MDS patient characteristics associated with use of disease-modifying therapy: results of a patient survey

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INTRODUCTION

- Annual MDS incidence is estimated at 30 per 100,000 persons ages 70 and older.[1]
- As MDS progresses, most patients experience worsening cytopenias that may lead to life-threatening complications such as infection and bleeding.[2,3]
- 1st-line treatment of intermediate to high-risk MDS patients with hypomethylating agents (HMAs) has been demonstrated to improve survival and/or delay progression.
- This work represents results of one patient survey conducted to evaluate patient-reported use of disease-modifying therapy (DMT).

METHODS

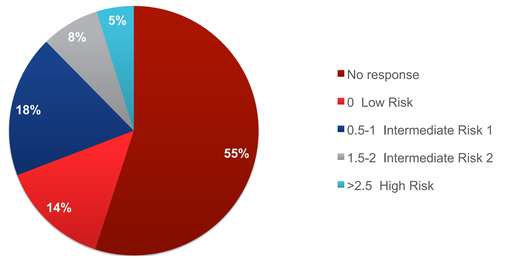
- Sponsor: MDS Foundation, Inc.
- Study design: One-time, web-based questionnaire of MDS patients
- Length of study: Responses were collected from July 2013 to June 2014
- Analysis:
  - Descriptive statistics were conducted for the following parameters:
    - Patient and disease characteristics
    - International Prognostic Scoring System (IPSS) risk
    - Overall mean QoL according to published FACT-G scoring algorithms (scale: 0 - 108)
    - Bone marrow biopsy (BMB) and DMT history
  - Statistical t-tests were used to evaluate the quality of life-DMT relationship
  - Responses to each question were voluntary, therefore the total number of respondents to each item varies; results exclude missing data

RESULTS

Demographics

- N = 727 patients
- Mean patient age = 68 years
- Mean FACT-G score = 73.1 (scale: 0 - 108)
- 47% of responders were female
- 19% of responders claimed full or part-time employment
- Less than half of respondents (46%) had been diagnosed for <3 years
- 43% of patients' primary insurance provider was Medicare
- Only 45% of patients reported knowledge of their IPSS risk score, of these:
  - 72% were lower risk (IPSS "low" and "intermediate 1")
  - 28% were higher risk (IPSS "intermediate 2" and "high")

Figure 1. Patient-reported IPSS Risk Score (N=727)



RESULTS (Cont.)

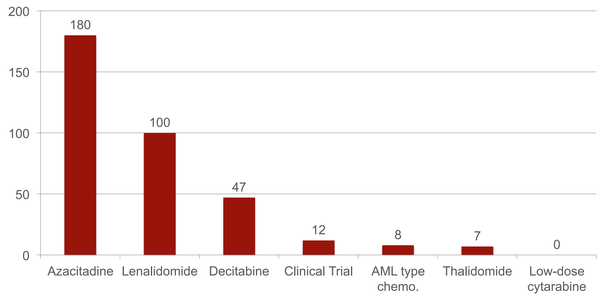
Table 1. MDS Patient Demographics (N=727)

Parameter	N (%)	Parameter	N (%)
Gender		**Time since diagnosis (years)**	
Female	234 (32.2)	10 or more years	39 (5.4)
Male	268 (36.9)	6 to <10 years	74 (10.2)
No response	225 (30.9)	3 to <6 years	126 (17.3)
Age		1 to <3 years	186 (25.6)
00-54	52 (7.2)	<1 year	145 (19.9)
55-64	85 (11.7)	No response	157 (21.6)
65-74	237 (32.6)	**Current employment status**	
75+	128 (17.6)	Disability	51 (7.0)
No response	225 (30.9)	Employed full-time	60 (8.3)
Primary Insurance Carrier		Employed part-time	37 (5.1)
Medicare	309 (42.5)	Retired	316 (43.5)
Private Insurance	137 (18.8)	Unemployed	16 (2.2)
Other	72 (9.9)	Other	18 (2.5)
None	11 (1.5)	No response	229 (31.5)
No Response	198 (27.2)		

DMT

- Nearly 40% (286) of respondents had ever received DMT, of those:
  - More patients received azacitidine (63%) than decitabine (16%)
  - Only 12 (4.2%) patients participated in a clinical trial
  - Most (73%) received only 1 DMT
  - 22% received 2 or more types of DMT
  - A majority (63%) of patients were receiving DMT at the time of survey administration

Figure 2. Number of Patients Who Received Each DMT Type at Least Once (N=286)[a]



[a] Because some patients received more than one DMT, there is an overlap between treatment types.

