concluded that Elevidys gene therapy for treatment of the appellant's DMD is medically necessary. According to the appellant's attorney, since the general drug formulary for MassHealth does not authorize coverage of Elevidys gene therapy for children [redacted] and above, in order to receive payment for this service (which is not specifically included as a covered service under any MassHealth regulation, service code list, or contract), [redacted] the appellant's treating neurologists, correctly submitted a request for prior authorization to Tufts in accordance with 130 CMR 450.303.[9]

[7] EPSDT is a federal law implemented in 1967 focusing on the child health component of Medicaid.

[8] The State plan is a comprehensive written statement submitted by the Medicaid agency describing the nature and scope of its Medicaid program and giving assurance that it will be administered in conformity with the specific requirements of title XIX, the regulations in . . . Chapter IV, and other applicable official issuances of the Department [of Health and Human Services]. The State plan contains all information necessary for the Centers for Medicare and Medicaid Services (CMS) to determine whether the plan can be approved to serve as a basis for Federal financial participation (FFP) in the State program. 42 Code of Federal Regulations (CFR) § 430.10.

[9] As mentioned *supra*, the appellant's total score on the NSAA, as of June 28, 2024, was 25 out of a possible 34. He

The appellant's grandmother testified that the appellant's treating neurologists believe that the appellant is an excellent candidate for gene therapy with Elevidys, and that the medication will slow the progression of the appellant's devastating diagnosis. She added that the appellant currently cannot keep up with his peers in terms of physical activities. She noted that the appellant, who is in the [redacted] has not been told of his diagnosis. The appellant's mother testified that his teachers initially noticed functional mobility deficits in the appellant and apprised the appellant's family of same (Testimony).

The appellant's attorney asserted that Tufts did not follow the EPSDT mandate to provide all medically necessary services to the appellant, a child under age [redacted] enrolled in MassHealth, regardless of whether such services are covered under the State Plan, by initially failing to conduct an individual assessment of the appellant. He asked whether Tufts wished to rescind its denial decision and start fresh by performing a new assessment of the appellant. Tufts' attorney did not agree that Tufts had violated EPSDT regarding the appellant, and the Tufts physicians who testified at hearing declined to agree to perform, or to authorize, a new assessment of the appellant. Also, [redacted] did not agree with the appellant's neurologist's opinion that the appellant's relative strength when compared with younger children diagnosed with DMD make him "similar" to [redacted] who are, in fact, eligible for the drug under the MassHealth criteria.[10]

At the close of the hearing, the Deputy Director/Hearing Officer agreed to keep the record of this appeal open until November 20, 2024, for the appellant's attorney to file a post-hearing memorandum of law, including proposed findings of fact and proposed rulings of law (Exh. 8). Further, the Deputy Director/Hearing Officer agreed to keep the appeal record open until December 20, 2024, for Tufts to file a responsive memorandum of law, including proposed findings of fact and proposed rulings of law (*Id*.).

B. Appellant's Post-Hearing Brief

By electronic mail, on November 20, 2024, the Deputy Director/Hearing Officer received the appellant's Post-Hearing Memorandum of Law ("memo"), and attachments, which were copied to the Tufts attorney.[11] In the appellant's memo, he asserts that Tufts' denial of the appellant's PA request violates the federal Medicaid Act's requirement to cover outpatient drugs for any medically accepted any indication that is approved by the FDA. The appellant adds that the use of Elevidys gene therapy is medically approved by the FDA for all ambulatory and non-ambulatory DMD-diagnosed children [redacted] and above, which includes the appellant. He concludes that therefore, by not providing the requested coverage, Tufts violated the Medicaid Act (Exh. 9, p. 1, *citing to* 42 U.S.C. § 1396r-8(d)(1)(B)(i) and § 1396r-8(k)(6)).

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was able to walk 403 meters in 6 minutes; per the MassHealth Drug List entry for Elevidys, children are considered "ambulatory" if they can walk 200 meters or more in 6 minutes (Exh. 5, pp. 22-23).

[10] [redacted] also testified that those treated with Elevidys receive an intravenous infusion in an outpatient setting, one time only.

[11] No proposed findings of fact or proposed rulings of law were included.

Next, the appellant argues that Tufts violated the EPSDT laws requiring MassHealth to cover all medically necessary diagnosis and treatment services for MassHealth Standard members under age [redacted] (Exh. 9, p. 2, *citing to* 130 CMR 450.140 through 450.149, and 42 U.S.C. § 1396d(a)(4)(b) and § 1396d(r)).

The appellant also avers that where the manufacturer of a drug has entered into a rebate agreement with the U.S. Department of Health and Human Services ("HHS"), the Medicaid Act requires the state to cover the cost of the drug under its state plan, unless the state complies with one of the exclusion or restriction provisions set out in the Medicaid Act (*Id*., p. 4, *citing to* 42 U.S.C. § 1396r-8(d)). Here, the appellant notes that Sarepta Therapeutics, which makes Elevidys, has entered into a rebate agreement with HHS, a point not disputed by Tufts. Pursuant to 42 U.S.C. § 1396r-8(d), there are four exclusions/restrictions under which states can deny coverage of a drug approved by the FDA when the manufacturer has entered into a rebate agreement with HHS, as follows:

> (i) the prescribed use is not for a medically accepted indication (as defined in subsection (k)(6));
> (ii) the drug is contained in the list referred to in paragraph (2); [12]
> (iii) the drug is subject to restrictions pursuant to an agreement between a manufacturer and a State authorized by the HHS 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).

