Evaluating clinical outcome assessments in low-risk myelodysplastic syndromes through development of a comprehensive conceptual model based on targeted literature reviews and clinician interviews

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Based on a preliminary conceptual model of the patient experience, existing COA tools may not fully capture the signs, symptoms, and impacts of LR-MDS

Supplemental data

SCAN ME

MSR12

Introduction

- Myelodysplastic syndromes (MDS) are a group of hematopoietic stem cell disorders characterized by abnormal blood cell development which contributes to cytopenias and an increased risk of developing acute myeloid leukemia (AML)[1]
- The International Prognostic Scoring System (IPSS) stratifies MDS into higher risk (HR-MDS) and lower risk (LR-MDS) based on cytopenias, bone marrow blasts, cytogenics, chromosomal abnormalities, age, and gender[2,3]
  - LR-MDS accounts for approximately 75% of US cases[4]; while these patients have a lower risk of AML progression than those with HR-MDS, they often live with malignant hematopoiesis for many years[1]
- Patients with LR-MDS present with heterogeneous manifestations of signs, symptoms, and impacts of the condition, ranging from mild anemia with close-to-normal performance status to life-threatening cytopenia, transfusion dependence, and, in some cases, disability[3]
  - Clinical outcome assessment (COA) research conducted in MDS has focused on patients with HR-MDS, with limited evidence focusing on patient experience in LR-MDS
- **Objective:** To develop a preliminary conceptual model (PCM) for LR-MDS and evaluate the ability of current COA tools to adequately capture patient experiences

Methods

Targeted Literature Reviews

- Targeted literature reviews (TLRs) were conducted to review available publications and patient blogs for LR-MDS–related signs, symptoms, impacts, and the landscape of current clinical outcome tools:
  1. One TLR was conducted to develop a **PCM documenting a set of signs, symptoms, and impacts specific to LR-MDS** (**Table 1**)

Table 1: LR-MDS Target Literature Review Inclusion Criteria

Criteria	Research articles	Patient stories or testimonials
Population	• Adult patients with a confirmed diagnosis of low-risk MDS • Caregivers of adult patients with a primary diagnosis of low-risk MDS	• Adult patients with a reported diagnosis of low-risk MDS • Caregivers of adult patients with a reported diagnosis of low-risk MDS
Studies of interest	• Noninterventional/qualitative studies (interviews, focus groups, or surveys with patients, caregivers, or clinicians, including interviews during questionnaire development) • Observational studies (including registries and real-world data) • Patient or caregiver panels	• Online posts/blogs on patient experience
Outcomes	• Studies reporting disease- and/or treatment-related symptoms and/or impacts experienced by patients with low-risk MDS	• Online blog posts reporting disease- and/or treatment-related symptoms and/or impacts experienced by patients with low-risk MDS
Sources reviewed	• Medline, Embase, and conference abstracts via OVID • Manual search via review of references from selected articles	• MDS Foundation • MDS UK Patient Support Group • Aplastic Anemia and MDS International Foundation (AAMDSIF)

MDS, myelodysplastic syndrome; PCM, preliminary conceptual model.

  2. The second TLR focused on evaluating the **landscape of COA tools used in LR-MDS clinical trials and regulatory labels** based on searches of OVID/ClinicalTrials.gov/PROQOLID/PROLABELS (**Table 2**)
     - A gap analysis was conducted to understand and compare COA tool coverage against concepts from the PCM and the shortlisted COA tools were reviewed further for content validity and psychometrics

Table 2: COA Tool Targeted Literature Review

Resource	TLR findings
Tool-focused TLR	**OVID** was used to identify **published literature (11 articles)** involving COA tools in LR-MDS
Clinical trial analysis	COA tools were used in 7 clinical trials for LR-MDS, as reported on **ClinicalTrials.gov**
PROQOLID	**PROQOLID** was searched to identify COA tools used in MDS (no results reported for LR-MDS)
PROLABELS	Two products approved for MDS included information on COA tools in their EMA SmPCs, as reported using **PROLABELS** (no results reported for LR-MDS)

COA, clinical outcome assessment; EMA, European Medicines Agency; LR-MDS, low-risk myelodysplastic syndrome; MDS, myelodysplastic syndrome; SmPC, summary of product characteristics; TLR, targeted literature review.