IPSS Risk Score and DMT

- Of those that received DMT at any point prior to survey (N=286):
  - 31% were IPSS lower risk (low risk and intermediate risk 1)
  - 23% were IPSS higher risk (intermediate risk 2 and high risk)
  - 46% did not report a risk score
- Targeted MDS therapy was received by a greater proportion of higher risk patients (67/90) than lower risk patients (88/237)
- The most common DMT reported was azacitidine and lenalidomide in high-risk and low-risk patients, respectively

Figure 3. Number of DMT Ever Received, per Patient (N=286)

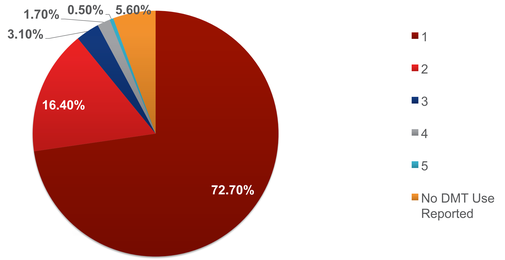
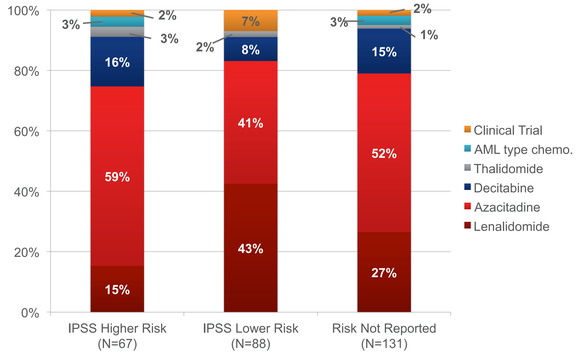


Table 2. Patient-reported DMT[a], by IPSS risk, N (%)

	All (N=286)	Higher-risk (N=67)	Lower-risk (N=88)	Risk not reported (N=131)
Azacitidine	180 (62.9)	54 (80.6)	41 (46.6)	85 (64.9)
Decitabine	47 (16.4)	15 (22.4)	8 (9.1)	24 (18.3)
Clinical trial medication	12 (4.2)	2 (3.0)	7 (8.0)	3 (2.3)
Immunomodulatory agents	107 (37.4)	17 (25.4)	45 (51.1)	45 (34.4)
Other[b]	8 (2.8)	3 (4.5)	0 (0)	5 (3.8)
Still receiving DMT	179 (62.6)	37 (55.2)	51 (58.0)	91 (69.5)

[a] Patients could report use of >1 DMT.
[b] Low-dose cytarabine, AML-type chemotherapy.

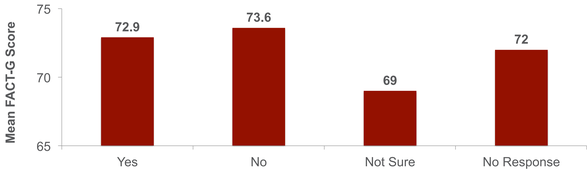
Figure 4. DMT Type by IPSS Risk Score



FACT-G Score

- Mean FACT-G for patients treated with DMT (72.9) was less than that of all patients 73.1 (p-value =0.671)
- Respondents still receiving DMT had a mean FACT-G score 2.08 greater than those that never received DMT treatment (P-value = 0.259)
- Patients who had received DMT (but were not using at time of survey) reported a mean FACT-G score 2.96 lower than those who never received DMT (p-value = 0.619)
- DMT use was not significantly associated with QoL (p=0.67)

Figure 5. Mean FACT-G Total Score in Patients Who Received DMT (P-value: 0.671)



Bone Marrow Biopsy

- 81% of patients received BMB at the time of MDS diagnosis
- However, just under half of patients reported having BMB performed since initial MDS diagnosis

Table 3. Bone Marrow Biopsy at, and Following, Diagnosis

	N (%)
BMB performed at diagnosis:	
Yes	587 (80.7)
No	17 (2.3)
Not sure	5 (0.7)
No response	118 (16.2)
BMB performed since diagnosis:	
Yes	352 (48.4)
No	254 (34.9)
Not sure	3 (0.4)
No response	118 (16.2)

CONCLUSION

- Less than half (40%) of MDS patients receive DMT.
- A small proportion of MDS patients (4%) participate in clinical trials.
- Our findings suggest that DMT use was not significantly associated with QoL (p=0.67).
- More research is needed to determine the barriers to DMT use and trial pazrticipation, and to demonstrate the value of adding more options to the existing therapeutic paradigm.

REFERENCES

1. Gracia-Manera G, List A, Kantarjian H, et al. Cancer Network. Myelodysplastic Syndromes. http://www.cancernetwork.com/cancer-management/mds Date published May 1 2014. Date accessed March 20, 2015.
2. Sekeres MA, Steenma DP. Defining prior therapy in myelodysplastic syndromes and criteria for relapsed and refractory disease: implications for clinical trial design and enrollment. Blood. 2009;114:2575-2580.
3. Greenberg PL. Current therapeutic approaches for patients with myelodysplastic syndromes. Br J Haematol. 2010;150:131-143.

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