MERCI Brief
October 2024

MEDICAID EVIDENCE REVIEW AND COST INITIATIVE BRIEF

Exon-Skipping Pharmaceutical Treatments for Duchenne Muscular Dystrophy

OVERVIEW

Between 2016 and 2021, the US Food and Drug Administration (FDA) gave accelerated approval to 4 exon-skipping pharmaceutical treatments for Duchenne muscular dystrophy (DMD): eteplirsen (Exondys 51), golodirsen (Vyondys 53), viltolarsen (Viltepso), and casimersen (Amondys 45).[1,2,3,4] DMD is a rare genetic condition characterized by progressive loss of muscle function due to mutations in the dystrophin gene.[5] The median life expectancy for an individual with DMD is 22 years, with symptoms beginning to appear in childhood, mostly between 2 to 11 years of age.[6] In our analysis of 81 million members enrolled in Medicaid in 2021, we identified 6,041 as having DMD. Within Medicaid, DMD was most common among male non-Hispanic White members, and members who identify as Hispanic or Latino (54.5% and 30.3%, respectively).

Standard medications for DMD include corticosteroids, administered in the early phase of DMD when patients are still able to walk, as well as continuing throughout the course of the dis-

MERCI Aims

The Medicaid Evidence and Review of Cost Initiative (MERCI) describes policy considerations for drugs approved by the US Food and Drug Administration (FDA) through the accelerated approval pathway. This brief provides information on:

- The estimated prevalence of target diagnoses (the accelerated approval drug's indication[s]) among Medicaid members;
- The clinical trial population used to support FDA approval, and how similar it is to Medicaid members overall; and
- Projected drug costs for state Medicaid programs, including a breakdown of state and federal funds using the Federal Medical Assistance Percentage (FMAP).

MERCI analyses include national and state-level data where available to inform budget and policy decisions by state and federal policymakers.

ease.[5] Because of the complexity of the disease, a multidisciplinary team approach is needed to manage the condition[5]; while there is a recently FDA-approved gene therapy for DMD, the clinical outcomes for improvement in physical function and mobility have not been established.[7] As a result, the new exon-skipping therapies (eteplirsen, golodirsen, viltolarsen, and casimersen) have the potential to play a role.[1-4] These therapies allow the body to skip over a mutated section in the dystrophin gene and produce a functional dystrophin protein.[1-4] As muscle damage in individuals with DMD is caused by an absence of this protein, increasing dystrophin is hoped to slow down damage but improved motor function has not been established.[1-4]

All 4 exon-skipping therapies were approved via the FDA's accelerated approval pathway, primarily based on small increases in dystrophin levels in biopsied muscle tissue, a surrogate endpoint of DMD progression.[8-11] Our analysis estimated that total 2021 Medicaid spending nationally on exon-skipping treatments was $252.1 million (excluding spending in 3 states for which we do not have data), with $171.2 million coming from federal funds and $80.8 million from state funds. Despite these significant expenditures, none of these exon-skipping therapies have been converted to full FDA approval, nor have the results of their confirmatory trials been published at the time of writing this brief. As a result, individuals with DMD, their families, and clinicians do not have clear evidence of whether these treatments are effective.

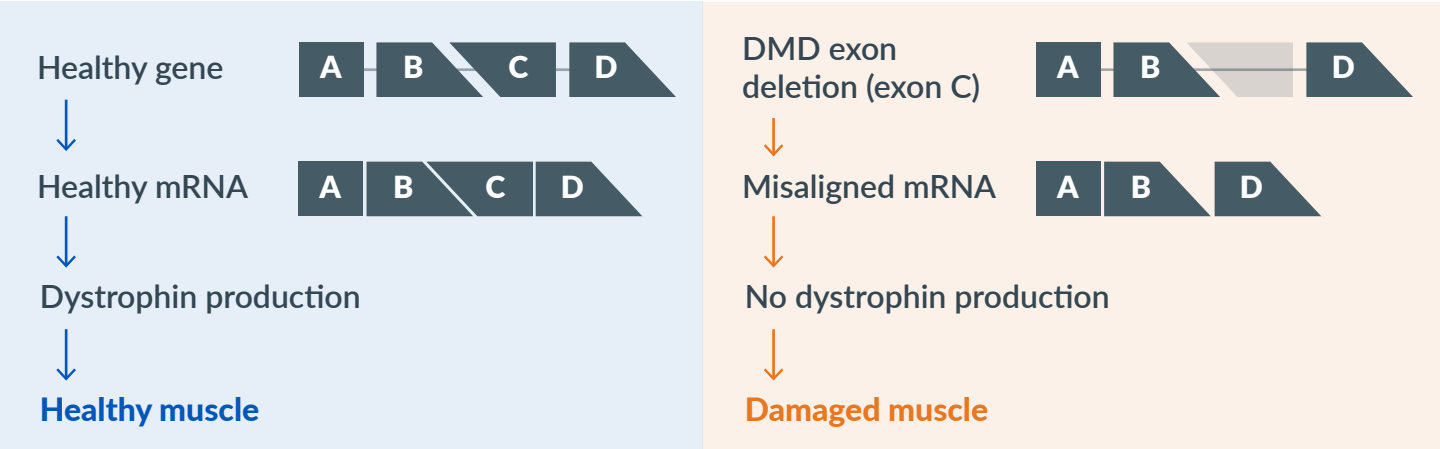
What Is Duchenne Muscular Dystrophy?

DMD is a rare genetic condition characterized by progressive loss of muscle function in childhood due to variants in the dystrophin gene, affecting multiple parts of the body (Exhibit 1).[5] The variants limit the production of dystrophin, a critical protein required for muscle structure and function.[5,12] The gene for dystrophin resides on the X chromosome and DMD mutations to the dystrophin gene are typically inherited, although they can also arise spontaneously.[13] As an X chromosome–linked disorder, it affects males almost exclusively, as they have only a single X chromosome; however, female carriers can also develop symptoms of muscle weakness.[14]

Symptoms of DMD begin to appear in childhood, mostly between 2 to 11 years of age, and include progressive muscle weakness, paradoxically enlarged calves (due to replacement of the calf muscles with fat and connective tissue), skeletal muscle wasting (decreased muscle size and strength), scoliosis (sideways curvature of the spine), loss of joint mobility, as well as cognitive

EXHIBIT 1

How DMD affects muscle function



Abbreviation. DMD: Duchenne muscular dystrophy.

