

# Myelodysplastic Syndrome (MDS): What is it and How Does it Impact on Quality of Life?

**Dr. Robert Klaassen**



**Associate Professor**  
Department of Pediatrics  
University of Ottawa



**Pediatric Hematologist/Oncologist**  
Department of Pediatrics, Division of  
Hematology/Oncology  
Children's Hospital of Eastern Ontario

# Outline

- What is MDS?
- Classification
- Treatment
- What is QOL?
- How Can You Measure QOL?
- Measuring Quality of Life
- Conclusion

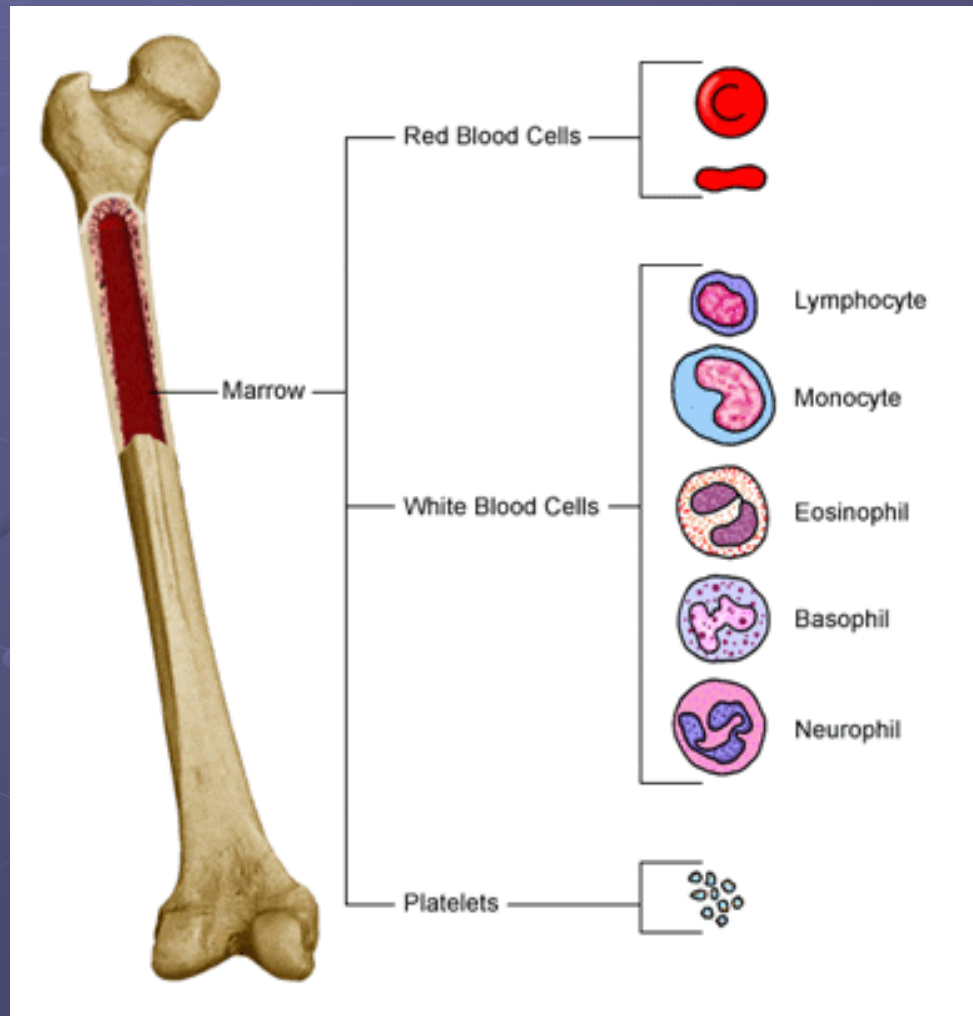
# What Is MDS?

