Policy and Procedure	
PHARMACY PRIOR AUTHORIZATION POLICY AND CRITERIA ORPTCHEM020.0226	**HEMATOLOGICAL AGENTS** **REBLOZYL** (luspatercept-aamt vial) **RYTELO** (imetelstat vial)
Effective Date: 3/1/2026	**Review/Revised Date**: 05/20, 11/20, 10/21, 10/22, 11/23, 10/24, 11/24, 10/25, 02/26 (snm)
Original Effective Date: 04/20	**P&T Committee Meeting Date**: 02/20, 06/20, 12/20, 12/21, 12/22, 12/23, 10/23, 10/24, 12/24, 12/25, 02/26
Approved by: Oregon Region Pharmacy and Therapeutics Committee	

SCOPE:
Providence Health Plan and Providence Health Assurance as applicable (referred to individually as “Company” and collectively as “Companies”).

APPLIES TO:
Commercial
Medicaid

POLICY CRITERIA:

COVERED USES:
All Food and Drug Administration (FDA) approved indications not otherwise excluded from the benefit.

REQUIRED MEDICAL INFORMATION:
Beta-Thalassemia:

A. For initiation of therapy (new starts) for beta-thalassemia, Reblozyl may be covered when all the following are met (supporting documentation required):
    1. Diagnosis of beta-thalassemia confirmed by hemoglobin analysis such as high-performance liquid chromatography (HPLC) or genetic testing
    2. Symptomatic anemia defined as a pretreatment or pretransfusion hemoglobin level less than or equal to 11 grams per deciliter
    3. Member has transfusion-dependent anemia requiring RBC transfusion of at least six units within the previous 24 weeks

B. For patients that are established on therapy for beta-thalassemia beyond nine weeks, ongoing documentation of patient response to therapy must include maintenance of reduced transfusion levels

Myelodysplastic Syndrome (MDS):

A. For initiation of therapy (new starts) for MDS, all the following must be met (supporting documentation required):
    1. Meets National Comprehensive Cancer Network guidelines with recommendation 2A or higher

B. For patients that are established on therapy for MDS: Documentation of a positive response to therapy (such as a decrease in transfusion burden)

Other Indications:

A. For initiation of therapy (new starts) for all other indications, use must be for an FDA approved indication or indication supported by National Comprehensive Cancer Network guidelines with recommendation 2A or higher

B. For patients that are established on therapy for other indications: Documentation of positive response to therapy

Note: Medications obtained as samples, coupons, or any other method of obtaining medications outside of an established health plan benefit are NOT considered established on therapy

EXCLUSION CRITERIA:

Concurrent use of imetelstat and luspatercept

AGE RESTRICTIONS:

Age must be appropriate based on FDA-approved indication

PRESCRIBER RESTRICTIONS:

Must be prescribed by or in consultation with a hematologist/oncologist

COVERAGE DURATION:

Beta-thalassemia: For initiation of therapy, authorization will be for nine weeks. For continuation of therapy, authorization will be for one year.

All other conditions including MDS: For initiation of therapy authorization will be for six months. For continuation of therapy, authorization will be for one year.

QUANTITY LIMIT:

Dose must be appropriate based on FDA-approved indication

Requests for indications that were approved by the FDA within the previous six (6) months may not have been reviewed by the health plan for safety and effectiveness and inclusion on this policy document. These requests will be reviewed using the New Drug and or Indication Awaiting P&T Review; Prior Authorization Request ORPTCOPS047.

Requests for a non-FDA approved (off-label) indication requires the proposed indication be listed in either the American Hospital Formulary System (AHFS), Drugdex, or the National Comprehensive Cancer Network (NCCN) and is considered subject to evaluation of the prescriber's medical rationale,

formulary alternatives, the available published evidence-based research and whether the proposed use is determined to be experimental/investigational.

Coverage for Medicaid is limited to a condition that has been designated a covered line item number by the Oregon Health Services Commission listed on the Prioritized List of Health Care Services.

Coverage decisions are made on the basis of individualized determinations of medical necessity and the experimental or investigational character of the treatment in the individual case.

INTRODUCTION:

Luspatercept-aamt is a recombinant fusion protein that binds to several endogenous TGF-β super family ligands, thereby weakening abnormal Smad2/3 signaling. Inhibition of TGF-β leads to increased differentiation and proliferation of erythroid precursors. Imetelstat is an oligonucleotide telomerase inhibitor. High telomerase activity and human telomerase reverse transcriptase (hTERT) RNA expression have been reported in lower-risk myelodysplastic syndrome (MDS). Inhibiting telomerase enzymatic activity and preventing telomerase binding leads to destruction of abnormal cells with high telomerase activity.

