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UTILIZATION MANAGEMENT MEDICAL POLICY

POLICY: Muscular Dystrophy – Gene Therapy – Elevidys Utilization Management Medical Policy
- Elevidys® (delandistrogene moxeparvovec-rokl intravenous infusion – Sarepta)

REVIEW DATE: 03/11/2026; 08/26/2026 selected revision

OVERVIEW

Elevidys, an adeno-associated virus (AAV) vector-based gene therapy, is indicated for the treatment of individuals 4 years of age and older with **Duchenne muscular dystrophy (DMD)**.[1] It is specifically indicated in patients who are **ambulatory** and have a confirmed mutation in the *DMD* gene.

Limitations of Use[1]:
Elevidys is not recommended in patients with the following:
1. Preexisting liver impairment (defined as gamma-glutamyl transferase [GGT] greater than 2 times the upper limit of normal or total bilirubin above the upper limit of normal not due to Gilbert's syndrome) or active hepatic viral infection due to the high risk of acute serious liver injury and acute liver failure.
2. Recent vaccination (within 4 weeks of treatment) due to immunogenicity and potential safety concerns.
3. Active or recent (within 4 weeks) infections due to safety concerns.

Disease Overview

DMD is a rare, progressive X-linked disease resulting from mutation(s) of the *DMD* gene, also known as the *Dystrophin* gene.[2,3] The incidence of DMD in the US is approximately 1 in 5,000 live male births. The *DMD* gene is the largest known human gene, totaling 2.3 megabases in size. The gene encodes for a functional dystrophin protein, which is part of a transmembrane protein complex that spans the sarcolemma of skeletal and cardiac muscle cells. This complex links the cytoskeleton to the extracellular matrix providing structural integrity to the sarcolemma and helps to transmit and absorb the shock associated with muscle contraction. Mutations in the *DMD* gene can prevent the production of dystrophin, creating either completely non-functional dystrophin protein or dystrophin isoforms with partial function. Without dystrophin, normal activity in patients with DMD causes excessive damage to muscle fiber cells. Over time, the muscle cells are replaced with fat and fibrotic tissue. Progressive muscle weakness is the primary manifestation of DMD. This leads to loss of ambulation, associated motor delays, respiratory impairment, and progressive decline in cardiac function. The first clinical symptoms of DMD are delay in motor development milestones, such as walking, which is observed around 2 years of age. Often there is a delay in diagnosis until the age of 3 years to 5 years. Age is an important prognostic factor in the progression of DMD. There is no cure for DMD currently. The goal of treatment is to manage symptoms, slow disease progression, and to delay disability. Boys with DMD typically lose the ability to walk by age 12 years or 13 years. In the past, mortality occurred by late adolescence or early twenties; however, with advances in respiratory and cardiac management, some patients are living into the fourth decade. The most common cause of death for patients with DMD are respiratory failure, respiratory infection, cardiomyopathy, and cardiac arrhythmias. Corticosteroids are a mainstay of therapy in DMD; however, its mechanism of action in DMD is unknown. Corticosteroids ameliorate the symptoms of the disease and delay time to loss of ambulation and other sequelae.

Guidelines

Elevidys is not addressed in current guidelines for DMD. The guidelines from the DMD Care Considerations Working Group (2018) notes that genetic testing for confirming DMD diagnosis is always required.[3-5] In patients with no mutations identified, but with signs/symptoms of DMD, muscle biopsy is

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clinically indicated. Glucocorticoids and physical therapy are the mainstays of treatment and should be continued even after the patient is non-ambulatory. Corticosteroids reduce the risk of scoliosis and stabilize pulmonary function. In patients who are non-ambulatory, continuing corticosteroid treatment provides a reduction in the risk of progressive scoliosis and stabilization of pulmonary function tests. Due to this benefit, glucocorticoids should be considered in all patients with DMD.

North Star Ambulatory Assessment (NSAA): NSAA is a validated functional rating scale specifically developed to assess ambulatory tasks in boys with DMD.[6] The NSAA scale is composed of 17 items and measures changes in gross motor ability (i.e., ability to rise from floor, move from sitting to standing, jump, run, and climb steps). Scores range from 0 to 34, with higher scores indicating better motor function.

