# The Role of Erythropoietin Stimulating Agents (ESAs) in the Treatment Landscape of Myelodysplastic Syndrome (MDS)

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## Background

- Myelodysplastic syndromes (MDS) are a family of myeloid cancers with diverse genotypes and phenotypes characterized by ineffective hematopoiesis and risk of transformation to acute myeloid leukemia (AML).[1]
- It is estimated that 10,000 people in the United States are diagnosed annually with MDS, with the prevalence more common amongst those greater than 70 years of age.[2]
- Anemia is the primary cytopenia observed in lower-risk MDS, with standard treatment consisting of red blood cell transfusions and ESA therapy.[3,4]

## Objectives

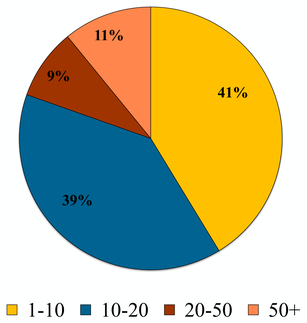
- To elucidate the unique role of ESAs in the treatment landscape of MDS, with a specific emphasis on understanding treatment failure with ESAs.
- To evaluate how practices treating low-risk MDS patients determine ESA treatment needs, average time to treatment failure, and future treatment direction following ESA treatment failure.

## Methods

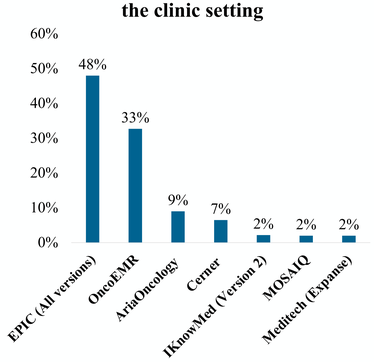
- A survey conducted from September 16, 2022, through December 12, 2022. The surveys were shared via member emails, a poll during an NCODA International Monthly Webinar and posted within all NCODA CONNECT member communities.
- Following the surveys, subgroup analyses were performed to determine electronic medical record (EMR) systems used by the various institutions, the preferred ESA used in the treatment of MDS patients, criteria used to determine lack of treatment responses and the average time to treatment failure with ESA therapy.

## Results

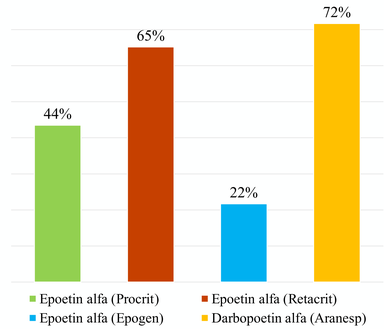
**Number of MDS patients treated on a monthly basis**



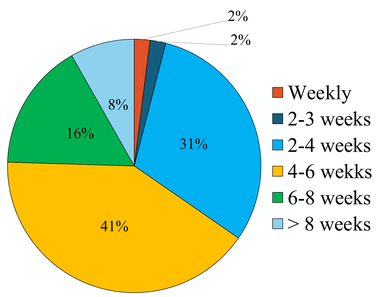

**EMR System currently used in the clinic setting**



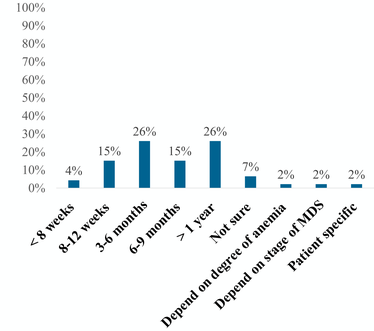

**ESA agent used to treat anemia associated with MDS**



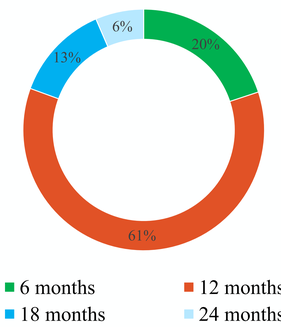

**Timeframe to assess for ESA response once given**




**Average time an MDS patient is treated with an ESA**




**MDS patients average time on ESA treatment before treatment failure**




- All 51 practice sites use ESAs to treat MDS associated anemia.
- 48/51 practice sites assess ESA response as both ≥ 1.5 g/dL rise in hemoglobin (Hgb) and a decrease in red blood cell (RBC) transfusion.
  - –2/51 sites assess ESA response only as ≥ 1.5 g/dL rise in Hgb
  - –1/51 sites assess response only as decrease in red blood cell (RBC) transfusion
- 29/46 sites define a lack of ESA response as lack of rise of 1.5 g/dL rise in hemoglobin or lack of a decrease in RBC transfusion requirement by 3-6 months of treatment and 17/46 sites define lack of response as no rise in hemoglobin by 6-8 weeks of treatment.
- Target hemoglobin goal was any target 10-11 g/dL for 25/46 practice sites, 1/46 sites had a goal of 8-10 g/dL, 18/46 sites had a goal of 10 g/dL, 1/46 sites had a goal of 11 g/dL, and 1/46 sites had a target of primarily monitoring for decrease in transfusion burden.
- 24/46 practice sites are interested in participating with NCODA in a project focused on ESA response in MDS patients.

## Discussion

- The 51 respondents are from different practice sites in the United States. Some respondents did not answer all the questions of the survey.
- On average, MDS patients were treated with ESAs for ≥ 3 months (67%) and response assessed at 4-6 weeks (41%)
- 52% of respondents agreed to participate in NCODA's project evaluating ESA responses in MDS patients.
- Epic (all versions) (48%) and OncoEMR (33%) were the most used EMR systems among respondents.
- The average time to ESA treatment failure was 12 months.
- Lack of ESA response was defined as a lack of rise of 1.5 g/dL in hemoglobin or no decrease in RBC transfusion requirement by 3-6 months of treatment by 63% of respondents. In contrast, 37% percent defined lack of response as no rise in hemoglobin at 6-8 weeks.

## Conclusion

- The survey results reflect current guidelines in treating MDS patients with ESAs
- Most practices surveyed define lack of ESA response with both Hgb levels and RBC transfusions, which is currently the standard of care.
- Most patients show lack of response to ESA therapy between 6-12 months - finding a way to easily track this information within the EMR system would be helpful in identifying MDS patients who require next steps in therapy.
- The NCODA PQI in Action published in alignment with this topic "Erythropoietin Stimulating Agent Ineligibility in MDS" offers further guidance in this treatment landscape and includes insights from 2 NCODA member practices.

## References

1. Li H, Hu F, Gale RP, Sekeres MA, Liang Y. Myelodysplastic syndromes. *Nat Rev Dis Primers*. 2022;8(1):74. Published 2022 Nov 17.
2. *Key statistics for myelodysplastic syndromes*. American Cancer Society. (n.d.). https://www.cancer.org/cancer/types/myelodysplastic-syndrome/about/key-statistics.html
3. Steensma DP, Bennett JM. The myelodysplastic syndromes: diagnosis and treatment. *Mayo Clin Proc*. 2006;81(1):104-130.
4. Fenaux P, Adès L. How we treat lower-risk myelodysplastic syndromes. *Blood*. 2013;121(21):4280-4286.