

Effect of splenectomy, stem cell transplantation, gene therapy or no definite treatment on frequency of disease related complications and physician-perceived quality of life in WAS

unique patient identifier (<u>not</u> patient initials):
<u>year</u> of birth:

center:

GENERAL DISEASE DATA

date of diagnosis:				
WAS mutation:				
WASP expression (by flow <input type="checkbox"/> /by western <input type="checkbox"/>	absent <input type="checkbox"/>	decreased <input type="checkbox"/>	normal <input type="checkbox"/>	not done <input type="checkbox"/>
No procedure (splenectomy, SCT, GT) <input type="checkbox"/>				
Reason:				

DISEASE DATA BEFORE FIRST PROCEDURE (or at last follow-up, if no procedure performed)

average platelet count (before any procedure):				
absolute lymphocyte count (/μl) (before any procedure):				
WAS score** (before first procedure):		date of scoring:		
quality of life (QOL) (before any procedure)	very good <input type="checkbox"/>	good <input type="checkbox"/>	limited <input type="checkbox"/>	unacceptable <input type="checkbox"/>
Karnofsky/Lansky Score* (before any procedure):				

PROCEDURES

splenectomy	no <input type="checkbox"/>	yes <input type="checkbox"/>	date:	
average platelet count after splenectomy:				
change of QOL(after splenectomy):	much improved <input type="checkbox"/>	improved <input type="checkbox"/>	unchanged <input type="checkbox"/>	worsened <input type="checkbox"/>
Karnofsky/Lansky Score* (after splenectomy and reasonable time of recovery):				

stem cell transplantation(SCT)	no <input type="checkbox"/>	yes <input type="checkbox"/>	date:		center:		
donor:	MSD <input type="checkbox"/>	MUD <input type="checkbox"/>	MMFD <input type="checkbox"/>	UCB <input type="checkbox"/>			
conditioning:							
GVHD-prophylaxis:							
acute GVHD <input type="checkbox"/>	grade:		chronic GVHD	resolved <input type="checkbox"/>	ongoing <input type="checkbox"/>	not severe <input type="checkbox"/>	severe <input type="checkbox"/>
chimerism	> 90% donor <input type="checkbox"/>		50-90% donor <input type="checkbox"/>	10-50% donor <input type="checkbox"/>	< 10% donor <input type="checkbox"/>		
average platelet count after SCT:							
change of QOL (after SCT):	much improved <input type="checkbox"/>	improved <input type="checkbox"/>	unchanged <input type="checkbox"/>	worsened <input type="checkbox"/>			
Karnofsky/Lansky Score* (after SCT and reasonable time of recovery):							

gene therapy (GT)	no <input type="checkbox"/>	yes <input type="checkbox"/>	date:		center:		
type of vector:							
outcome:	corrected cell populations (%)	lymphocytes:		myeloid:		platelets:	
	post-GT leukemia	no <input type="checkbox"/>		yes <input type="checkbox"/>		type:	
average platelet count after GT:							
change of QOL(after GT):	much improved <input type="checkbox"/>	improved <input type="checkbox"/>	unchanged <input type="checkbox"/>	worsened <input type="checkbox"/>			
Karnofsky/Lansky Score* (after GT and reasonable time of recovery):							

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DISEASE RELATED COMPLICATIONS (BEFORE, AFTER, DURING OR WITHOUT ANY PROCEDURE!)

Please list only serious events (requiring hospitalization) e. g. sepsis, meningitis, pneumonia requiring respiratory support, systemic viral/fungal infections, serious bleeding episodes (in particular intracranial, GI, requiring RBC-transfusions) and all fatal events, **regardless of whether they occurred before or after any procedure.**

bacterial infections			
date (month/year):	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>

viral infections			
date (month/year):	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>

fungal infections			
date (month/year):	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>
date:	type:	organism:	fatal <input type="checkbox"/>

bleeding			
date (month/year):	type:	requiring transfusion of (e.g. RBC, platelets):	fatal <input type="checkbox"/>
date:	type:	requiring transfusion of:	fatal <input type="checkbox"/>
date:	type:	requiring transfusion of:	fatal <input type="checkbox"/>
date:	type:	requiring transfusion of:	fatal <input type="checkbox"/>

malignancy (lymphoma, leukemia, EBV-LPD, other)			
date (month/year):	type:	treatment:	fatal <input type="checkbox"/>
date:	type:	treatment:	fatal <input type="checkbox"/>
date:	type:	treatment:	fatal <input type="checkbox"/>

autoimmunity (hemolytic anemia, neutropenia, ITP, vasculitis, other)			
date (month/year):	type:	treatment:	fatal <input type="checkbox"/>
date:	type:	treatment:	fatal <input type="checkbox"/>
date:	type:	treatment:	fatal <input type="checkbox"/>

other serious event			
date (month/year):	type:	treatment:	fatal <input type="checkbox"/>
date:	type:	treatment:	fatal <input type="checkbox"/>
date:	type:	treatment:	fatal <input type="checkbox"/>

IMMUNIZATIONS (BEFORE, AFTER, DURING OR WITHOUT ANY PROCEDURE!)

pneumovax	<input type="checkbox"/>	dates:	no complications <input type="checkbox"/>	protective titers <input type="checkbox"/>
prevnar	<input type="checkbox"/>	dates:	no complications <input type="checkbox"/>	protective titers <input type="checkbox"/>
meningococcal	<input type="checkbox"/>	dates:	no complications <input type="checkbox"/>	protective titers <input type="checkbox"/>
H.i.-B	<input type="checkbox"/>	dates:	no complications <input type="checkbox"/>	protective titers <input type="checkbox"/>
MMR	<input type="checkbox"/>	dates:	no complications <input type="checkbox"/>	protective titers <input type="checkbox"/>
Varivax	<input type="checkbox"/>	dates:	no complications <input type="checkbox"/>	protective titers <input type="checkbox"/>
Rota	<input type="checkbox"/>	dates:	no complications <input type="checkbox"/>	protective titers <input type="checkbox"/>

