EE11

Burden of Myelodysplastic Syndromes Part II: Systematic Literature Review of Economic Burden

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Background

- Myelodysplastic syndromes (MDS) are a group of clonal neoplastic bone marrow disorders characterized by ineffective hematopoiesis and cytopenia[1,2]
  - The progression of MDS varies across patients; some experience relatively slow disease progression, while others experience progressive cytopenia that eventually leads to death[1,2]
  - Hematopoietic stem cell transplant is the only curative therapy for MDS but is limited to select patients with favorable health status. Treatments to achieve remission for patients ineligible for hematopoietic stem cell transplant include supportive care, immunotherapy, and chemotherapy
- New treatments and treatment regimens have played a pivotal role in MDS by inducing sustained hematologic responses and delaying progression to leukemia

Objective

- This study sought to systematically identify, review, and synthesize the evidence for the economic burden of MDS as part of a larger study objective to determine the overall burden of MDS

Methods

Approach

- A systematic literature search was conducted using MEDLINE and EMBASE to identify studies that evaluated patients with MDS and reported healthcare utilization costs

PICOS and Study Selection

- Screening of studies identified in the initial search was done independently and in duplicate by 2 reviewers. Disagreements were resolved through discussion
- Studies meeting the PICOS criteria (**Table 1**) in the title/abstract phase had their full-text publications retrieved and were re-evaluated in duplicate and independently by 2 reviewers. Disagreements were resolved through discussion between the reviewers
- Relevant systematic reviews identified during screening were reviewed to cross-reference the search strategy and identify missed publications
- A study mapping exercise was conducted to match publications reporting the same study. This was accomplished using registration numbers, study authors, and sample size. This exercise was used to prevent double counting outcomes in the final data set and to show that reported outcomes are from distinct patients

Table 1. PICOS Criteria of Economic Burden

Eligibility criteria	
Patients	MDS
Interventions	Not applicable
Comparators	Not applicable
Outcomes	Studies reporting data for: • Healthcare resource use • Direct medical costs • Nonmedical costs • Out-of-pocket costs for patients • Loss of productivity (patients or family caregivers)
Study design	Randomized controlled trials Nonrandomized clinical trials Single-arm trials Database analyses Cross-sectional studies Observational studies
Language	English
Year range	No limit

Data Extraction

- Studies meeting the complete PICOS criteria after full-text screening had their details on study characteristics, outcomes, and patient characteristics extracted by 2 investigators independently and in duplicate. Discrepancies in data extraction between reviewers were resolved through discussion and a reevaluation of the full text

Quality Assessment

- Prospective observational studies had their quality assessed via the Newcastle Ottawa Scale[3]
- Randomized controlled studies were assessed via the Risk of Bias 2 instrument endorsed by the Cochrane Collaboration[4]
- The observational studies in epidemiology (STROBE) checklist was used to evaluate the quality of reporting of the selected studies

Data Analysis

- To syntheize the collected data, a descriptive analysis was conducted to summarize the outcomes for the economic burden of MDS
- All costs were converted to 2020 USD. No adjustment was made for purchasing power parity between countries
- When sufficient data were available, a subgroup analysis based on risk status was conducted

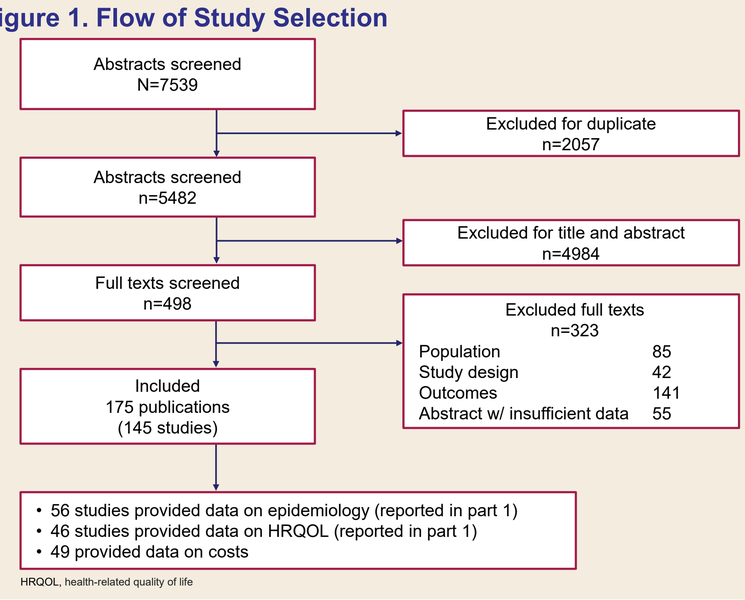
Cost categories

- Drug-related cost refers to the cost of procuring the drug therapy
- Medical cost refers to the cost associated with medical treatment (eg, surgical operations, hospital visits, treatment administration, emergency department [ED] visits), excluding drug-related costs
- Treatment cost refers to the cost associated with the treatment of disease including both medical and drug-related costs
- Transfusion cost refers to the cost associated with receiving blood transfusions (eg, blood products, cost of hospital stay)
- Cost of best supportive care refers to the cost associated with providing care that manage symptoms from complications of MDS (eg, erythropoietin-stimulating agents, transfusions, granulocyte colony-stimulating factor, iron chelation therapy)

