Pharmacy Management Drug Policy

SUBJECT: Duchenne Muscular Dystrophy (DMD): Amondys 45 (casimersen), Emflaza (deflazacort), Elevidys (delandistrogene moxeparvovec-rokl), Exondys 51 (eteplirsen), Viltepso (vilotarsen) and Vyondys 53 (golodirsen)
POLICY NUMBER: PHARMACY-85
EFFECTIVE DATE: 12/30/2019
LAST REVIEW DATE: 01/02/2024

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☒ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☒ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☒ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

Duchenne muscular dystrophy (DMD) is a rare genetic disease that affects approximately 20,000 boys and young men in the United States. A mutation in the gene for dystrophin causes progressive muscle wasting, leading to the need for wheelchairs and ventilators.[4] There are numerous mutations in the DMD gene that have been identified and the type of mutation and its effect on the production of dystrophin accounts for variable symptoms/progression rates in each affected patient.[3] Approximately 13% of patients with DMD contain genetic mutations that are amenable to exon 51 skipping, approximately 8% of patients with DMD contain genetic mutations that are amenable to exon 45 and 53 skipping, each. In general, death occurs around 19 years of age if no interventions are made.[5] Respiratory, cardiac, orthopedic, and rehabilitative interventions and the use of corticosteroids can lead to an extended life expectancy of up to 40 years.

Amondys 45 (casimersen) is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 skipping. It is designed to bind to exon 45 of dystrophin pre-mRNA, allowing for production of an internally truncated dystrophin protein in these patients. The drug is approved under accelerated approval which allows a surrogate endpoint (dystrophin level) to be used for serious disease in which there is an unmet need for therapy.

The accelerated approval of Amondys 45 was based upon interim results form an ongoing global, randomized, double-blind, placebo-controlled Phase 3 trial (ESSENCE). 43 patients were randomized in a 2:1 manner to receive either Amondys 45 or placebo for 96 weeks (interim results were assessed at 48 weeks). All patients were ambulatory and were required to have been on stable doses of oral corticosteroids for at least 24 weeks prior to the start of treatment. The efficacy endpoint was based on change from baseline in the dystrophin protein level at week 48. Patients who received Amondys 45 showed a significantly greater increase in dystrophin protein levels (mean 0.81% of normal) cmpared to those who received placebo (0.22% of normal). It is not yet known whether this increase in dystrophin contributes to clinical benefit in patients. No confirmatory trials have yet been conducted to demonstrate that Amondys 45 provides clinical benefit in DMD patients amenable to exon 45 skipping.

Elevidys (delandistrogene moxeparvovec-rokl) is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years of age. It is designed to deliver the gene encoding the micro-dystrophin protein. The micro-dystrophin expressed by Elevidys is a shortened version that contains selected domains of dystrophin expressed in normal muscle cells. The drug is approved under accelerated approval which allows a surrogate endpoint (micro-dystrophin level) to be used for serious disease in which there is an unmet need for therapy.

The accelerated approval of Elevidys was based upon data from two ongoing clinical studies (Study 102 and Study 103) and safety data from three ongoing trials (Study 101, Study 102, and Study 103). Study 102 is a multicenter three-part Phase 2 study and Study 3 is a two-part open-label phase 1 study in five cohorts of boys with DMD defined by age and ambulatory status. For the subset of patients 4-5 years of age who received the FDA approved dosage of Elevidys, the mean change from baseline in Elevidys micro-dystrophin expression levels at Week 12 following Elevidys infusion was 95.7% (n=3; standard deviation [SD]: 17.9%) in Study 102 Parts 1 and 2, and 51.7% (n=11; SD: 41.0%) in Study 103 Cohort 1. Elevidys did not demonstrate a statistically significant treatment effect on functional outcomes; however, an exploratory subgroup analysis of the 16 participants (Elevidys: n=8; placebo: n=8) 4 through 5 years of age showed a numerical advantage for Elevidys compared to placebo in the change in North Star Ambulatory Assessment (NSAA) total score.