FDA APPROVED INDICATIONS:

Reblozyl (luspatercept)

- Treatment of anemia in adults with β-thalassemia requiring regular RBC transfusions
- Treatment of anemia failing an erythropoiesis stimulating agent and requiring two or more red blood cell units over eight weeks in adult patients with very low- to intermediate-risk myelodysplastic syndromes with ring sideroblasts (MDS-RS) or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T).
- Treatment of anemia without previous erythropoiesis stimulating agent use (ESA-naïve) in adult patients with very low- to intermediate-risk myelodysplastic syndromes (MDS) who may require regular red blood cell (RBC) transfusions.

Rytelo (imetelstat)

- Treatment of adult patients with low- to intermediate-1 risk myelodysplastic syndromes (MDS) with transfusion-dependent anemia requiring four or more red blood cell units over eight weeks who have not responded to or have lost response to or are ineligible for erythropoiesis-stimulating agents

POSITION STATEMENT:

Beta-thalassemia

Beta-thalassemia is a rare genetic blood disorder characterized by partial or complete deficiency of beta globin chain synthesis, leading to reduced red blood cell

(RBC) production. The reduced amount (beta+) or absence (beta0) of beta globin chains result in an unbalanced excess of alpha globin chains, which leads to a cascade of events through the generation of ROS. ROS activates apoptotic pathways, hemolysis of mature red blood cells and degradation of immature erythroid precursors in the bone marrow (also known as ineffective erythropoiesis).

Clinical presentation of β-thalassemia manifests usually within the first two years of life. Transfusion-dependent beta thalassemia (TDT) is a severe manifestation of beta thalassemia in which affected patients require regular blood transfusion to maintain adequate hemoglobin levels. If patients are left untreated or poorly transfused, the clinical picture is characterized by growth retardation, pallor, jaundice, leg ulcers and skeletal changes from expansion of the bone marrow. Current standards of care include regular RBC transfusions and iron chelation therapy to manage iron overload. TDT affects about 2,000 patients in the United States.

The European Hematology Association published guidelines for the management of transfusion dependent thalassemia in 2021. These guidelines recommend offering HSCT to patients at a young age if an HLA identical sibling is an available and willing donor. A matched unrelated donor (MUD) can also be used, provided high compatibility in both HLA classes. The guidelines recommend use of luspatercept in patients requiring regular blood transfusions who are 18 years of age or older. Luspatercept should be discontinued if a patient does not experience a decrease in transfusion burden after 9 weeks of treatment.[17]

There are two FDA approved gene therapies for the treatment of transfusion dependent beta-thalassemia (TDT), betibeglogene autotemcel (beti-cel, Zynteglo) and exagamglogene autotemcel (exa-cel, Casgevy). Beti-cel is a lentiviral vector gene therapy which adds a modified beta-globin gene to autologous hematopoietic stem cells, which enables the production of a modified functional adult hemoglobin. Exa-cel is the first FDA approved gene therapy utilizing CRISPR/Cas9 technology and modifies BCL11A expression in autologous hematopoietic stem cells which increases fetal hemoglobin (γ-globin) production which in turn increases functional hemoglobin production. Casgevy and Zynteglo have demonstrated similar efficacy and safety.

Summary of Clinical Trials for luspatercept in TDT:
BELIEVE Trial (NCT02604433)

- Study Design: R, DB, PC, Phase 3
- Study Duration: 48 weeks
- Patient population (N=336)

  - Inclusion criteria: Adult patients with beta thalassemia requiring regular blood transfusions (6-20 units in the 24 weeks prior to randomization with no greater than 35-day transfusion-free period at that time)
  - Exclusion criteria: Patients with diagnosis of hemoglobin S/β-thalassemia or alpha-thalassemia, DVT or stroke requiring medical intervention ≤24 weeks prior to randomization, chronic anticoagulation, platelet count <100 x 10^9/L, poorly controlled diabetes, use of ESA, ≤24 weeks prior to randomization, hydroxyurea treatment ≤24 weeks prior to randomization, pregnant or lactating females, uncontrolled hypertension, major organ damage, history of malignancy
- Intervention: Luspatercept 1 mg/kg subcutaneously once every 21 days plus best supportive care vs. Normal saline solution and best supportive care
- Primary endpoint: Proportion of subjects with ≥33% reduction from baseline in RBC transfusion burden with a reduction of at least two units from Week 13 to Week 24 compared to Week 12 prior to randomization
- Secondary endpoint:
  - Proportion of subjects with ≥33% reduction from baseline in RBC transfusion burden during weeks 37 to 48
  - ≥50% reduction from baseline in RBC transfusion burden during weeks 13 to 24
  - ≥50% reduction from baseline in RBC transfusion burden during weeks 37 to 48
  - Mean change from baseline in RBC transfusion burden during weeks 13 to 24