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  - an acquired problem (not inherited)
  - that results in ineffective blood production
  - due to a clonal disorder of the blood stem cells
  - considered to be a premalignant condition that often progresses to leukemia over the span of a number of years

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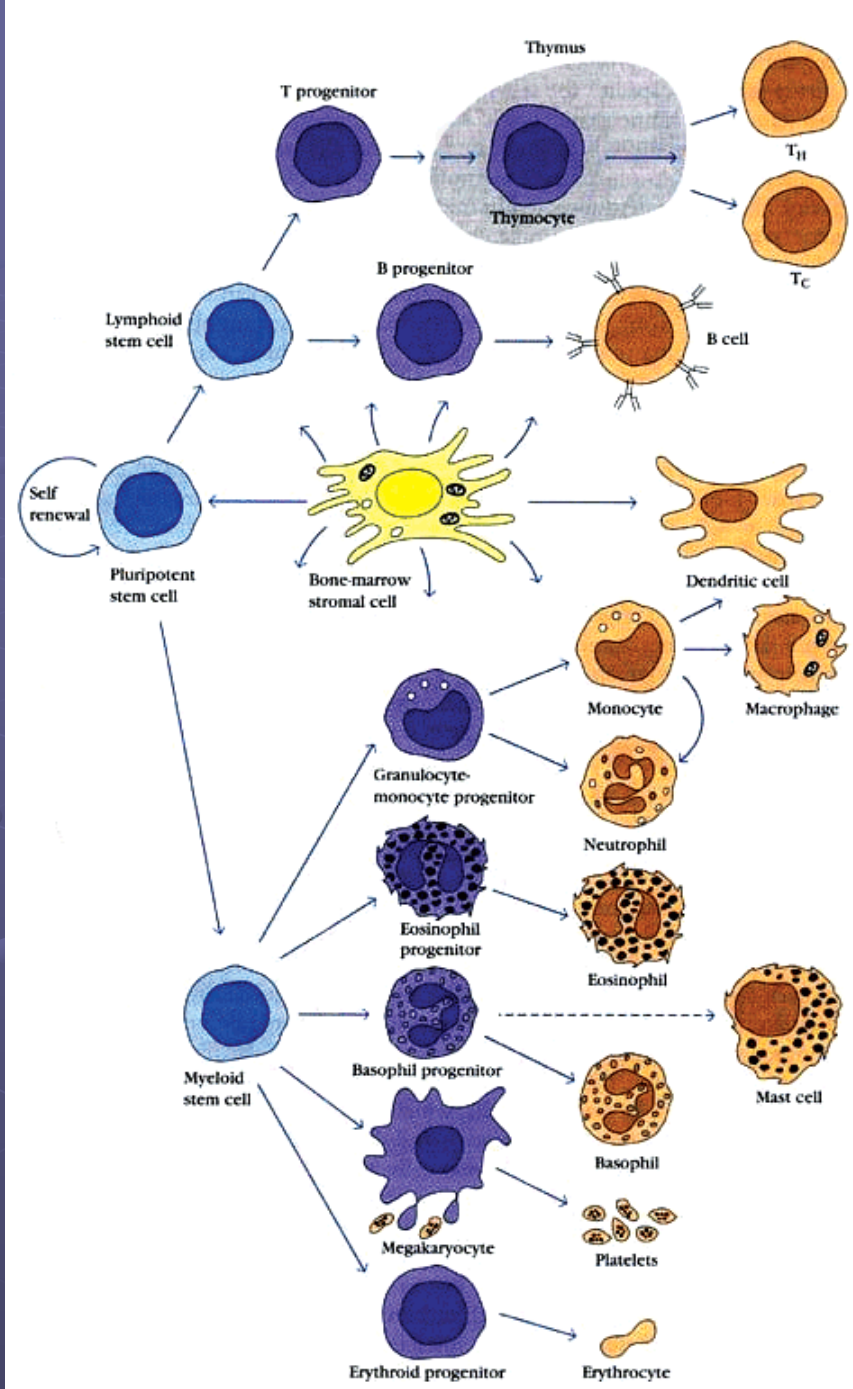
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# Classification

## CCC system

- Category- idiopathic, syndrome, treatment
- Cytology
  - Single/multiple cell types
  - With or without abnormal shapes
  - With or without extra blasts
- Cytogenetics