Endorsement by Clinicians

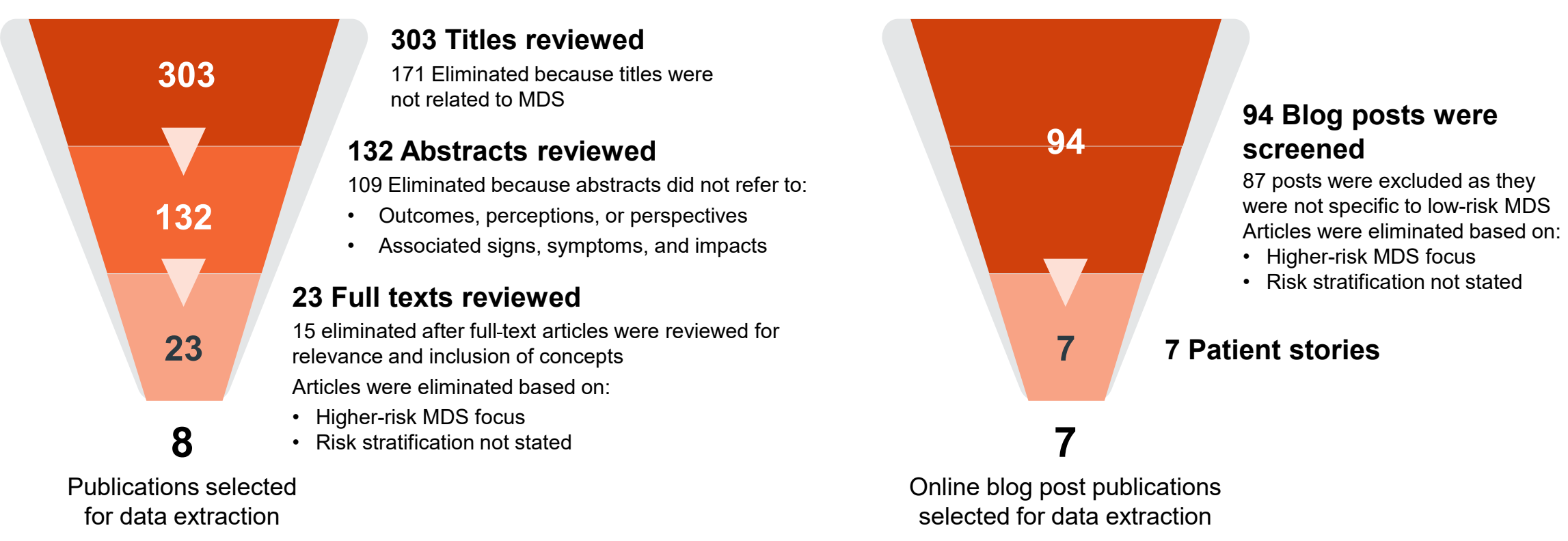
- Structured clinician interviews (n=3) were conducted with clinicians with expertise and experience in treating patients with LR-MDS
- Clinicians had an average experience of 16 years (range, 9-25 years) treating patients with MDS, handling an average of 50 patients with LR-MDS (range, 40-60) per month
- Interviews (60 minutes each) were conducted using a semistructured discussion guide with open-ended and probing questions, including the following:
  - Clinicians' background and experience caring for patients with LR-MDS
  - Clinicians' insights on commonly occurring signs, symptoms, and impacts that patients report experiencing as a result of LR-MDS
  - Clinicians reviewed the initial PCM, developed from the TLR, and prioritized concepts important to patients
  - Interviews gathered insights on characteristics and appropriateness of current COA tools that may be fit to use in patients with LR-MDS from the clinicians' perspective
- The PCM was revised to add, update, and/or remove concepts as applicable; using this updated PCM, the COA tool mapping previously conducted in the landscape assessment was refined

Results

PCM Development

- A total of 46 signs and symptoms as well as 40 impacts were included in the initial PCM based on TLR of 8 peer-reviewed articles and 7 patient blogs (**Figure 1**)
  - Fatigue, dyspnea, anemia, and social impacts were most common

Figure 1: LR-MDS TLR Identified Research Articles and Patient Stories or Testimonials Relevant to the PCM



303 Titles reviewed
171 Eliminated because titles were not related to MDS

132 Abstracts reviewed
109 Eliminated because abstracts did not refer to:
- Outcomes, perceptions, or perspectives
- Associated signs, symptoms, and impacts

23 Full texts reviewed
15 eliminated after full-text articles were reviewed for relevance and inclusion of concepts
Articles were eliminated based on:
- Higher-risk MDS focus
- Risk stratification not stated

8 Publications selected for data extraction

94 Blog posts were screened
87 posts were excluded as they were not specific to low-risk MDS
Articles were eliminated based on:
- Higher-risk MDS focus
- Risk stratification not stated

7 Patient stories

7 Online blog post publications selected for data extraction

MDS, myelodysplastic syndrome; PCM, preliminary conceptual model; TLR, targeted literature review.