Efficacy Results:

		Luspatercept (n=224)	Placebo (n=112)	Odds Ratio (CI)	P value
Primary Endpoint	≥33% reduction in blood transfusion in Week 13-24	21.4% (n=48)	4.5% (n=5)	5.79 (2.24-14.97)	<0.0001
Secondary Endpoints	≥33% reduction in blood transfusion in Week 37-48	19.6%	3.6%	6.44 (2.27-18.26)	<0.0001
	≥50% reduction in blood transfusion in Week 13-24	7.6%	1.8%	4.55 (1.03-20.11)	0.0303
	≥50% reduction in blood transfusion in Week 37-48	10.3%	0.9%	11.92 (1.65-86.29)	0.0017

- Mean change in transfusion burden from baseline to weeks 13-24: -1.35 RBC units/12 weeks (95% CI -1.77 to -0.93; P<0.001)

Safety Results:

Adverse Event	Luspatercept	Placebo
Bone pain	19.7%	8.3%
Arthralgia	19.3%	11.9%
Cough	14.3%	11%
Dizziness	11.2%	4.6%

Hypertension	1.8%	0%
Thromboembolic events	3.6%	0.9%

- GRADE evidence rating: C
    - Strengths: Multicenter study from 15 countries, double-blinded, randomized, placebo-controlled
    - Limitations: Uncertain if threshold of 33% reduction of transfusion burden is associated with long-term benefit

Myelodysplastic syndrome (MDS)

MDS is a heterogenous group of hematopoietic stem/progenitor cell (HSPC) disorders characterized by poor marrow cell function. This syndrome leads to ineffective hematopoiesis, cytopenias, increased risk of transformation to acute myeloid leukemia and reduced survival.[10,11] Myelodysplastic syndromes also referred to as myelodysplastic neoplasms (MDS) in the 5th edition of the World Health Organization (WHO) Classification of Haematolymphoid Tumours.[22]

Two commonly used classification systems for MDS include the International Consensus Classification (ICC)[24] and the World Health Organization 5th edition (WHO5)[22]. Classification considers cytogenetic and molecular abnormalities, bone marrow blast count and dysplasia (≥10% for any hematopoietic lineage).

- Disease-typing based on the ICC classifies MDS according to either specific cytogenetic or karyotypic abnormalities, the degree of dysplasia, or presence of excess blasts. The WHO classification system considers either delineating by genetic abnormalities or morphology.
- Treatment of MDS is based on prognostic scoring systems.[11,14] MDS risk scoring is often done using the Revised International Prognostic Scoring System (IPSS-R). This score considers cytogenetics, bone marrow blasts, hemoglobin, platelets, and absolute neutrophil count. The score predicts median survival, and time to disease progression to AML.[14] Patients with MDS are classified into five risk groups based on this risk score (very low, low, intermediate, high, very high).[11,14]
    - High to very-high risk MDS requires intensive therapy with allogenic hematopoietic stem cell transplant, immune-modulating therapies, or enrollment in clinical trials
    - Very low to intermediate risk MDS is often treated with a disease management strategy rather than intensive therapy with a curative goal; focus on preventing bleeds/infections and treating anemia.[11] Around 40% of low-risk MDS patients develop transfusion dependent anemia.[20,21]
    - The National Comprehensive Cancer Network (NCCN) Guidelines for Myelodysplastic Syndromes[14] give recommendations for the treatment of symptomatic anemia in lower-risk disease. Treatment recommendations

consider cytogenetic abnormalities such as deletion 5q, serum erythropoietin level and ring sideroblasts to help guide treatment.
- Neither luspatercept nor imetelstat have been studied in deletion 5q [del(5q)] cytogenic abnormalities. Lenalidomide is usually the preferred agent for those with del(5q) subtype.