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# Classification

**Table 3. Etiologic Classification of 877 Patients With Pediatric Myelodysplastic Syndrome**

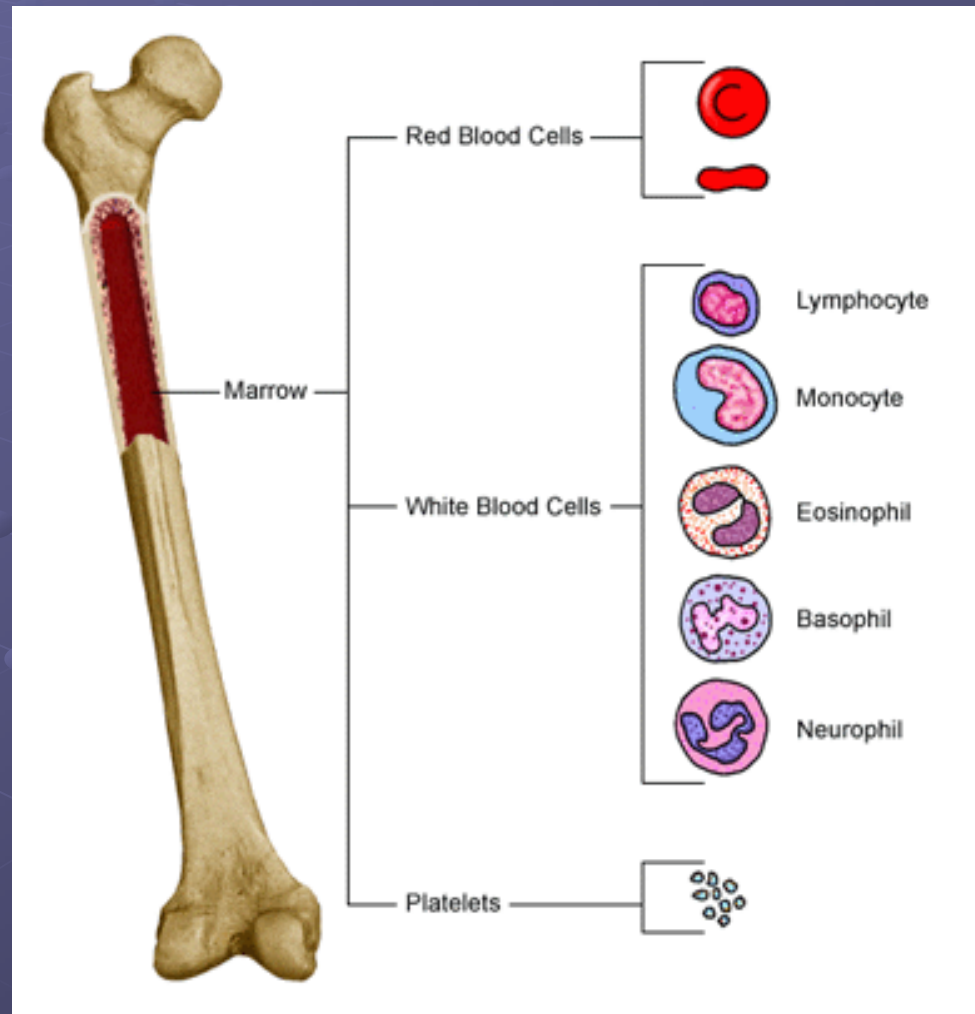
<b>Etiologic Category</b>	<b>% (No./Total No.)</b>
1. Idiopathic/primary/de novo	68.7 (609/877)
2. Therapy related*	7.4 (66/887)
3. Constitutional/syndrome related	23.9 (212/877)
Down syndrome	7.6 (56/740)
Fanconi anemia	3.9 (26/659)
Neurofibromatosis 1	2.3 (20/887)
Familial	2.0 (18/887)
Mitochondrial cytopathies	1.4 (12/887)
Shwachman-Diamond syndrome	1.2 (11/887)
Other inherited bone marrow failure	1.3 (13/887)
Miscellaneous anomalies†	6.3 (56/887)