Results

- Systematic searches of MEDLINE and EMBASE identified 7539 abstracts, and 175 publications representing 145 unique studies met the inclusion criteria defined in the PICOS. The evidence base of this review is summarized in the PRISMA diagram in **Figure 1**. Of these, 49 unique studies provided evidence on the economic burden of MDS

Figure 1. Flow of Study Selection



HRQOL, health-related quality of life

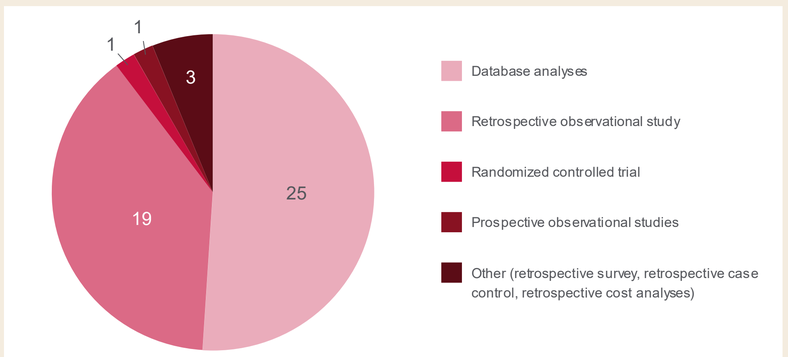
An online version of this poster and supplementary material can be found through this QR code.

Results (cont)

Study Characteristics

- Study designs are presented in **Figure 2**; 4 studies provided data on both epidemiology and cost, and 2 studies provided data on both cost and health-related quality of life
- Included studies were conducted in Australia, Canada, China, Croatia, France, Germany, Greece, Italy, Japan, Nigeria, Spain, Sweden, and the US
- Database analysis was the most frequently used study design, followed by retrospective observational study

Figure 2. Design of Included Studies



Overall Costs

- Overall costs were reported in 22 unique studies. For those regimen containing hypomethylating agent (HMA), the cost ranged from $33,506 to $243,802, while for other regiments the cost varied from $21,502 to $233,714 (**Figure 3)**
- **Figure 4** presents 7 studies separating overall costs for MDS-related and non-MDS–related. MDS-related costs ranged from $2,659 to $142,196. Non-MDS–related costs varied from $1698 to $151,801

Figure 3. Overall Costs in Treating MDS

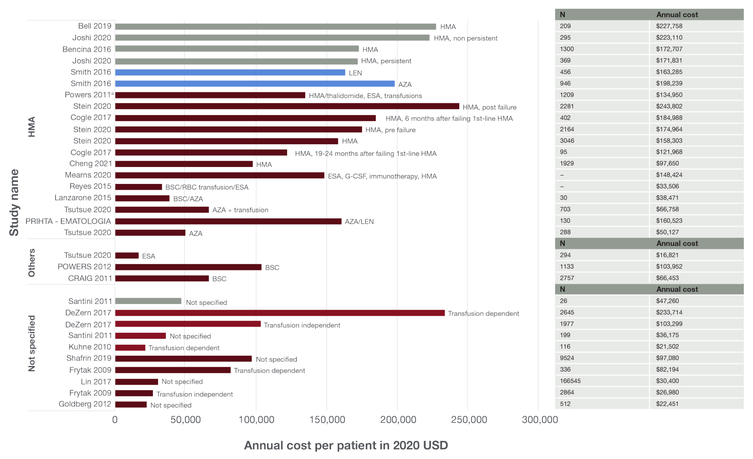
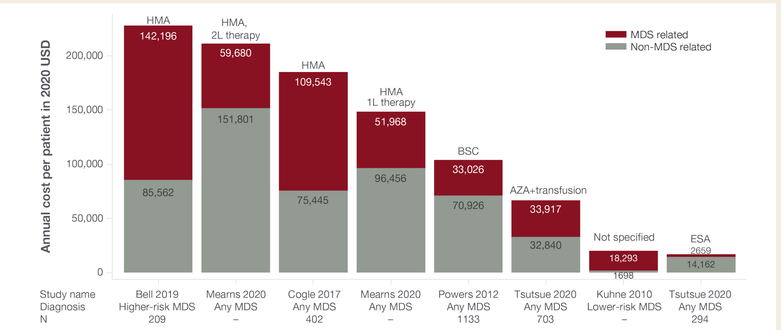