Revised international prognostic scoring system (IPSS-R) for myelodysplastic syndromes prognostic risk categories and scores.[16]

IPSS-R Risk Score Prognosis*

Risk Category	Risk Scores	Median survival in years without treatment	25% AML progression in years without treatment
Very low	≤ 1.5	8.8	Not reached
Low	> 1.5-3	5.3	10.8
Intermediate	> 3-4.5	3	3.2
High	> 4.5-6	1.6	1.4
Very high	> 6	0.8	0.7

*Adapted from NCCN[14]

The pivotal trial used for the FDA approval of luspatercept was a double-blind, placebo-controlled phase 3 randomized clinical trial. Eligible patients were adults very low to intermediate risk MDS-RS requiring RBC transfusions (at least 2 units per 8 weeks) and had failed or were unlikely to respond to ESAs (owing to an endogenous erythropoietin level of >200 IU/L).

- Intervention: Patients were randomized in a 2:1 ratio to luspatercept or placebo SQ every three weeks for 24 weeks. Luspatercept was dosed initially at 1 mg/kg but could be titrated to 1.33 mg/kg and then 1.75 mg/kg.
- Primary outcome: transfusion independence for at least eight weeks during the study period
- Key secondary endpoint: transfusion independence for at least 12 weeks.
- Results: A total of 229 patients were enrolled in the trial
  - Significantly more patients in the luspatercept met the primary outcome than in the placebo group (38 vs 13%; p<0.001).
  - More patients in the luspatercept group met the key secondary endpoint (28 vs 8%; p<0.001).
- Safety: The most common adverse events (luspatercept vs placebo) were fatigue (27 vs 13%), diarrhea (22 vs 9%), asthenia (20 vs 12%), nausea (20 vs 8%), dizziness (20 vs 5%), and back pain.
  - 31% of patients receiving luspatercept and 30% of patients receiving placebo experienced a serious adverse event.[187]

Luspatercept was studied in ESA- naïve patients with very low to intermediate risk MDS in the COMMANDS trial of 356 patients randomized 1:1 to luspatercept or epoetin alfa. Key eligibility criteria included adult patients who were ESA-naïve with endogenous serum EPO levels of < 500 U/L and who required regular RBC transfusions (2–6 packed red blood cell units per 8 weeks for ≥8 weeks). Patients were stratified by baseline red blood cell transfusion burden (<4 units per 8 weeks vs ≥4 units per 8 weeks), endogenous serum erythropoietin concentration (≤200 U/L vs >200 to <500 U/L), and ring sideroblast status (positive [~73%] vs negative)[1,15].

- Primary endpoint: proportion of patients who experienced both red blood cell transfusion independence (defined as the absence of any RBC transfusion during any consecutive 12-week period) and an associated concurrent mean improvement in hemoglobin by at least 1.5 g/dL for any consecutive 12 week period during Weeks 1-24
- Results: 86 (59%) of 147 patients in the luspatercept group and 48 (31%) of 154 patients in the epoetin alfa group reached the primary endpoint (common risk difference on response rate 26.6; 95% CI 15.8–37.4; p<0·0001)
- In the ring sideroblast population, responses were 65% with luspatercept versus 26% with epoetin alfa and in the ring sideroblast-negative population responses were similar between treatment arms, 41% with luspatercept versus 46% for epoetin alfa.

Imetelstat approval was supported by a phase 3 randomized, double-blind, placebo-controlled IMerge trial of 178 patients with low-to-intermediate risk non del(5q) MDS. Participants must have been transfusion-dependent (requiring ≥ 4 red blood cell (RBC) units over an 8-week period during the 16 weeks prior to randomization) and must have failed to respond or have lost response or be ineligible for erythropoiesis-stimulating agents. This trial showed that imetelstat improved RBC transfusion independence in patients with low- to intermediate-1 risk MDS who required four or more red blood cell units over eight weeks who had not responded to or had lost response to or were ineligible for erythropoiesis-stimulating agents.

- Primary endpoint of RBC transfusion independence for at least eight weeks
    - 40% (95% CI: 30.9 to 49.3) patients in the imetelstat group vs.15% (95% CI: 7.1 to 26.6) in the placebo group [rate difference 25%, 9.9 to 36.9; p=0.0008]
- Secondary endpoint of RBC transfusion independence for at least 24 weeks
    - 33% (95% CI: 20.1 to 37.0) patients in the imetelstat group vs. 2% (95% CI: 0.4-11.5) patients in the placebo group [rate difference 25%; 12.6 to 34.2; p=0.001]
- The median duration of RBC transfusion independence was 51.6 weeks (95% CI 26.9 to 83.9) vs 13.3 weeks (8.0 to 24.9) in the placebo group (HR 0.23, 0.09 to 0.57).