(42 U.S.C. § 1396r-8(d)(1)(B))

None of these four circumstances apply in the case of Elevidys coverage by MassHealth, according to the appellant. Therefore, according to his argument, Tufts must cover the cost of the drug for the appellant (Exh. 9, pp. 4-5).

Next, the appellant argues that it is unlawful for Tufts, on behalf of MassHealth, to deny coverage of Elevidys based on Tufts' medical judgment about the drug's purported lack of effectiveness, where the FDA has already spoken on this issue and has approved the drug for children with DMD [redacted] and older. The appellant cites to two court decisions, [redacted] 1323, 1334-38 (S.D. Fla. 2006), and Arkansas Department of Human Services v. Sarepta [redacted] in support of his argument. In these decisions, both courts held that the state Medicaid programs in those states could not rely on new or different clinical data to decide whether certain drugs should be covered, where the FDA had already evaluated clinical data concerning the efficacy and safety standards of the drugs in question (*Id*., pp. 5-6).

[12] The drugs listed in paragraph 2 of this statute are: drugs used for weight loss or anorexia, cosmetic or hair growth drugs, fertility drugs, cough and cold medications, prescription vitamins and minerals, drugs for sexual dysfunction, nonprescription drugs, and covered outpatient drugs for which the manufacturer seeks to require, as a condition of sale, that associated tests or monitoring services be purchased exclusively from the manufacturer or its designee.

The appellant also argues that under EPSDT, MassHealth or its agents must cover all "necessary health care, diagnostic services, treatment and other measures. . . to correct or ameliorate defects and physical and mental illnesses and conditions discovered by the screening services, whether or not such services are covered under the State plan" (*Id.*, pp. 6-7, *citing to* 42 U.S.C. § 1396d(r)(5) and 130 CMR 450.144(A)(1)). The appellant cites to the First Circuit court decision in the case of [redacted] where the court held that a certified class of plaintiff children in Massachusetts, all of whom were diagnosed with severe emotional disturbances, were entitled to coverage of a broad scope of services under EPSDT regulations, including comprehensive assessments, coordination of care, crisis services, and in-home support services, among others, which various state agencies had failed to effectively provide. The appellant notes that the [redacted] summarized as follows:

> Thus, if a licensed clinician finds a particular service to be medically necessary to help a child improve his or her functional level, this service must be paid for by a state's Medicaid plan pursuant to the EPSDT mandate.

[redacted]

Finally, the appellant avers that at least one court has held that "the EPSDT contemplates individualized review of a child's condition." [redacted] In the [redacted] case, a PA request for growth hormone treatment for a child diagnosed with short stature was denied; the state appeals court reversed a hearing officer's decision to uphold the denial of such treatment by the [redacted] remanding the case to the state for further proceedings. In its decision, the court indicated that "simply reapplying the prior authorization criteria was not an individualized determination of the child's needs" (*Id.*).

The appellant concludes his memo by noting that here, the Tufts physicians, in their testimony, acknowledged not having conducted an individualized assessment of the appellant's needs, and asserts that they did not give sufficient weight to the medical opinion of the appellant's treating neurologist that the appellant would benefit from Elevidys. Further, he argues that Tufts ignored the requirement of the Medicaid Act to cover FDA-approved treatments (*Id.*, p. 8).

C. Tufts' Post-Hearing Brief

By electronic mail, on December 19, 2024, the Deputy Director/Hearing Officer received Tufts' Post-Hearing Memorandum of Law ("Tufts memo"), and attachments, which were copied to the appellant's attorney (Exh. 10).[13]

In the Tufts memo, Tufts argues that under federal law, MassHealth is permitted to impose medical necessity criteria for Elevidys pursuant to a prior authorization requirement (Exh. 10, p. 1). In addition, under MassHealth regulations at 130 CMR 406.422(A), "Prior Authorization:"

[13] Again, no proposed findings of fact or proposed rulings of law were included.

> Prescribers must obtain prior authorization from the MassHealth agency for drugs identified by MassHealth in accordance with 130 CMR 450.303, Prior Authorization. If the limitations on covered drugs specified in 130 CMR 406.412(A) and 406.413(A) and (C) would result in inadequate treatment for a diagnosed medical condition, the prescriber may submit a written request, including written documentation of medical necessity, to the MassHealth agency for prior authorization for an otherwise non-covered drug. . . .

(*Id.*, p. 3)

Tufts also asserts that "[i]t is undisputed that the [appellant] was over the age of [redacted] at the time of the prior authorization request" (Exh. 10, p. 5). Tufts states that "[w]hile the [appellant] argues that he meets all of the other criteria set forth in the prior authorization requirements, it is abundantly clear that the [appellant] does not meet the age criteria. Prior authorization requires that all criteria be met, not some or most" (*Id.*).

Tufts adds that "[w]hile [Tufts'] witnesses, [redacted] provided personal opinions on the effectiveness of Elevidys, the decision to deny coverage was based on review of the [appellant's] clinical records and the coverage criteria for Elevidys" (*Id.*, *citing to* Exh. 5, Tufts pre-hearing submission, pp. 92, 120).

Tufts also avers that MCMC Consult, a third-party independent reviewer, agreed with Tufts' initial decision to deny coverage of Elevidys for the appellant, finding that "there were no unique clinical circumstances applicable to the [appellant] that would make coverage of Elevidys medically appropriate. . . ." (*Id.*).