The Phase 3 EMBARK study is being conducted as the confirmatory trial for Elevidys to assess clinical benefit. On October 30, 2023, Sarepta announced topline results from EMBARK, which enrolled 125 patients with DMD between the ages of 4-7 years of age. The primary endpoint was not met as the change in NSAA total score from baseline at Week 52 (2.6 points in Elevidys-treated patients patients vs 1.9 points in placebo-treated) did not reach statistical significance (n=125, p=0.24). Key secondary endpoints, including Time to rise (TTR) and 10-meter walk test, showed statistically significant improvement (Change vs placebo LSM difference in seconds was -0.64 for TTR and -0.42 for 10-meter walk test). Full results from EMBARK will be shared at future medical meetings and publications will be pursued in a medical journal.

Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the DMD gene due to risk for immune-mediated myositis. Warnings/precautions involve acute serious liver injury, immune-mediated myositis, myocarditis, and pre-existing immunity against AAVrh74.

Emflaza (deflazacort) is indicated for patients 2 years of age and older with DMD. It is a corticosteroid for symptomatic therapy that works by decreasing inflammation and reducing the activity of the immune system. It is an oxazoline derivative of prednisone and has an estimated dosage equivalency of 1:1.3 compared to prednisone. It was approved by the FDA in 2017 but has been available outside the US for decades.

Exondys 51 (eteplirsen) is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. It is designed to skip exon 51, allowing for the synthesis of a truncated partial functioning form of dystrophin protein. The drug is approved under accelerated approval which allows a surrogate endpoint (dystrophin level) to be used for serious disease in which there is an unmet need for therapy.

The efficacy of Exondys 51 was evaluated in three open-label pivotal studies (designated as studies 1,2, and 3 in the package insert) in patients with DMD that is amenable to exon 51 skipping. Study 1 assessed the effect of Exondys on dystrophin levels and on improved distance walked in 12 patients, Study 2 was an extension study evaluating the same patients from study 1 as compared to a matched

historical control population, and study 3 is an ongoing study that included 12 different patients with DMD and studied the effect of Exondys 51 on dystrophin levels. In all 3 clinical trials, patients were required to be on stable doses of corticosteroids for at least 6 months prior to trial enrollment. Additionally, patients in all 3 clinical trials were ambulatory with a baseline minimum 6-minute walk test of at least 200 meters in study 1/2. It has been proposed that the 6MWT is a reliable tool used to measure progression in DMD.

Study 1 and 2 reported that the use of Exondys contributed to an increase in dystrophin levels and 6MWT results, however the FDA has recommended retraction of this study due to concerns related to methodology and interpretation of its findings. The FDA approval of Exondys 51 was based on results from Study 3, which reported a statistically significant increase in dystrophin levels. The median increase after 48 weeks was 0.1%. It is not yet known whether this increase in dystrophin contributes to clinical benefit in patients. The FDA labeling for Exondys 51 specifically states that a clinical benefit has not yet been established and continued approval is contingent upon verification of a clinical benefit in ongoing confirmatory clinical trials.

Following FDA approval of Exondys 51, Kinane, et al published a 2018 analysis that evaluated the effect of eteplirsen on lung function in DMD patients that participated in the aforementioned Study 1 and 2. This analysis compared Forced Vital Capacity (FVC) to historical controls from the United Dystrophinopathy Project (UDP, N=34) and Maximum Expiratory Pressure (MEP)/ Maximum Inspiratory Pressure (MIP) were compared to published natural history. The data showed a decline in FVC of 2.3% predicted per year in eteplirsen-treated patients compared with a decline of 4.1% predicted per year in the natural history cohort. There was an annual decline in MEP% predicted of 2.6% compared to a decline of 2.7%-3.6% in historical published reports of DMD and an increase in MIP% predicted of 0.6% vs a decline of 3.8-3.8% in eteplirsen-treated patients vs historic published reports. However, this analysis only reported pulmonary function endpoints that were collected as exploratory assessments from previously reported trials (Study 1 and 2) and there are no other published prospective trials that have compared these pulmonary endpoints to an active placebo control. Due to potential heterogeneity in the historical population that was used for comparison in this analysis, the benefit of Eteplirsen on pulmonary function cannot be fully assessed and the FDA labeling for Exondys 51 continues to state that a clinical benefit has not yet been established for Exondys 51.