DUCHENNE MUSCULAR DYSTROPHY (DMD) and EXON-SKIPPING THERAPIES

OVERVIEW

DMD FREQUENCY

INCIDENCE IN THE US

Approximately 17.24 per 100,000 live male births, corresponding to approximately 362 individuals diagnosed in 2019[17]

PREVALENCE IN MEDICAID

6,041 Medicaid members (or 7.4 per 100,000 members) have DMD in 47 states

EXON-SKIPPING THERAPIES FACTS

DRUG PRICE PER PATIENT $703,872 to $748,800 per year

FDA ACCELERATED APPROVAL DATE *None have yet been converted to full FDA Approval

eteplirsen	golodirsen	viltolarsen	casimersen
September 2016	December 2019	August 2020	Februrary 2021

DRUG UPTAKE

In 2021, the percentages of individual Medicaid members with DMD undergoing treatment were 27.5% for eteplirsen, 12.2% for golodirsen, 5.8% for viltolarsen, and 15.1% for casimersen

DRUG ADHERENCE

Of those receiving eteplirsen in 2021, 76% used the medication consistently over time. The adherence rates in 2021 were 74.6% for golodirsen, 69.9% for casimersen, and 67.9% for viltolarsen

MEDICAID COST ESTIMATES

2021 ESTIMATED COST TO MEDICAID

$252.1 million, with $175.5 million coming from federal funds and $76.5 million from state funds

PROJECTED ANNUAL COST TO MEDICAID

National estimate to treat eligible Medicaid members at full uptake: $315.2 million, with $200.1 million coming from federal funds associated with non–dually eligible Medicaid beneficiaries and $115.1 million from state funds

Sources. Information sourced from IPD Analytics and the FDA websites, unless otherwise referenced. Estimates of costs to Medicaid are based on our analysis presented in the historical and potential Medicaid spending on exon-skipping therapies sections.
Abbreviations. DMD: Duchenne muscular dystrophy; FDA: US Food and Drug Administration.

impairment including delayed speech and language development.[13] People with DMD also develop impairment of the heart and respiratory muscles, leading to fatigue, shortness of breath, lung collapse due to weak inspiration, cardiomyopathy (weakening of the heart muscle), and ultimately, respiratory failure.[15]

Over time, people with DMD typically transition from being able to walk (i.e., ambulatory), to being able to walk only some of the time, to being unable to walk (i.e., nonambulatory).[5] People with DMD typically need a wheelchair by 8 to 14 years of age.[16] For individuals with DMD, the median life expectancy is 22 years.[6] People born more recently have a significantly higher life expectancy (patients born after 1990 have a median life expectancy of 28 years).[6]

In the US, the diagnosed incidence of DMD is approximately 17.24 per 100,000 live male births, corresponding to approximately 362 new cases in 2019.[17] Based on data from 10 states, the prevalence of DMD is highest among people who identify as non-Hispanic White, (who account for around 60% of all people with *any* muscular dystrophy, the majority of which have DMD), followed by people who identify as Hispanic or Latino (around 20% of all people with any muscular dystrophy).[18]

Individuals with DMD are typically identified after suggestive signs and symptoms are noticed, such as weakness, clumsiness, difficulty with stair climbing, or toe walking.[5] Less common symptoms, which include developmental delay or increased concentrations of routine blood test markers (including creatine kinase), can also lead health care providers to a diagnosis of DMD.[5]

How Is DMD Managed?

Standard medications for DMD include corticosteroids (including prednisone and deflazacort) administered in the early ambulatory phase of DMD when patients are still able to walk, as well as continuing throughout the course of the disease.[5] Corticosteroid treatment has been associated with improved strength and physical functioning; however, these therapies often have negative side effects, such as weight gain, metabolic changes, lower bone density, and behavioral problems.[5,19] Because of the complexity of the disease, a multidisciplinary team approach is needed to manage the condition.[5] While there is an FDA-approved gene therapy (delandistrogene moxeparvovec) for DMD, the clinical outcomes for improvement in physical function and mobility have not been established.[7]

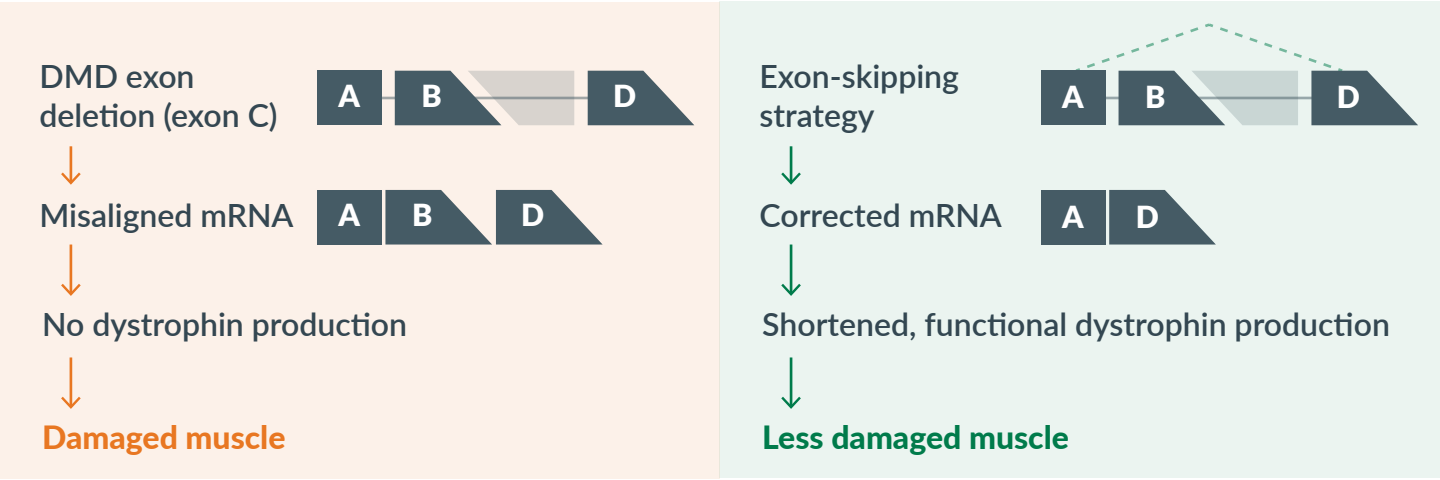
With the availability of new treatment options for DMD, including exon-skipping therapies (eteplirsen, golodirsen, viltolarsen, and casimersen) and gene therapy (delandistrogene moxeparvovec), standard of care is likely to change. Each of the exon-skipping therapies has a wholesale acquisition cost of over $700,000 per year, and the gene therapy has a one-time cost of $3.2 million.[20]

