INTRODUCTION

- Annual MDS incidence is estimated at 30 per 100,000 persons ages 70 and older.
- As MDS progresses, most patients experience worsening complications such as infection and bleeding.1
- Interim treatment of 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 (Std. according to published FACT-G scoring algorithm): (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

- Year = 727 patients
- Mean patient age = 68 years
- Mean FACT-G score = 73.1 (scale: 0 – 108)
- 47% of respondents were female
- 19% of respondents claimed full-time employment
- Less than half (46%) had been diagnosed for <3 years
- 43% of patients received any previous therapies
- Only 45% of patients reported knowledge of their IPSS risk score; of those:
  - 72% were lower risk (IPSS “low” and “intermediate 1”)
  - 28% were higher risk (IPSS “intermediate 2” and “high”)

Table 1. MDS Patient Demographics (N=727)

<table>
<thead>
<tr>
<th>Parameter</th>
<th>N (%)</th>
<th>Yes, ever since diagnosis (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age</td>
<td>Mean = 68.3</td>
<td></td>
</tr>
<tr>
<td>Gender</td>
<td>Female = 54%</td>
<td></td>
</tr>
<tr>
<td>Employment Status</td>
<td>Full-time = 47%</td>
<td></td>
</tr>
<tr>
<td>Time since diagnosis</td>
<td>&lt;3 years = 46%</td>
<td></td>
</tr>
<tr>
<td>Prior Therapy</td>
<td>Yes = 43%</td>
<td></td>
</tr>
<tr>
<td>IPSS Risk Score</td>
<td>Lower = 45%</td>
<td></td>
</tr>
</tbody>
</table>

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

DMT

- 40% (290) of respondents had ever received DMT, of those:
  - More patients received azacitidine (33%) than decitabine (16%)
  - Only 12.4% 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

Table 2. Patient-reported DMT*, by IPSS risk, N (%) (N=728)

<table>
<thead>
<tr>
<th>Parameter</th>
<th>0 (%)</th>
<th>1 (%)</th>
<th>2 (%)</th>
<th>3 (%)</th>
<th>4 (%)</th>
<th>5 (%)</th>
<th>Risk Not Reported</th>
<th>Total DMT (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>DMT</td>
<td>No response</td>
<td>Low Risk</td>
<td>S-1 Intern. Risk</td>
<td>S-2 Intern. Risk</td>
<td>High Risk</td>
<td>No response</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Azacitidine</td>
<td>180 (25)</td>
<td>54 (7.5)</td>
<td>47 (6.3)</td>
<td>268 (36.9)</td>
<td>72 (9.9)</td>
<td>26 (3.5)</td>
<td>11 (1.5)</td>
<td>658 (90.1)</td>
</tr>
<tr>
<td>Decitabine</td>
<td>128 (17.6)</td>
<td>39 (5.4)</td>
<td>47 (6.3)</td>
<td>268 (36.9)</td>
<td>72 (9.9)</td>
<td>26 (3.5)</td>
<td>11 (1.5)</td>
<td>658 (90.1)</td>
</tr>
<tr>
<td>Immunomodulatory agents</td>
<td>157 (21.6)</td>
<td>47 (6.3)</td>
<td>47 (6.3)</td>
<td>268 (36.9)</td>
<td>72 (9.9)</td>
<td>26 (3.5)</td>
<td>11 (1.5)</td>
<td>658 (90.1)</td>
</tr>
<tr>
<td>Clinical Trial</td>
<td>157 (21.6)</td>
<td>58 (7.6)</td>
<td>47 (6.3)</td>
<td>268 (36.9)</td>
<td>72 (9.9)</td>
<td>26 (3.5)</td>
<td>11 (1.5)</td>
<td>658 (90.1)</td>
</tr>
<tr>
<td>Bone Marrow Biopsy</td>
<td>157 (21.6)</td>
<td>58 (7.6)</td>
<td>47 (6.3)</td>
<td>268 (36.9)</td>
<td>72 (9.9)</td>
<td>26 (3.5)</td>
<td>11 (1.5)</td>
<td>658 (90.1)</td>
</tr>
<tr>
<td>Thalidomide</td>
<td>72 (9.9)</td>
<td>18 (2.5)</td>
<td>18 (2.5)</td>
<td>39 (5.4)</td>
<td>18 (2.5)</td>
<td>18 (2.5)</td>
<td>18 (2.5)</td>
<td>174 (24.0)</td>
</tr>
<tr>
<td>Low-dose cytarabine, AML-type chemo.</td>
<td>72 (9.9)</td>
<td>18 (2.5)</td>
<td>18 (2.5)</td>
<td>39 (5.4)</td>
<td>18 (2.5)</td>
<td>18 (2.5)</td>
<td>18 (2.5)</td>
<td>174 (24.0)</td>
</tr>
</tbody>
</table>

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

Conclusions

- More patients received Decitabine (16%) than other DMTs
- Thalidomide (16%) was the least common DMT received
- There was a higher proportion of patients receiving DMT in high-risk patients
- There was no significant association between IPSS risk score and DMT use

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

<table>
<thead>
<tr>
<th>Parameter</th>
<th>No (%)</th>
<th>Yes (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bone Marrow Biopsy</td>
<td>No Response</td>
<td>68</td>
</tr>
<tr>
<td>Diagnosis</td>
<td>No Response</td>
<td>72</td>
</tr>
</tbody>
</table>

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 participation, and to demonstrate the value of adding more options to the existing therapeutic paradigm.

REFERENCES


This research was funded by Onconova Therapeutics, Inc. and conducted by Partnership for Health Analytic Research, LLC.