Real-World Outcomes With Early Adoption of Imetelstat in Patients With Myelodysplastic Syndromes: Insights From a United States Community Oncology Chart Review

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Background

- Imetelstat is approved for select patients (pts) with lower-risk myelodysplastic syndromes (MDS) and red blood cell (RBC) transfusion-dependent anemia after erythropoiesis-stimulating agent (ESA) failure, ineligibility, or relapse[1,2]

We evaluated early real-world (RW) treatment (tx) patterns and hematologic outcomes in pts with MDS receiving imetelstat in community oncology settings

Methods

- **Design:** Retrospective electronic health record–based chart review of 30 eligible adults with MDS treated with imetelstat in US community oncology practices (**Table 1**)
- **Hemoglobin (Hb) outcomes/sensitivity:** Baseline (BL) Hb was a minimum of ≤8 weeks pre-index; post-index Hb/change from BL were assessed through tx plus a 30- to 45-day grace period, excluding Hb values ≤14 days after transfusion
- **RBC transfusion independence:** Exploratory; no chart-documented RBC transfusion over any ≥8-week post-index interval
- **Thrombocytopenia/Neutropenia:** Defined by platelet/absolute neutrophil count (ANC) labs and graded per CTCAE, version 6.0
- **Statistics:** All analyses were descriptive

Results

Tx Patterns

- Imetelstat was initiated in heavily pretreated pts (**Table 1**) at a median 42.6 months after MDS diagnosis; median BL Hb was 7.7 g/dL

Hb Outcomes (Figure 1)

- Median Hb increased by 2.3-2.7 g/dL, with peak Hb 9.0-9.3 g/dL; findings were consistent across sensitivity and ≥2 dose analyses

Exploratory RBC Transfusion Outcomes

- A chart-documented ≥8-week transfusion-free interval was observed in 73.3% of pts

Early Cytopenia Pattern

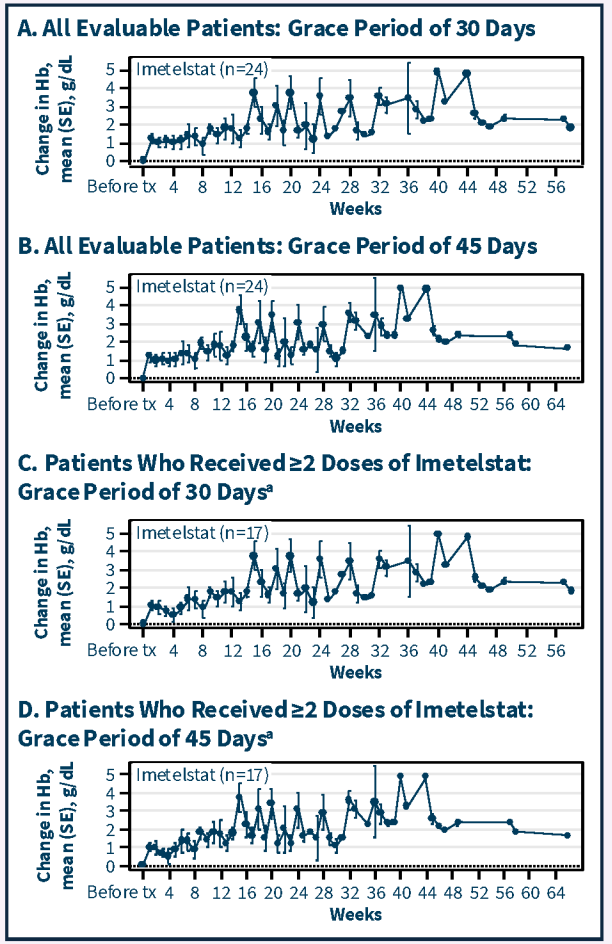
- At ~60 days, grade 3/4 cytopenias were more common among pts with low BL counts (neutropenia: 27.8% with ANC <1.5×10^9/L vs 5.6% with ANC ≥1.5×10^9/L; thrombocytopenia: 30.8% with platelets ≤150×10^9/L vs 7.7% with platelets >150×10^9/L)
- Cytopenias were managed with tx interruption/dose modification; 20% of pts discontinued tx

Table 1. Baseline Demographic and Clinical Characteristics

	Imetelstat (N=30)
Age group at index, n (%), y	
≤75	7 (23.3)
>75	23 (76.7)
Sex, n (%)	
Female	7 (23.3)
Male	23 (76.7)
Cytogenetic abnormality at diagnosis, n (%)[a]	
del(5q)	4 (13.3)
Other	26 (86.7)
RS status, n (%)[a]	
RS positive	12 (40.0)
RS negative	13 (43.3)
IPSS-R risk category at diagnosis (physician documentation), n (%)[a]	
Intermediate (>3.0 to ≤4.5 points)	3 (10.0)
High (>4.5 to ≤6.0 points)	14 (46.7)
Hb, g/dL, median	7.7
ANC, ×10^9/L, n (%)[a]	
≥1.5	18 (60.0)
≥1.0 to <1.5	6 (20.0)
≥0.5 to <1.0	<3 (<10.0)
<0.5	<3 (<10.0)
Platelet count, ×10^9/L, n (%)	
>150	13 (43.3)
100 to 150	7 (23.3)
75 to <100	<3 (<10.0)
50 to <75	3 (10.0)
25 to <50	0
<25	3 (10.0)
Serum EPO, n (%), mU/mL[a]	
<200	<3 (<10.0)
200 to 500	7 (23.3)
>500	9 (30.0)
Prior therapy, %	
ESA	100
Luspatercept	72.4
HMA	52.6
Lenalidomide	28.6
≥2 prior lines of therapy at initiation	83.3

ANC, absolute neutrophil count; EPO, erythropoietin; ESA, erythropoiesis-stimulating agent; Hb, hemoglobin; HMA, hypomethylating agent; IPSS-R, Revised International Prognostic Scoring System; RS, ring sideroblast.
[a]The remaining of the 30 patients were not documented.

Figure 1. Hb Change Over Time in Patients Pre- and Post-Index Hb Data Treated With Imetelstat Excluding Values ≤14 Days of Transfusion



Hb, hemoglobin; tx, treatment.
[a]Hb values at later post-index time points reflect the subset of patients with longer imetelstat exposure and available laboratory follow-up. The maximum observed imetelstat treatment duration was 57 weeks, with Hb follow-up extending beyond treatment during the pre-specified period.

Exploratory Analysis in Pts With del(5q) Disease

- Four pts with del(5q) MDS previously exposed to lenalidomide, ESAs, and luspatercept had a median Hb increase of 1.85 g/dL ≤45 days post-index, excluding Hb values ≤14 days after transfusion; median peak Hb was 8.7 g/dL
- No chart-documented RBC transfusions occurred during any ≥8-week post-index interval

Study Highlights

Evidence Gap

Early post-approval RW evidence for imetelstat is limited, particularly in community oncology settings that often differ from clinical trial settings

Main Finding

In an older, heavily pretreated community-practice population, imetelstat was associated with a consistent Hb improvement signal despite short tx exposure and later-line use

Effectiveness Signal

Hb improvement was consistent across analytic definitions, including analyses that excluded Hb values obtained within 14 days of transfusion

Practice Implications

The preserved Hb signal supports continued evaluation of imetelstat benefit in routine practice while reinforcing the need for early cytopenia monitoring and timely dose modification to help appropriate pts remain on therapy

References

1. RYTELO® (imetelstat) for injection, for intravenous use. Package insert. Geron Corporation; 2024.
2. RYTELO® imetelstat summary of product characteristics. Geron Corporation; 2025.

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