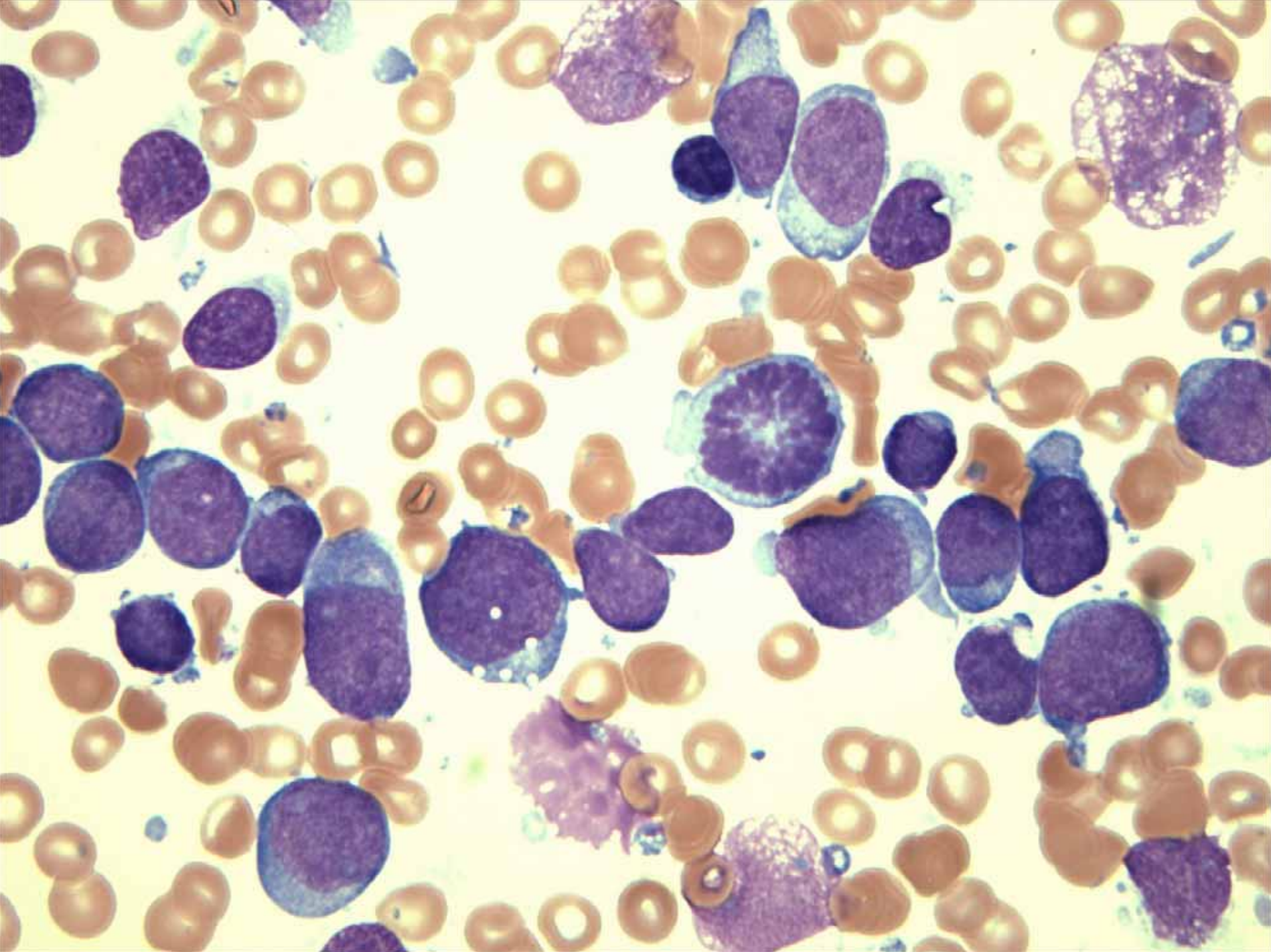
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