- No statistically significant improvement in fatigue or other anemia-related symptoms were found during the trial.
- Safety: Grade 3 and Grade 4 cytopenias occurred during the IMerge trial. Imetelstat had significantly higher rates of both thrombocytopenia and neutropenia compared to placebo: thrombocytopenia (65% versus 8%), neutropenia (71% versus 7%). In the IMerge trial, high rates of grade 3/4 reactions in treatment arm did not result in higher rates of clinical sequelae such as infections or bleeding events. These cytopenias were most often managed with dose delays and dose reductions

Currently there is no direct comparison between luspatercept (Reblozyl) and imetelstat (Rytelo). Differences in study designs and patient populations make indirect comparisons difficult. The Institute for Clinical and Economic Review did do an indirect comparison of the two medications in the ring-sideroblast positive (RS+) subpopulation. They found no evidence for superiority in efficacy, i.e., reductions in RBC transfusion or improvements in quality of life, for imetelstat compared to luspatercept. Imetelstat was found to be more costly than luspatercept over the lifetime horizon.[21]

2024 Institute for Clinical and Economic Review (ICER) report on imetelstat for anemia in myelodysplastic syndrome[21]:

- Clear benefit in reducing RBC transfusions; however, improvement in fatigue is modest with high rates of grade 3 and grade 4 adverse events.
- Inconclusive net benefit of imetelstat compared to best supportive care and insufficient evidence compared to luspatercept for ring sideroblast (RS+) subgroup
- Current list price exceeds commonly used threshold for cost-effectiveness (69% – 74% price decrease to reach cost-effectiveness price thresholds of $94,800–$113,000 per year)

BILLING GUIDELINES AND CODING:

HCPCS	Coding Description	Brand Name
J0870	Injection, imetelstat, 1 mg	Rytelo
J0896	Injection, luspatercept-aamt, 0.25 mg	Reblozyl
ADMINISTRATION CODES◊		
96372	Ther/proph/diag inj sc/im	
96413	Chemo iv infusion 1 hr	
96415	Chemo iv infusion addl hr	
96417	Chemo in infus each addl seq	

*Coding Notes:

• The above code list is provided as a courtesy and may not be all-inclusive. Inclusion or omission of a code from this policy neither implies nor guarantees reimbursement or coverage. Some codes may not require routine review for medical necessity, but they are subject to provider contracts, as well as member benefits, eligibility and potential utilization audit.
• HCPCS/CPT code(s) may be subject to National Correct Coding Initiative (NCCI) procedure-to-procedure (PTP) bundling edits and daily maximum edits known as "medically unlikely edits" (MUEs) published by the Centers for Medicare and Medicaid Services (CMS). This policy does not take precedence over NCCI edits or MUEs. Please refer to the CMS website for coding guidelines and applicable code combinations.

REFERENCE/RESOURCES:

1. Relevant package inserts.
2. Luspatercept. In: Lexi-Drugs Online [Internet database]. Hudson, OH: Lexi-Comp, Inc. Updated periodically. Accessed November 6, 2025.
3. Fibach E, Rachmilewitz EA. Pathophysiology and treatment of patients with beta-thalassemia—an update. *F1000Res*. 2017;6:2156.
4. Galanello R, Origa R. Beta-thalassemia. *Orphanet J Rare Dis*. 2010;5:11.
5. ClinicalTrials.gov. An efficacy and safety study of luspatercept (ACE-536) versus placebo in adults who require regular red blood cell transfusions due to beta (β) thalassemia (BELIEVE). https://clinicaltrials.gov/ct2/show/NCT02604433 (accessed November 6, 2025).
6. Sun D, Abraham I. Cost of allogeneic blood transfusion. *World J Hematol*. 2012;1(3):8-13.
7. Weiss M, Parisi Jun M, Sheth S. Clinical and economic burden of regularly transfused adult patients with β-thalassemia in the United States: a retrospective cohort study using payer claims. *Am J Hematol*. 2019;94(5):E129-132.
8. Piga A, Perotta S, Gamberini MR et al. Luspatercept improves hemoglobin levels and blood transfusion requirements in a study of patients with β-thalassemia. *Blood*. 2019;133(12):1279-89.
9. Cappellini MD, Viprakasit V, Taher A et al. The BELIEVE trial: results of a phase 3, randomized, double-blind, place-controlled study of luspatercept in adult beta-thalassemia patients who require regular red blood cell (RBC) transfusions. Presented at the 60th Annual Meeting of the American Society of Hematology (ASH); December 1-4, 2018; San Diego, CA. (accessed 2019 Nov 29).
10. Kubasch AS and Platzbecker U. Setting fire to ESA and EMA resistance: New targeted treatment options in lower risk myelodysplastic syndromes. International journal of molecular sciences, 2019.
11. Hellström-Lindberg E, et al. A validated decision model for treating the anaemia of myelodysplastic syndromes with erythropoietin+ granulocyte colony-stimulating factor: significant effects on quality of life. British journal of haematology, 2003.

