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Development of a Core Outcome Set for Myelodysplastic Syndromes – A Delphi Study from the EUMDS Registry Group

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Supplementary Table 1. Study characteristics

	Number of studies (%
Study type	
CTP 0	4 (0.9
CTP I	60 (14.1
CTP I & II	58 (13.6
CTP II	184 (43.3
CTP II & III	6 (1.4
CTP III	23 (5.4
CTP IV	8 (1.9
Observational study	9 (2.1
Not stated	73 (17.2
Examined intervention	
Medications	289 (68.0
Transplantation	28 (6.6
Drug & Transplantation	81 (19.1
Transfusion	6 (1.4
Procedures	6 (1.4
Health/Molecular prognostic values	15 (3.5
Sample size	
<30	98 (23.1
30-100	222 (52.2
>100	100 (23.5
Not stated	5 (1.2
Region	
North America	246 (57.9
South America	2 (0.5
Europe	66 (15.5
Asia	73 (17.2
Oceania	6 (1.4
Intercontinental	32 (7.5
Year	
2012	102 (24.0
2013	105 (24.7
2014	120 (28.2
2015	96 (22.6
2016	2 (0.5
Enrolled patients	
Minimum one MDS patient among others	358 (84.2
Solely MDS patients	67 (15.8

CTP: Clinical Trial Phase.

DELPHI SURVEY (First Round)

MDS-RIGHT - Providing the right care to the right patient with MyeloDysplastic Syndrome at the right time





Establishment of a new Core Outcome Set for MDS*

MDS-RIGHT project: Providing the right care to the right patient with MyeloDysplastic Syndrome at the right time*

The aim of our study is to identify and select the most relevant outcomes to be included in an MDS-specific core outcomes set (COS).

The COMET (Core Outcome Measures in Effectiveness Trials) initiative defined a COS as follows: "A core outcome set (COS) is an agreed minimum set of outcomes that should be measured and reported in all clinical trials, audits of practice or other forms of research for a specific condition" [1].

In the following section, we present 26 patient-relevant outcomes based on our previously performed MDS systematic literature search. For each outcome, please answer the following two categories:

- 1. Rank, on a scale from 1-9, the general importance of including the outcome in the COS for MDS patients:
 - 1-3 Low importance for decision-making
 - 4-6 Important, but not critical for decision-making
 - 7-9 Critical for decision-making
- 2. For each outcome, select the preferred/target MDS patient group/intervention and study type.

The survey will be used to identify and select important and critical outcomes. In further steps, these selected outcomes will be discussed and operationalized in detail in an expert panel.

Additional information: https://mds-europe.eu/right http://www.comet-initiative.org/ Technical support: mdsonlinesurvey@gmail.com

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Establishment of a new Core Outcome Set for MDS

Welcome to the MDS Delphi survey.

Please complete the following questionnaire (participant's general information) and proceed to the next section.

*Required

Mark only one square.

General information from the participant:

1. Choose your work/p	ractice country*.							
□ Austria	□ France	□ Italy	□ Romania					
□ Croatia	□ Germany	□ Netherlands	□ Serbia					
□ Czech Republic	□ Greece	□ Poland	□ Spain					
□ Denmark	□ Israel	□ Portugal	□ Sweden					
□ United Kingdom								
2. Gender*								
□ Male □	Female							
3. Age*								
4. Specialty*								
□ Geriatric Medicine	□ Hematology	□ Oncolo	ogy					
□ Hematology & Oncolo	ogy Internal Medic	ine Others						
5. Work experience*								
□ <5 years	□ 5-10 years	□ >10 ye	ars					
6. Experience with MDS patients*								
□ <5 years	□ 5-10 years	□ >10 ye	ars					

Please fill out both question categories for all outcomes. A general/non-MDS specific outcome definition has been provided for several of the outcomes.

