

Imetelstat (RYTELO)

Criteria for Use

July 2025

VA Pharmacy Benefits Management Services and National Formulary Committee

The following recommendations are based on medical evidence, clinician input, and expert opinion. The content of the document is dynamic and will be revised as new information becomes available. The purpose of this document is to assist practitioners in clinical decision-making, to standardize and improve the quality of patient care, and to promote cost-effective drug prescribing. THE CLINICIAN SHOULD USE THIS GUIDANCE AND INTERPRET IT IN THE CLINICAL CONTEXT OF THE INDIVIDUAL PATIENT. INDIVIDUAL CASES THAT ARE EXCEPTIONS TO THE EXCLUSION AND INCLUSION CRITERIA SHOULD BE ADJUDICATED AT THE LOCAL FACILITY ACCORDING TO THE POLICY AND PROCEDURES OF ITS P&T COMMITTEE AND PHARMACY SERVICES.

The Product Information should be consulted for detailed prescribing information.

Exclusion Criteria

If ANY of the following are selected, the patient will NOT meet criteria for imetelstat.

- Concurrent use of erythropoiesis stimulating agents (ESAs)
- Platelet count less than $75 \times 10^9/L$
- Absolute neutrophil count less than $1.5 \times 10^9/L$ unless with Duffy Null phenotype
- Severe hepatic impairment defined as total bilirubin $> 3x$ upper limit of normal (ULN) unless with Gilbert syndrome
- Severe renal impairment defined as serum creatinine < 30 ml/min
- Deletion 5q (del[5q]) karyotype
- Myelodysplastic syndromes / myeloproliferative neoplasm overlap syndrome
- Known pregnancy
- Lactation

Inclusion Criteria

All the following criteria must be selected to meet criteria.

- Confirmed diagnosis of low- to intermediate-1 risk myelodysplastic syndrome (MDS)
- Transfusion-dependent, requiring regular red blood cell (RBC) transfusions of ≥ 4 RBC units over 8 weeks
- Documentation of inadequate response, loss of response, intolerance or contraindication to an 8-week course of an **erythropoiesis stimulating agent (ESA)**
- Documentation of inadequate response, loss of response, intolerance or contraindication to a 9-week course of **luspatercept-aamt** at maximal tolerated doses (i.e. 1.75 mg/kg subcutaneously every 3 weeks)

Additional Inclusion Criteria – Select If Applicable

- For females who can become pregnant: Pregnancy should be excluded prior to receiving imetelstat.
- For females who can become pregnant: Counseling provided on potential risks vs. benefits of treatment and the use of effective contraception during therapy and for 1 week after stopping treatment.
- For lactating females: Advise against breastfeeding during treatment and for 1 week after the last dose

Other Justification