Data from the Muscular Dystrophy Surveillance, Tracking, and Research Network (MD STAR*net*), a multisite and multistate population-based surveillance system funded by the Centers for Disease Control and Prevention (CDC), show disparities in treatment by race or ethnicity.[21] MD STAR*net* and other researchers found that males identifying as non-Hispanic Black experienced delays across the DMD diagnostic journey.[21] They were typically older at initial evaluation, consultation with a neurology or neuromuscular specialist, receipt of confirmatory diagnosis, and first offer and initiation of corticosteroid treatment.[21] Another study found that Medicaid members and uninsured individuals tended to be diagnosed later than those with private insurance, with a mean age at diagnosis of 4.8 years compared with 4.3 years.[22]

DRUG INFORMATION

The FDA has approved 4 exon-skipping therapies: eteplirsen (Exondys 51), golodirsen (Vyondys 53), viltolarsen (Viltepso), and casimersen (Amondys 45).[1,2,3,4] These therapies are administered by a

EXHIBIT 2
How exon-skipping works



Abbreviation. DMD: Duchenne muscular dystrophy.

once-weekly intravenous infusion and do not cure DMD, but aim to stabilize the progressive symptoms of the disease by causing production of more functional dystrophin protein (Exhibit 2).[4] All therapies received fast-track priority review and orphan drug designation by the FDA, and were approved via the accelerated approval pathway, primarily based on small increases in dystrophin levels in biopsied muscle tissue, a surrogate end-point of DMD progression.[8-11]

FINDINGS

What Evidence Was Used by the FDA to Approve Exon-Skipping Treatments for DMD?

For eteplirsen (Exondys 51), the FDA considered 3 studies that included 25 boys with DMD amenable to exon 51 skipping; the studies provided information on dystrophin production in response to eteplirsen.[10] See Appendix A, Exhibit A1 for a summary of the studies used to approve eteplirsen through the accelerated pathway.

For golodirsen (Vyondys 53), the FDA considered a 2-part trial for boys with DMD amenable to exon 53 skipping.[9] Phase 1 included 12 boys and Phase 2 added an additional 13 boys for a total of 25 trial participants.[9] See Appendix A, Exhibit A2 for a summary of the study used to approve golodirsen through the accelerated pathway.

For viltolarsen (Viltepso), the FDA considered a dose-finding study that included 16 boys with DMD amenable to exon 53 skipping, aged 4 to 9 years.[11] See Appendix A, Exhibit A3 for a summary of the study used to approve viltolarsen through the accelerated pathway.

For casimersen (Amondys 45), the FDA considered data from a study that included 229 boys with DMD amenable to exon 45 or exon 53 skipping, aged 6 to 13 years; interim data from 43 participants who received casimersen was available at the time of accelerated approval.[8,23] See Appendix A, Exhibit A4 for a summary of the study used to approve casimersen through the accelerated pathway.

Why Did the FDA Grant Eteplirsen Accelerated Approval?

In *Study 1* (NCT01396239), 12 participants received eteplirsen (30 mg/kg or 50 mg/kg) or placebo.[10] However, the FDA stated that because of insufficient information on baseline dystrophin protein levels, it was not possible to estimate

ETEPLIRSEN Exondys 51®

BASIC INFORMATION

DRUG CLASS
Antisense oligonucleotide

MANUFACTURER
Sarepta Therapeutics

PRICE PER PATIENT
$748,800 per year

APPROVED INDICATION(S)

DMD patients with a confirmed mutation of the DMD gene amenable to exon-51 skipping

FDA APPROVAL

PATHWAY
Accelerated approval

DATE
September 2016

DOSING

ROUTE Intravenous (IV)

FORMULATIONS
100 mg/2 mL (50 mg/mL) in a single-dose vial
500 mg/10 mL (50 mg/mL) in a single-dose vial

RECOMMENDED DOSAGE
30 mg/kg of body weight, once weekly
Administered as IV infusion over 35 to 60 minutes via an inline 0.2-micron filter

SAFETY

BOXED WARNINGS None

PRECAUTIONS
If hypersensitivity reactions occur, give appropriate medical treatment and consider slowing the infusion or interrupting therapy.

ADVERSE REACTIONS
Balance disorder and vomiting

GOLODIRSEN Vyondys 53®

BASIC INFORMATION

DRUG CLASS
Antisense oligonucleotide

MANUFACTURER
Sarepta Therapeutics

PRICE PER PATIENT
$748,800 per year

APPROVED INDICATION(S)

DMD patients with a confirmed mutation of the DMD gene amenable to exon-53 skipping

FDA APPROVAL

PATHWAY
Accelerated approval

DATE
December 2019

DOSING

ROUTE Intravenous (IV)

FORMULATIONS
100 mg/2 mL (50 mg/mL) in a single-dose vial

RECOMMENDED DOSAGE
30 mg/kg of body weight, once weekly
Administered as IV infusion over 35 to 60 minutes via an inline 0.2-micron filter

SAFETY

BOXED WARNINGS None

PRECAUTIONS
If hypersensitivity reactions occur, give appropriate medical treatment and consider slowing the infusion or interrupting therapy.
Kidney function should be monitored; creatinine may not be a reliable measure of renal function in DMD patients.

ADVERSE REACTIONS
Headache, fever, fall, abdominal pain, head cold, cough, vomiting, and nausea

Abbreviations. DMD: Duchenne muscular dystrophy; FDA: US Food and Drug Administration.

VITOLARSEN Viltepso®

BASIC INFORMATION

DRUG CLASS
Antisense oligonucleotide

MANUFACTURER
NS Pharma

PRICE PER PATIENT
$703,872 per year

APPROVED INDICATION(S)

DMD patients with a confirmed mutation of the DMD gene amenable to exon-53 skipping

FDA APPROVAL

PATHWAY
Accelerated approval

DATE
August 2020

DOSING

ROUTE Intravenous (IV)

FORMULATIONS
250 mg/5 mL (50 mg/mL) in a single-dose vial

RECOMMENDED DOSAGE
80 mg/kg of body weight, once weekly
Administered as IV infusion over 60 minutes

SAFETY

BOXED WARNINGS None

PRECAUTIONS
Kidney function should be monitored; creatinine may not be a reliable measure of renal function in DMD patients.

