

Patterns of Treatment Among Patients with Recently-Diagnosed Myelodysplastic Syndromes in a National Registry, 2006-2008

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Disclosures

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General Objective of Slone Epidemiology Center MDS Registry

Long term follow-up of recently-diagnosed patients with myelodysplastic syndromes (MDS) to provide “real world” information on clinical, economic, and quality of life outcomes

Overview of Methods - Enrollment

- *Patient-centered observational registry*
- Recruitment -- patients self-enroll
 - Materials provided in doctors' offices -- 381 physicians agreed to participate
 - Patients find registry and enroll via internet
 - 20% of enrollees have some “doctor involvement”
- Eligibility
 - Patient informed of diagnosis of MDS within past 4 months
 - No other criteria -- goal is nationally representative series of patients with respect to demographics, disease characteristics, and treatment

Overview of Methods - Data Collection

- **Self-administered questionnaires filled out by patient -- covers demographics, treatment, medical events, quality of life**
 - **Enrollment**
 - **Baseline -- sent after enrollment received**
 - **Follow-up every six months**
 - **Medical records**
 - **Requested at baseline and follow-up with release signed by patient**
 - **Abstracted by nurses at Slone Epidemiology Center**
 - **No physician case report forms**
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Inclusion of Subjects in the Analysis

Included: patients who, as of 10/31/08, had completed a baseline questionnaire ≤ 6 months since diagnosis (n=303)

Excluded: patients with MDS/MPD according to WHO criteria (n=14)

Final total: **289** subjects

Subset analysis based on MDS classification from medical records: **202** subjects

Treatment Information

Reported by patients on baseline questionnaire

Covers period from diagnosis to completion of the questionnaire -- median = **60 days** (range 6-180)

Characteristics of 289 MDS Patients

Characteristic	No.	(%)
Male	148	(51)
Female	141	(49)
<i>Median age (range)</i>	<i>66 yr (19-88)</i>	
Level of education*		
High school or less	50	(18)
Some college	105	(37)
College graduate	133	(46)
Race/ethnicity*		
Non-Hispanic white	262	(92)
Other	24	(8)

*1 patient had unknown education, and 3 were of unknown race/ethnicity.



Distribution of 289 MDS Patients According to Geographic Region Of Residence

Region	MDS		U.S. Population*
	No.	(%)	(%)
New England	18	(6)	(5)
Mid-Atlantic	32	(11)	(14)
South Atlantic	73	(25)	(18)
East North Central	32	(11)	(16)
West North Central	22	(8)	(7)
East South Central	16	(6)	(6)
West South Central	28	(10)	(11)
Mountain	26	(9)	(7)
Pacific	41	(14)	(15)
Other	1	(0.3)	(1)

*U.S. Census, 2000.

WHO Subtypes Based on Information Obtained from Medical Records of 202 MDS Patients at Baseline

Subtype*	No.	(% of Classified)
Refractory anemia (RA)	10	(7)
Refractory anemia with ringed sideroblasts (RARS)	8	(5)
Refractory cytopenia with multilineage dysplasia (RCMD)	42	(28)
Refractory cytopenia with multilineage dysplasia and ringed sideroblasts (RCMD-RS)	13	(9)
Refractory anemia with excess blasts-1 (RAEB-1)	36	(24)
Refractory anemia with excess blasts-2 (RAEB-2)	22	(14)
Myelodysplastic syndrome, unclassified (MDS-U)	9	(6)
MDS associated with isolated del (5q)	12 [†]	(8)
<i>SEC unable to classify / not found in record</i>	50	(25) [‡]

*Classification by SEC if possible, otherwise from record.

[†]8 additional patients had 5q- but did not meet the WHO criteria.

[‡]Percent of total.