Tufts argues that federal law "grants state Medicaid agencies the right to 'place appropriate limits on a service based on such criteria as medical necessity or utilization control procedures'" [redacted]

Tufts further asserts that 42 U.S.C. § 1396r-8(d)(1)(A), the federal Medicaid Act, permits a state Medicaid agency to impose prior authorization (PA) requirements for any covered outpatient drug, so long as the agency provides a response by telephone or other telecommunication device within 24 hours of a request for prior authorization, and so long as the agency provides for the dispensing of at least a 72-hour supply of a covered outpatient prescription drug in an emergency situation. These rules do not apply to outpatient drugs used for weight loss or anorexia, cosmetic or hair growth drugs, fertility drugs, cough and cold medications, prescription vitamins and minerals, drugs for sexual dysfunction, and nonprescription drugs (42 U.S.C. § 1396r-8(d)(2)). Tufts avers that this PA requirement is applicable to all drugs required as part of EPSDT diagnosis and treatment services of a MassHealth-eligible child under age [redacted] following a screening of that child, and is also subject to a finding of medical necessity. Tufts notes that the term "medically necessary" is not explicitly defined in the Medicaid Act and quotes the court decision in Moore ex rel. Moore v. Reese, 637 F. 3d 1220, 1232 (11th Cir. 2011), for the proposition that even coverage of drugs that is mandatory under the federal Medicaid Act is subject to a medical necessity determination for a particular individual.

Next, Tufts contends that federal law also permits MassHealth to establish criteria concerning the medical necessity for drugs requiring prior authorization (*Id*., p. 6). Tufts cites to the "Introduction to the MassHealth Drug List," which states that "the [medical necessity] 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" (*Id*.). Tufts also cites to the "MassHealth Guidelines for Medical Necessity Determination Overview," which reflects that MassHealth develops its criteria for medical necessity and prior authorization via "an ongoing process that includes a rigorous review of the most current evidence-based literature and input from clinical and program staff, and often from external clinical experts" (*Id*.).

Finally, Tufts argues that under 42 U.S.C. § 1396r-8(d)(1)(B)(iv), a state Medicaid agency such as MassHealth may exclude or restrict coverage of a covered outpatient drug if the drug has been excluded by a state-established formulary. Citing to 42 U.S.C. § 1396r-8(d)(4)(C), Tufts asserts that MassHealth appropriately excluded Elevidys from coverage for children with DMD [redacted] and older, based on the following:

> 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 (or, in the case of a drug the prescribed use of which is not approved under the Federal Food, Drug, and Cosmetic Act [21 U.S.C. 301 et seq.] but is a medically accepted indication, based on information from the appropriate compendia described in subsection (k)(6)), 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.

(Id. at 6-7 (italics in Tufts memo))

In the Tufts memo, Tufts clarifies that the "identified population" for whom MassHealth has excluded coverage of Elevidys, pursuant to this statute, is children with DMD [redacted] and older (*Id*., p. 7).[14]

Findings of Fact

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

1. The appellant, a [redacted] boy who lives in the community, is a MassHealth Standard member enrolled in Tufts Health Together, a managed care organization (Testimony).

2. The appellant was diagnosed with Duchenne muscular dystrophy ("DMD") on June 11, 2024, just after his [redacted] birthday.

3. DMD results from a genetic mutation in the gene that makes dystrophin, a vital protein in the human body. This protein is crucial for the development and function of muscle tissue. DMD severely impairs the production of dystrophin, leading to the destruction of muscle tissue, eventually including heart and lung muscle. Patients are typically boys. Over time, the loss of dystrophin leads to an inability to walk, and a loss of cardiac and lung function (Testimony of [redacted]).

4. A diagnosis of DMD is typically associated with early death (*Id*.).

5. The appellant's pediatric neurologist at [redacted] submitted a prior authorization request to Tufts on July 10, 2024 on the appellant's behalf, seeking coverage of Elevidys gene therapy infusion for DMD (Exh. 5).

6. By letter dated July 12, 2024, Tufts denied the PA request; the denial letter stated in relevant part: "[Tufts] utilizes the MassHealth United Formulary. MassHealth's coverage guideline entitled [DMD] Disease Modifying Agents for the coverage of Elevidys (delandistrogene moxeparvovec-rokl) require that you are at least [redacted] and less than [redacted] at the time of administration. . . ." (Exh. 5, p. 95).

7. The appellant filed an expedited internal appeal of the denial decision with Tufts on July 16, 2024 (*Id*., p. 108).

8. Prior to making a decision on the expedited internal appeal filed by the appellant, Tufts referred the case to a consulting organization, [redacted] (Exh. 5).

[14] The compendia referred to at subsection k(6) of this statute are as follows: (a) American Hospital Formulary Service Drug Information; (b) United States Pharmacopeia-Drug Information (or its successor publications); and (c) the DRUGDEX Information System. *See also*, 42 U.S.C. § 1396r-8(g)(1)(B)(i).

9. A pediatric neurologist with [redacted] had a peer-to-peer discussion with [redacted] a second pediatric neurologist at [redacted] familiar with the appellant's case. After this peer-to-peer discussion with [redacted] recommended upholding the denial of the PA request (*Id.*, at pp. 122-125).

10. On July 17, 2024, Tufts notified the appellant by letter that it was upholding the original PA request denial because "the reviewer's findings indicate that the [MassHealth criteria] have not been met as the [appellant] is outside the age range of [redacted] at the time of administration" (*Id*. at 127).