Scale 1-9:

1-3 Low importance for decision-making

4-6 Importar 7-9 Critical f	•			r decisi	on-mal	king				
Overall sur	vival									
General definit	tion: "Th	e length	of time	from eith	ner the d	ate of dia	agnosis o	or the sta	art of tr	eatment for a disease,
that patients d	iagnose	d with tl	ne disea	se are s	till alive"	[2]. Dea	th is con	sidered	irrespe	ective of the cause.*
Mark only one	numbe	r								
Low importance for decision- making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk	group,	interv	ention	and stu	idy type	e where	e this o	utcome	e is m	ost relevant
□ Very low □ Intermedi □ High and □ Supportiv □ Disease-r □ Hematop □ Clinical st □ Registry □ Daily prace □ Other:	ate ris very he there modify oietic s tudy	k (IPS) igh risl apy ing the	S-R >3 〈(IPSS	-4,5) S-R 4,5	,					
Quality of I	ife									
Inclusion of the	e outcor	ne into	the gene	eral MDS	COS:*					
Mark only one	numbe	r								
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk	group,	interv	ention	and stu	ıdy type	e where	e this o	utcome	e is m	ost relevant
□ Very low □ Intermedi □ High and □ Supportiv □ Disease-r □ Hematop □ Clinical st □ Registry	ate ris very he thera modify oietic s	k (IPS) igh risl apy ing the	S-R >3 〈(IPSS	-4,5) S-R 4,5	·					

□ Daily prac	tice									
□ Other: _										
Duration of	hospit	talizatio	on							
Inclusion of the	outcom	e into the	e genera	II MDS C	COS:*					
Mark only one	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk	group,	interve	ntion a	nd stud	ly type	where	this ou	tcome	is m	ost relevant
□ Very low a □ Intermedia □ High and □ Supportive □ Disease-n □ Hematope □ Clinical st □ Registry □ Daily prace □ Other:	ate risk very hig e thera nodifyir pietic st udy etice	(IPSS-gh risk py ng thera em cell	·R >3-4 (IPSS-I apy	,5) R 4,5->	•					
Cytogenetic	-									
Inclusion of the		e into the	e genera	II MDS C	OS:*					
Mark only one	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk (group, i	interver	ntion ar	nd stud	y type	where t	this out	come i	s mo	st relevant
□ Very low a □ Intermedia □ High and □ Supportive □ Disease-n □ Hematope □ Clinical st □ Registry □ Daily prace	ate risk very hig e thera nodifyir pietic st udy	(IPSS-gh risk py ng thera	·R >3-4 (IPSS-I apy	,5) R 4,5->	•					
□ Other: _										

Hematological improvement

making

General definiti	ion: Hae	matologi	cal impr	ovemen	ts of cyto	openias.	*			
Mark only one	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk (group, i	interver	ntion ai	nd stud	ly type	where	this out	come i	s ma	st relevant
□ Very low a	and low	risk (II	PSS-R	0-3)						
□ Intermedia	ate risk	(IPSS-	R >3-4	1,5)						
□ High and	very hig	gh risk	(IPSS-	R 4,5->	>6,5)					
□ Supportive	e thera	ру								
□ Disease-n	nodifyir	ng thera	ару							
□ Hematopo	pietic st	em cell	l transp	olantati	on					
□ Clinical st	udy									
□ Registry										
□ Daily prac	tice									
□ Other:										
Response/F	Remiss	ion								
General definit	ion: Ren	nission i	n terms	of comp	plete rer	mission,	partial re	emission	and/	or marrow remission
etc.*										
Mark only one	number									
Low										Critical
importance for decision-	1	2	3	4	5	6	7	8	9	for decision- making
making										making
Patient risk of	group, i	interver	ntion ai	nd stud	ly type	where	this out	come i	s mo	st relevant
□ Very low a	and low	risk (II	PSS-R	0-3)						
□ Intermedia	ate risk	(IPSS-	R >3-4	1,5)						
□ High and	very hiç	gh risk	(IPSS-	R 4,5->	>6,5)					
□ Supportive	e thera	ру								
□ Disease-n	nodifyir	ng thera	ару							
□ Hematopo	oietic st	em cel	l transp	olantati	on					
□ Clinical st	udy									
□ Registry										
□ Daily prac	tice									
□ Other:										
Time to res	ponse									
Inclusion of the										
	outcom	e into the	e genera	al MDS (COS:*					
Mark only one		e into the	e genera	al MDS (COS:*					
Mark only one Low		e into th	e genera	al MDS (COS:*					Critical