Clinical Efficacy

EMBARK is a randomized, two-part, crossover, placebo-controlled, Phase III confirmatory trial assessing the efficacy and safety of Elevidys in ambulatory patients with DMD (n = 125).[1,7,8] Patients were ≥ 4 years to < 8 years of age and were on a stable dose of corticosteroids for at least 12 weeks. All enrolled patients had baseline anti-adeno-associated virus serotype rh 74 (AAVrh74) antibody titers < 1:400. Part I of the study was double-blinded; patients received Elevidys infusion or placebo and were followed for 52 weeks. In Part II, patients who received placebo in Part I received Elevidys infusion with a follow-up duration of another 52 weeks. Some of the key inclusion criteria for the EMBARK trial were patients with a NSAA score > 16 and < 29 and the time-to-rise from floor (TTR) < 5 seconds at the screening visit.[1,7] Patients were excluded if they met any of the following relevant exclusion criteria noted in the EMBARK study: left ventricular ejection fraction of < 40%; abnormal laboratory values considered clinically significant, including, but not limited to GGT > two times the upper limit of normal; glutamate dehydrogenase > 15 U/L; total bilirubin > the upper limit of normal (elevations in total bilirubin confirmed to be due to Gilbert's syndrome are not exclusionary); white blood cell count > 18,500 per microliter; and platelets ≤ 150,000 per microliter. Patients should not have any serologic evidence of current, chronic or active human immunodeficiency virus (HIV), hepatitis C, or hepatitis B infection. Another exclusion criteria was the use of any investigational medication or any treatment designed to increase dystrophin expression (e.g., exon-skipping therapies) within 6 months of Elevidys administration and during the study.

***Year 1 Results*:** The primary endpoint of change from baseline to Week 52 in the NSAA total score was not significantly different for the Elevidys and placebo-treated groups.[7] The between-group difference least squares mean (LSM) was 0.65 points (95% confidence interval [CI]: -0.45, 1.74; P = not significant). The key secondary endpoints of change from baseline to Week 52 in TTR and the 10 meter walk/run (10MWR) were statistically significantly different between Elevidys and placebo. However, since the primary endpoint failed to meet statistical significance, these secondary endpoint results are thought to be hypothesis generating.

Updated 2-year data from the EMBARK study are published.[8] Due to the crossover study design in EMBARK, patients treated with placebo in Part I were treated with Elevidys in Part II after the first 52 weeks. For this reason, there is no longer a placebo arm in the EMBARK study. The Elevidys-treated patients in Part I were compared with a propensity score-weighted matched external control (EC) cohort of patients with DMD. Patients for EC were selected from the FOR-DMD, BioMarin PRO-DMD-01, and CINRG DNHS studies. Based on the baseline characteristics, the EC cohort and patients in the EMBARK study were well-matched. Patients in the EC had received only corticosteroids, although the exact dose is not available. In Part II, a higher peri-infusion corticosteroid dose (~1.6 mg/kg/day) was administered to Elevidys-treated patients before the placebo infusion; this was in addition to the baseline stable oral standard of care corticosteroids.

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***Year 2 Results*:** At 2 years, Elevidys-treated patients demonstrated statistically significant differences in functional outcome scores compared with the EC cohort.[8] For the NSAA score, the LSM change was 2.63 for Elevidys vs. -0.25 for the EC. The between-group LSM difference for the primary endpoint of NSAA score improved by 2.88 points with Elevidys (95% CI: 1.43, 4.33; P = 0.0001). For the TTR secondary endpoint, there was a decrease of -2.06 seconds (95% CI: -3.43, -0.70; P = 0.0033) in the TTR with Elevidys (+0.65 seconds for Elevidys vs +2.71 seconds for EC). For the 10MWR, for Elevidys-treated patients it decreased by -0.04 seconds vs. +1.32 seconds for EC for a difference of -1.36 seconds (95% CI: -2.24, -0.47; P = 0.0028). The rise from floor velocity and the 10MWR velocity also favored Elevidys (difference +0.055 rise/s; +0.239 m/s, respectively; P ≤ 0.0001 for both). P-values are noted as nominal values and have not been adjusted for multiple comparisons. In patients treated with Elevidys, the micro-dystrophin expression increased from 34.29% at Week 12 (n = 17) to 45.68% at Week 64 (n = 16). Sarcolemmal localization, as measured by percent dystrophin-positive fibers (PDPF), increased from 28.13% at Week 12 to 38.60% at Week 64.