Khan, et al published a 2019 analysis evaluating the effect of eteplirsen on lung function in DMD patients in studies 1/ 2 as well as two additional open-label, multicenter studies (204 and 301). Study 204 is an unpublished study that enrolled boys aged 7 to 21 who were primarily non-ambulatory or minimally ambulatory (6MWT < 300 meters). Study 301 is an ongoing phase 3 trial in boys aged 7 to 16 years old who are ambulatory (6MWT distance of at least 300 meters). The analysis of these 3 studies compared Percent Predicted Forced Vital Capacity (FVC% p) annual change to matched Cooperative International Neuromuscular Research Group Duchenne Natural History Study (CINRG DNHS) controls. Statistical analysis was presented for patients aged 10 to<18 years who were expected to be in a linear phase of FEV%p decline based on a 2015 study by Mayer, et al which demonstrated that DMD patients between the ages of 10 to 18 years will have a decline in FEV%p of 5% annually. The results of studies 204 and 301 demonstrated a FEV%p annual change of -3.66% in Study 204 participants and -3.79% in Study 301 participants compared to a range of 5.5-6% in the historical control group. Like the Kinane article, this analysis only reported FVC values collected as exploratory assessments from previously reported trials with no active placebo control. The FDA labeling for Exondys 51 continues to state that a clinical benefit has not yet been established for Exondys 51.

Viltepso (vilotarsen) is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. It is designed to bind to exon 53, resulting in production of an internally truncated dystrophin protein in patients with genetic mutations that are amenable to exon 53 skipping. The drug is approved under accelerated approval which allows a surrogate endpoint (dystrophin level) to be used for serious disease in which there is an unmet need for therapy.

The accelerated approval of Viltepso was based on results from a multicenter, 2-period, dose-fining study in patients with a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. During the initial period, patients were randomized to Viltepso or placebo and then all patients received 20 weeks of open-label Viltepso (40mg/kg once weekly or 80mg/kg once weekly) in the second period. Efficacy was assessed based on change from baseline in dystrophin protein level at Week 25. In patients who received Viltepso 80mg/kg once weekly, mean dystrophin levels increased from 0.6% of normal at baseline to 5.9% of normal by week 25, with a mean change of 5.3% of normal. All patient demonstrated an increase in dystrophin levels over their baseline values. It is not yet known whether this increase in dystrophin contributes to clinical benefit in patients. No confirmatory trials have yet been conducted to demonstrate that Viltepso provides clinical benefit in DMD patients amenable to exon 53 skipping.

Vyondys 53 (golodirsen) is indicated for the treatment of DMD in patients with a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. It is designed to skip exon 53, allowing for the synthesis of a truncated partial functioning form of dystrophin protein. The drug is approved under accelerated approval which allows a surrogate endpoint (dystrophin level) to be used for serious disease in which there is an unmet need for therapy.

The accelerated approval of Vyondys 53 was based on results from a two-part Phase I/II clinical study in patients with DMD amenable to exon 53 skipping. Part I was a randomized, placebo-controlled study to assess the safety and pharmacokinetics of Vyondys 53 (SRP-4053) in 12 patients. Part II of this study was a 168-week, open-label evaluation of 25 patients including eligible patients from part 1 of the study as well as 13 additional patients with DMD amenable to exon 53 skipping. Primary endpoints for part 2 of this study were change from baseline in dystrophin protein levels at week 48 and change from baseline on a 6-minute walk test (6MWT) at week 144. All patients were required to be on a stable dose of corticosteroids for at least 6 months prior to initiation of therapy and demonstrate minimum performance on a 6MWT, North Star Ambulatory Assessment, and Gowers test. Results of this study showed an increase in mean dystrophin levels from 0.10% of normal at baseline to 1.02% at week 48 (mean change of 0.92%). It is not yet known whether this increase in dystrophin contributes to clinical benefit in patients. No confirmatory trials have yet been conducted to demonstrate that Vyondys 53 provides clinical benefit in DMD patients amenable to exon 53 skipping.