11. The appellant filed this external appeal of the denials with the BOH on July 18, 2024 (Exh. 2).

12. Elevidys was added to the MassHealth Drug List effective March 4, 2024 for ambulatory children with DMD [redacted] and the eligibility criteria have not been updated since that time (Testimony, Exh. 11).

13. Per the MassHealth Drug List, to be approved for Elevidys therapy, documentation of all of the following is required:
    - appropriate diagnosis; **and**
    - member is [redacted] at the time of administration; **and**
    - prescriber is a neuromuscular specialist; **and**
    - copy of genetic test with a confirmed mutation in the DMD gene; **and**
    - member does not have any deletion in exon 8 or exon 9 of the DMD gene; **and**
    - copy of baseline anti-AAVrh74 total binding antibody titers < 1:400; **and**
    - member has a baseline measurement for both of the following:
        - North Star Ambulatory Assessment, including scores and times on individual items (within the past three months); **and**
        - Six-minute walk test (within the past three months); **and**
    - member is ambulatory as defined by a current six-minute walk test (6MWT – distance walked in six minutes in meters) ≥ 200 meters; **and**
    - one of the following:
        - member is on a stable dose of corticosteroid; **or**
        - attestation that the member will continue to utilize chronic corticosteroids after Elevidys infusion; **or**
        - demonstrated contraindication to corticosteroids; **and**
    - member has not previously received treatment with a gene therapy for DMD; **and**
    - infusion will take place in a qualified treatment facility; **and**
    - member is not currently utilizing antisense oligonucleotides; **and**
    - appropriate dosing.

14. In June, 2023, the FDA approved Elevidys gene therapy on an accelerated basis for ambulatory children with DMD aged [redacted] (Testimony).

15. In June 2024, the FDA expanded its approval of Elevidys for all children aged [redacted] and older with DMD, regardless of whether the children are ambulatory (Exh. 7).

16. In connection with the expanded approval of Elevidys for all children with DMD aged [redacted] and over, the FDA wrote in its June 20, 2024 press release: "While the large, randomized study of Elevidys failed to meet its statistical primary endpoint of improvement versus placebo in the North Star Ambulatory Assessment, the FDA found the observations regarding the secondary endpoints and exploratory endpoints to be compelling and to indicate clinical benefit compared to placebo. These endpoints include improvements in time to rise from the floor, 10-meter walk/run, time to ascend four steps and creatine kinase levels" (*Id*.).

17. According to Tufts' testimony, this expanded approval was "controversial;" the FDA scientific panel that examined the clinical trial results in children over [redacted] did not find significant clinical improvement in any age group which received the drug (Testimony of [redacted]).

18. Tufts, as an agent of MassHealth, adheres to the medical necessity and prior authorization criteria set forth in MassHealth regulations, and on the MassHealth Drug List (Testimony).

19. The appellant is prescribed prednisone, a glucocorticoid medication, by one of his [redacted] neurologists, [redacted] (Exh. 5, pp. 26-27).

20. A letter of medical necessity ("LOMN") for Elevidys for the appellant dated July 2, 2024 from [redacted] states: "[The appellant] [who has a history of gross motor delays] is a [redacted] with newly diagnosed DMD, confirmed by genetic testing showing a deletion of exons 46-51 in DMD. Clinically, he has proximal muscle weakness, calf hypertrophy, and markedly elevated creatine kinase. Since our last visit, Elevidys gene therapy has been approved for DMD boys [redacted] and older. We had a follow-up discussion today regarding gene therapy. We reviewed the . . . consent form, discussed specific logistics around eligibility – [the appellant's] AAVrh74 antibody is negative, alternative therapies including exon skipping, Givinostat, and continuing care with prednisone alone. While therapies such as exon skipping and Givinostat may medically and conceptually be given concurrently to Elevidys, I cannot guarantee that insurance would approve such combinations. We explicitly discussed the limited efficacy data and limited long-term safety data for Elevidys. The family confirmed that they are interested in moving forward with treatment" (*Id*., pp. 26-27).

21. Following the internal PA appeal denial by Tufts, in July, 2024, [redacted] submitted a second letter, which states in pertinent part: "Of note, [the appellant] is relatively strong for a boy his age with DMD. His North Star Ambulatory Assessment (NSAA), a comprehensive measure of strength and motor function, score is 25. By comparison, the mean NSAA for [redacted] the children that showed the most benefit in study one. . . was 20. Based on our experience with neurodegenerative diseases and gene therapy, treating early before muscle deterioration is likely why the younger boys showed more improvement. [The appellant's] relatively preserved strength (similar or even better than a typical [redacted] DMD boy) makes him an excellent candidate for gene therapy and increases the likelihood of a clinically meaningful benefit" (Exh. 9).

22. The appellant's total score on the NSAA, as of June 28, 2024, was 25 out of a possible 34. He was able to walk 403 meters in 6 minutes; per the MassHealth Drug List entry for Elevidys, children are considered "ambulatory" if they can walk 200 meters or more in 6 minutes (Exh. 5, pp. 22-23).

23. Medical documentation in the record reflects that as of July 2, 2024, the appellant's anti-AAVrh74 total binding antibody titers were less than 1:400 (considered a negative result), and that he has no deletion in exon 8 and/or exon 9 in the DMD gene. In addition, the appellant has not previously been treated with gene therapy for DMD, he is on a corticosteroid, and he is currently not utilizing antisense oligonucleotide medications such as Amondys 45 or Exondys 51 (Exh. 5).

24. The appellant, who has not been told of his DMD diagnosis, currently cannot keep up with his peers in terms of physical activities (Testimony of [redacted]).