Dosing

The recommended dose is 1.33 x 10^{14} vg/kg of body weight (or 10 mL/kg body weight) for patients weighing < 70 kg or 9.31 x 10^{15} vg total fixed dose for patients ≥ 70 kg.[1] Re-administration of Elevidys is not recommended. Immune responses to the AAVrh74 vector can occur after Elevidys administration. To reduce this risk, corticosteroids should be administered starting 1 day prior to Elevidys infusion and continued for a minimum of 60 days after the infusion, unless earlier tapering is clinically indicated.

Safety

Elevidys has a Boxed Warning for acute serious liver injury and acute liver failure.[1] Acute serious liver injury, including life-threatening and fatal acute liver failure has occurred with Elevidys. Patients with preexisting liver impairment may be at higher risk. Liver function by clinical examination and laboratory testing should be assessed prior to Elevidys infusion for aspartate aminotransferase (AST), alanine aminotransferase (ALT), GGT, albumin, activated partial thromboplastin time (aPTT), international normalized ratio (INR), and total bilirubin. Platelet counts and troponin-I levels should also be assessed. Systemic corticosteroids should be administered before and after Elevidys infusion. Liver function should be monitored weekly for the first 3 months after infusion and should continue to be monitored until results are unremarkable. Patients should maintain proximity to an appropriate healthcare facility for at least 2 months following Elevidys infusion. Patients should consult with a specialist (e.g., gastroenterologist or hepatologist) if acute serious liver injury or impending acute liver failure is suspected.

Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene.[1] Warnings/Precautions include acute serious liver injury and acute liver failure, serious infections, immune-mediated myositis, myocarditis, infusion-related reactions, and pre-existing immunity against AAVrh74. For the administration of Elevidys, the anti-AAVrh74 total antibody binding titer should be < 1:400.

POLICY STATEMENT

Prior Authorization is recommended for medical benefit coverage of Elevidys. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indication. Because of the specialized skills required for evaluation and diagnosis of patients treated with Elevidys as well as the specialized training required for administration of Elevidys, approval requires Elevidys to be prescribed by a physician who specializes in the condition being treated. All approvals are provided for one-time (per lifetime) as a single dose. The approval duration is 90 days to allow for an adequate timeframe to prepare and administer one dose of therapy. If claims history is available, verification is required for certain criteria as noted by **[verification in claims history required]**. For the dosing criteria, verification of the appropriate weight-

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based dosing is required by a Medical Director as noted by **[verification required]**. In the criteria for Elevidys, as appropriate, an asterisk (*) is noted next to the specified gender. In this context, the specified gender is defined as follows: males are defined as individuals with the biological traits of a man, regardless of the individual's gender identity or gender expression. All reviews (approvals and denials) will be forwarded to the Medical Director for evaluation.