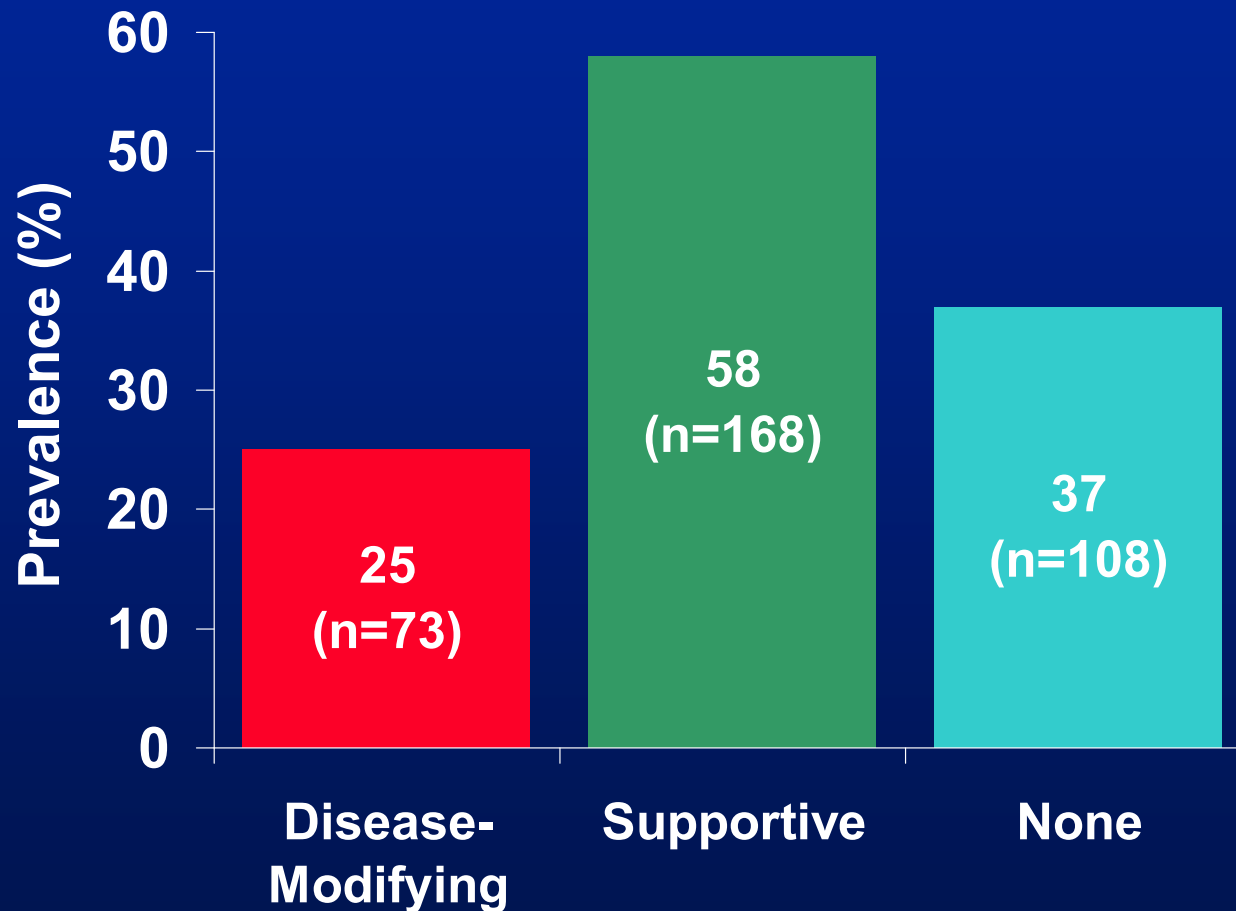
IPSS Risk Classification Based on Information Obtained from Medical Records of 202 MDS Patients at Baseline

Risk Level*	No.	(% of Classified)
Low	24	(14)
Intermediate-1	78	(46)
Intermediate-2	46	(27)
High	20	(12)
<i>SEC unable to classify / not found in record</i>	34	<i>(17)[†]</i>

*Classification by SEC if possible, otherwise from record.

[†]Percent of total.

Type of Treatment Among 289 Recently-Diagnosed MDS Patients



Specific Disease-Modifying Treatments Among 289 Recently-Diagnosed MDS Patients

Agent	No.	(%)
Azacitidine	29	(10)
Decitabine	21	(7)
Lenalidomide	18	(6)
Multiple drugs*	5	(2)

*During the period from diagnosis to completion of the baseline questionnaire, 2 patients reported taking azacitidine and lenalidomide, 2 decitabine and lenalidomide, and 1 decitabine and thalidomide.