25. Tufts did not perform an individualized assessment of the appellant when deciding whether to authorize Elevidys for treatment of his DMD (Testimony of [redacted]).

26. Sarepta Therapeutics, which makes Elevidys, has entered into a rebate agreement with the U.S. Department of Health and Human Services.

Analysis and Conclusions of Law

Pursuant to regulation 130 CMR 508.001, "MassHealth Member Participation in Managed Care:"

> (A) Mandatory Enrollment with a MassHealth Managed Care Provider. MassHealth members who are younger than [redacted] years old must enroll in a MassHealth managed care provider available for their coverage type. Members described in 130 CMR 508.001(B) or who are excluded from participation in a MassHealth managed care provider pursuant to 130 CMR 508.002(A) are not required to enroll with a MassHealth managed care provider.

(B) Voluntary Enrollment in a MassHealth Managed Care Provider. The following MassHealth members who are younger than [redacted] years old may, but are not required to, enroll with a MassHealth managed care provider available for their coverage type:
(1) MassHealth members who are receiving services from DCF or DYS;
(2) MassHealth members who are enrolled in the Kaileigh Mulligan Program, described in 130 CMR 519.007(A): The Kaileigh Mulligan Program. Such members may choose to receive all services on a fee-for-service basis;
(3) MassHealth members who are enrolled in a home- and community-based services waiver. Such members may choose to receive all services on a fee-for-service basis; or
(4) MassHealth members who are receiving Title IV-E adoption assistance as described at 130 CMR 522.003: Adoption Assistance and Foster Care Maintenance. Such members may choose to receive all services on a fee-for-service basis.

(C) Senior Care Organizations (SCO). MassHealth members who are [redacted] years of age or older may enroll in a SCO pursuant to 130 CMR 508.008(A).

(D) Integrated Care Organizations (ICO). Also referred to as "One Care plans." Members enrolled in an ICO (One Care plan) are participants in the Duals Demonstration, also known as "One Care." MassHealth members who are [redacted] years of age at time of enrollment may enroll in an ICO pursuant to 130 CMR 508.007(A).

…

Next, MassHealth regulation 130 CMR 508.004(B) states as follows:

Obtaining Services when Enrolled in an MCO.
(1) Primary Care Services. When the member selects or is assigned to an MCO, that MCO will deliver the member's primary care, determine if the member needs medical or other specialty care from other providers, and determine referral requirements for such necessary medical services. An MCO may provide a member's primary care through an MCO-administered ACO.
(2) Other Medical Services. All medical services to members enrolled in an MCO (except those services not covered under the MassHealth contract with the MCO, family planning services, and emergency services) are subject to the authorization and referral requirements of the MCO. MassHealth members enrolled in an MCO may receive family planning services from any MassHealth family planning provider and do not need an authorization or referral in order to receive such services. Members enrolled with an MCO should contact their MCO for information about covered services, authorization requirements, and referral requirements.

(Emphases added)

MassHealth regulation 130 CMR 508.010, "Right to a Fair Hearing," states as follows:

> Members are entitled to a fair hearing under 130 CMR 610.000: MassHealth: Fair Hearing Rules to appeal:
> (A) the MassHealth agency's determination that the MassHealth member is required to enroll with a MassHealth managed care provider under 130 CMR 508.001;
> **(B) a determination by the MassHealth behavioral health contractor, by one of the MCOs, Accountable Care Partnership Plans, or SCOs as further described in 130 CMR 610.032(B), if the member has exhausted all remedies available through the contractor's internal appeals process;**
> (C) the MassHealth agency's disenrollment of a member under 130 CMR 508.003(D)(1), (D)(2)(a), or (D)(2)(b), or discharge of a member from a SCO under 130 CMR 508.008(E); or
> (D) the MassHealth agency's determination that the requirements for a member transfer under 130 CMR 508.003(C)(3) have not been met.

(Emphasis added)

The appellant exhausted the internal appeal process offered through his MCO, Tufts Health Together. Thereafter, he requested a fair hearing with BOH, to which he is entitled pursuant to the above regulations.

As MassHealth's agent, Tufts is required to follow MassHealth laws and regulations pertaining to a member's care.

Generally, MassHealth will not pay for any services or prescriptions that are not medically necessary (130 CMR 450.204). 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.

(130 CMR 450.204(A))

Specifically, regarding pharmaceuticals, MassHealth publishes a Drug List that specifies the drugs

that are payable by MassHealth, and these drugs must be "approved by the U.S. Food and Drug Administration and manufactured by companies that have signed rebate agreements with the U.S. Secretary of Health and Human Services pursuant to 42 U.S.C. 1396r-8" (130 CMR 406.412(A)).

Here, the evidence shows that Sarepta Therapeutics, Inc., the manufacturer of Elevidys gene therapy, has signed a rebate agreement with the Secretary of Health and Human Services. As of June 2024, Elevidys is approved by the FDA for all children [redacted] and older, whether ambulatory or non-ambulatory, who are diagnosed with DMD.