Clinician Endorsement of Concept Mapping

- The PCM was refined based on clinician report of the commonly occurring signs, symptoms, and impacts that patients reported experiencing due to LR-MDS. The final PCM included a **total of 88 concepts** (**Figures 2, 3**)

Signs and Symptoms:

✓ **14 signs and symptoms** were **spontaneously endorsed** as relevant to LR-MDS by clinicians
- ○ Fatigue, shortness of breath, and easy bruising were mentioned by all 3 clinicians

⊕ **No new signs or symptoms** were mentioned beyond those already included in the initial PCM

⊖ **6 signs and symptoms** from the PCM were noted as **not relevant** to LR-MDS (or indicative of another disease or condition), and were removed[a]
- ○ Night sweats, splenomegaly, and discomfort to spleen were mentioned by 2 clinicians; deteriorated eyesight, eye strain, and weight loss were mentioned by 1 clinician

Impacts:

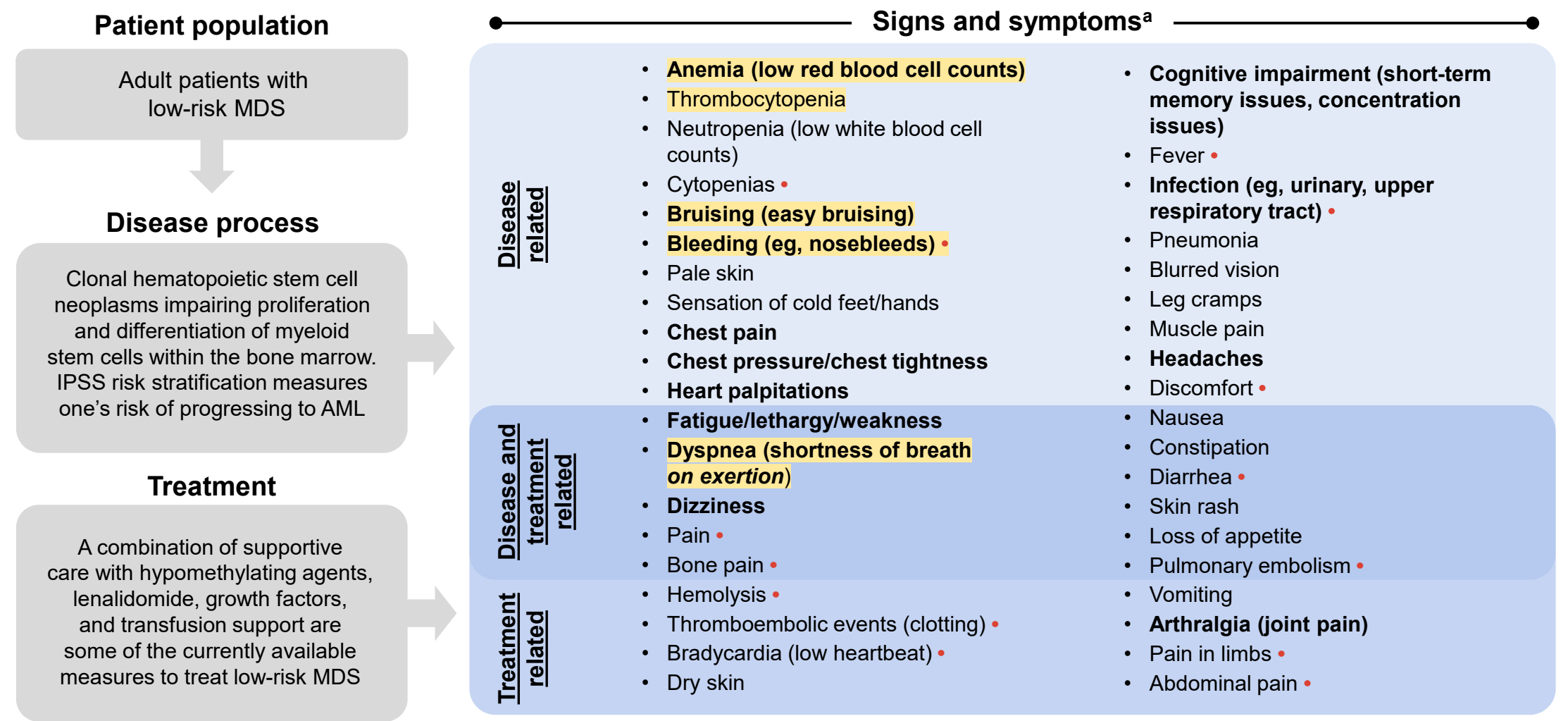
✓ **15 impacts spontaneously endorsed** as relevant to LR-MDS
- ○ Impacts reported by at least 2 clinicians included difficulty walking (eg, risk of falling), difficulty carrying things (eg, laundry), difficulty with self-care (cooking, cleaning, showering), inability to participate in desired activities (inability to travel), worry (feeling like a burden on family), negative impacts on relationships (missing out on family events), and interference with ability to work