Documentation: Documentation is required for use of Elevidys as noted in the criteria as **[documentation required]**. Documentation may include, but is not limited to chart notes, laboratory results, medical test results, claims records, prescription receipts, and/or other information. All documentation must include patient-specific identifying information.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Elevidys is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Duchenne Muscular Dystrophy – Treatment.** Approve a one-time (per lifetime) single dose if the patient meets ALL of the following (A, B, C, D, E, F, G, H, I, J, K, L, M, N, O, P, and Q):
   - **A)** Patient is a male*; AND
   - **B)** Patient age is greater than or equal to 4 years 0 days and less than 8 years 0 days; AND
   - **C)** Patient had a genetic test confirming the diagnosis of Duchenne muscular dystrophy involving a pathogenic variant in the *DMD* gene **[documentation required]**; AND
   - **D)** Patient does not have any deletions in exon 8 or exon 9 in the *DMD* gene **[documentation required]**; AND
   - **E)** Patient has not received Elevidys in the past **[verification in claims history required]**; AND
     Note: If no claim for Elevidys is present (or if claims history is not available), the prescribing physician confirms that the patient has not previously received Elevidys.
   - **F)** Patient is ambulatory **[documentation required]**; AND
   - **G)** Anti-adeno-associated virus serotype rh74 (AAVrh74) total binding antibody titers are less than 1:400 **[documentation required]**; AND
   - **H)** Patient screening is negative for ALL of the following (i, ii, and iii):
     - **i.** Human immunodeficiency virus-1 and -2 **[documentation required]**; AND
     - **ii.** Hepatitis B virus **[documentation required]**; AND
     - **iii.** Hepatitis C virus **[documentation required]**; AND
   - **I)** The prescribing physician confirms that the patient has not received or will not receive any vaccinations within 4 weeks of Elevidys treatment; AND
   - **J)** The prescribing physician confirms that the patient does not have an active infection (e.g., bacterial, viral) or a recent infection within the past 4 weeks; AND
   - **K)** Patient has left ventricular ejection fraction that is greater than or equal to 40% **[documentation required]**; AND
   - **L)** Patient has undergone liver function testing within the past 30 days and meets ALL of the following (i, ii, and iii):
     - **i.** Gamma-glutamyl transferase level is less than or equal to 2 times the upper limit of normal **[documentation required]**; AND
     - **ii.** Total bilirubin is less than the upper limit of normal **[documentation required]**; AND
       Note: A patient with Gilbert's syndrome does not have to meet this requirement.

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iii. Coagulation parameters, such as the activated partial thromboplastin time (aPTT) and international normalized ratio (INR) are within the normal laboratory reference ranges **[documentation required]**; AND

M) A complete blood cell count has been obtained within the past 30 days and the patient meets BOTH of the following (i and ii):
- **i.** White blood cell count is less than 18.5 x 10^9/L **[documentation required]**; AND
- **ii.** Platelet count is greater than 150 x 10^9/L **[documentation required]**; AND

N) The medication is prescribed by a neurologist, neuromuscular specialist, or a physician who specializes in the management of Duchenne muscular dystrophy; AND

O) According to the prescribing physician, patient has started or will receive systemic corticosteroids equivalent to oral prednisone at a dose of 1 mg/kg per day or more, commencing 1 day prior to Elevidys infusion and continuing for at least 60 days; AND

P) Current patient body weight has been obtained within the past 30 days **[documentation required]**; AND

Q) If criteria A through P are met, approve one dose of Elevidys by intravenous infusion to provide a one-time (per lifetime) single dose, according to ONE of the following (i or ii):
- **i.** For a patient weighing less than 70 kg, the Elevidys dose is 1.33 x 10^{14} vector genomes per kg (vg/kg) based on the current patient weight in kg (or 10 mL/kg body weight) **[verification required]**; OR
- **ii.** For a patient weighing 70 kg or more, the Elevidys dose is 9.31 x 10^{15} vg total fixed dose **[verification required]**.
  Elevidys is provided as a customized kit to meet dosing requirements for each patient based on their weight (in kilograms). Elevidys kit sizes (per the cited NDC) are in Table 1.

* Refer to the Policy Statement

Dosing. The recommended dose of Elevidys is one dose given by intravenous infusion to provide a one-time (per lifetime) single dose based on ONE of the following (A or B):

A) For a patient weighing less than 70 kg, the Elevidys dose is 1.33 x 10^{14} vector genomes per kilogram (vg/kg) of body weight (or 10 mL/kg body weight) **[verification required]**; OR

B) For a patient weighing 70 kg or more, the Elevidys dose is 9.31 x 10^{15} vg total fixed dose **[verification required]**.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Elevidys is not recommended in the following situations:

1. **Becker Muscular Dystrophy.** Elevidys is not approved for use in this condition.