ADVERSE REACTIONS
Upper respiratory tract infection, injection site reaction, cough, and fever

CASIMERSEN Amondys 45®

BASIC INFORMATION

DRUG CLASS
Antisense oligonucleotide

MANUFACTURER
Sarepta Therapeutics

PRICE PER PATIENT
$748,800 per year

APPROVED INDICATION(S)

DMD patients with a confirmed mutation of the DMD gene amenable to exon-45 skipping

FDA APPROVAL

PATHWAY
Accelerated approval

DATE
February 2021

DOSING

ROUTE Intravenous (IV)

FORMULATIONS
100 mg/2 mL (50 mg/mL) in a single-dose vial

RECOMMENDED DOSAGE
30 mg/kg of body weight, once weekly
Administered as IV infusion over 35 to 60 minutes via an inline 0.2-micron filter

SAFETY

BOXED WARNINGS None

PRECAUTIONS
If hypersensitivity reactions occur, give appropriate medical treatment and consider slowing the infusion or interrupting therapy.
Kidney function should be monitored; creatinine may not be a reliable measure of renal function in DMD patients.

ADVERSE REACTIONS
Upper respiratory tract infection, cough, fever, headache, joint pain, and oropharyngeal (throat or back of mouth) pain

Abbreviations. DMD: Duchenne muscular dystrophy; FDA: US Food and Drug Administration.

dystrophin production in response to eteplirsen in this study.[10]

Study 2 (also called Study 202; NCT01540409), continued to follow all the participants from *Study 1* for 4 years.[24] Participants who were in the placebo group in *Study 1* were rerandomized to 30 mg/kg or 50 mg/kg of eteplirsen per week, and investigational treatment continued on an open-label basis (i.e., the participants, clinicians, and outcome assessors were aware that all participants were now receiving eteplirsen).[24] Data considered by the FDA showed no evidence of a clinical benefit when comparing the participants in Study 2 to an external control group; the average dystrophin level after 180 weeks of treatment was less than 1% (0.93%) of dystrophin levels in people without DMD.[10]

Study 3 (also called Study 301) included 13 patients who received eteplirsen (30 mg/kg) weekly for 48 weeks; in the 12 participants with evaluable dystrophin levels increased significantly from a mean of 0.16% of dystrophin levels in people without DMD to 0.44% (a median increase of 0.1%).[10]

Eteplirsen does not have any boxed warning; however, the original prescribing label highlighted the most common adverse reactions (incidence of ≥ 35% compared with placebo) as balance disorder and vomiting.[10] The most recent label highlights the same warnings and precautions, with the addition of hypersensitivity reactions.[10]

Why Did the FDA Grant Golodirsen Accelerated Approval?

Study 1 Part 1 (NCT02310906) was a randomized dose titration trial conducted over 12 weeks (with an additional 8 weeks possible for evaluation of safety), which was extended to 168 weeks as *Study 1* Part 2 (NCT02310906).[25] *Study 1* Part 2 included 13 additional individuals who had never been administered golodirsen, along with the original 12 participants from *Study 1* Part 1.[9,26] At week 48, participants in the golodirsen group had significantly higher levels of dystrophin, with a mean percent normal dystrophin level of 1.02% compared with 0.1% at baseline (a mean increase of 0.92%).[10,26]

Golodirsen does not have any boxed warning; however, the original prescribing label highlighted hypersensitivity reactions and kidney toxicity.[9] The most common adverse reactions (incidence of ≥ 20% compared with placebo) were headache, fever, falls, abdominal pain, nasopharyngitis, cough, vomiting, and nausea. The most recent label highlights the same warnings and precautions.[9]

Why Did the FDA Grant Viltolarsen Accelerated Approval?

For NCT02740972, in participants who received viltolarsen 80 mg/kg once a week, mean dystrophin levels increased from 0.6% of normal at baseline to 5.9% of normal by week 25.[11] In participants who received viltolarsen 40 mg/kg once a week, mean dystrophin levels increased from 0.3% of normal at baseline to 5.7% of normal by week 25.[27] The prescribing label only cited the findings from the high-dose group.[11]

Viltolarsen does not have any boxed warning; however, the original prescribing label highlighted kidney toxicity.[11] The most common adverse reactions (incidence of ≥ 15%) were upper respiratory tract infection, injection site reaction, cough, and fever.[11] The most recent label highlights the same warnings and precuations.[11]

Why Did the FDA Grant Casimersen Accelerated Approval?

Study 1 (NCT02500381; ESSENCE) is ongoing; hence, the FDA used the interim outcome of dystrophin level at 48 weeks in a subset of 43 participants.[8] No results are yet posted on ClinicalTrials.gov nor were we able to identify any publications reporting the results of this study.[23] Data considered by the FDA showed participants in the casimersen group had a significantly greater increase in dystrophin of 0.81% (from a baseline of 0.93% to 1.74% of normal) compared with

participants in the placebo group (a mean increase of 0.22%, from 0.54% to 0.76% of normal).[8]

Casimersen does not have any boxed warning; however, the original prescribing label highlighted kidney toxicity.[8] The most common adverse reactions (incidence of ≥ 20% and at least 5% higher than placebo) were upper respiratory tract infection, cough, fever, headache, arthralgia, and oropharyngeal pain. The most recent label highlights the same warnings and precautions, with the addition of hypersensitivity reactions.[8]

What Studies Were Requested to Convert Exon-Skipping Treatments to Full Approval?

At the time of writing this brief, none of the exon skipping therapies for DMD have been converted to full FDA approval. Also, on ClinicalTrials.gov the trials for eteplirsen, golodirsen and casimersen are listed as active, not recruiting, and the viltolarsen trial is reported as completed but we did not identify any publications at the time of writing this brief.[23,28,29] .

Eteplirsen (Exondys 51)

As part of the accelerated approval in 2016, the FDA requested the completion of a 2-year randomized, double-blind, controlled trial of eteplirsen in participants with a confirmed mutation of the DMD gene amenable to exon-51 skipping (Exhibit XX).[30] The FDA recommended that participants be randomized to the approved dosage of eteplirsen (30 mg/kg/week) or to a dosage that provides significantly higher exposure (e.g.,30 mg/kg/day).[30] The FDA also requested the primary endpoint be the North Star Ambulatory Assessment, a validated and widely used clinical assessment scale to measure functional ability in ambulant individuals with DMD.[30,31] See Appendix B, Exhibit B1 for a summary of the study requested to support full approval of eteplirsen.

Golodirsen (Vyondys 53) and Casimersen (Amondys 45)

As part of the accelerated approvals in 2019 and 2021, the FDA requested the completion of Study 4045-301, also known as ESSENCE (NCT02500381); interim findings in a subset of 43 participants were used to support the approval regarding effectiveness for casimersen.[8,30] See Appendix B, Exhibit B2 for a summary of the study requested to support full approval of golodirsen and casimersen.