making

Patient risk	group,	interv	ention	and stu	dy type	e where	e this o	utcome	is mo	ost relevan	t
□ Very low □ Intermed □ High and	iate risl very h	k (IPS igh risl	S-R >3	-4,5)	->6,5)						
□ Supportiv											
 □ Disease-modifying therapy □ Hematopoietic stem cell transplantation 											
□ Hematop□ Clinical s		stem ce	eli trans	spianta	lion						
□ Registry	luuy										
□ Daily pra	ctice										
□ Other:	000										
-											
Overall res	sponse										
General defin	ition: Sui	mmary o	of compl	ete, part	ial, marr	ow and/	or hema	tological	respor	nses.*	
Mark only one	e numbei	r									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making	
Patient risk	k group	, interv	ention	and st	udy typ	e wher	re this c	outcom	e is m	ost relevar	nt
□ Very low			•	,							
□ Intermed		•			. C E\						
☐ High and	•	•	K (IPSS	5-R 4,5	->6,5)						
□ Supportiv□ Disease-			rany								
□ Hematop	•	-		solanta	tion						
□ Clinical s			on train	эргагна							
□ Registry	,										
□ Daily pra	ctice										
□ Other:											
Safety											
General defin	nition: S	afety c	an inclu	ıde: adv	erse ev	vents, la	aboratory	/ evalua	ations,	vital signs,	physical
examinations											
Mark only one	e numbei	r									
Low importance for decision- making	1	2	3	4	5	6	7	8	9	Critical for decision- making	
Patient risk	group,	interv	ention a	and stu	dy type	e where	e this o	utcome	is m	ost relevan	t
□ Very low	and lov	w risk ((IPSS-I	R 0-3)							
□ Intermediate risk (IPSS-R >3-4,5)											
□ High and	very h	igh risl	k (IPSS	S-R 4,5	->6,5)						
□ Supportiv	ve thera	ару									

 □ Disease-modifying therapy □ Hematopoietic stem cell transplantation □ Clinical study □ Registry □ Daily practice □ Other:
Adverse event
General definition: Any occurrence or worsening of an undesirable or unintended sign, symptom (including
an abnormal laboratory finding), or disease temporally associated with the use of a medicinal
product/procedure, whether or not related to the medicinal product/procedure [4].*
Mark only one number
Low importance 1 2 3 4 5 6 7 8 9 for decision-making
Patient risk group, intervention and study type where this outcome is most relevant
□ Very low and low risk (IPSS-R 0-3) □ Intermediate risk (IPSS-R >3-4,5) □ High and very high risk (IPSS-R 4,5->6,5) □ Supportive therapy □ Disease-modifying therapy □ Hematopoietic stem cell transplantation □ Clinical study □ Registry □ Daily practice □ Other:
Infectious event
Inclusion of the outcome into the general MDS COS:*
Mark only one number
Low importance 1 2 3 4 5 6 7 8 9 for decision-making
Patient risk group, intervention and study type where this outcome is most relevant
 □ Very low and low risk (IPSS-R 0-3) □ Intermediate risk (IPSS-R >3-4,5) □ High and very high risk (IPSS-R 4,5->6,5) □ Supportive therapy □ Disease-modifying therapy □ Hematopoietic stem cell transplantation □ Clinical study □ Registry □ Daily practice □ Other:

Iron overload

Mark only one Low	numhai									
Low	TUTTIOG	r								
importance for decision- making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk g	group,	interve	ention a	and stu	idy typ	e where	e this o	utcome	e is m	ost relevant
□ Very low a□ Intermedia□ High and v□ Supportive	ate risl very h	k (IPSS igh risl	S-R >3	-4,5) [^]	->6,5)					
□ Disease-m			rany							
□ Hematopo	-	-		splanta	tion					
□ Clinical stu				•						
□ Registry	Ĭ									
□ Daily prac	tice									
□ Other:				_						
Secondary I	marhi	-1:4								
Joodinaar y	HOIDI	aity								
-		•	ence of	morbiditi	ies after	the time	of diagn	osing a	nd/or tr	eating the patient wi
-		•	ence of I	morbiditi	ies after	the time	of diagn	osing a	nd/or tr	eating the patient wi
General definiti	on: The	e occurr	ence of I	morbiditi	ies after	the time	of diagn	osing a	nd/or tr	eating the patient wi
General definiti	on: The	e occurr	ence of r	morbiditi 4	ies after	the time	of diagn	osing a	nd/or tr 9	eating the patient wi Critical for decision- making
General definiti MDS* Mark only one in the control of the control	on: The	e occurr	3	4	5	6	7	8	9	Critical for decision- making
General definiti MDS* Mark only one I Low importance for decision-making	on: The	e occurr	3	4	5	6	7	8	9	Critical for decision- making

General definition: Iron overload indicates excess of iron in the body from any cause. This can be defined by

Need for supportive therapy

for decision-

making

General definiti	on: The	necessity	y of impl	ementin	g suppor	tive thera	apy amoi	ng MDS	patie	nts (e.g., transfusions
of erythrocytes	and/or t	hromboc	ytes, int	erventio	nal G-C	SF, antib	iotic ther	apy etc.) [6]. <mark>*</mark>	
Mark only one	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk g	group, i	nterver	ntion ai	nd stud	ly type	where t	this out	come i	s mo	st relevant
□ Very low a □ Intermedia □ High and v □ Supportive □ Disease-n □ Hematopo □ Clinical sto □ Registry □ Daily pracc □ Other:	ate risk very hig therap nodifyin vietic st udy	(IPSS-gh risk (py ng thera	R >3-4 (IPSS- apy	l,5) R 4,5->	•					
Treatment-r	elated	morta	lity							
Inclusion of the	outcom	e into the	e genera	al MDS (COS:*					
Mark only one	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk g	group, i	nterver	ntion ai	nd stud	ly type	where t	this out	come i	s mo	st relevant
□ Very low a □ Intermedia □ High and v □ Supportive □ Disease-m □ Hematopo □ Clinical str □ Registry □ Daily prac □ Other:	ate risk very hig theral nodifyir vietic st udy	(IPSS-gh risk (py ng thera	R >3-4 (IPSS- apy	l,5) R 4,5->						
Acute/Chro	nic ara	ift-vers	us-ho	st dise	ase					
Inclusion of the	_									
Mark only one			<u> </u>							
Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision-

making

Patient risk g	iroup, i	nterver	ıtıon ar	ia stua _.	y type i	wnere i	inis out	come is	s mo	st reievant
□ Very low a □ Intermedia □ High and v □ Supportive □ Disease-m	and low ate risk very high therap nodifyin	risk (IF (IPSS- gh risk (Dy g thera	PSS-R R >3-4 (IPSS-I	0-3) .,5) R 4,5->	6,5)		341		•	2
□ Hematopo		em ceii	transp	nantatio	on					
□ Clinical stu	uay									
□ Registry	lioo									
□ Daily pract	uce									
□ Other:										
Frank france										
Event-free s										
		•				_				eatment for a disease
until experienci	ng a part	icular gr	oup of pr	edefined	d events	(e.g., fra	cture, inf	ection, la	abora	tory test abnormality,
particular kind of	of progre	ssion, d	eath fror	n any ca	use) [7].	*				
Mark only one i	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk g	group, i	nterver	ntion ar	nd stud	y type	where t	this out	come is	s mo	st relevant
	te risk very high very high very high very hodifying ietic structure. survivon: The ogression eatment,	(IPSS-gh risk (Dy g therate cell length of the cell	R >3-4 (IPSS-I	,5) R 4,5-> lantation	on the dated	Ū				eatment for a disease tional line of therapy,
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making