Elevidys was added to the MassHealth Drug List on March 4, 2024, and is subject to prior authorization and medical necessity criteria, as follows:

- Documentation of all of the following is required:
    - appropriate diagnosis; **and**
    - member is [redacted] administration; **and**
    - prescriber is a neuromuscular specialist; **and**
    - copy of genetic test with a confirmed mutation in the DMD gene; **and**
    - member does not have any deletion in exon 8 or exon 9 of the DMD gene; **and**
    - copy of baseline anti-AAVrh74 total binding antibody titers < 1:400; **and**
    - member has a baseline measurement for both of the following:
        - North Star Ambulatory Assessment, including scores and times on individual items (within the past three months); **and**
        - Six-minute walk test (within the past three months); **and**
    - member is ambulatory as defined by a current six-minute walk test (6MWT – distance walked in six minutes in meters) ≥ 200 meters; **and**
    - one of the following:
        - member is on a stable dose of corticosteroid; **or**
        - attestation that the member will continue to utilize chronic corticosteroids after Elevidys infusion; **or**
        - demonstrated contraindication to corticosteroids; **and**
    - member has not previously received treatment with a gene therapy for DMD; **and**
    - infusion will take place in a qualified treatment facility; **and**
    - member is not currently utilizing antisense oligonucleotides; **and**
        - appropriate dosing.

(MassHealth Drug List)

MassHealth regulation 130 CMR 406.422(A) states in relevant part:

> Prescribers must obtain prior authorization from the MassHealth agency for drugs identified by MassHealth in accordance with 130 CMR 450.303: Prior Authorization. If the limitations on covered drugs specified in 130 CMR 406.412(A) and 406.413(A) and (C) would result in inadequate treatment for a diagnosed medical condition, the

prescriber may submit a written request, including written documentation of medical necessity, to the MassHealth agency for prior authorization for an otherwise noncovered drug.

The appellant is a MassHealth Standard member who was diagnosed with DMD just after he turned [redacted] in June, 2024. The appellant's neurologist, [redacted] submitted a prior authorization request for Elevidys, the only gene therapy approved for DMD, to Tufts in July, 2024. A second neurologist, [redacted] submitted a LOMN for Elevidys and a detailed letter requesting an appeal after the initial PA denial. Tufts denied the PA request on the basis that MassHealth does not cover this drug for children with DMD [redacted] and older. An internal appeal was requested, which upheld the denial decision.

A. Analysis under the Early and Periodic Screening Diagnostic and Testing (EPSDT) Services Regulations

The appellant is enrolled in MassHealth Standard and enrolled in Tufts, a MCO. As such, he is eligible for EPSDT services, as set forth at 130 CMR 450.140 though 149. The EPDST regulations, binding on MassHealth, state in relevant part:

> 130 CMR 450.140 "Early and Periodic Screening, Diagnostic and Treatment (EPSDT) Services: Introduction"
> (A) Legal Basis.
> **(1) In accordance with federal law at 42 U.S.C. 1396d(a)(4)(b) and 1396d(r), and 42 CFR 441.50, and notwithstanding any limitations implied or expressed elsewhere in MassHealth regulations or other publications, the MassHealth agency has established a program of Early and Periodic Screening, Diagnostic and Treatment (EPSDT) for MassHealth Standard and MassHealth CommonHealth members younger than [redacted] years old, including those who are parents.**
> (2) Any qualified MassHealth provider may deliver EPSDT services. However, in delivering well-child care, providers must follow the EPSDT Medical Protocol and Periodicity Schedule.
> (3) EPSDT screening services include among other things, health, vision, dental, hearing, behavioral health, developmental and immunization status screening services.
> (4) The regulations governing the EPSDT program are set forth in 130 CMR 450.140 through 450.149.
> **(B) Program Objectives. The objectives of the EPSDT program are**
> **(1) to provide comprehensive and continuous health care designed to prevent illness and disability;**
> **(2) to foster early detection and prompt treatment of health problems before they become chronic or cause irreversible damage;**
> (3) to create an awareness of the availability and value of preventive well-child care services; and
> (4) to create an awareness of the services available under the EPSDT program, and

where and how to obtain those services.

…

450.144: "EPSDT Services: Diagnosis and Treatment"
(A) (1) EPSDT diagnosis and treatment services consist of all medically necessary services listed in 1905(a) of the Social Security Act (42 U.S.C. 1396d(a) and (r)) that are
(a) needed to correct or ameliorate physical or mental illnesses and conditions discovered by a screening, whether or not such services are covered under the State Plan; and
(b) payable for MassHealth Standard and MassHealth CommonHealth members younger than [redacted] years of age, if the service is determined by the MassHealth agency to be medically necessary.
(2) To receive payment for any service described in 130 CMR 450.144(A)(1) that is not specifically included as a covered service under any MassHealth regulation, service code list, or contract, the requester must submit a request for prior authorization in accordance with 130 CMR 450.303. This request must include, without limitation, a letter and supporting documentation from a MassHealth-enrolled physician, physician assistant, certified nurse practitioner, certified nurse midwife, or certified clinical nurse specialist documenting the medical need for the requested service. If the MassHealth agency approves such a request for service for which there is no established payment rate, the MassHealth agency will establish the appropriate payment rate for such service on an individual-consideration basis in accordance with 130 CMR 450.271. If the request is for a member who is enrolled in an MCO or Accountable Care Partnership Plan, as defined in 130 CMR 450.000, the requestor must submit the request to the MCO or Accountable Care Partnership Plan according to the MCO's or Accountable Care Partnership Plan's prior authorization process. . .

(Emphases added)[15]

[15] *See also*, Smith v. Benson, 703 F. Supp. 2d 1262, 1260 (S.D. Fla. 2010) ("the scope of EPSDT services is defined at 42 U.S.C. § 1396d(r)(5). In particular, § 1396d(r)(5) provides that EPSDT services are mandated if those services are a type of 'medical assistance,' as defined in § 1396d(a), that is 'necessary ... to correct or ameliorate defects and physical and mental illnesses and conditions discovered by the screening services, whether or not such services are covered under the State plan'").