Vitolarsen (Viltepso)

As part of the accelerated approval in 2020, the FDA requested the completion of Study NS065/NCNP-01-301, also known as RACER53 (NCT04060199).[30] See Appendix B, Exhibit B3 for a summary of the study requested to support full approval of viltolarsen.

DATA METHODS SUMMARY

Researchers at the Center for Evidence-based Policy (Center) used Centers for Medicare & Medicaid Services (CMS) Transformed Medicaid Statistical Information System (T-MSIS) and associated T-MSIS analytic files to identify Medicaid members with DMD whose specific diagnosis likely matched drugs' indicated subtype. Specifically, we were interested in males under 30 years of age with at least 1 inpatient or at least 2 outpatient claims at least 30 days apart, with a DMD diagnosis [ICD-10 G71.01] or at least 1 claim for a DMD disease-modifying treatment. We recorded patient demographic characteristics, comorbidity status, and health care service use using applicable ICD-10 (International Classification of Diseases, 10th revision) diagnosis codes, procedure codes, and NDCs (National Drug Codes).

The initial population for analysis included all Medicaid members aged 64 years or younger. As our focus was on Medicaid expenditures, and members with both Medicaid and Medicare (i.e., dual eligible) have pharmacy benefits under Medicare Part D, we excluded members with evidence of dual enrollment in the years 2019, 2020, and 2021. Using these criteria, Utah and Alabama were excluded as they do not report dual-enrollment status using this method. Other state-based analysis exclusions were determined based on recommendations from the CMS Data Quality Atlas, which reports state-specific data availability and completeness; we generally excluded data elements identified as `unusable' or 'high concern' data quality according to Data Quality Atlas.

DMD analysis cohorts were anchored in 2021, with a 3 year lookback period to ensure comprehensive Medicaid member identification. To make comparisons with the Medicaid-insured population at-large, a 3-to-1 exact matching method based on state, age (in years), sex, and race/ethnicity was used. Our cost model estimated annual costs for each of the 4 drugs, using inputs of drug indication prevalence, drug uptake, and adherence observed in the analytic dataset, as well as reported drug acquisition costs and statutorily required rebates. Our utilization metrics do not have a continuous enrollment requirement. Refer to Appendix C for additional detail on how we conducted this study.

How Common Is Duchenne Muscular Dystrophy Among Medicaid Members?

Our analytic cohort included 81,439,193 non-dually eligible Medicaid members aged 0 to 64 in 2021 (members in Alabama, Mississippi, and Utah were excluded because of data availability). Of these, 6,041 (or 7.4 per 100,000 members) were identified as having DMD.

DMD was most common among non-Hispanic White and Hispanic Medicaid members (54.5% and 30.3%, respectively; Exhibit 3). All DMD patients are under 30 years of age. Nearly two-thirds (60.9%) of DMD patients were under the age of 18 and 20% were under the age of 10.

The prevalence of DMD among state Medicaid populations varied somewhat among the states with available data, from around 4 cases per 100,000 members in Hawaii, to around 19 cases per 100,000 members in Nebraska. DMD was most common in Midwestern states, with Nebraska, New Hampshire, Delaware, Kansas, Iowa, Missouri, Texas, South Dakota, Pennsylvania, Minnesota experiencing DMD rates above 10 cases per 100,000 members (Exhibit 4). Still, even in states with higher DMD rates, those with the disease accounted for less than 0.1% of states' total Medicaid members (see Appendix D for state prevalence).

EXHIBIT 3

Characteristics of Medicaid members with and without DMD, 2021

	with DMD[a]	%[b]	without DMD[a]	%[b]
Total members	*6,041*	-	*81,433,152*	-
Age, in years				
0 to 9	1,208	20.0	20,424,512	25.1
10 to 17	2,470	40.9	16,145,286	19.8
18 to 29	2,363	39.1	16,559,233	20.3
30 to 64[c]	0	0	28,304,116	34.8
Race and ethnicity				
American Indian or Alaska Native, non-Hispanic	43	1.0	865,977	1.5
Asian, non-Hispanic	138	3.3	2,342,557	4.2
Black, non-Hispanic	363	8.8	11,769,790	20.9
Hispanic	1,248	30.3	17,000,236	30.1
Native Hawaiian or Pacific Islander, non-Hispanic	19	< 1	272,954	< 1
White, non-Hispanic	2,247	54.5	23,449,744	41.5
Multiracial, non-Hispanic	33	< 1	550,964	1.0
Other race or ethnicity	30	< 1	191,967	< 1

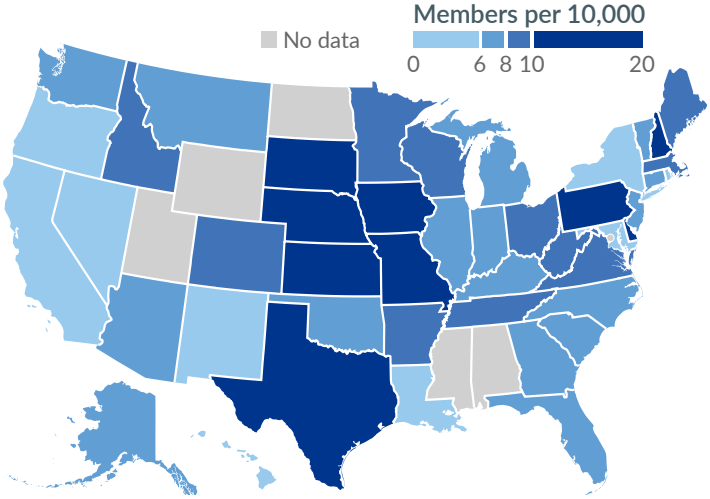
Notes. [a] Excluding dually eligible members and members in Mississippi, Utah, and Alabama. [b] Percentage of members with nonmissing data on demographic characteristics. 4,121 (68.2%) of the members DMD and 56,444,189 (69.3%) of the members without DMD had nonmissing race/ethnicity data. For more detail see Appendix E. [c] The DMD case-finding algorithm used limited the search for DMD patients to those who are male and aged < 30 years. See Appendix C for detail.

How Do Medicaid Members With DMD Compare With Exon-Skipping Trial Populations?