⊕ **4 new impacts** were mentioned by clinicians
- ○ Treatment burden and its associated factors (ie, medical costs such as expensive medications, co-pay, and parking; inability to travel due to transfusion appointments and doctors' visits burden); inability to initiate activities; caregiver dependence; and grappling with loss of independence

⊖ **No impacts** were identified as **not relevant** by clinicians

[a] *A total of 14 signs and symptoms were reported by at least 1 clinician as not commonly occurring in LR-MDS, including fever, infection, pain, hemolysis, diarrhea, and bleeding. Although reported by clinicians as not very common in LR-MDS, these signs and symptoms were retained in the updated PCM, and decisions on their relevance will be confirmed after patient interviews in LR-MDS are completed.*

Figure 2: PCM Signs and Symptoms



Patient population

Adult patients with low-risk MDS

Disease process

Clonal hematopoietic stem cell neoplasms impairing proliferation and differentiation of myeloid stem cells within the bone marrow. IPSS risk stratification measures one's risk of progressing to AML

Treatment

A combination of supportive care with hypomethylating agents, lenalidomide, growth factors, and transfusion support are some of the currently available measures to treat low-risk MDS

Signs and symptoms[a]

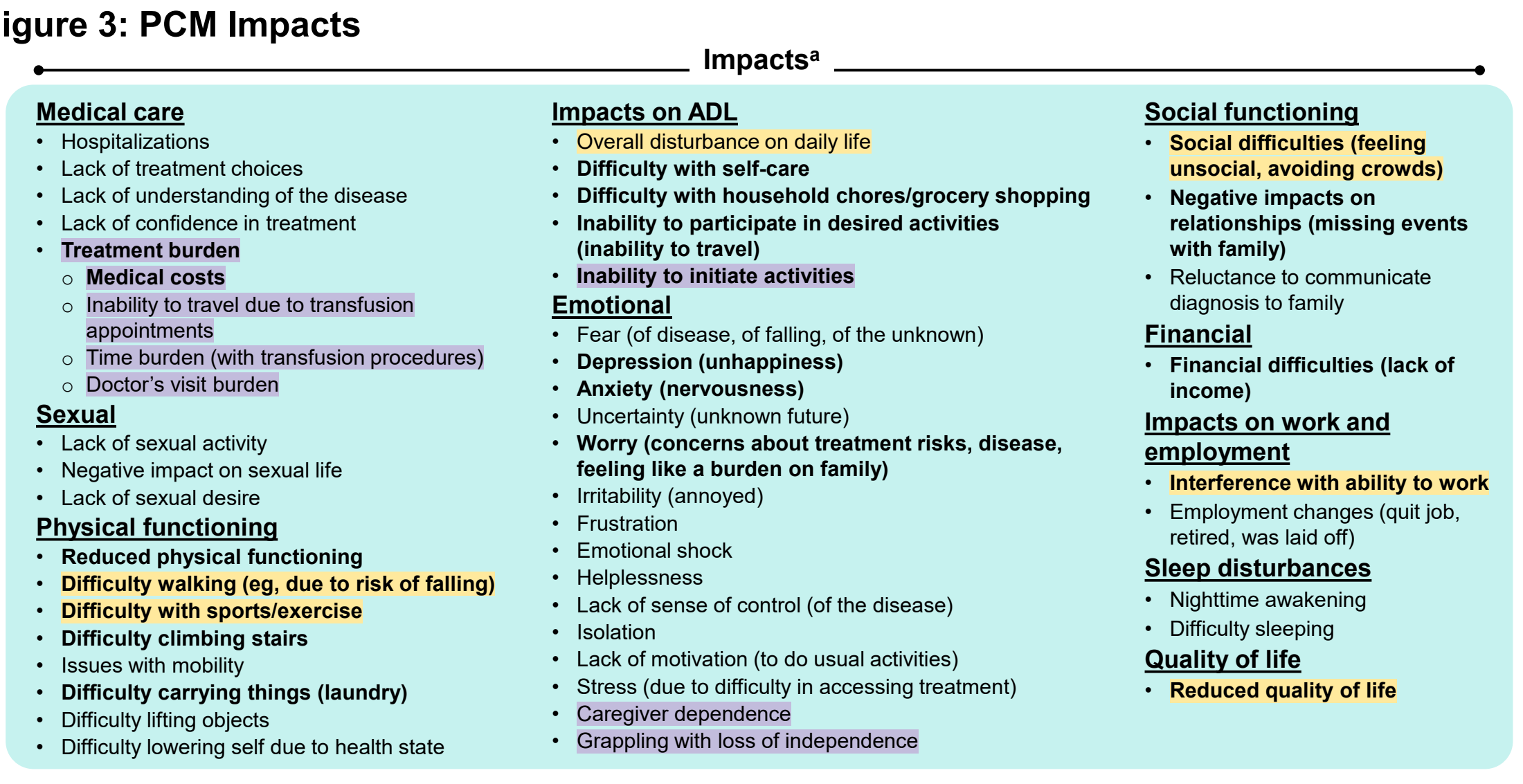
Disease related
- **Anemia (low red blood cell counts)**
- Thrombocytopenia
- Neutropenia (low white blood cell counts)
- Cytopenias ●
- **Bruising (easy bruising)**
- **Bleeding (eg, nosebleeds)** ●
- Pale skin
- Sensation of cold feet/hands
- **Chest pain**
- **Chest pressure/chest tightness**
- **Heart palpitations**
- **Cognitive impairment (short-term memory issues, concentration issues)**
- Fever ●
- **Infection (eg, urinary, upper respiratory tract)** ●
- Pneumonia
- Blurred vision
- Leg cramps
- Muscle pain
- **Headaches**
- Discomfort ●