Figure 4. MDS- and Non-MDS–Related Overall Costs



Treatment-Related Costs

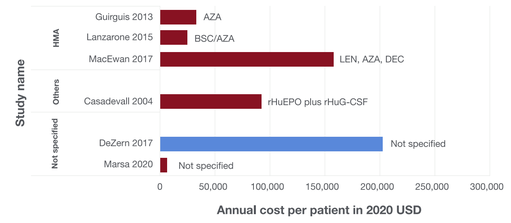
- **Figure 5** reports various costs of treating MDS in patients stratified by treatment modality (HMA based or best supportive care [BSC] based) and risk level where available
  - Treatment cost varied from $24,431 to $157,252 in HMA-based therapies and $5622 to $200,576 for others or nonspecified (**Figure 5A**)
  - Drug-related cost varied from to $60,344 to $71,802 for HMA and $4098 to $6854 for others (**Figure 5B**)
  - Medical cost ranged from $53,648 to $78,930 for low-risk patients vs $215,820 for high-risk patients in HMA-based regimens. The medical cost was $22,882 for transfusion-independent patients vs $75,338 for transfusion-dependent patients (**Figure 5C**)
  - Transfusion costs varied from $4451 to $25,684 in HMA-based regimens and from $4236 to $49,785 for other therapies (**Figure 5D**)
  - Inpatient cost varied from $27,957 to $44,277 in HMA-treated refractory anemia with excess blasts patients and from $1961 to $55,178 in other regimens (**Figure 5E**). Across studies reporting the cost by transfusion dependence, transfusion dependent patients had higher inpatient costs
  - Only 1 study reported outpatient cost for HMA regimens ($108,090). Outpatient cost for other regimens varied from $1702 to $22,365 (**Figure 5F**)
  - Costs of ED visits for HMA regimens in higher-risk patients ranged from $35,460 to $68,235. ED costs for other treatments ranged from $348 to $1092 (**Figure 5G**)
  - Hospitalization days for HMA regimens ranged from 1.6 to 52.8 days, while for BSC it varied from 7.8 to 15.0 days (**Figure 5H**)

Cost of Physician Visits

- 2 studies reported physician visit costs. One study (n=2864) estimated $4730 for transfusion-dependent patients to $12,901 for transfusion-independent patients. The other study (n=166,545) reported physician visit cost of $5822

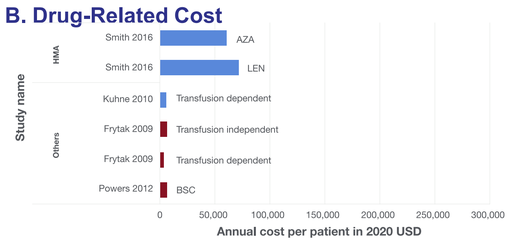
Figure 5. Treatment-Related Costs for MDS

A. Treatment Cost



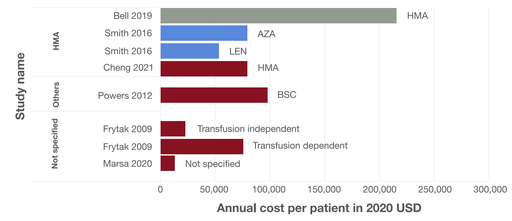
N	Annual treatment cost
31	$31,903
30	$24,431
38,025	$157,252
N	**Annual treatment cost**
6	$91,132
N	**Annual treatment cost**
2645	$200,576
33,091	$5622

B. Drug-Related Cost



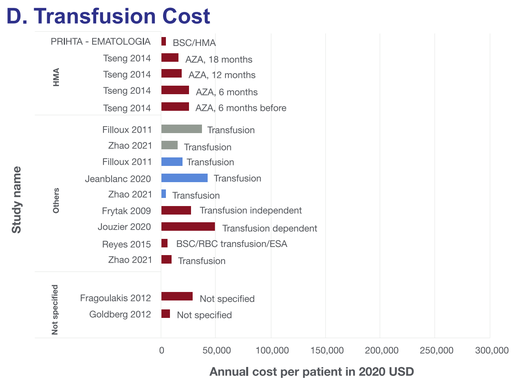
N	Annual drug-related cost
946	$60,344
456	$71,802
N	**Annual drug-related cost**
–	$6033
336	$6854
2864	$4098
1133	$6447