The demographic characteristics of patients included in the drug trials were somewhat different than those observed in our analysis of Medicaid data. For example, our Medicaid-insured cohort was 54.5% non-Hispanic White, 30.3% Hispanic, 8.8% Black, and 3.3% Asian, with other race and ethnicity categories represented at less than 1%; the participants in eteplirsen *Study 1* and *2* trials were 92% White and 8% Asian, and the viltolarsen study cohort was 94% White and 6% Hispanic.[10,24,27,32] The average age of drug trial participants varied, with 7.4 years in the viltolarsen trial and 9.3 years in eteplirsen *Study 1* and *2*

EXHIBIT 4

DMD prevalence among Medicaid members, per 100,000 members, by state, 2021



Note. Data not available for Alabama, Utah, and Mississippi; data suppressed for the District of Columbia, North Dakota, and Wyoming (N < 11).

trials; the average age of Medicaid members with DMD was 15.4 years.[10,24,27,32]

Exhibit 5 describes the prevalence of select comorbidities and body system–level impairments for Medicaid members aged 0-29 with DMD compared to members without DMD (matched 1:3 on state, age, sex, and race and ethnicity). Individuals with DMD were significantly more likely to have comorbid diagnoses across all major body systems, particularly the central nervous, musculoskeletal, and pulmonary systems, compared to members without DMD.

Members with DMD had substantially higher hospital and emergency use than their matched comparisons (Exhibit 6). Specifically, about 10% of Medicaid members with DMD experienced at least 1 hospitalization in 2021, compared to 2.3% of members without DMD. Members with DMD also experienced more total inpatient days (1,271 vs. 268 per 1,000 members), were more likely to experience hospital stays lasting at least 5 days (5.4% vs. 1%), and had higher emergency department use across multiple measures. Members with DMD were also more likely to have claims for outpatient physical therapy, occupational therapy, and durable medical equipment.

How Common Is Use of Exon-Skipping Drugs Among Medicaid Members With DMD?

To calculate uptake of each treatment in Medicaid, we first estimated the total number of Medicaid members eligible for exon-skipping treatment based on the prevalence of DMD we observed in the T-MSIS data and the percentage of DMD patients with the corresponding gene mutation reported by the FDA.[1,2,3,4] We then identified members with at least 1 claim for these treatments. In 2021, eteplirsen uptake among Medicaid members was 27.5%. In comparison, uptake for casimersen, golodirsen, and viltolarsen were lower at 15.1%, 12.2%, and 5.8%, respectively (Exhibit 7).

EXHIBIT 5

Prevalence of affected body systems and specific conditions in matched Medicaid members with and without DMD, 2021

System or condition	Medicaid members with DMD	%	Matched Medicaid members without DMD[c]	%
Total members[a]	*6,019*	-	*18,057*	-
Cardiomyopathy	735	12.2	13	< 1
Cardiovascular	2,484	41.3	519	2.9
Central nervous system[b]	5,799	96.3	523	2.9
Musculoskeletal	2,275	37.8	923	5.1
Osteoporosis/osteopenia	266	4.4	- [d]	- [d]
Pneumonia	236	3.9	74	< 1
Pressure wounds	124	2.1	15	< 1
Psychological	1,505	25.0	3,103	17.1
Pulmonary	2,310	38.4	1,018	5.6

Notes.[a] No 2021 health care information available for 22 members identified as having DMD. For the purposes of this calculation, they were eliminated from analysis along with their matched counterparts. [b] DMD is categorized as a central nervous system disorder, not a musculoskeletal disorder, by the CDPS. [c] Medicaid members without DMD matched to members with DMD at 3:1 on state, age, sex, race, and ethnicity. [d] Suppressed (N < 11).
Abbreviations. CDPS: Chronic Illness and Disability Payment System; DMD: Duchenne muscular dystrophy.

EXHIBIT 6

Health service use by matched Medicaid members with and without DMD, 2021

	Members with DMD	Matched members without DMD[a]
Total members	*6,041*	*18,123*
Hospitalizations		
% with ≥ 1 hospitalization	9.9	2.3
% with ≥ 2 hospitalizations	3.0	< 1
Total hospitalizations, per 1,000 members	154	35
Total inpatient days, per 1,000 members	1,271	268
Average length of stay per hospitalization, days	8.2	7.6
% with ≥ 1 hospitalization lasting ≥ 5 days	5.4	1.0
Emergency visits		
% with ≥ 1 ED visit	26.4	20.1
% with ≥ 5 ED visits	1.1	< 1
Total ED visits, per 1,000 members	464	327
Rehabilitation		
% with ≥1 outpatient PT or OT claim	48.4	3.78
% with ≥ 1 ventilator/respiratory assist claim	18.9	<1
% with ≥ 1 wheelchair[b] claim	23.3	<1
% with ≥ 1 home health claim		