Disease and treatment related
- **Fatigue/lethargy/weakness**
- **Dyspnea (shortness of breath *on exertion*)**
- **Dizziness**
- Pain ●
- Bone pain ●
- Nausea
- Constipation
- Diarrhea ●
- Skin rash
- Loss of appetite
- Pulmonary embolism ●

Treatment related
- Hemolysis ●
- Thromboembolic events (clotting) ●
- Bradycardia (low heartbeat) ●
- Dry skin
- Vomiting
- **Arthralgia (joint pain)**
- Pain in limbs ●
- Abdominal pain ●

AML, acute myeloid leukemia; IPSS, International Prognostic Scoring System; MDS, myelodysplastic syndrome; PCM, preliminary conceptual model.
[a] *Highlighted in yellow are concepts with reported prevalence ≥50% from the prior TLR. **Bolded** concepts are clinician-endorsed concepts, that is, mentioned spontaneously by clinicians. ● concepts are not common in patients with LR-MDS as reported by clinicians.*

Figure 3: PCM Impacts



Impacts[a]

Medical care
- Hospitalizations
- Lack of treatment choices
- Lack of understanding of the disease
- Lack of confidence in treatment
- **Treatment burden**
  - ○ **Medical costs**
  - ○ Inability to travel due to transfusion appointments
  - ○ Time burden (with transfusion procedures)
  - ○ Doctor's visit burden

Sexual
- Lack of sexual activity
- Negative impact on sexual life
- Lack of sexual desire

Physical functioning
- **Reduced physical functioning**
- **Difficulty walking (eg, due to risk of falling)**
- **Difficulty with sports/exercise**
- **Difficulty climbing stairs**
- Issues with mobility
- **Difficulty carrying things (laundry)**
- Difficulty lifting objects
- Difficulty lowering self due to health state

Impacts on ADL
- Overall disturbance on daily life
- **Difficulty with self-care**
- **Difficulty with household chores/grocery shopping**
- **Inability to participate in desired activities (inability to travel)**
- **Inability to initiate activities**

Emotional
- Fear (of disease, of falling, of the unknown)
- **Depression (unhappiness)**
- **Anxiety (nervousness)**
- Uncertainty (unknown future)
- **Worry (concerns about treatment risks, disease, feeling like a burden on family)**
- Irritability (annoyed)
- Frustration
- Emotional shock
- Helplessness
- Lack of sense of control (of the disease)
- Isolation
- Lack of motivation (to do usual activities)
- Stress (due to difficulty in accessing treatment)
- Caregiver dependence
- Grappling with loss of independence