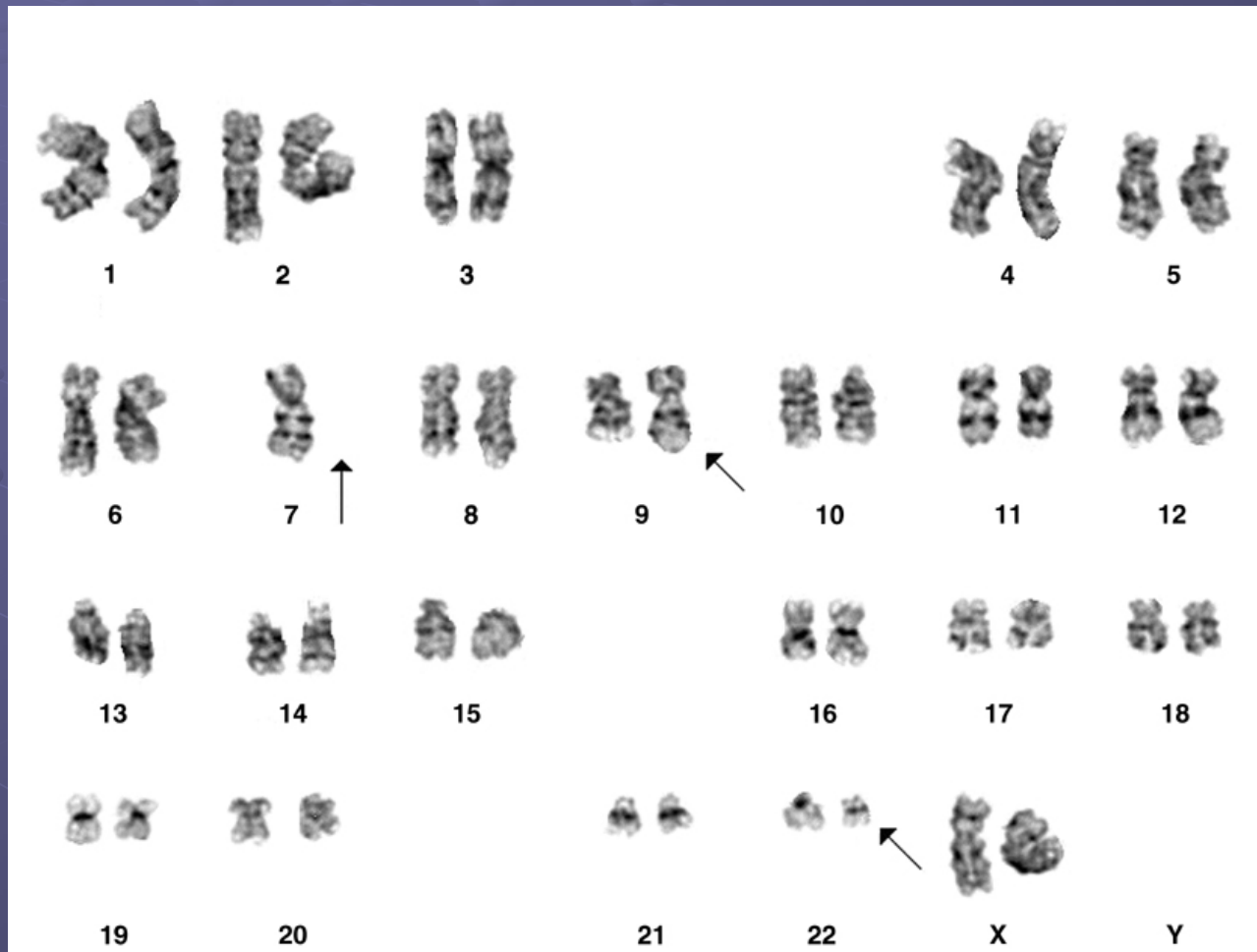
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# Classification