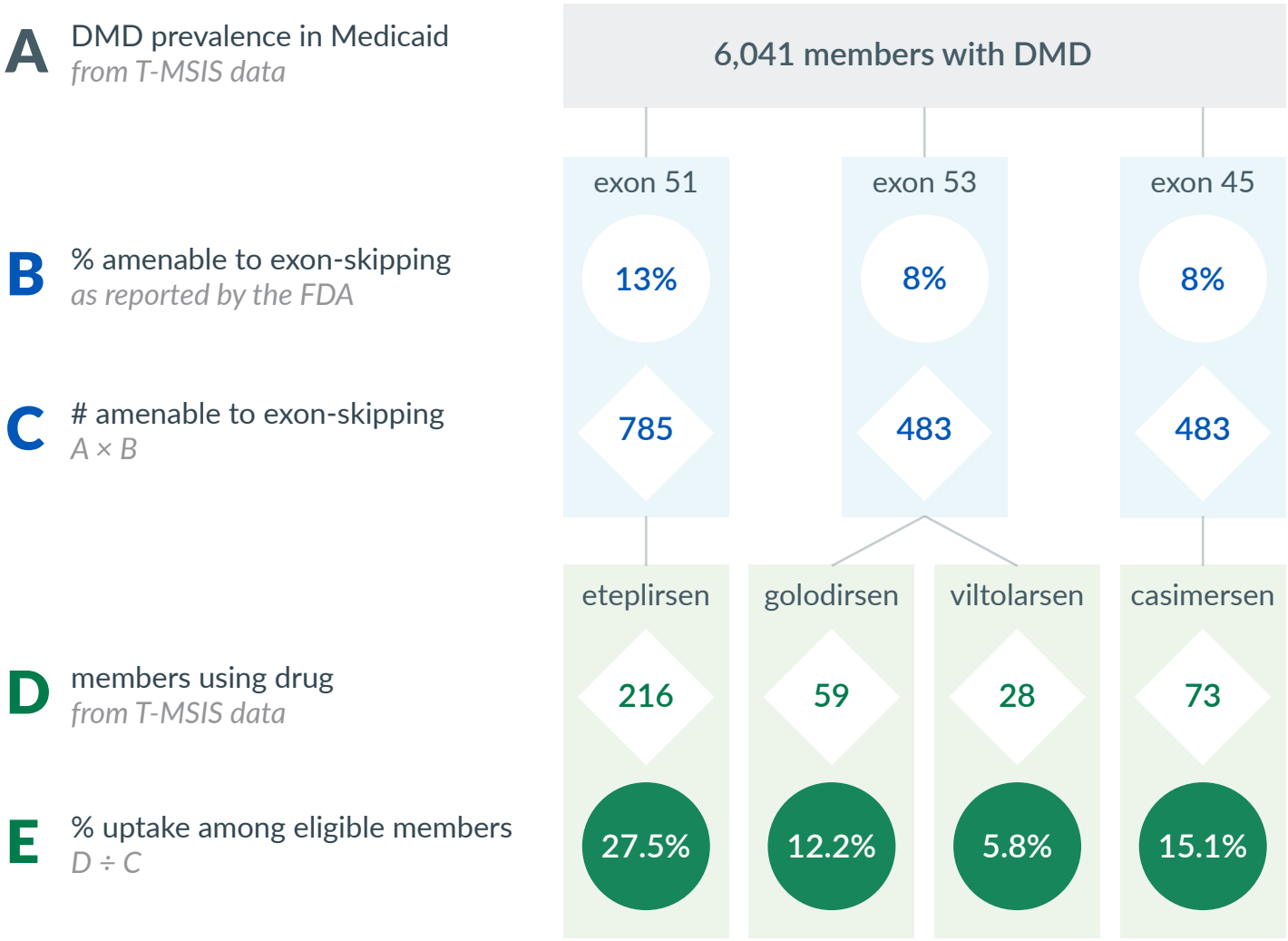
Note. [a] Medicaid members without DMD matched to members with DMD at 3:1 on state, age, sex, race, and ethnicity. [b] Includes claims for both wheelchairs and required equipment.
Abbreviations. ED: emergency department; DMD: Duchenne muscular dystrophy; DME: durable medical equipment; OT: occupational therapy; PT: physical therapy.

The average age of Medicaid members using these treatments (13.9 years) was slightly lower compared to the average age of all Medicaid members with DMD (15.6 years) but significantly higher than the average age of clinical trial participants (8.5 years). Another study comparing participants in the approval trials with the real-world DMD patients receiving these treatments indicated the same age difference and also noted that nearly all patients in the trials were in earlier stages (stage 1 or stage 2), while 47% of Medicaid insured patients were more advanced (stage 3 or 4) when initiating these DMD treatments.[33]

We identified 370 members with claims for any of the 4 exon-skipping medications. We considered medication adherence among eligible members in 2021 using the Medication Possession Ratio (MPR), a member-level metric describing the ratio of total days supplied to days in the measurement year. Each member's measurement year began at the date of their first medication fill and ended on the last day of 2021. A member was considered "adherent" if their individual MPR was at least 0.8, meaning they possessed their medication on at least 80% of the days in their measurement year. In 2021, 74% of eligible members prescribed any of the 4 medications demonstrated adherence at this level; the mean MPR for the cohort was 0.89 (89% of the days in the measurement year). When only considering members fully enrolled for the

EXHIBIT 7

Uptake of Exon-skipping drugs among Medicaid members with DMD, 2021



Abbreviations. DMD: Duchenne muscular dystrophy; FDA: US Food and Drug Administration; T-MSIS: Transformed Medicaid Statistical Information System.

measurement year, adherence (76%) and mean MPR (0.90) were similar. The adherence rates and mean MPRs were similar across treatments. The lowest adherence rate was 68% for viltolarsen and the highest rate was 76% for eteplirsen. See Appendix F for detail.

It is unknown from the MPR why a patient might stop taking the drug; for example, they may have experienced adverse effects, experienced a change in medical status, or moved to a different medication regimen for their condition. An MPR is also known to be affected by enrollment status, although there were not significant differences in adherence or mean MPR by enrollment status identified in this cohort.

What Was the Impact of Exon-Skipping Drugs on Medicaid Spending in 2021?

We estimated that total 2021 Medicaid spending among non–dually eligible beneficiaries on the exon-skipping treatments for DMD was $252.1 million nationally (excluding any spending in Alabama, Mississippi, and Utah, for which we do not have this data); $171.5 million for eteplirsen,

EXHIBIT 8

Total estimated Medicaid spending on exon-skipping drugs by drug, 2021

	Estimated 2021 Medicaid spending, in $millions
Total spending[a]	*252.1*
Casimersen	27.3
Eteplirsen	171.4
Golodirsen	43.3
Viltolarsen	10

Note. [a] Excludes any spending in Alabama, Mississippi, and Utah.

$43.3 million for golodirsen, $10.0 million for viltolarsen, and $27.3 million for casimersen (Exhibit 8). We estimated $175.5 million of the total cost was paid by federal funds and the remaining $76.5 million by state funds. These total cost estimates are based on converting the "total days supply" in 2021 pharmacy and outpatient services claims for each of these drugs into a cost, using an estimated dosage based on the patient age, the median body weight for their age, and the corresponding cost of the drug. Refer to the Methods Appendix (Appendix C) for additional detail on how the costs were calculated.

What Is the Potential Impact of Exon-Skipping Drugs on State Medicaid Spending?

We estimated that the total annual cost of exon-skipping treatments for DMD in Medicaid would be $315.2 million nationally (95% confidence bounds, $234.1 million and $430.1 million). This corresponds to a per-member per-month (PMPM) cost of $0.34 (95% confidence bounds, $0.25 and $0.47) for all Medicaid members. Based on the enrollment composition of the DMD patients in CHIP (Children's Health Insurance Program) and Medicaid expansion, and the weighted national average of corresponding federal match rates, we estimated that $200.1 million of the total costs would come from federal funds and the remaining $115.5 million would be paid for by the states.

Overall, $175.2 million of the total costs would be for eteplirsen, $46.7 million for golodirsen, $21.1 million for viltolarsen, and $72.1 million for casimersen (Exhibit 9). These estimates are calculated based on the number of estimated patients eligible for each treatment by age, the drug price (from the treatment dose needed based on median weight for patient age), and the uptake and use patterns observed in 2021 data (with some modifications to these inputs for newer drugs). Refer to the Methods Appendix (Appendix C) for additional detail on model inputs and assumptions, and Appendix G for cost estimates under different uptake and adherence scenarios.