Specific Supportive Therapies*

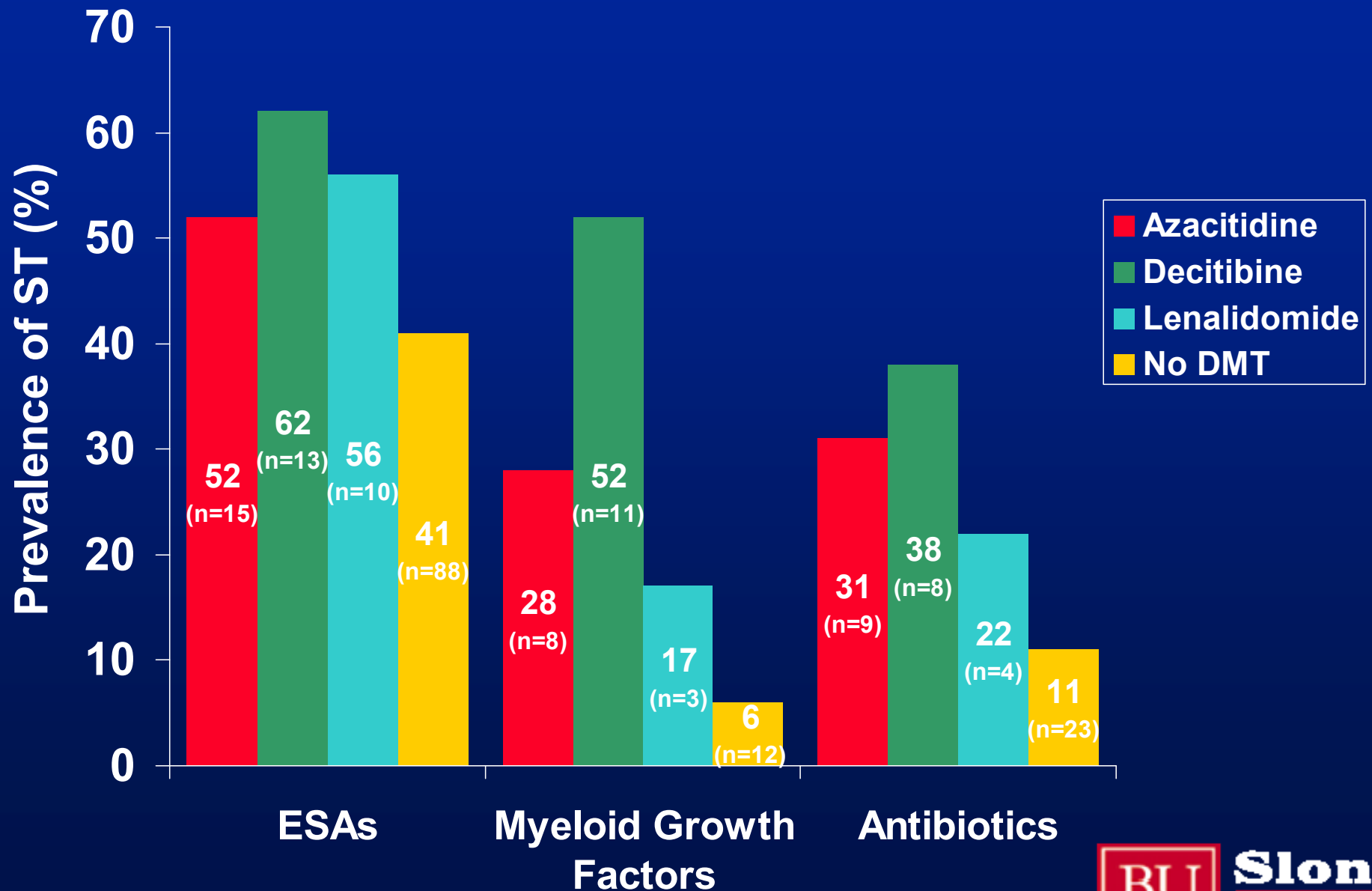
Among 289 Recently-Diagnosed MDS Patients

Agent	No.	(%)
ESAs	128	(44)
Darbepoetin	58	(20)
Epoetin	71	(25)
Myeloid growth factors	35	(12)
Filgrastim	27	(9)
Pegfilgrastim	9	(3)
Sargramostim	1	(0.3)
Iron chelators	4	(1)
ATG	4	(1)
Antibiotics	46	(16)
One or more other agents	37	(13)