12. Fenaux P, et al. Luspatercept for the treatment of anemia in myelodysplastic syndromes and primary myelofibrosis. *Blood*, 2019.
13. Fenaux P, et al. Luspatercept in Patients with Lower-Risk Myelodysplastic Syndromes. *New Engl J Med*, 2020.
14. National Comprehensive Cancer Network (NCCN). NCCN Clinical Practice Guidelines in Oncology: Myelodysplastic Syndromes, version 1.2026.
15. Platzbecker U, Della Porta MG, Santini V, *et al*. Efficacy and safety of luspatercept versus epoetin alfa in erythropoiesis-stimulating agent-naive, transfusion-dependent, lower-risk myelodysplastic syndromes (COMMANDS): interim analysis of a phase 3, open-label, randomised controlled trial. Lancet. 2023 Jul 29;402(10399):373-385.
16. Greenberg PL, Tuechler H, Schanz J, *et al*. Revised international prognostic scoring system for myelodysplastic syndromes. Blood. 2012 Sep 20;120(12):2454-65. doi: 10.1182/blood-2012-03-420489.
17. Farmakis D, Porter J, Taher A, Domenica Cappellini M, Angastiniotis M, Eleftheriou A. 2021 Thalassaemia International Federation Guidelines for the Management of Transfusion-dependent Thalassemia. Hemasphere. 2022 Jul 29;6(8):e732.
18. Association of Public Health Laboratories (U.S.);Centers for Disease Control and Prevention (U.S.). Hemoglobinopathies: current practices for screening, confirmation and follow-up. December 2015. Available from https://stacks.cdc.gov/view/cdc/42387 (accessed November 6, 2025).
19. Platzbecker U, Santini V, Fenaux P, et al. Imetelstat in patients with lower-risk myelodysplastic syndromes who have relapsed or are refractory to erythropoiesis-stimulating agents (IMerge): a multinational, randomised, double-blind, placebo-controlled, phase 3 trial. Lancet. 2024 Jan 20;403(10423):249-260. Epub 2023 Dec 1. Erratum in: Lancet. 2024 Jan 20;403(10423):248.
20. Food and Drug Administration. Center for Drug Evaluation and Research. Application number: 217779Orig1s000 Multi-discipline review. Available at https://www.accessdata.fda.gov/drugsatfda_docs/nda/2024/217779Orig1s000TOC.cfm (accessed November 6, 2025).
21. Tice JA, Luu L, Fahim SM, et al. Imetelstat for Anemia in Myelodysplastic Syndrome: Effectiveness and Value, Evidence Report. Institute for Clinical and Economic Review, July 2, 2024. Available from https://icer.org/assessment/myelodysplastic-syndrome-2024/ (accessed November 6, 2025)
22. Khoury JD, Solary E, Abla O, et al. The 5th edition of the World Health Organization Classification of Haematolymphoid Tumours: Myeloid and Histiocytic/Dendritic Neoplasms. Leukemia. 2022 Jul;36(7):1703-1719.
23. Food and Drug Administration. FDA Briefing Document. NDA # 217779. Oncology Drugs Advisory Committee Meeting. Available at

https://www.fda.gov/media/176966/download (accessed August 23, 2024)
24. Arber DA, Orazi A, Hasserjian RP, et al. International Consensus Classification of Myeloid Neoplasms and Acute Leukemias: integrating morphologic, clinical, and genomic data. Blood. 2022 Sep 15;140(11):1200-1228.