# Treatment

- Supportive care
  - Aggressively treat infections
  - Transfusions if needed – primarily red cell
- BMT is the only curative option, but need to have a donor
- Other approved treatments in adults, such as 5 azacitidine (Vidaza), decitabine (Dacogen) and lenalidomide (Revlimid) have very little experience in children

# Treatment

## ● International BMT registry results

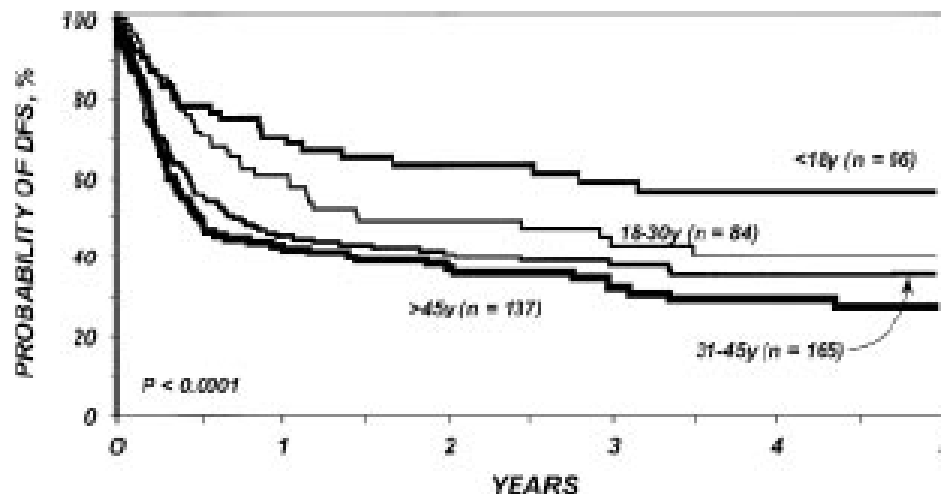


Figure 4. Probability of DFS after bone marrow transplantation for myelodysplasia, according to age at transplantation.

# Treatment

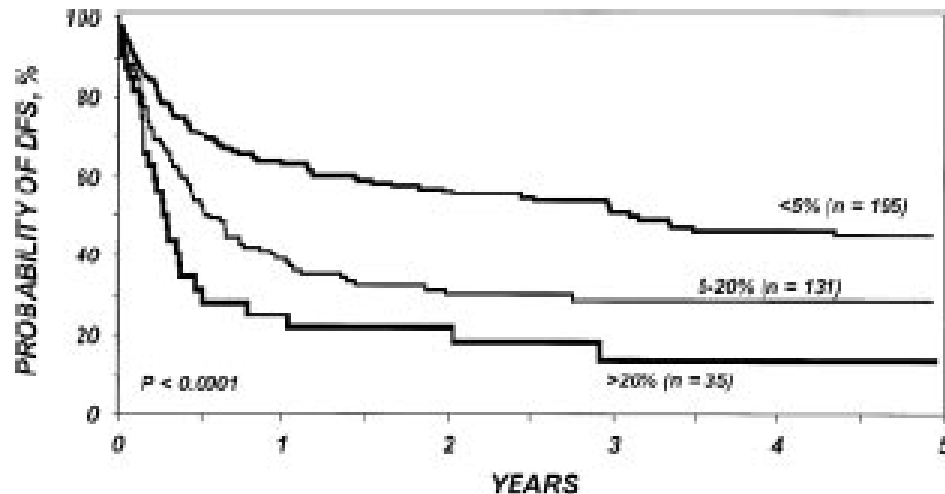


Figure 2. Probability of DFS after bone marrow transplantation for myelodysplasia, according to blasts in bone marrow before transplantation.

- Seattle group reported 74% success with BMT in 27 children with good type MDS

# Measuring Quality of Life

- MDS is a chronic disorder which can have a significant impact on a patient's ability to function normally
- In particular patients who need ongoing red blood cell transfusion with iron removal therapy can be significantly affected
- We decide to try to measure this issue

# What is QOL?

*“Individuals’ perceptions of their position in life, in the context of the culture and value systems in which they live, and in relation to their goals, expectations, standards, and concerns.”*

“The gap between ones life expectations and life experiences.”

(WHOQOL group, 1993 & 1995)

**Quality of Life Research 1993 ; 2 : 153 – 159**  
**Social Science and Medicine 1995 ; 41 : 1403 - 1409**



# What is “Health-Related” Quality of Life?

## Health-Related Quality of Life

- often refers to the specific impact a condition or illness may have on overall QoL
- does not typically include non-medical constraints such as religion and finance
- is often used interchangeably with QoL

# How Can You Measure QOL?

- There is no gold standard
- Typically a questionnaire is used
- A simple way is to just have the patient plot it on a visual analogue scale (VAS)
- A more detailed picture comes from multiple questions looking at different areas (domains), such as mental, physical or social functioning



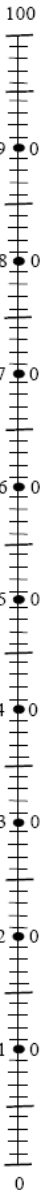
# EuroQOL- EQ-5D

To help people say how good or bad their state of health is, we have drawn a scale (rather like a thermometer) on which the best state you can imagine is marked 100 and the worst state you can imagine is marked 0.