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OTHER TREATMENT (BEFORE OR AFTER ANY PROCEDURE!)

antibiotics:	no <input type="checkbox"/>	continuously/prophylactically <input type="checkbox"/>	sporadic/prn <input type="checkbox"/>	both <input type="checkbox"/>
list types and dose of antibiotics				
1.	from _____ till _____		still on <input type="checkbox"/>	
2.	from _____ till _____		still on <input type="checkbox"/>	
3.	from _____ till _____		still on <input type="checkbox"/>	

antifungals:	no <input type="checkbox"/>	continuously/prophylactically <input type="checkbox"/>	sporadic/prn <input type="checkbox"/>	both <input type="checkbox"/>
list types and dose of antifungals				
1.	from _____ till _____		still on <input type="checkbox"/>	
2.	from _____ till _____		still on <input type="checkbox"/>	

antivirals:	no <input type="checkbox"/>	continuously/prophylactically <input type="checkbox"/>	sporadic/prn <input type="checkbox"/>	both <input type="checkbox"/>
list types and dose of antivirals				
1.	from _____ till _____		still on <input type="checkbox"/>	
2.	from _____ till _____		still on <input type="checkbox"/>	

steroids:	no <input type="checkbox"/>	continuously/prophylactically <input type="checkbox"/>	sporadic/prn <input type="checkbox"/>	both <input type="checkbox"/>
list types and dose of steroids				
1.	from _____ till _____		still on <input type="checkbox"/>	
2.	from _____ till _____		still on <input type="checkbox"/>	

IVIG/SCIG:	no <input type="checkbox"/>	continuously/prophylactically <input type="checkbox"/>	sporadic/prn <input type="checkbox"/>	both <input type="checkbox"/>
list dose, frequency and route (iv/subcut)				
1.	from _____ till _____		still on <input type="checkbox"/>	
2.	from _____ till _____		still on <input type="checkbox"/>	

transfusions:	date (month/year)	quantity	reason
RBC			
platelets			

CURRENT STATUS

patient alive <input type="checkbox"/>	last follow-up (date):			
QOL (at last follow-up):	very good <input type="checkbox"/>	good <input type="checkbox"/>	limited <input type="checkbox"/>	unacceptable <input type="checkbox"/>
Karnofsky/Lansky Score* (at last follow-up):				
patient dead <input type="checkbox"/>	date of death:		cause of death:	

local IRB issues	exempt <input type="checkbox"/>	approved <input type="checkbox"/>
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COMMENTS

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SUBMITTING PHYSICIAN

name:	or stamp:
e-mail:	
address:	

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Table 1. Karnofsky/Lansky Scale

Karnofsky Scale (recipient age ≥ 16 years)		Lansky Scale (recipient age <16 years)	
Able to carry on normal activity; no special care is needed		Able to carry on normal activity; no special care is needed	
100	Normal, no complaints, no evidence of disease	100	Fully active
90	Able to carry on normal activity	90	Minor restriction in physically strenuous play
80	Normal activity with effort	80	Restricted in strenuous play, tires more easily, otherwise active
Unable to work, able to live at home cares for most personal needs, a varying amount of assistance is needed		Mild to moderate restriction	
70	Cares for self, unable to carry on normal activity or to do active work	70	Both greater restrictions of, and less time spent in active play
60	Requires occasional assistance but is able to care for most needs	60	Ambulatory up to 50% of time, limited active play with assistance/supervision
50	Requires considerable assistance and frequent medical care	50	Considerable assistance required for any active play, fully able to engage in quiet play
Unable to care for self, requires equivalent of institutional or hospital care, disease may be progressing rapidly		Moderate to severe restriction	
40	Disabled, requires special care and assistance	40	Able to initiate quite activities
30	Severely disabled, hospitalization indicated, although death not imminent	30	Needs considerable assistance for quiet activity
20	Very sick, hospitalization necessary	20	Limited to very passive activity initiated by others (e.g., TV)
10	Moribund, fatal process progressing rapidly	10	Completely disabled, not even passive play

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** WAS scoring system:

Score	XLN 0	iXLT <1	XLT		Classic WAS		
			1	2	3	4	5
Thrombocytopenia	-	-/+	+	+	+	+	+
Small platelets	-	+	+	+	+	+	+
Eczema	-	-	-	(+)	+	++	-/(+)/+/+++
Immunodeficiency	-/(+)	-	-/(+)	(+)	+	+	(+)/+
Infections	-/(+)	-	-	(+)	+	+/+++	-/(+)/+/+++
Autoimmunity and/or malignancy	-	-	-	-	-	-	+
Congenital neutropenia	+	-	-	-	-	-	-
Myelodysplasia	-/+	-	-	-	-	-	-

Scoring system: -/(+), absent or mild; -/+, intermittent thrombocytopenia, possible myelodysplasia; (+), mild, transient eczema or mild, infrequent infections not resulting in sequelae; +, thrombocytopenia, persistent but therapy-responsive eczema, and recurrent infections requiring antibiotics and often intravenous immunoglobulin prophylaxis; ++, eczema that is difficult to control and severe, life-threatening infections. WAS, Wiskott-Aldrich syndrome; XLN, X-linked neutropenia; XLT, X-linked thrombocytopenia. Modified with permission from [6].

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