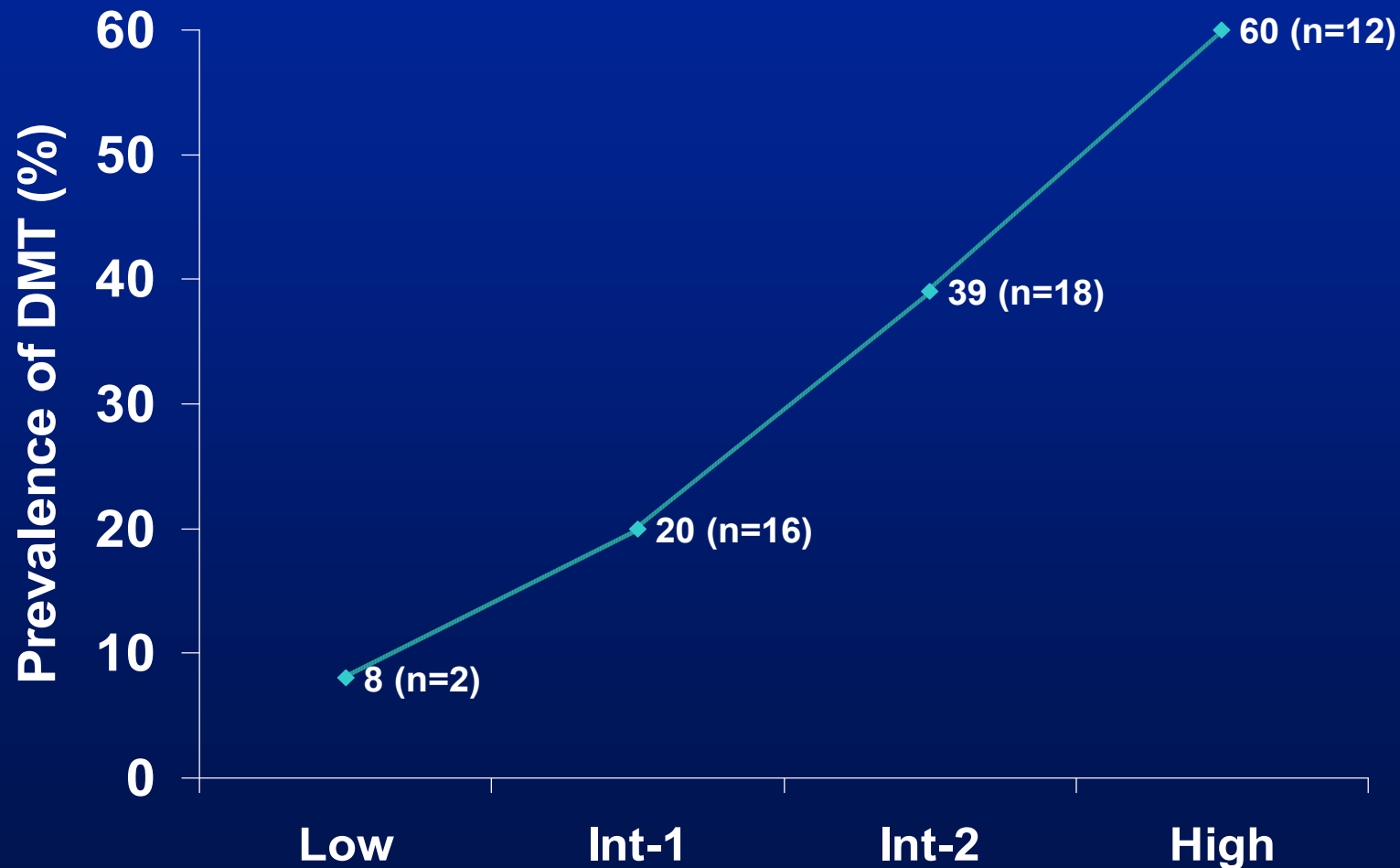
*Categories not mutually exclusive.



Type of Supportive Therapy (ST) According to Disease-Modifying Treatment (DMT)

