Why it is Important to Ensure the Price is Right: Access Restrictions for Therapies Treating Duchenne Muscular Dystrophy

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OBJECTIVES

Duchenne muscular dystrophy (DMD) is a rare and highly burdensome condition, yet some insurers elect to not cover these life-changing therapies. With increasing therapeutic competition, additional barriers may impact existing treatments. This research aims to provide an updated evaluation of trends between price and coverage outcomes for DMD therapies in the United States compared to a 2024 analysis, incorporating the recent approval of Duvyzat.

METHODS

FDA databases were used to identify targeted therapies approved in DMD. Corticosteroids (Emflaza and Amagree) are excluded as they have significantly different value and price considerations.

DMD THERAPIES ASSESSED

PRODUCT	LAUNCH YEAR	Estimated US REVENUE*	INDICATED DMD PATIENT POPULATION
Exondys 51 (eteplirsen)	2016	~$450M	Confirmed DMD gene mutation amenable to exon 51 skipping
Vyondys 53 (golodirsen)	2019	~$264M	Confirmed DMD gene mutation amenable to exon 53 skipping
Viltepso (viltolarsen)	2020	~$117M	
Amondys 45 (casimersen)	2021	~$263M	Confirmed DMD gene mutation amenable to exon 45 skipping
Elevidys (delandistrogene moxeparvovec-rokl)	2023	~$820M	Ambulatory pediatric patients aged 4 through 5 years with confirmed mutation in DMD gene
Duvyzat (givinostat)	2024	~N/A	Patients aged 6 years and older with a confirmed diagnosis of DMD

Table 1. Summary of DMD targeted therapies included in analysis
**Estimated US revenue is extrapolated/estimated from 2024 fiscal year per product global revenue and the proportion of the products' collated US revenue to rest of world revenue*

Therapies' annual Wholesale Acquisition Costs (WACs) were calculated and analyzed together to understand key differences. An ICER report was used to analyze perceptions of price-value alignment. Finally, publicly available coverage policies at 10 of the largest commercial plans (by covered lives) were used to analyze the access of DMD therapies

RESULTS

ANNUAL WAC OF DMD THERAPIES

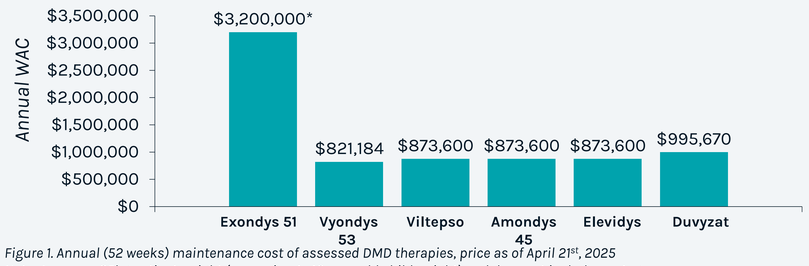

Figure 1. Annual (52 weeks) maintenance cost of assessed DMD therapies, price as of April 21st, 2025
Note: Assumes 35kg patient weight (approximate 10-year-old child weight) and does not include wastage
**Elevidys is administered as a one-time treatment, and is priced flat regardless of weight*

Prices of DMD therapies are generally $800k - $1M annually; Elevidys is a gene therapy and was 1st all-comer treatment for DMD. Most of the larger plans (by covered lives) cover use, but some smaller plans do not provide coverage. Across therapies, most plans manage to the same level of restriction; however, plans may prefer or exclude certain entrants (e.g., Molina only covers Elevidys). Most plans require PAs based on trial criteria to assess ambulatory status; however, levels of evidence required may vary by plan (i.e., variations in 6-minute walk test benchmark and/or requiring documentation of additional ambulatory tests). Plans not providing coverage consider therapies to have insufficient evidence due to the use of surrogate endpoints (i.e., increase in dystrophin). Duvyzat coverage is favorable, likely driven by payers' increased familiarity with DMD and Duzyvat's nonsteroidal treatment of all DMD mutations.

