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

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OBJECTIVES

Over the last 10 years, there have been a number of new therapeutic options approved for the treatment of Duchenne muscular dystrophy (DMD). Despite the severity and rarity of DMD, some health plans are choosing not to cover these life-prolonging therapies. This research aims to evaluate trends in price and payer coverage outcomes of DMD therapies in the United States.

METHODS

FDA databases were used to identify targeted therapies approved in DMD. Emflaza (deflazacort), was excluded from this poster as it is a corticosteroid, which have significantly different value and price considerations.

DMD THERAPIES ASSESSED

PRODUCT	LAUNCH YEAR	ESTIMATED 2023 US REVENUE*	INDICATED DMD PATIENT POPULATION
Exondys 51 (eteplirsen)	2016	$457M	Confirmed DMD gene mutation amenable to exon 51 skipping
Vyondys 53 (golodirsen)	2019	$110M	Confirmed DMD gene mutation amenable to exon 53 skipping
Viltepso (viltolarsen)	2020	$90M	
Amondys 45 (casimersen)	2021	$231M	Confirmed DMD gene mutation amenable to exon 45 skipping
Elevidys (delandistrogene moxeparvovec-rokl)	2023	$200M	Ambulatory pediatric patients aged 4 through 5 years with a confirmed DMD gene mutation

Table 1. Summary of DMD targeted therapies included in analysis
**Exondys, Vyondys, Amondys estimated US revenue is extrapolated/estimated from 2023 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

Prices of current DMD therapies vary greatly from several hundreds of thousands to millions of dollars; all but Elevidys vary linearly based on weight (kg).

ANNUAL WAC OF DMD THERAPIES

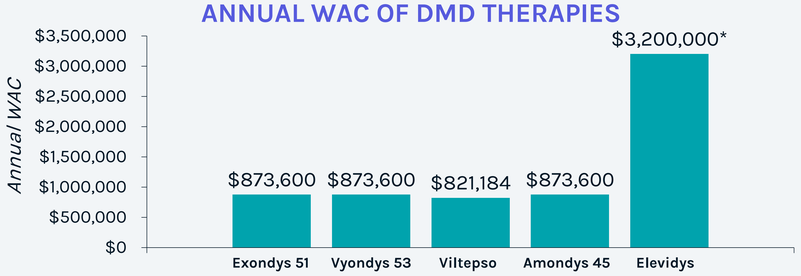

Figure 1. Annual (52 weeks) maintenance cost of assessed DMD therapies, price as of March 22nd, 2024
Note: Assumes 35kg patient weight (approximate 10-year-old child weight) and does not include wastage
**Elevidys is administered as a one-time treatment, and is priced flat regardless of weight*

Most of the larger plans (by covered lives) allow use of DMD therapies, but some smaller plans do not provide coverage. Across therapies, most manage to the same level of restriction. However, Cigna covers all but Elevidys, and Molina covers Elevidys but none of the others. Most plans require prior authorizations based on trial criteria to assess ambulatory status. To be eligible for coverage across plans, different levels of evidence are required to demonstrate ambulatory status (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 of clinical benefit due to the use of surrogate endpoints (i.e., increase in dystrophin).

PRIOR AUTHORIZATION COVERAGE OF DMD THERAPIES

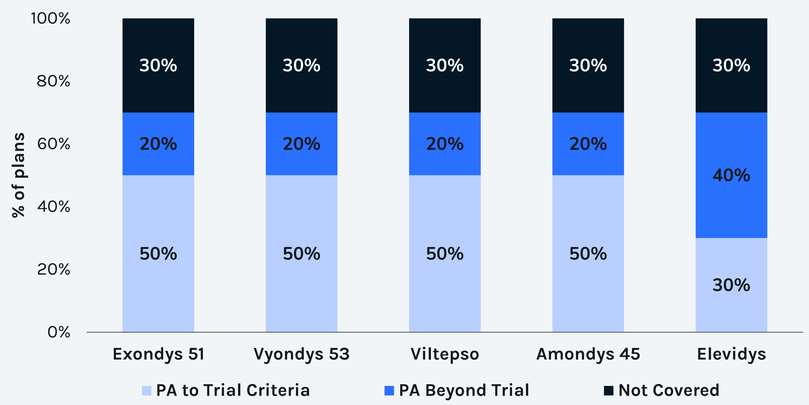

Figure 2. Commercial coverage for DMD therapies at ten of the largest plans by lives
Public commercial plans analyzed: United Healthcare, Anthem, Aetna, Centene Corporation, Health Care Service Corporation (HCSC), Cigna, Humana, Kaiser Permanente, BlueCross BlueShield Michigan, Molina Healthcare
Analysis conducted on 03/01/2024, 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

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), particularly for Elevidys due to its high-cost.

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. With over 75 therapies in development to treat DMD, payers are likely to soon have additional leverage for coverage decisions. Contracting may increase in prominence and undifferentiated products may subsequently step through formulary preferred options. As the development of rare disease therapies continues to progress, the future DMD access and treatment landscape highlights the need for manufacturers to create and continually refine a comprehensive commercial strategy throughout product development to optimize the commercial opportunity.

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