Comparing these estimates against Medicaid total 2022 spending estimates reported elsewhere, the total spending on exon-skipping treatments would potentially account for about 0.7% of total Medicaid prescription drug spending.

EXHIBIT 9

Total estimated potential Medicaid spending on exon-skipping drugs, by drug

	Estimated potential Medicaid spending, in $millions	95% lower bound, in $millions	95% upper bound, in $millions
Total spending[a]	*315.2*	*234.1*	*430.1*
Eteplirsen	175.2	135.4	219.7
Golodirsen	46.7	32.5	68.4
Viltolarsen	21.1	16.3	40.9
Casimersen	72.1	49.9	101.0

Note. [a] Excludes any spending in Alabama, Mississippi, and Utah.

CONSIDERATIONS

DMD is a rare genetic condition characterized by progressive loss of muscle function and early death.[5] The median life expectancy for an individual with DMD is 22 years, with symptoms beginning to appear in childhood, mostly between 2 to 11 years of age.[6] Out of 81 million members enrolled in Medicaid in 2021, excluding those dually eligible for Medicare, we identified 6,041 as having DMD (7.4 in 100,000).

Our analysis shows that exon-skipping therapies are being administered to a Medicaid population that is, on average, 7 years older than clinical trial participants (15.4 vs. 8.5 years). This age gaps signals more than just years, it signals a more advanced disease stage at time of treatment. It appears that many Medicaid enrollees may be in disease progression stage 3 or 4 when starting treatment, as opposed to the clinical trial participants who were generally in earlier stage 1 or stage 2.[33]

Uptake of these exon-skipping drugs is high, illustrating the extraordinary need for treatment options in this population. But gaps in evidence resulting from outstanding confirmatory trials have implications for patients, clinicians, and Medicaid program officials. In 2021, nearly 400 children and young adults enrolled in Medicaid were taking exon-skipping drugs without clear understanding of these therapies' effectiveness. While waiting for FDA-requested confirmatory trial results, state officials should plan for annual multi-million-dollar expenditures for exon-skipping drugs, and federal officials should plan for annual expenditures of $200 million to $300 million per year.

Individuals with DMD and their caregivers may wonder whether once-weekly infusions, administered for years, are slowing the progression of their disease or not really making a difference. Clinicians may wonder whether the therapies they prescribe are truly effective. Medicaid officials may wonder how best to support individuals with DMD, and the many care needs they have as their disease progresses. Medicaid officials may also be concerned about the financial burden of these drugs if millions of taxpayer dollars are being spent on ineffective care. Results of confirmatory trials and full FDA drug approval, as appropriate, are needed to allow DMD patients, their families, clinicians, and Medicaid program administrators to make important decisions about care and policy. Moreover, federal policymakers should revisit their options to require timely and complete sharing of confirmatory trial results for accelerated-approval drugs.

PROJECT TEAM

RHONDA ANDERSON, RPH
Director of Pharmacy

MARCUS BACHHUBER, MD, MSHP
Clinical Epidemiologist

CAITLIN BURBANK, PT, PHD
Research Associate III

GÜLCAN ÇIL, PHD
Senior Statistician

GALEN GAMBLE, BA
Project Coordinator

DAVID RADLEY, PHD, MPH
Research Director, Data and Analytics

MOIRA RAY, MD, MPH
Clinical Epidemiologist

JENNIFER RYAN, ND, MS, CCRP
Research Associate II

BETH SHAW, MSC
Research Director, Evidence Synthesis

SUSAN STUARD, MBA
Director, State Technical Assistance

SNEHA YEDDALA, PHARM D, MS
Research Associate I

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APPENDIX A

SUMMARY OF STUDIES USED TO SUPPORT EFFICACY OF EXON-SKIPPING DRUGS

See attachment for tables summarizing the studies for each exon-skipping drug.

EXHIBIT A1
Summary characteristics of studies used to support efficacy of eteplirsen

EXHIBIT A2
Summary characteristics of studies used to support efficacy of golodirsen

EXHIBIT A3
Summary characteristics of studies used to support efficacy of viltolarsen

EXHIBIT A4
Summary characteristics of studies used to support efficacy of casimersen

APPENDIX B

STUDIES REQUESTED TO SUPPORT FULL APPROVAL OF EXON-SKIPPING DRUGS

See attachment for tables summarizing the studies for each exon-skipping drug.

EXHIBIT B1
Summary characteristics of study requested to support full approval of eteplirsen

EXHIBIT B2
Summary characteristics of study requested to support full approval of golodirsen

EXHIBIT B3
Summary characteristics of study requested to support full approval of viltolarsen

EXHIBIT B4
Summary characteristics of study requested to support full approval of casimersen

APPENDIX C

METHODS

See attachment for a full description of the methods used to prepare this brief.

EXHIBIT C1
Exon-skipping treatments and prices

EXHIBIT C2
Cost-modeling inputs: prevalence and uptake

EXHIBIT C3
Cost-modeling inputs: drug costs

EXHIBIT C4
Cost-modeling inputs: adherence

APPENDIX D

MEMBERS WITH AND WITHOUT DMD, 2021

See attachment for this table.

APPENDIX E

DEMOGRAPHIC INFORMATION

See attachment for a table describing the availability of demographic information, and demographic characteristics of Medicaid members included in our study.

APPENDIX F

TREATMENT UPTAKE, 2021

See attachment for tables describing voxelotor uptake and use patterns by Medicaid members.

TABLE F1
Uptake of exon-skipping drugs among Medicaid members with DMD, 2021

TABLE F2
Medication adherence, means, and proportions, stratified by treatment, 2021

APPENDIX G

COST SCENARIO ANALYSES

See attachment for tables describing cost scenarios for each exon-skipping drug.

TABLE G1

Estimated annual cost of eteplirsen (in $M) under different uptake and adherence scenarios

TABLE G2

Estimated annual cost of golodirsen (in $M) under different uptake and adherence scenarios

TABLE G3

Estimated annual cost of vitolarsen (in $M) under different uptake and adherence scenarios

TABLE G4

Estimated annual cost of casimersen (in $M) under different uptake and adherence scenarios

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centerforevidencebasedpolicy.org

Center for Evidence-based Policy

3030 South Moody Avenue, Suite 250
Portland, OR 97201

Phone: (503) 494-2182
Fax: (503) 494-3807

centerforevidencebasedpolicy.org