POLICY:

AMONDYS 45

Based upon our assessment and review of the peer-reviewed literature, Amondys 45 has been medically proven to be effective and therefore, **medically appropriate** for the following:

1. Must have a diagnosis of DMD with a confirmed mutation of the DMD gene that is amenable to exon 45 skipping **AND**
2. Must be on a stable dose of corticosteroids for at least 6 months prior to therapy or a documented reason not to be on corticosteroids **AND**
3. Documentation must indicate kidney function testing prior to starting therapy **AND**
4. Patient must not concurrently be on another exon skipping therapy for DMD **AND**

5. Amondys 45 will not be approved for use in patients that have previously been treated with Elevidys **AND**
6. All the below criteria must be met (The below criteria are NOT applicable to **Medicaid Managed Care**)
   a. Prescribed by or in consultation with a provider who specializes in the treatment of Duchenne Muscular dystrophy (DMD) and/or neuromuscular disorders **AND**
   b. The patients must be ambulatory and must have a baseline unassisted 6-minute walk test (6MWT) of at least 300 meters **AND**
   c. Must have stable cardiac function with left ventricular ejection fraction (LVEF) of >40% **AND**
   d. Must have stable pulmonary function with forced vital capacity ≥ 50% of predicted and not require supplemental oxygen **AND**
   e. Dose must not exceed 30mg/kg infused over 35-60 minutes, once weekly **AND**
   f. Initial approval for new starts will be for 3 months. Continued approval in non-new starts for additional 6-month periods will require recent documentation that the patient continues to remain ambulatory with an unassisted 6MWT of at least 100 meters. In addition, the patient must meet the following criteria (Progress notes/test results must be dated after the most recent drug approval and within 3 months of the continuation period that is being requested):
      i. Must have stable cardiac function with left ventricular ejection fraction (LVEF) of >40% **AND**
      ii. Must have stable pulmonary function with forced vital capacity ≥ 50% of predicted and not require supplemental oxygen **AND**
      iii. Must have a < 5% annual decline in percent predicted FVC (FVC% p)

ELEVIDYS

Based upon our criteria and assessment of the peer-reviewed evidence, the use of Elevidys (delandistrogene moxeparvovec-rokl) has not been medically proven to be effective and, therefore, is considered investigational for the treatment of Duchenne muscular dystrophy (DMD). The justification for Elevidys (delandistrogene moxeparvovec-rokl) to be considered investigational is as follows:

1. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug has a definite positive effect on health outcomes.
2. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug, over time, leads to improvement in health outcomes (e.g., the beneficial effects of the service outweigh any harmful effects).
3. Based upon our assessment of peer-reviewed medical literature, there is inconclusive evidence that the drug provides improvement in health outcomes in standard conditions of medical practice, outside the clinical investigatory settings.

Refer to Corporate Medical Policy #11.01.03 Experimental or Investigational Services

EMFLAZA

Based upon our assessment and review of the peer-reviewed literature, Emflaza has been medically proven to be effective and therefore, **medically appropriate** for the following:

1. Must be prescribed by or in consultation with a provider who specializes in the treatment of Duchene Muscular dystrophy (DMD) and/or neuromuscular disorders **AND**
2. Must have a diagnosis of DMD with a confirmed mutation of the DMD gene **AND**
3. Must have had trial of prednisone for ≥ 6 months (documentation required) and at least ONE of the following intolerable adverse effects:
   a. Cushingoid appearance (documentation required) **OR**
   b. Central (truncal) obesity (documentation required) **OR**

   c. Undesirable weight gain defined as a ≥10% of body weight gain increase over a 6-month period (documentation required) **OR**
   d. Diabetes and/or hypertension that is unable to be managed **OR**
   e. Experienced unmanageable side effects that required hospitalization or significant clinical intervention at any point during prednisone therapy (a full 6-month trial is not required)
      i. Examples included steroid induced mania, sepsis, or severe behavioral changes which negatively impact school, home, etc.
4. Initial approval will be 12 months. Subsequent approvals for 12 months at a time will require documentation of a stabilization or slowing of decline in motor function, pulmonary function, or orthopedic outcomes as measured from pretreatment baseline status
5. The dose of Emflaza must not exceed 0.9 mg/kg/day. Quantity of Emflaza tablets will be limited to 30 tablets per 30 days and Emflaza suspension will be limited to 30 ml per 30 days
   a. If tablets are used, dose should be rounded up to the nearest possible dose. If suspension is used, dose should be rounded up to the nearest tenth of a milliliter
   b. Requests for additional quantity of Emflaza liquid suspension will only be allowed for children aged 7 years and under whose weight warrants an increased quantity
      i. Children over 8 years of age will require documentation of an attempt and inability to swallow an oral pill (whole or crushed)