C. Medical Cost



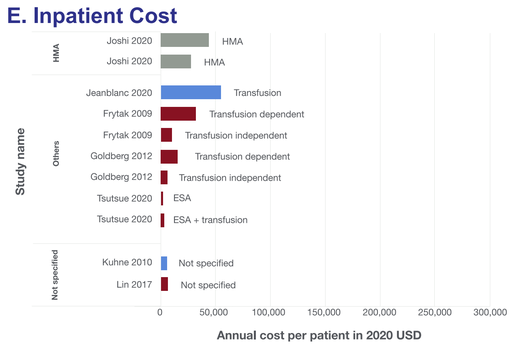
N	Annual medical cost
209	$215,820
946	$78,930
456	$53,648
2400	$79,251
N	**Annual medical cost**
1133	$97,504
N	**Annual medical cost**
2864	$22,882
336	$75,338
33,091	$12,633

D. Transfusion Cost



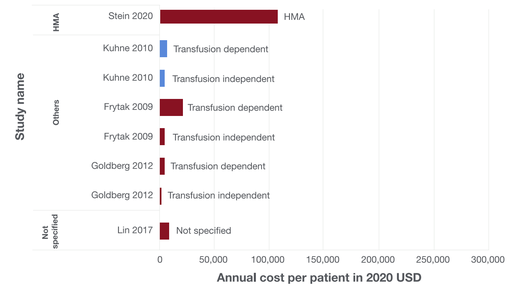
N	Annual transfusion cost
130	$4451
51	$15,986
51	$19,027
51	$25,411
51	$25,684
N	**Annual transfusion cost**
43	$36,813
1147	$14,598
111	$19,754
5081	$42,556
1164	$4236
336	$27,087
100	$49,785
–	$5757
2311	$9379
N	**Annual transfusion cost**
–	$28,841
512	$7873

E. Inpatient Cost



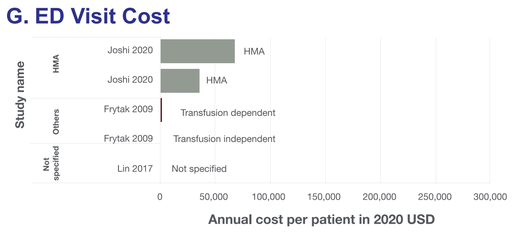
N	Annual inpatient cost
295	$44,277
369	$27,957
N	**Annual inpatient cost**
5081	$55,178
336	$32,526
2864	$10,479
206	$16,315
307	$6506
294	$1961
–	$3560
N	**Annual inpatient cost**
–	$6019
116,545	$7307

F. Outpatient Cost



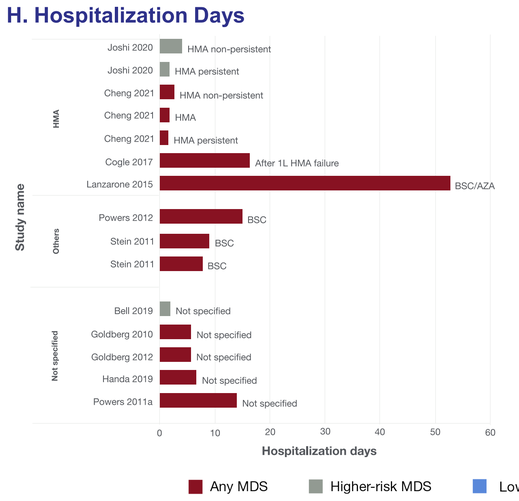
N	Annual outpatient cost
3046	$108,090
N	**Annual outpatient cost**
–	$7387
–	$5489
336	$22,365
2864	$5,743
206	$4889
307	$1702
N	**Annual outpatient cost**
166,545	$9961

G. ED Visit Cost



N	Annual ED visits cost
295	$68,235
369	$35,460
N	**Annual ED visits cost**
336	$1092
2864	$348
N	**Annual ED visits cost**
166,545	$326

H. Hospitalization Days



N	Hospitalization days
295	4.1
363	2.7
1929	1.7
1534	1.6
402	16.4
30	52.8
N	**Hospitalization days**
1133	15.0
912	9.0
221	7.8
N	**Hospitalization days**
209	2.0
512	5.7
512	5.7
855,726	6.7
578	14.0

Conclusions

- MDS has imposed a significant economic burden, with overall costs ranging from $16,821 to $243,802
- There were significant variations in the costs across the studies included in this review
- The costs associated with HMA treatments were generally higher than other regimens. The cost of treating transfusion dependent patients was greater than that for transfusion independent patients
- Studies that estimated costs by risk profile were limited. Further research on the economic burden of MDS stratified by risk-level is needed

References

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Bibliographic information for the papers included in this review are accessible through the QR code.

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