Patient risk group, intervention and study type where this outcome is most relevant
□ Very low and low risk (IPSS-R 0-3)
□ Intermediate risk (IPSS-R >3-4,5)
☐ High and very high risk (IPSS-R 4,5->6,5)
□ Supportive therapy
□ Disease-modifying therapy
□ Hematopoietic stem cell transplantation
□ Clinical study
□ Registry
□ Daily practice
□ Other:
Li Other.
Disease free survival
Disease-free survival
General definition: The length of time from either the date of diagnosis or the start of treatment for a disease
until recurrence of the disease, diagnosing other diseases (related/not related to the primary disease of
interest) or death from any cause [9].*
Mark only one number
Low importance 1 2 3 4 5 6 7 8 9 for decision-
for decision-
making Patient risk group, intervention and study type where this outcome is most relevant
Patient risk group, intervention and study type where this outcome is most relevant
□ Very low and low risk (IPSS-R 0-3)
□ Intermediate risk (IPSS-R >3-4,5)
□ High and very high risk (IPSS-R 4,5->6,5)
□ Supportive therapy
□ Disease-modifying therapy
□ Hematopoietic stem cell transplantation
□ Clinical study
□ Registry
□ Daily practice
□ Other:
Relapse-free survival
General definition: The length of time from either the date of diagnosis or the start of treatment for a disease
until disease recurrence or death from any cause [10].*
Mark only one number
Low Critical importance 1 2 3 4 5 6 7 8 9 for decision-
for decision- making making
Patient risk group, intervention and study type where this outcome is most relevant
□ Very low and low risk (IPSS-R 0-3)
□ Intermediate risk (IPSS-R >3-4,5)

☐ High and ☐ Supportive ☐ Disease-r ☐ Hematope ☐ Clinical st ☐ Registry ☐ Daily prace ☐ Other:	e thera nodifyir pietic st udy	py ng thera	ару		,					
Progression	n-free s	surviva	al .							
General definit	ion: The	length o	f time fro	om eithe	r the dat	e of diag	nosis or	the start	of tre	eatment for a disease
until disease p	rogressio	on or dea	ath, irres	pective	of the ca	use [11]	*			
Mark only one	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk (group, i	intervei	ntion ai	nd stud	ly type	where	this out	come is	s ma	st relevant
□ Very low a □ Intermedia □ High and □ Supportive □ Disease-r □ Hematope □ Clinical st □ Registry □ Daily prace □ Other: Time to processors	ate risk very hig e thera nodifyir pietic st udy etice	(IPSS-gh risk py ng thera cel	-R >3-4 (IPSS- apy	ŀ,5) [°] R 4,5->	ŕ					
General definit	ion: The	length o	f time fro	om eithe	r the dat	e of diag	nosis or	the start	of tre	eatment for a disease
until disease p	rogressio	on or dea	ath due t	o diseas	se progre	ession [1	1].*			
Mark only one	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk	group, i	intervei	ntion ai	nd stud	ly type	where	this out	come is	s mo	st relevant
□ Very low a □ Intermedia □ High and □ Supportive □ Disease-r □ Hematopo	ate risk very hiç e thera nodifyir	(IPSS-gh risk py ng thera	-R >3-4 (IPSS- apy	ŀ,5) R 4,5->	ŕ					