The appellant here is entitled, under EPSDT, to medically necessary services to correct or ameliorate a physical condition, DMD, discovered upon being screened by his neurologists.[16] DMD is a progressive neuromuscular disease in boys that, if untreated, typically leads to an early death from wasting of muscle, including heart and lung muscle. While there are other drugs approved for treatment of children with DMD, such as Exondys and Givinostat, the appellant's neurologist has chosen Elevidys for him.[17] She writes that the appellant's relatively preserved strength (similar or even better than a typical [redacted] year old DMD boy) makes him an excellent candidate for gene therapy and increases the likelihood of a clinically meaningful benefit. While studies of the benefits and efficacy of Elevidys in slowing the progression of DMD are ongoing, the FDA announced, when expanding authorization for Elevidys in June 2024, that the secondary endpoints and exploratory endpoints for Elevidys therapy were "compelling," and indicated clinical benefit compared to placebo. These clinical benefits included improvements in time to rise from the floor, 10-meter walk/run, time to ascend four steps and creatine kinase levels (Exh. 7). In addition, [redacted] the appellant's neurologist, documented that based on [redacted] experience with neurodegenerative diseases and gene therapy, treating early with gene therapy, before muscle deterioration, leads to stabilization in muscle strength.

Further, although the appellant does not meet the age requirement set forth in the PA criteria contained in the MassHealth Drug List [redacted] the appellant meets *all other* PA criteria set forth in the MassHealth Drug List for this medication: having anti-AAVrh74 total binding antibody titers less than 1:400 (considered a negative result), being on a corticosteroid (prednisone), having no deletion in exon 8 and/or exon 9 in the DMD gene, having not previously been treated with gene therapy for DMD, and not utilizing antisense oligonucleotide medications such as Amondys 45 or Exondys 51.

The appellant is also still ambulatory, as defined by the results of his 6MWT administered as part of the NSAA. When approving Elevidys on an accelerated basis for *all children with DMD* [redacted] *and older* in June 2024, the FDA did not require that such children continue to be ambulatory.

[16] Section 1905 of the Social Security Act (codified at 42 U.S.C. § 1396d) specifically identifies medically necessary prescribed drugs as being payable under Medicaid pursuant to 42 U.S.C. § 1396d(a)(12), and also identifies the EPSDT services listed at 42 U.S.C. § 1396d(r) as being payable for children under age [redacted] enrolled in Medicaid, including "[s]uch other necessary health care, diagnostic services, treatment, and other measures described in section 1905(a) to correct or ameliorate defects and physical and mental illnesses and conditions discovered by the screening services, whether or not such services are covered under the State plan" (42 U.S.C. § 1396d(r)(5)).

[17] Exondys is an antisense oligonucleotide medication, and Givinostat is a nonsteroidal histone deacetylase (HDAC) inhibitor that works by targeting pathogenic processes to reduce inflammation and loss of muscle. Neither is "gene therapy," as Elevidys is. Antisense oligonucleotides (ASOs) such as Exondys facilitate exon skipping for specific DMD gene mutations, but the ASOs can only address a minority of the gene mutations and require repeated administration. Elevidys, on the other hand, is a recombinant gene therapy designed to deliver into the body a gene that leads to production of Elevidys micro-dystrophin, a shortened protein (138 kDa, compared to the 427 kDa dystrophin protein of normal muscle cells) that contains selected domains of the dystrophin protein present in normal muscle cells; it is administered as a single IV dose. *See*, www.fda.gov (last checked January 14, 2025).

Under EPSDT, an individual assessment of each Medicaid-eligible child must be made by his or her physicians. *See*, [redacted] (children diagnosed with severe emotional disturbances in Massachusetts were entitled to individual screening and evaluation services under EPSDT, and to a broad scope of state services under EPSDT regulations, including comprehensive assessments, coordination of care, crisis services, and in-home support services);[redacted]

While Tufts, in its post-hearing memo, asserts that it considered the appellant's particular NSAA test results and other clinical data when deciding whether to approve Elevidys for him, despite his age, this assertion is belied by the hearing testimony of [redacted] The latter was unequivocal when he testified that only the appellant's age, and not his individual circumstances, was considered when Tufts made the decision to deny the requested treatment.

[redacted] the appellant's neurologist, has satisfied the criteria set forth at 130 CMR 450.144(A)(2), where a particular service -- in this case, an infusion of Elevidys for a [redacted] with DMD -- is not specifically included as a covered service under any MassHealth regulation, service code list, or contract. [redacted] is a MassHealth provider, and she has submitted a PA request, LOMN and supporting clinical documentation in support of this medication.[18] Tufts, as MassHealth's agent, may set an appropriate rate of reimbursement for the infusion procedure, informed by the following regulation:

> (A) The MassHealth agency may identify certain services as requiring individual consideration (I.C.) in program regulations, associated lists of service codes and service descriptions, billing instructions, provider bulletins, and other written issuances from the MassHealth agency. For services requiring individual consideration, the MassHealth agency establishes the appropriate amount of payment based on the standards and criteria set forth in 130 CMR 450.271(B). Providers claiming payment for any I.C.-designated service must submit with such claim a report that includes a detailed description of the service, and is accompanied by supporting documentation that must minimally include where applicable, but is not limited to, an operative report, pathology report, or in the case of a purchase, a copy of the supplier's invoice. The MassHealth agency does not pay claims for "I.C." services unless it is satisfied that the report and documentation submitted by the provider are adequate to support the claim.
> **(B) The MassHealth agency determines the appropriate payment for an I.C. service in accordance with the following standards and criteria:**
> **(1) the amount of time required to perform the service;**
> **(2) the degree of skill required to perform the service;**
> **(3) the severity and complexity of the member's disease, disorder, or disability;**
> **(4) any applicable relative-value studies; and**

[18] *See*, Internet website of the Massachusetts Board of Registration in Medicine, https://findmydoctor.mass.gov/profiles (last checked January 14, 2025).