EXONDYS 51

Based upon our assessment and review of the peer-reviewed literature, Exondys 51 has been medically proven to be effective and therefore, **medically appropriate** for the following:

1. Must have a diagnosis of DMD with a confirmed mutation of the DMD gene that is amenable to exon 51 skipping **AND**
2. Must be on a stable dose of corticosteroids for at least 6 months prior to therapy or a documented reason not to be on corticosteroids **AND**
3. Patient must not concurrently be on another exon skipping therapy for DMD **AND**
4. Exondys 51 will not be approved for use in patients that have previously been treated with Elevidys **AND**
5. All the below criteria must be met (The below criteria is NOT applicable to **Medicaid Managed Care**)
   a. Prescribed by or in consultation with a provider who specializes in the treatment of Duchene Muscular dystrophy (DMD) and/or neuromuscular disorders **AND**
   b. The patient must be ambulatory with a baseline unassisted 6-minutewalk test (6MWT) of at least 200 meters **AND**
   c. Must have stable cardiac function with left ventricular ejection fraction (LVEF) of >40% **AND**
   d. Must have stable pulmonary function with forced vital capacity ≥ 50% of predicted and not require supplemental oxygen **AND**
   e. Dose must not exceed 30mg/kg of body weight infused once weekly **AND**
   f. Initial approval for new starts will be for 3 months. Continued approval in non-new starts for additional 6-month periods will require documentation that the patient continues to remain ambulatory with an unassisted 6MWT of at least 100 meters. In addition, the patient must meet the following criteria (Progress notes/test results must be dated after the most recent drug approval and within 3 months of the continuation period that is being requested):
      i. Must have stable cardiac function with left ventricular ejection fraction (LVEF) of > 40% **AND**
      ii. Must have stable pulmonary function with forced vital capacity ≥ 50% of predicted and not require supplemental oxygen **AND**
      iii. Must have a < 5% annual decline in percent predicted FVC (FVC% p)

VILTEPSO

Based upon our assessment and review of the peer-reviewed literature, Viltepso has been medically proven to be effective and therefore, **medically appropriate** for the following:

1. Must have a diagnosis of DMD with a confirmed mutation of the DMD gene that is amenable to exon 53 skipping **AND**
2. Must be on a stable dose of corticosteroids for at least 3 months prior to therapy or a documented reason not to be on a corticosteroid **AND**
3. Documentation must indicate kidney function testing prior to starting therapy **AND**
4. Patient must not concurrently be on another exon skipping therapy for DMD **AND**
5. Viltepso will not be approved for use in patients that have previously been treated with Elevidys **AND**
6. All the below criteria must be met (The below criteria are NOT applicable to **Medicaid Managed Care**)
    a. Prescribed by or in consultation with a provider who specializes in the treatment of Duchenne Muscular dystrophy (DMD) and/or neuromuscular disorders **AND**
    b. The patients must be ambulatory and must have a baseline unassisted 6-minute walk test (6MWT) of at least 250 meters **AND**
    c. Must have stable cardiac function with left ventricular ejection fraction (LVEF) of >40% **AND**
    d. Must have stable pulmonary function with forced vital capacity ≥ 50% of predicted and not require supplemental oxygen **AND**
    e. Dose must not exceed 80mg/kg infused over 60 minutes, once weekly **AND**
    f. Initial approval for new starts will be for 3 months Continued approval in non-new starts for additional 6-month periods will require recent documentation that the patients continue to be ambulatory with an unassisted 6MWT of at least 100 meters. In addition, the patient must meet the following criteria (Progress notes/test results must be dated after the most recent drug approval and within 3 months of the continuation period that is being requested):
        i. Must have stable cardiac function with left ventricular ejection fraction (LVEF) of >40% **AND**
        ii. Must have stable pulmonary function with forced vital capacity ≥ 50% of predicted and not require supplemental oxygen **AND**
        iii. Must have a < 5% annual decline in percent predicted FVC (FVC% p)

VYONDYS 53

Based upon our assessment and review of the peer-reviewed literature, Vyondys 53 has been medically proven to be effective and therefore, **medically appropriate** for the following:

1. Must have a diagnosis of DMD with a confirmed mutation of the DMD gene that is amenable to exon 53 skipping **AND**
2. Must be on a stable dose of corticosteroids for at least 6 months prior to therapy or a documented reason not to be on corticosteroids **AND**
3. Documentation must indicate kidney function testing prior to starting therapy **AND**
4. Patient must not concurrently be on another exon skipping therapy for DMD **AND**
5. Vyondys 53 will not be approved for use in patients that have previously been treated with Elevidys **AND**
6. All the below criteria must be met (The below criteria are NOT applicable to **Medicaid Managed Care**)
    a. Prescribed by or in consultation with a provider who specializes in the treatment of Duchene Muscular dystrophy (DMD) and/or neuromuscular disorders **AND**
    b. The patient must be ambulatory must have a baseline unassisted 6-minutewalk test (6MWT) of at least 250 meters **AND**
    c. Must have stable cardiac function with left ventricular ejection fraction (LVEF) of >50% **AND**

d. Must have stable pulmonary function with forced vital capacity ≥ 50% of predicted and not require supplemental oxygen **AND**
e. There must be a proven contraindication to Viltepso **AND**
f. Dose must not exceed 30 mg/kg infused IV over 35 to 60 minutes, once weekly
g. Initial approval for new starts will be for 3 months. Continued approval in non-new starts for additional 6-month periods will require documentation that the patient continues to be ambulatory with an unassisted 6MWT of at least 100 meters. In addition, the patient must meet the following criteria (Progress notes/test results must be dated after the most recent drug approval and within 3 months of the continuation period that is being requested):
    i. Must have stable cardiac function with left ventricular ejection fraction (LVEF) of >50% **AND**
    ii. Must have stable pulmonary function with forced vital capacity ≥ 50% of predicted and not require supplemental oxygen **AND**
    iii. Must have a < 5% annual decline in percent predicted FVC (FVC% p)

POLICY GUIDELINES:

1. Amondys 45, Elevidys, Exondys 51, Vyondys 53, and Viltepso are administered as an IV infusion and will be covered under the medical benefit. Emflaza is a self-administered oral medication and will be covered under the pharmacy benefit.
2. Amondys 45, Elevidys, Exondys 51, Vyondys 53 and Viltepso will not be approved for patients who have lost ambulation or who have LVEF or FVC% predicted test results that indicate severe decompensation. These medications have only been evaluated in ambulatory patients and it is unknown if patients with more advanced disease and greater muscle deterioration would receive any benefit with treatment. Pivotal studies do not provide adequate support to determine if the drug provides clinical benefit related to cardiac and respiratory complications, which can lead to morbidity and mortality in DMD. (Not applicable to **Medicaid Managed Care**)
3. Continued approval at time of recertification will require documentation via current progress notes (dated after the most recent drug approval and within 3 months of the continuation period that is being requested) that the drug is providing ongoing benefit to the patient in terms of improvement or stability in disease state or condition and meets all other criteria under the policy. Such documentation may include progress notes, imaging or laboratory findings, and other objective or subjective measures of benefit which support that continued use of the requested product is medically necessary. Also, ongoing use of the requested product must continue to reflect the current policy's preferred formulary. Recertification reviews may result in the requirement to try more cost-effective treatment alternatives as they become available (i.e., generics, biosimilars, or other guideline-supported treatment options). Requested dosing must continue to be consistent with FDA-approved or off-label/guideline-supported dosing recommendations.

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract.

CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.

Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

HCPCS:

C9075	Amondys 45 (Effective 7/1/21)
J1428	Exondys 51
J3490	Viltepso
J1429	Vyondys 53

UPDATES:

Date	Revision
01/02/2024	Revised
11/30/2023	P&T Committee Approval
9/22/2022	P&T Committee Approval
3/31/2022	Revised
12/3/2021	Revised
9/16/2021	Reviewed/P&T Committee Approval
6/15/2021	Revised
5/6/2021	P&T Committee Approval
4/15/2021	Revised
1/29/2021	Revised
9/16/2020	P & T Committee Approval
9/3/2020	Revised
7/1/2020	Revised
2/20/2020	Revised
12/30/2019	Created

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