☐ Clinical st☐ Registry☐ Daily prac☐ Other:☐	·			,						
Performand	e statu	IS								
General definit	ion: The	perform	ance sta	atus desc	cribes th	e status	of symp	toms and	d fun	ctions with respect to
ambulatory sta	tus and r	need for	care [12	.]. *						
Mark only one	number									
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk	group, i	nterver	ntion ar	nd stud	y type	where	this out	come is	s ma	st relevant
instrumental a	ate risk very hig e therap modifyin pietic st udy etice activiti ion: Functivities of chold, pro	(IPSS-gh risk by ag thera em cell es ctional a of daily li	R >3-4 (IPSS- apy I transp	include a	on activities es funct	ions which	ch are es	ssential t	o live	dressing, eating) and a autonomously (e.g., n, taking prescribed
Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
Patient risk	group, i	interver	ntion ai	nd stud	y type	where	this out	come is	s mo	st relevant
□ Very low a □ Intermedia □ High and □ Supportiv □ Disease-r □ Hematopo □ Clinical st □ Registry	ate risk very hiç e therap nodifyin pietic st	(IPSS-gh risk py ng thera	·R >3-4 (IPSS- apy	.,5) R 4,5->	•					

			_						
tranef	usion	s nar i	nationt						
		uie gen		000.					
1	2	3	4	5	6	7	8	9	Critical for decision- making
group,	interv	ention	and stu	ıdy typ	e wher	e this o	utcome	e is m	ost relevant
ate risl very h e thera nodifyi pietic s	k (IPS: igh ris apy ng the	S-R >3 k (IPSS erapy	3-4,5) S-R 4,5	,					
ctice			_						
	ooietic	c stem	cell tra	anspla	ntatioi	1			
ematoj			cell tra	-	ntatioi	า			
ematoj	ne into			-	ntatioi	า			
e mato j	ne into			-	ntatioi 6	1 7	8	9	Critical for decision- making
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Additiona	l outcomes?		
Comment	ts/suggestions?		

Thank you for taking the time to fill out this survey!

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- you have read and understood the above mentioned project description and instructions
- you voluntarily agree to participate
- you are at least 18 years of age
- you fill and submit the survey only once
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References

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DELPHI SURVEY (Second Round)

MDS-RIGHT - Providing the right care to the right patient with MyeloDysplastic Syndrome at the right time





Establishment of a new Core Outcome Set for MDS* (Second round)

MDS-RIGHT project: Providing the right care to the right patient with MyeloDysplastic Syndrome at the right time*

The aim of our study is to identify and select the most relevant outcomes to be included in an MDS-specific core outcomes set (COS).

The COMET (Core Outcome Measures in Effectiveness Trials) initiative defined a COS as follows: "A core outcome set (COS) is an agreed minimum set of outcomes that should be measured and reported in all clinical trials, audits of practice or other forms of research for a specific condition" [1].

In this second round, we present 26 patient- and clinically-relevant outcomes based on our previously performed MDS systematic literature search and one additional outcome, which has been included as a suggestion of at least two participants ("Secondary malignancy"). For each outcome, please answer the following question:

- 1. Rank, on a scale from 1-9, the general importance of including the outcome in the COS for MDS patients:
 - 1-3 Low importance for decision-making
 - 4-6 Important, but not critical for decision-making
 - 7-9 Critical for decision-making

This second round should enable the participants to reconsider their opinion based on the group's results from the first round. The results of the survey will be used to identify and select important and critical outcomes. In further steps, these selected outcomes will be discussed and operationalized in detail in an expert panel.

Additional information: https://mds-europe.eu/right http://www.comet-initiative.org/ Technical support: mdsonlinesurvey@gmail.com

*This work was supported by MDS-RIGHT: "Providing the right care to the right patient with MyeloDysplastic Syndrome at the right time". The project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No. 634789.

Establishment of a new Core Outcome Set for MDS

(Second round)

Welcome to the MDS Delphi survey.

Please complete the following questionnaire (participant's general information) and proceed to the next section.