Social functioning
- **Social difficulties (feeling unsocial, avoiding crowds)**
- **Negative impacts on relationships (missing events with family)**
- Reluctance to communicate diagnosis to family

Financial
- **Financial difficulties (lack of income)**

Impacts on work and employment
- **Interference with ability to work**
- Employment changes (quit job, retired, was laid off)

Sleep disturbances
- Nighttime awakening
- Difficulty sleeping

Quality of life
- **Reduced quality of life**

ADL, activities of daily living; PCM, preliminary conceptual model.
[a] *Highlighted in yellow are concepts with reported prevalence ≥50% from the prior TLR. Highlighted in purple are concepts that were not reported in the original TLR and added to the updated PCM based on clinician interviews. **Bolded** concepts are clinician-endorsed concepts, that is, mentioned spontaneously by clinicians.*

COA Tool Refinement and Analyses

- COA Targeted TLR identified 6 tools from OVID, 5 from ClinicalTrials.gov, 4 from PROQOLID, and 4 from PROLABELS
- Based on searches conducted across 4 databases, 10 distinct COA tools were identified and reviewed for LR-MDS evidence (**Table 3**)

Table 3: COA Tools Identified and Reviewed for LR-MDS Evidence

COA Tool	Sources in which COA tool was reported
QUALMS	🔴🟢🟡
EORTC QLQ-C30	🔴🟢🟣
FACT-An	🔴🟢🟣
QOL-E	🔴🟡
MDASI-AML/MDS	🟡
SF-36	🔴
EQ-5D	🔴🟢🟣
EORTC QLQ-FA12	🔴
FACT-G	🟣
LIFE-H	🟡

🔴 TLR 🟢 Clinical trials 🟡 PROQOLID 🟣 PROLABELS

10 tools were identified
- Tools were selected for further review based on the following criteria:
  - ○ Tools reported in searches specifically for LR-MDS or
  - ○ Tools developed for patients with MDS
- Based on these criteria, 2 tools that were not found in LR-MDS–specific searches and not developed in patients with MDS were excluded for further review (FACT-G and LIFE-H)

8 tools were considered for further review
- Among the eight tools mapped to the preliminary conceptual model, three were removed, as they provided the least coverage of the relevant concepts (SF-36, EORTC QLQ-FA12, and EQ-5D)

5 tools were selected for evaluation of content validity and psychometric properties

AML, acute myeloid leukemia; COA, clinical outcome assessment; EORTC QLQ, European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire; EQ-5D, EuroQol; FACT-An, Functional Assessment of Cancer Therapy – Anemia; FACT-G, Functional Assessment of Cancer Therapy – General; LIFE-H, Assessment of Life Habits; LR-MDS, low-risk myelodysplastic syndrome; MDASI-AML/MDS, MD Anderson Symptoms Inventory for acute myeloid leukemia/myelodysplastic syndrome; MDS, myelodysplastic syndrome; PCM, preliminary conceptual model; QOL-E, Quality of Life E; QUALMS, Quality of Life in Myelodysplasia Scale; SF-36, 36-Item Short Form Survey.

Concept Mapping Coverage, Content Validity, and Psychometrics

- The COA tools used to map concepts in the COA landscape review work were assessed
  - Across all 5 COA tools, no tools had strong coverage across all signs, symptoms, and impacts (**Tables 4, 5**):
    1. **FACT-An** covered 49% (43/88) of the total concepts, or 73% (16/22) of the concept categories
    2. **EORTC QLQ C-30** covered 40% (35/88) of the total concepts, or 68% (15/22) of the concept categories
    3. **QOL-E** covered 33% (29/88) of the total concepts, or 55% (12/22) of the concept categories
    4. **MDASI-AML/MDS** covered 29% (26/88) of the total concepts, or 55% (12/22) of the concept categories
    5. **QUALMS** covered 27% (24/88) of the total concepts, or 45% (10/22) of the concept categories
- Evidence of content validity and psychometrics of these COA tools in LR-MDS populations were reviewed with the conclusion that most COA tools were not suitable in patients with LR-MDS due to not being developed for these patients, and most tools had no evidence on psychometric properties assessed in patients with LR-MDS