2. **Patients with Duchenne Muscular Dystrophy Who are Non-Ambulatory.** Elevidys is not approved for use in patients who are non-ambulatory.

3. **Concurrent Use with Anti-Sense Oligonucleotide (Exon-Skipping) Therapies.** Patients should have discontinued use of exon-skipping therapies prior to the administration of Elevidys. The EMBARK confirmatory trial excluded patients who had used exon-skipping therapies within 6 months of Elevidys administration and at any time during the study. There are no data available with concomitant use of any of the exon-skipping therapies with Elevidys.

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Note: Examples of anti-sense oligonucleotide (exon-skipping) therapies include Exondys 51 (eteplirsen intravenous infusion), Vyondys 53 (golodirsen intravenous infusion), Viltepso (viltolarsen intravenous infusion), and Amondys 45 (casimersen intravenous infusion).

4. **Prior Receipt of Gene Therapy.** Elevidys has not been studied in a patient who has received prior gene therapy. Treatment with Elevidys is not recommended.

5. **Prior Hematopoietic Stem Cell Transplantation.** Elevidys has not been studied in a patient who has received prior stem cell transplant. Treatment with Elevidys is not recommended.

6. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Elevidys® intravenous infusion [prescribing information]. Cambridge, MA: Sarepta; November 2025.
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3. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *Lancet Neurol*. 2018;17(3):251-267.
4. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurol*. 2018;17(4):347-361.
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6. Muntoni F, Domingos J, Manzur AY, et al. Categorising trajectories and individual item changes of the North Star Ambulatory Assessment in patients with Duchenne muscular dystrophy. *PLoS ONE*. 2019;14(9):e0221097.
7. Mendell JR, Muntoni F, McDonald CM, et al. AAV gene therapy for Duchenne muscular dystrophy: the EMBARK phase 3 randomized trial. *Nat Med.* 2025;31(1):332-341.
8. Mendell JR, Muntoni F, McDonald CM, et al. Two-year outcomes following delandistrogene moxeparvovec treatment in ambulatory patients with Duchenne muscular dystrophy: Phase 3 EMBARK trial. *Neurol Ther.* 2026;15(2):545-559.

Table 1. Elevidys Multi-Vial Kits.[1]

Patient Weight (kg)	Total Vials per Kit	Total Dose Volume Per Kit (mL)	NDC Number
10.0 to 10.4	10	100	60923-501-10
10.5 to 11.4	11	110	60923-502-11
11.5 to 12.4	12	120	60923-503-12
12.5 to 13.4	13	130	60923-504-13
13.5 to 14.4	14	140	60923-505-14
14.5 to 15.4	15	150	60923-506-15
15.5 to 16.4	16	160	60923-507-16
16.5 to 17.4	17	170	60923-508-17
17.5 to 18.4	18	180	60923-509-18
18.5 to 19.4	19	190	60923-510-19
19.5 to 20.4	20	200	60923-511-20
20.5 to 21.4	21	210	60923-512-21
21.5 to 22.4	22	220	60923-513-22
22.5 to 23.4	23	230	60923-514-23

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Table 1 (continued). Elevidys Multi-Vial Kits.[1]