(5) any complications or other circumstances that the MassHealth agency deems relevant.

(130 CMR 450.271) (emphasis added)

Based on the medical opinions of his neurology team at [redacted] it is abundantly clear that the appellant will benefit as much as, or more than, other younger children diagnosed with DMD from treatment with Elevidys gene therapy. Elevidys is thus medically necessary for the appellant, under EPSDT and pursuant to 130 CMR 450.204(A).

As such, under the EPDST mandate incorporated into MassHealth regulations, MassHealth must pay for the cost of such treatment for the appellant.

B. Analysis under federal Medicaid Law (42 U.S.C. § 1396d *et seq*.)

The appellant asserts the use of Elevidys gene therapy is medically approved by the FDA for all ambulatory and non-ambulatory DMD-diagnosed children [redacted] and above, which includes the appellant. He concludes that, therefore, by not providing the requested coverage, Tufts violated the Medicaid Act. Under the Medicaid Act, 42 U.S.C. § 1396d *et seq*., states are given considerable latitude in fashioning their Medicaid programs, and must consider utilization management practices, and cost control factors, including mandating the use of less expensive generic drugs over brand-name drugs unless a prescriber specifies otherwise.

Pursuant to 42 U.S.C. § 1396r-8(d)(1)(B), there are four exclusions/restrictions under which individual states can deny coverage of a drug approved by the FDA when the manufacturer has entered into a rebate agreement with HHS, as follows:

(i) the prescribed use is not for a medically accepted indication (as defined in subsection (k)(6));
(ii) the drug is contained in the list referred to in paragraph (2);
(iii) the drug is subject to restrictions pursuant to an agreement between a manufacturer and a State authorized by the HHS 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).

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

Paragraph 4(C) of this section of the law, as cross-referenced in 42 U.S.C. § 1396r-8(d)(1)(B)(iv), above, states:

> **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** (or, in the case of a drug the prescribed use of which is not approved under the Federal Food, Drug, and Cosmetic Act [21 U.S.C. 301 et seq.] but is a medically accepted indication, based on information from the appropriate compendia described in subsection (k)(6)), **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.**

42 U.S.C. § 1396r-8(d)(4)(C) (emphasis added)

Tufts argued that MassHealth has made such a permitted exclusion for an identified population in the instant matter, *to wit*, children with DMD over [redacted] and older. However, neither MassHealth nor Tufts has explicitly stated, in a written explanation available to the public, that Elevidys does not have a significant, clinically meaningful therapeutic advantage in terms of safety, effectiveness, or clinical outcome for children [redacted] and older with DMD, over other drugs included in the MassHealth formulary, as required by federal law, above.

Further, neither MassHealth nor Tufts has identified which other drugs in the formulary used to treat DMD, if any, it believes are more effective and beneficial in treating DMD in children [redacted] and over. Pursuant to the above law, it must do so before it excludes Elevidys from treatment for a specific disease (DMD) and for an identified population (children [redacted] and over).

Federal and state case law also supports that state Medicaid programs may not deny coverage of a covered outpatient drug based on the state's medical judgment about the drug's purported lack of effectiveness, where the FDA has already spoken on this issue and has approved the drug for all members of a certain identified population. *See,* [redacted] In these decisions, both courts held that the state Medicaid programs in those states could not rely on new or different clinical data to decide whether certain drugs should be covered, where the FDA had already evaluated clinical data concerning the efficacy and safety standards of the drugs in question (*Id*., pp. 5-6).

The court in [redacted] wrote that "the [federal] statutory scheme is carefully constructed in such a way to precisely circumscribe the only methods by which a state may remove a Medicaid-eligible drug from coverage and prevent it from either arbitrarily removing a drug or adopting its own *ad hoc* procedure for removing a drug from coverage." That is precisely what MassHealth has done in the instant matter, bypassing the processes set forth in 42 U.S.C. § 1396r-8(d)(4)(C), above, before arbitrarily excluding coverage of Elevidys for children [redacted] and over with DMD.

In failing to cover Elevidys for all children, both ambulatory and non-ambulatory, [redacted] and above, and by arbitrarily denying coverage of this drug for children [redacted] and above, MassHealth and/or Tufts have, in fact, violated the requirements set forth above in the federal Medicaid Act.

MassHealth must cover the cost of Elevidys for the appellant.

The appeal is therefore **APPROVED**.

Order for Tufts Health Plan

Send written notices of approval for Elevidys treatment to the appellant's parent/guardian, and to his treating neurologists at [redacted]

Implementation of this Decision

If this decision is not implemented within 30 days after the date of this decision, you should contact your MassHealth Enrollment Center. If you experience problems with the implementation of this decision, you should report this in writing to the Director of the Board of Hearings, at the address on the first page of this decision.

Paul C. Moore
Deputy Director/Hearing Officer
Board of Hearings

cc: Tufts Health Plan, Nicole Dally, Program Manager, Appeals and Grievances, One Wellness Way, Canton, MA 02021

[redacted]

[redacted]