We would like you to indicate on this scale how good or bad your own health is today, in your opinion. Please do this by drawing a line from the box below to whichever point on the scale indicates how good or bad your state of health is today.

**Your own  
state of health  
today**

Best  
imaginable  
state of health



Worst  
imaginable  
state of health

# Measuring Quality of Life

## Objectives

- Generate potential items – using expert group, interviews patients/families
- Select the most important items to be included in the measure
- Pilot the newly developed measure in a new group of patients

# Measuring Quality of Life

Initial Meeting of Investigators in Toronto (Phase 1)



Item Generation Interviews/Focus Groups

15 thalassemia children age 7.0–18  
25 parents (10 child age 2-6.9, 15  
child age 7.0–18)

15 thalassemia adults

15 MDS adults



Toronto Meeting to Develop Initial Measures



Cognitive Debriefing of the Developed Measures (Phase 2)

15 thalassemia children age 7.0–18  
25 parents (10 child age 2-6.9, 15  
child age 7.0–18)

15 thalassemia adults

15 MDS adults



Consensus Meeting in Toronto to Refine Measures

# Measuring Quality of Life

			Patient #1	Patient #2	Patient #3 M-M
			1	2	3
Category	Expert Item	Item	Importance Rank	Importance Rank	Importance Rank
		Ranking#			
Schooling	Does having Thalassemia and having blood transfusions affect your academic performance?	1	1	.	7
	Does having Thalassemia and having blood transfusions affect your social life at school ( i.e. dances, parties, special events)	2	1	.	5
Anxiety	Do you worry about your future?	3	8	.	10
Sleep	I have trouble sleeping because of my infusions	4	7	.	8
Self esteem	Do you feel different from other children?	5	6	.	6
Disclosure	Have you worried about telling a boyfriend/girlfriend about your Thalassemia?	6	.	.	18
Pain	To what extent do you feel that physical pain prevents you from doing what you need to do?	7	5	.	19
Parent/ Child interaction	I fight with my parent (s) about my treatment	8	.	.	17
	I wish my parent/parents would allow me to make more decisions about my treatment	9	.	.	16



# Progress to Date

- A total of 31 children, 43 parents and 30 adults with thalassemia have been interviewed
- We have talked to 11 adults with MDS and plan to interview a further 14 over the next two months
- We are meeting in December at the beginning of the ASH meeting in San Francisco to rap the study up and plan the next phase

# TranQo!



# TranQo!

First, for each item please tell us how often these things happened to you in the past 4 weeks...

We would like to know something about how you are feeling...(physically)	Never	Almost never	Some-times	Often	Always
1. I have had trouble sleeping...	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
2. I was free of pain or discomfort...	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
3. Pain prevented me from doing what I needed to do....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
4. I had enough energy for daily activities...	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
5. I was limited in my ability to do the kind of moderate work or activities I take part in...	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
6. I was limited in my ability to do the kind of vigorous work or activities I take part in...	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

# Validation Study

- In San Francisco we will be bringing together thalassemia doctors from across North America – so far Vancouver, Montreal, Boston, Oakland, New York have agreed to be study sites
- We will test the TranQol to make sure it is reliable and is valid (measures what is supposed to measure)

# Conclusion

- MDS is a serious problem which affects blood production and can lead to leukemia
- Treatment in children is limited primarily to BMT, but if done in the early phases of the disease has a good success rate.
- MDS can have a significant impact on quality of life.
- We have created a tool to try to better understand quality of life.
- A future study is planned to test that the TranQol is reliable and works properly

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## Investigators:

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- Dr Ian Quirt, UHN
- Dr. Melissa Forgie, Ottawa Hospital
- Dr. Melanie Kirby, HSC
- Dr. Rena Buckstein, Toronto-Sunnybrook
- Dr. Shabbir Alibhai, UHN
- Dr. Issac Odame, HSC
- Durhane Wong-Rieger, Anemia Institute
- Dr. Victor Blanchette, HSC

## Research Coordinators:

- Manuela Merelles-Pulcini (Toronto)
- Nagina Parmar (Toronto)
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