Patient Weight (kg)	Total Vials per Kit	Total Dose Volume Per Kit (mL)	NDC Number
23.5 to 24.4	24	240	60923-515-24
24.5 to 25.4	25	250	60923-516-25
25.5 to 26.4	26	260	60923-517-26
26.5 to 27.4	27	270	60923-518-27
27.5 to 28.4	28	280	60923-519-28
28.5 to 29.4	29	290	60923-520-29
29.5 to 30.4	30	300	60923-521-30
30.5 to 31.4	31	310	60923-522-31
31.5 to 32.4	32	320	60923-523-32
32.5 to 33.4	33	330	60923-524-33
33.5 to 34.4	34	340	60923-525-34
34.5 to 35.4	35	350	60923-526-35
35.5 to 36.4	36	360	60923-527-36
36.5 to 37.4	37	370	60923-528-37
37.5 to 38.4	38	380	60923-529-38
38.5 to 39.4	39	390	60923-530-39
39.5 to 40.4	40	400	60923-531-40
40.5 to 41.4	41	410	60923-532-41
41.5 to 42.4	42	420	60923-533-42
42.5 to 43.4	43	430	60923-534-43
43.5 to 44.4	44	440	60923-535-44
44.5 to 45.4	45	450	60923-536-45
45.5 to 46.4	46	460	60923-537-46
46.5 to 47.4	47	470	60923-538-47
47.5 to 48.4	48	480	60923-539-48
48.5 to 49.4	49	490	60923-540-49
49.5 to 50.4	50	500	60923-541-50
50.5 to 51.4	51	510	60923-542-51
51.5 to 52.4	52	520	60923-543-52
52.5 to 53.4	53	530	60923-544-53
53.5 to 54.4	54	540	60923-545-54
54.5 to 55.4	55	550	60923-546-55
55.5 to 56.4	56	560	60923-547-56
56.5 to 57.4	57	570	60923-548-57
57.5 to 58.4	58	580	60923-549-58
58.5 to 59.4	59	590	60923-550-59
59.5 to 60.4	60	600	60923-551-60
60.5 to 61.4	61	610	60923-552-61
61.5 to 62.4	62	620	60923-553-62
62.5 to 63.4	63	630	60923-554-63
63.5 to 64.4	64	640	60923-555-64
64.5 to 65.4	65	650	60923-556-65
65.5 to 66.4	66	660	60923-557-66
66.5 to 67.4	67	670	60923-558-67
67.5 to 68.4	68	680	60923-559-68
68.5 to 69.4	69	690	60923-560-69
69.5 and above	70	700	60923-561-70

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HISTORY

Type of Revision	Summary of Changes	Review Date
Annual Revision	No criteria changes.	08/14/2024
Early Annual Revision	The Policy Statement was modified from "Due to the lack of clinical efficacy data, approval is not recommended for Elevidys" to as listed. **Duchenne Muscular Dystrophy – Treatment:** This condition of approval was added. **Conditions Not Recommended for Approval:** Removed Duchenne Muscular Dystrophy. Added Becker Muscular Dystrophy, Patients with Duchenne Muscular Dystrophy Who are Non-Ambulatory, Concurrent Use with Anti-Sense Oligonucleotides (Exon-Skipping) Therapies, Prior Receipt of Gene Therapy, and Prior Hematopoietic Stem Cell Transplantation.	06/25/2025
Selected Revision	**Duchenne Muscular Dystrophy – Treatment:** The Policy Statement was modified to state approval is not recommended. This condition of approval was moved to Conditions Not Recommended for Approval.	07/23/2025
Update	**07/29/2025:** Overview section updated with information on resuming Elevidys shipment for ambulatory patients.	--
Early Annual Revision	**Duchenne Muscular Dystrophy – Treatment:** This condition of approval was added. **Conditions Not Recommended for Approval:** Removed Duchenne Muscular Dystrophy. Added Becker Muscular Dystrophy, Patients with Duchenne Muscular Dystrophy Who are Non-Ambulatory, Concurrent Use with Anti-Sense Oligonucleotide (Exon-Skipping) Therapies, Prior Receipt of Gene Therapy, and Prior Hematopoietic Stem Cell Transplantation.	03/11/2026
Selected Revision	**Duchenne Muscular Dystrophy – Treatment:** In reference to left ventricular ejection fraction, spelled out "that is greater than or equal to" 40%. Previously it was "≥" 40%. Under liver function testing, removed requirements for alanine aminotransferase levels, aspartate aminotransferase levels, and glutamate dehydrogenase level. For gamma glutamyl transferase level spelled out "less than or equal to" 2 times the upper limit of normal; previously "≤" was used. For total bilirubin level, spelled out "less" than the upper limit of normal; previously it was "<" than the upper limit of normal. Under complete blood cell count, for white blood cell count spelled out "less than" 18.5 x 10^9/L. For platelet count spelled out "greater than" 150 x 10^9/L.	08/26/2026

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