Table 4: Concept Mapping for Signs and Symptoms

Concept category[a]	FACT-An	EORTC QLQ-C30	QOL-E	QUALMS	MDASI-AML/MDS
Fatigue (n=1)	100%	100%	100%	100%	100%
Respiratory (n=3)	33%	33%	33%	33%	33%
Neurological (n=1)	100%	0%	0%	0%	0%
Cardiovascular (n=6)	17%	8%	17%	0%	8%
Pain (n=8)	50%	38%	25%	13%	50%
Hematologic (n=7)	0%	0%	0%	14%	0%
Cognitive (n=1)	0%	100%	0%	100%	100%
Gastrointestinal (n=4)	25%	100%	0%	50%	75%
Dermatologic (n=3)	0%	0%	33%	0%	50%
Systemic (n=4)	25%	25%	0%	0%	38%
Ocular (n=1)	0%	0%	0%	0%	0%
Other: leg cramps (n=1)	0%	0%	0%	0%	0%

Concept coverage: 100% 75% 50% 25% 0%

EORTC QLQ, European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire; FACT-An, Functional Assessment of Cancer Therapy – Anemia; MDASI-AML/MDS, MD Anderson Symptoms Inventory for acute myeloid leukemia/myelodysplastic syndrome; QOL-E, Quality of Life E; QUALMS, Quality of Life in Myelodysplasia Scale.
[a] ***Bolded** categories include concepts that are clinician endorsed, that is, mentioned spontaneously by clinicians. Each category describes n number of related, individual signs and symptoms, comprising 1 to 8 concepts. Mapping for individual concepts is shown in supplementary data. Scoring was conducted at the concept level, with 1 point assigned for full coverage and 0.5 points for partial coverage. Category scores were calculated as the percentage of the maximum possible points achieved.*

Conclusion

- **Identified COA tools did not comprehensively cover the LR-MDS PCM developed**
  - No available tool adequately captured the signs and symptoms associated with LR-MDS
- **Patient interviews are ongoing** and will guide appropriate COA tool selection and finalize the conceptual model based on patient experience in LR-MDS (preliminary findings shown in supplemental data)
- There is a **need for de novo COA tools, modification of existing tools, or a combination of multiple tools** to ensure coverage of prioritized concepts that capture metrics tailored to LR-MDS disease risk and management strategies

Discussion

- **This work developed a preliminary conceptual model and then refined it based on clinician perspectives on the patient experience in LR-MDS**
- **Clinicians endorsed an updated PCM, provided recommendations for measurement of the patient experience of LR-MDS, and contextualized several concepts reported in the PCM based on their patients' experience of those concepts**
  - Clinician input guided the addition of new concepts, as well as refinements to the wording, grouping, and descriptions of existing concepts, and the removal of those deemed not relevant to LR-MDS, thereby supporting the development of the LR-MDS PCM
- **The COA tools from the second TLR were assessed against the clinician-endorsed PCM**
  - Across all 5 COA tools, no tool had strong coverage across all signs, symptoms, and impacts

Table 5: Concept Mapping for Impacts

Concept category[a]	FACT-An	EORTC QLQ-C30	QOL-E	QUALMS	MDASI-AML/MDS
Emotional (n=15)	43%	23%	23%	50%	7%
Activities of daily living (n=5)	100%	70%	80%	30%	0%
Social functioning (n=3)	100%	67%	33%	100%	67%
Physical functioning (n=8)	25%	38%	56%	0%	13%
Medical care (n=8)	19%	13%	19%	19%	0%
Sexual (n=3)	67%	0%	67%	0%	0%
Sleep disturbances (n=2)	50%	50%	50%	0%	0%
Financial (n=1)	0%	100%	0%	100%	0%
Work and employment (n=2)	100%	50%	100%	0%	75%
Quality of life (n=1)	100%	100%	0%	0%	0%

Concept coverage: 100% 75% 50% 25% 0%

EORTC QLQ, European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire; FACT-An, Functional Assessment of Cancer Therapy – Anemia; MDASI-AML/MDS, MD Anderson Symptoms Inventory for acute myeloid leukemia/myelodysplastic syndrome; QOL-E, Quality of Life E; QUALMS, Quality of Life in Myelodysplasia Scale.
[a] ***Bolded** categories include concepts that are clinician endorsed, that is, mentioned spontaneously by clinicians. Each category describes n number of related, individual impacts, comprising 1 to 15 concepts. Mapping for individual concepts is shown in supplementary data. Scoring was conducted at the concept level, with 1 point assigned for full coverage and 0.5 points for partial coverage. Category scores were calculated as the percentage of the maximum possible points achieved.*

Clinician Perspectives on COA Landscape

- Clinicians were asked to share their perspectives on COA tools. They also provided insight into which factors would make COAs most useful for them in clinical practice. Clinicians' insights on appropriate considerations for use of COA tools in LR-MDS:

Impressions of current COA tools
- 2 of the clinicians noted that they **do not use** any standard tools within their clinical practice to regularly monitor their patients
- 1 clinician highlighted several **challenges** with the use of COA tools in clinical practice, noting the following:
  - ○ Lack of a tool **specific to MDS** and its manifestations
  - ○ Difficulty integrating available tools into **routine workflow**
- COA tools can only assess the patient experience at **time of administration**, not accounting for **contextual factors**, potentially misrepresenting the patient experience
  - ○ Depending on the timing of administration, results may be **influenced by the patient experience of transfusions**
  - ○ Without MDS-specific tools, the **questions may not fit the context of use,** may be **long**, or have **irrelevant** concepts assessed

How best to measure these concepts
- Ensure COA tools are **short** and **succinct** tools to minimize patient burden
- Consider **timing of contextual factors** (eg, transfusions) to ensure that the experience captured by questions is about the experience of LR-MDS vs the experience of treatment
- **Continuous data capture** was noted as potentially useful to consider (eg, using wearable devices) to more fully understand concepts such as physical functioning (noting that such technology may have its challenges in an older patient population)

Abbreviations

AML, acute myeloid leukemia; COA, clinical outcome assessment; EMA, European Medicines Agency; EORTC QLQ, European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire; EQ-5D, EuroQol; FACT-An, Functional Assessment of Cancer Therapy – Anemia; FACT-G, Functional Assessment of Cancer Therapy – General; HR-MDS, high-risk myelodysplastic syndrome; IPSS, International Prognostic Scoring System; LIFE-H, Assessment of Life Habits; LR-MDS, low-risk myelodysplastic syndrome; MDS, myelodysplastic syndrome; MDASI-AML/MDS, MD Anderson Symptoms Inventory for acute myeloid leukemia/myelodysplastic syndrome; PCM, preliminary conceptual model; PRO, patient-reported outcome; QOL-E, Quality of Life E; QUALMS, Quality of Life in Myelodysplasia Scale; SF-36, 36-Item Short Form Survey; SmPC, summary of product characteristics; TLR, targeted literature review.

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Acknowledgments

Literature reviews and clinician interviews were undertaken by the IQVIA team, funded by GSK.

Medical writing support for this poster was provided by Devon Greer, PhD, of Nucleus Global, an Inizio company, and funded by GSK.

Disclosures

A. Cardellino: *GSK*: current employment, current equity holder in publicly traded company. **F. Sen Nikitas**: *GSK*: current employment, current equity holder in publicly traded company. **S. Zhang**: *GSK*: current employment, current equity holder in publicly traded company. **V. Santini:** participated in advisory boards of Ascentage, AbbVie, Curis, Bristol Myers Squibb, Geron, Novartis, Syros, and Servier. **A.M. Zeidan**: received research funding (institutional) from Bristol Myers Squibb/Celgene, a Bristol Myers Squibb Company, AbbVie, Astex Pharmaceuticals, Pfizer, Kura Oncology, MedImmune/AstraZeneca, Boehringer Ingelheim, Incyte Corporation, Takeda, Novartis, Shattuck Labs, Geron Corporation, Foran Pharmaceuticals, and Aprea Therapeutics; participated in advisory boards, had a consultancy with, or received honoraria from AbbVie, Pfizer, Bristol Myers Squibb/Celgene, a Bristol Myers Squibb Company, Jazz Pharmaceuticals, Incyte Corporation, Agios Pharmaceuticals, Servier, Boehringer Ingelheim, Novartis, Astellas Pharma, Daiichi Sankyo, Geron Corporation, Taiho Oncology, Seattle Genetics, BeyondSpring, Takeda, Ionis, Amgen, Janssen, Genentech, Epizyme, Syndax Pharmaceuticals, Gilead, Kura Oncology, Chiesi, ALX Oncology, BioCryst, Notable Labs, Orum Therapeutics, Mendus, Zentalis Pharmaceuticals, Regeneron, Syros Pharmaceuticals, Schrödinger, and Tyme Technologies; and served on clinical trial committees for Novartis, AbbVie, Gilead, Syros Pharmaceuticals, BioCryst, ALX Oncology, Kura Oncology, Geron Corporation, and Bristol Myers Squibb/Celgene, a Bristol Myers Squibb Company. **M. Alcantara, S. Shah, B. Williams,** and **J. Bailey**: *IQVIA*: current Employment. The remaining author(s) have nothing to report.

ISPOR 2026 | May 17-20, 2026 | Philadelphia, PA, USA

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