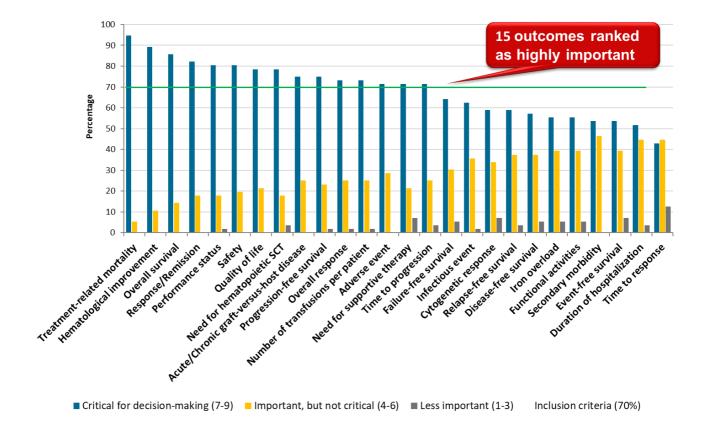
*Required

Mark only one square.

General information	from the	partici	pant:
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1. Choose your work/p	ractice country*.							
□ Austria	□ France	□ Italy	□ Romania					
□ Croatia	□ Germany	□ Netherlands	□ Serbia					
□ Czech Republic	□ Greece	□ Poland	□ Spain					
□ Denmark	□ Israel	□ Portugal	□ Sweden					
□ United Kingdom								
2. Gender*								
□ Male □	Female							
3. Age*								
4. Specialty*								
☐ Geriatric Medicine	□ Hematology	□ Oncolo	ogy					
□ Hematology & Oncolo	ogy 🗆 Internal Medic	ine □ Others						
5. Work experience*								
□ <5 years	□ 5-10 years	□ >10 ye	ars					
6. Experience with MD	S patients*							
□ <5 years	□ 5-10 years	□ >10 ye	ars					
7. Did you participate i	7. Did you participate in the previous, first round of the MDS-survey?*							
□ Yes □ No								

Figure 1 Results from the first round of the MDS-COS survey



Please fill out the questions for all outcomes. A general/non-MDS specific outcome definition has been provided for several of the outcomes.

Sca	le	1	-9
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- 1-3 Low importance for decision-making
- 4-6 Important, but not critical for decision-making
- 7-9 Critical for decision-making

Overall survival

General definition: "The length of time from either the date of diagnosis or the start of treatment for a disease, that patients diagnosed with the disease are still alive" [2]. Death is considered irrespective of the cause.*

Mark only one number

Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
------------------------------------	---	---	---	---	---	---	---	---	---	-------------------------------------

Quality of life

Inclusion of the outcome into the general MDS COS:*

Mark only one number

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										3

Duration of hospitalization

Inclusion of the outcome into the general MDS COS:*

Mark only one number

Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making	
------------------------------------	---	---	---	---	---	---	---	---	---	-------------------------------------	--

Cytogenetic response

Inclusion of the outcome into the general MDS COS:*

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										

^{*}Required

Hematological improvement

General definition: Haematological improvements of cytopenias.*

Mark only one number

Low Critical importance 2 5 6 1 3 4 7 8 9 for decisionfor decisionmaking making

Response/Remission

General definition: Remission in terms of complete remission, partial remission and/or marrow remission etc.*

Mark only one number

Low Critical importance 1 2 3 4 5 6 7 8 9 for decisionfor decisionmaking making

Time to response

Inclusion of the outcome into the general MDS COS:*

Mark only one number

Low importance 1 2 3 4 5 6 7 8 9 Critical for decision-making

Overall response

General definition: Summary of complete, partial, marrow and/or hematological responses.*

Mark only one number

Low Critical importance 1 2 3 4 5 6 7 8 9 for decisionfor decisionmaking making

Safety

General definition: Safety can include: adverse events, laboratory evaluations, vital signs, physical examinations etc. [3].*

Mark only one number

Low importance for decision-making 1 2 3 4 5 6 7 8 9 Critical for decision-making

Adverse event

General definition: Any occurrence or worsening of an undesirable or unintended sign, symptom (including an abnormal laboratory finding), or disease temporally associated with the use of a medicinal product/procedure, whether or not related to the medicinal product/procedure [4].*