PRIOR AUTHORIZATION COVERAGE OF DMD THERAPIES

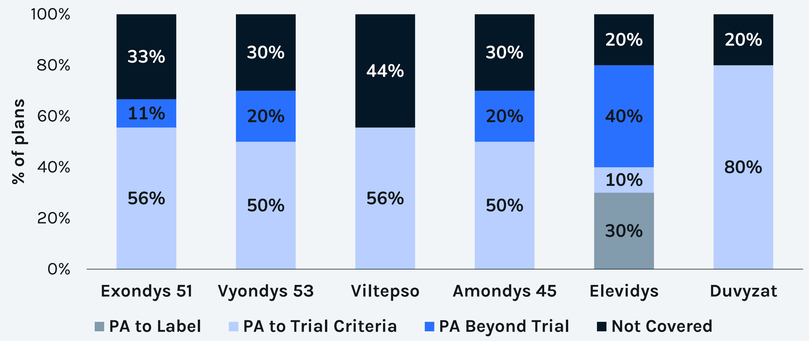

Figure 2. Commercial coverage for DMD therapies at ten of the largest plans by lives
Public commercial plans analyzed: United Healthcare, Anthem, Aetna, Centene Corporation, Health Care Service Corporation (HCSC), Cigna, Humana, Kaiser Permanente, BlueCross BlueShield Michigan, Molina Healthcare
Analysis conducted on 04/01/2025, updates to coverage may have occurred since initial analysis

TYPICAL CLINICAL CRITERIA FOR COVERAGE

PRODUCT	TRIAL CRITERIA	BEYOND TRIAL CRITERIA
Exondys 51	Age, 6MWT, FVC ≥50%, LVEF >40%, concurrent corticosteroid use	NSAA score of 17 or Gower's test of <7 seconds, dystrophin level, Brooke Upper Extremity Scale
Vyondys 53	Age, ambulatory, 6MWT, FVC ≥50%, concurrent corticosteroid use	Failure of corticosteroid, LVEF ≥50%, exclude patients with nocturnal ventilation
Viltepso	Age, ambulatory, 6MWT, standing test, climbing test, concurrent corticosteroid use	FVC ≥50%, LVEF ≥40%, failure of corticosteroid
Amondys 45	Age, ambulatory, 6MWT, FVC ≥50%, concurrent corticosteroid use	Failure of corticosteroid, exclude patients with nocturnal ventilation
Elevidys	Age, ambulatory status, anti-AAVrh74 total binding antibody titers < 1:400	Baseline liver function tests required, Failure of corticosteroid
Duvyzat	Age, ambulatory, 6MWT, FVC ≥50%, concurrent corticosteroid use	N/A

Table 2. Trial criteria required for coverage in plans assessed in analysis; 6MWT: 6-minute walk test, FVC: Forced vital capacity, LVEF: Left ventricular systolic function, NSAA: North Star Ambulatory Test

ICER analysis found two DMD therapies provided low long-term value but still recommend payers to cover the therapies with prior authorizations.

ICER OUTCOMES (2019)

DRUG	CLINICAL OUTCOME	ECONOMIC OUTCOME	PAYER RECOMMENDATION
Exondys 51	Lack of sufficient evidence to show net health benefit	Low long-term value considering cost	Prior authorization criteria should be based on clinical evidence, guidelines, and input from clinicians and patient advocacy groups
Vyondys 53		N/A; price not established	

Table 3. Summary of ICER report outcomes for DMD therapies
Note: Emflaza was also analyzed in this ICER report but is excluded from this poster for previously listed reasons

CONCLUSIONS

Despite the use of surrogate endpoints and unfavorable clinical and economic outcomes from ICER, most plans cover DMD therapies. As a rare, pediatric, and burdensome disease with an ongoing need for efficacious treatments, insurers are inclined to cover DMD therapies. Additionally, patient advocacy groups have likely played a large role in pressuring payers to cover DMD treatments due to its approval and high level of unmet need. To minimize impact on budget, plans attempt to restrict access to whom they determine are the most appropriate patients for treatment (i.e., population studied in trial) or only approve on a case-by-case basis (i.e., non-formulary). Despite the strict criteria that is implemented, patients are still receiving therapy evidenced by strong sales across treatments.

FUTURE IMPLICATIONS

Though current agents have largely been covered with surrogate outcomes, establishing differentiation on functional outcomes will likely be a key access driver in the future. Rare disease therapies have also increasingly been carved out from self-funded plans to help manage costs, making reinsurance more popular as well. As the development of more rare disease therapies continues, the DMD access and treatment landscape highlights the need for manufacturers to create and refine their commercial strategy throughout product development to optimize commercial opportunity. Manufacturers must also consider navigating and understanding reinsurance to improve patient access.

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PAG Influence on Reimbursement Decision

1. https://mdaquest.org/insurance-denials-for-gene-therapy-treatment-delay-access-to-care/

Coverage Policies

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2. Anthem: https://www.anthem.com/
3. Aetna: https://www.aetna.com/
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