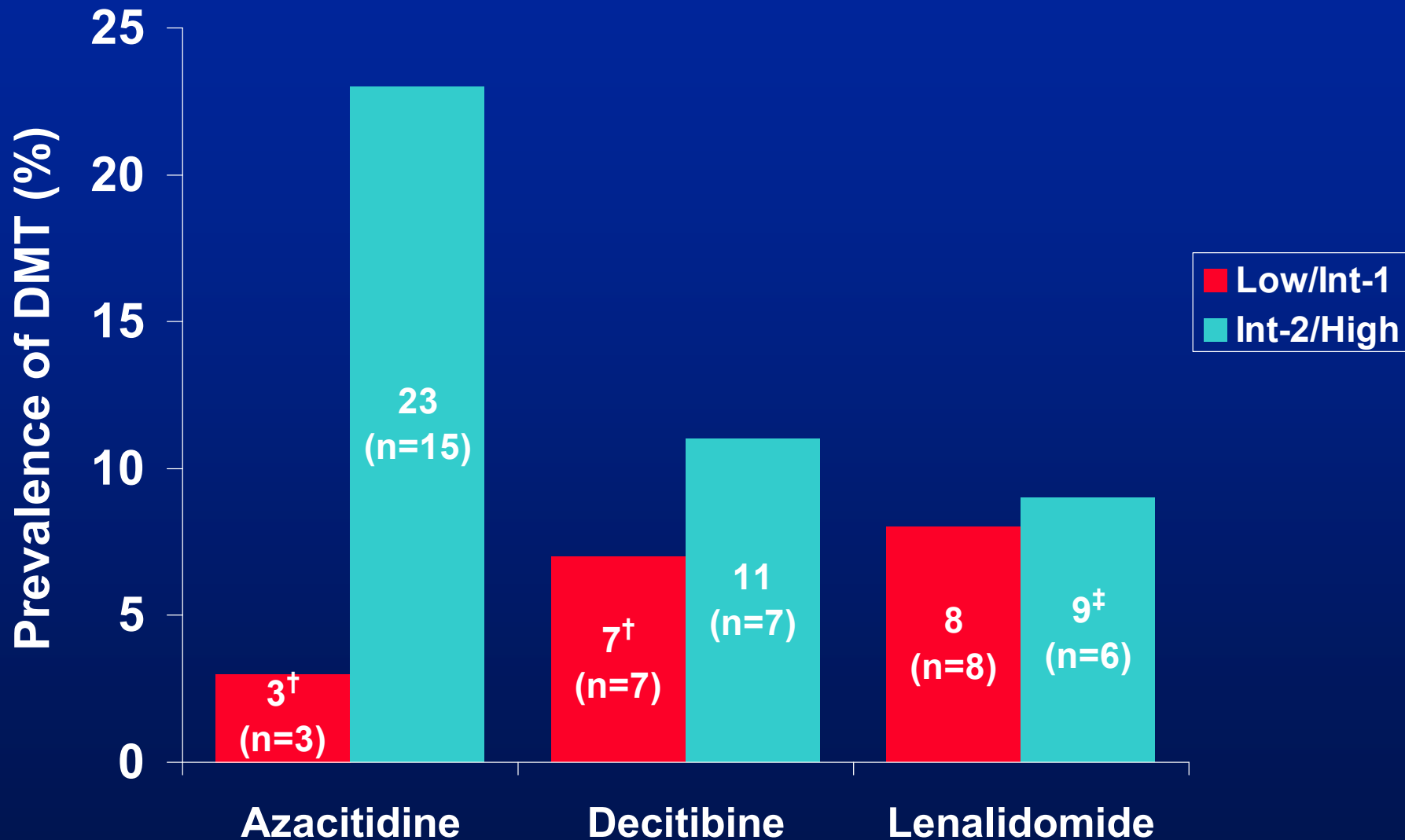


Disease-Modifying Treatment (DMT) According to IPSS Risk Category*



*Confined to 168 patients with known IPSS.

Type of Disease-Modifying Treatment (DMT) According to IPSS Risk Category*



*Confined to 168 patients with known IPSS.

[†]All patients were Int-1.

[‡]All patients were Int-2.

Lenalidomide and 5q-

- Proportion of patients with 5q- (with or without other cytogenetic abnormalities) treated with lenalidomide: **35% (7/20)**
 - Proportion of lenalidomide-treated patients who did not have 5q-: **53% (8/15)**
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Other Salient Findings

- *Transfusions*

39% of patients had at least one transfusion

- *Region*

Any disease-modifying treatment, **6%** of New England patients (1/18) vs. **27%** of those in other regions (199/271), $p < 0.001$

Limitations

- *The registry population is younger and of higher socioeconomic status than MDS patients as a whole*

There is some representation of older and low SES patients

- *The treatment information is self-reported by patients, and may underestimate the prevalence of specific therapies*

A comparison of self reports and records for a subset of patients showed good agreement

Conclusions

- A majority of MDS patients in the U.S. receive supportive therapy in the first few months after diagnosis
 - Relatively few recently-diagnosed patients receive disease-modifying treatment
 - Patients who receive disease-modifying treatment are also more likely to receive supportive therapy, perhaps due to hematologic side effects of the treatment
 - A substantial proportion of patients who receive lenalidomide either do not have 5q- or have intermediate-2 risk, outside the drug's approved indications
 - DNA hypomethylating agents are used not only in higher risk patients, but also in those with intermediate-1 risk
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