Mark only one number

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										3

Infectious event

Inclusion of the outcome into the general MDS COS:*

Mark only one number

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										· ·

Iron overload

General definition: Iron overload indicates excess of iron in the body from any cause. This can be defined by evaluation of serum ferritin and transferrin saturation or if available by magnetic resonance imaging [5].*

Mark only one number

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										_

Secondary morbidity

General definition: The occurrence of morbidities after the time of diagnosing and/or treating the patient with MDS*

Mark only one number

Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
------------------------------------	---	---	---	---	---	---	---	---	---	-------------------------------------

Need for supportive therapy

General definition: The necessity of implementing supportive therapy among MDS patients (e.g., transfusions of erythrocytes and/or thrombocytes, interventional G-CSF, antibiotic therapy etc.) [6].*

Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
------------------------------------	---	---	---	---	---	---	---	---	---	-------------------------------------

Treatment-related mortality

Inclusion of the outcome into the general MDS COS:*

Mark only one number

Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
------------------------------------	---	---	---	---	---	---	---	---	---	-------------------------------------

Acute/Chronic graft-versus-host disease

Inclusion of the outcome into the general MDS COS:*

Mark only one number

Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
------------------------------------	---	---	---	---	---	---	---	---	---	-------------------------------------

Event-free survival

General definition: The length of time from either the date of diagnosis or the start of treatment for a disease until experiencing a particular group of predefined events (e.g., fracture, infection, laboratory test abnormality, particular kind of progression, death from any cause) [7].*

Mark only one number

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										

Failure-free survival

General definition: The length of time from either the date of diagnosis or the start of treatment for a disease until disease progression, recurrence during predefined line treatment, absence of additional line of therapy, death during treatment, death from any cause etc. [8].*

Mark only one number

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										making

Disease-free survival

General definition: The length of time from either the date of diagnosis or the start of treatment for a disease until recurrence of the disease, diagnosing other diseases (related/not related to the primary disease of interest) or death from any cause [9].*

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										

Relapse-free survival

General definition: The length of time from either the date of diagnosis or the start of treatment for a disease until disease recurrence or death from any cause [10].*

Mark only one number

Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
------------------------------------	---	---	---	---	---	---	---	---	---	-------------------------------------

Progression-free survival

General definition: The length of time from either the date of diagnosis or the start of treatment for a disease until disease progression or death, irrespective of the cause [11].*

Mark only one number

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										

Time to progression

General definition: The length of time from either the date of diagnosis or the start of treatment for a disease until disease progression or death due to disease progression [11].*

Mark only one number

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										

Performance status

General definition: The performance status describes the status of symptoms and functions with respect to ambulatory status and need for care [12].*

Mark only one number

Low importance for decision-making	1	2	3	4	5	6	7	8	9	Critical for decision- making
------------------------------------	---	---	---	---	---	---	---	---	---	-------------------------------------

Functional activities

General definition: Functional activities include activities of daily living (e.g., bathing, dressing, eating) and instrumental activities of daily living which defines functions which are essential to live autonomously (e.g., making household, preparing meals, making phone calls or other communication, taking prescribed medications etc.) [13].*

Low importance for decision-	1	2	3	4	5	6	7	8	9	Critical for decision- making
making										

outco	ome into	the gene	eral MDS	S COS:*						
numb	er									
1	2	3	4	5	6	7	8	9	Critical for decision- making	
emato	poieti	c stem	cell tra	anspla	ntation	7				
outco	me into	the gene	eral MDS	COS:*						
numb	er									
1	2	3	4	5	6	7	8	9	Critical for decision- making	
maliç	gnancy	,								
ion: Se	econdary	/ maligna	ancy cou	ıld be de	fined as	a new ca	ancer tha	at has	occurred as a result of	
nent w	ith radia	tion or ch	nemothe	rapy [14].*					
numb	er									
1	2	3	4	5	6	7	8	9	Critical for decision- making	
outco	omes?							_		
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Number of transfusions per patient

Comments/suggestions?

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References

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