

Wiskott-Aldrich Syndrome Pre-HSCT Data

Registry Use Only

Sequence
 Number:

--	--

Date
 Received:

--	--

CIBMTR Center Number:

CIBMTR Recipient ID:

Has this patient's data been previously reported to USIDNET?

1 yes → USIDNET ID:

2 no


Today's Date: / /
 Month Day Year

Date of HSCT for which this form is being completed: / /
 Month Day Year

HSCT type: autologous allogeneic, unrelated allogeneic, related syngeneic (identical twin)

Product type: marrow PBSC cord blood other product, specify: _____

This form must be accompanied by Form 2000 – Recipient Baseline Data. All information in the box above, including the date, should be identical with the corresponding Form 2000. Information should come from an actual examination by the Transplant Center physician, or the physician who is following the recipient pre-HSCT, or abstraction of the recipient's medical records.

Questions followed by the symbol  indicate additional information necessary to complete the question is referenced in the forms instruction manual.

If this is a report of a second or subsequent transplant, check here and continue with question 79.

Disease Assessment at Diagnosis

Disease assessment at diagnosis includes disease characteristics observed within six weeks of the date of diagnosis.

1. What was the date of diagnosis of Wiskott-Aldrich Syndrome (WAS)? / /
 Month Day Year

2. Specify the WAS defining (diagnostic) criteria:

1 definitive → (definitive diagnosis defined as male patient with congenital thrombocytopenia (< 70,000 platelets/mm³), small platelets, and at least one of the additional criteria at questions 3–6)

Specify all additional criteria for definitive WAS diagnosis:	
3. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Mutation in WASp
4. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Absent WASp mRNA on northern blot analysis of lymphocytes
5. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Absent WASp protein in lymphocytes
6. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Maternal cousins, uncles or nephews with small platelets and thrombocytopenia

2 probable → (probable diagnosis defined as male patient with congenital thrombocytopenia (< 70,000 platelets/mm³), small platelets, and at least one of the additional criteria at questions 7–10)

Specify all additional criteria for probable / possible WAS diagnosis:	
7. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Eczema
8. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Abnormal antibody response to polysaccharide antigens
9. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Autoimmune disease(s)
10. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Lymphoma / leukemia

3 possible → (possible diagnosis defined as male patient with congenital thrombocytopenia (< 70,000 platelets/mm³) and small platelets; or with splenectomy for thrombocytopenia and at least one of the additional criteria at questions 7–10)

Mail this form to your designated campus (Milwaukee or Minneapolis). Retain the original at the transplant center.

CIBMTR Center Number: [][][][][][]

CIBMTR Recipient ID: [][][][][][][][][][][][][][][][]

11. Was a WAS gene mutation identified?

- 1 yes
- 2 no

12. Specify gene mutation identified:

- 1 nucleotides affected (e.g., 361C>T)
- 2 predicted amino acid change (e.g., W14R)

13. Was a WASp protein expressed?

- 1 yes
- 2 no
- 3 unknown

Laboratory Studies at Diagnosis

Report findings prior to any first treatment of Wiskott-Aldrich syndrome.

14. Date CBC tested: [][] [][] 20 [][] (testing done within 6 weeks of diagnosis)
Month Day Year

Specify units:

15. WBC: [][][][][][] • [][] 1 $\times 10^9/L$ ($\times 10^3/mm^3$) not tested
2 $\times 10^6/L$

16. Lymphocytes: [][] % not tested

17. Eosinophils: [][] % not tested

18. Polymorphonuclear leukocytes (PMN): [][] % not tested

19. Hemoglobin: [][][][][] • [][] 1 g/dL not tested transfused RBC < 30 days from date of test
2 g/L
3 mmol/L

20. Platelets: [][][][][][][][] 1 $\times 10^9/L$ ($\times 10^3/mm^3$) not tested transfused platelets < 7 days from date of test
2 $\times 10^6/L$

21. Mean platelet volume: [][] • [][] fl not tested

Immunoglobulin Analysis

Specify the following quantitative immunoglobulins measured prior to any disease treatment:

Value: [][][][][][] • [][] Specify units: 1 mg/dL 23. [][] [][] [][][][] not tested
2 g/dL Date tested: Month Day Year
3 g/L

24. IgM: [][][][][][] • [][] 1 mg/dL 25. [][] [][] [][][][] not tested
2 g/dL
3 g/L

26. IgA: [][][][][][] • [][] 1 mg/dL 27. [][] [][] [][][][] not tested
2 g/dL
3 g/L

28. IgE: [][][][] IU/mL 29. [][] [][] [][][][] not tested

30. Did the recipient receive supplemental intravenous immunoglobulins (IVIg) prior to any first treatment of WAS?

- 1 yes
- 2 no
- 3 unknown

31. Was therapy ongoing within one month of immunoglobulin testing?

- 1 yes
- 2 no

CIBMTR Center Number:

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Lymphocyte Analysis

Specify the following lymphocyte analyses performed prior to any disease treatment:

32. Were lymphocyte analyses performed? yes no

- 1 yes
- 2 no

33. Date of most recent testing performed:	<input type="text"/>	<input type="text"/>	<input type="text"/>	Month	Day	Year
34. Absolute lymphocyte count:	<input type="text"/>	cells / μL (cells / mm^3)				
	% of total lymphocytes:	Value:	Specify units:			
35. CD3 (T cells):	<input type="text"/>	- or -	<input type="text"/>	1 <input type="checkbox"/> $\times 10^9/\text{L}$	<input type="checkbox"/> not tested	
				($\times 10^3/\text{mm}^3$)		
36. CD4 (T helper cells):	<input type="text"/>	- or -	<input type="text"/>	2 <input type="checkbox"/> $\times 10^6/\text{L}$	<input type="checkbox"/> not tested	
				1 <input type="checkbox"/> $\times 10^9/\text{L}$	<input type="checkbox"/> not tested	
				($\times 10^3/\text{mm}^3$)		
37. CD8 (cytotoxic T cells):	<input type="text"/>	- or -	<input type="text"/>	2 <input type="checkbox"/> $\times 10^6/\text{L}$	<input type="checkbox"/> not tested	
				1 <input type="checkbox"/> $\times 10^9/\text{L}$	<input type="checkbox"/> not tested	
				($\times 10^3/\text{mm}^3$)		
38. CD20 (B lymphocyte cells):	<input type="text"/>	- or -	<input type="text"/>	2 <input type="checkbox"/> $\times 10^6/\text{L}$	<input type="checkbox"/> not tested	
				1 <input type="checkbox"/> $\times 10^9/\text{L}$	<input type="checkbox"/> not tested	
				($\times 10^3/\text{mm}^3$)		
39. CD56 (natural killer (NK) cells):	<input type="text"/>	- or -	<input type="text"/>	2 <input type="checkbox"/> $\times 10^6/\text{L}$	<input type="checkbox"/> not tested	
				1 <input type="checkbox"/> $\times 10^9/\text{L}$	<input type="checkbox"/> not tested	
				($\times 10^3/\text{mm}^3$)		
40. CD4+ / CD45RA+ (naive T cells):	<input type="text"/>	- or -	<input type="text"/>	2 <input type="checkbox"/> $\times 10^6/\text{L}$	<input type="checkbox"/> not tested	
				1 <input type="checkbox"/> $\times 10^9/\text{L}$	<input type="checkbox"/> not tested	
				($\times 10^3/\text{mm}^3$)		
41. CD4+ / CD45RO+ (memory T cells):	<input type="text"/>	- or -	<input type="text"/>	2 <input type="checkbox"/> $\times 10^6/\text{L}$	<input type="checkbox"/> not tested	
				1 <input type="checkbox"/> $\times 10^9/\text{L}$	<input type="checkbox"/> not tested	
				($\times 10^3/\text{mm}^3$)		

Clinical Features Assessed between Diagnosis and the Start of the Preparative Regimen

Infections Identified between Diagnosis and the Start of the Preparative Regimen

Specify the presence of all clinically significant infections identified between diagnosis and the start of the preparative regimen. If any given infection was identified, use the Codes for Commonly Reported Organisms on the following page to report the organism present. Only report an organism once, even if it was identified at the same site in subsequent infections.

For questions 66–78, also report any fungal infections in the Form 2000 – Recipient Baseline Data beginning at question 163.

Copy this chart to report more than three different infections identified at any one site; check here if additional pages are attached.

Site of infection?	First organism	Second organism	Third organism	Specify other organism
42. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Hepatitis	43. <input type="text"/>	44. <input type="text"/>	45. <input type="text"/>	46. _____
47. If hepatitis was present, was it a prominent feature of WAS?				
1 <input type="checkbox"/> yes				
2 <input type="checkbox"/> no				
48. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Meningitis / encephalitis	49. <input type="text"/>	50. <input type="text"/>	51. <input type="text"/>	52. _____
53. If meningitis / encephalitis was present, was it a prominent feature of WAS?				
1 <input type="checkbox"/> yes				
2 <input type="checkbox"/> no				

CIBMTR Center Number:

CIBMTR Recipient ID:

Site of infection? **First organism** **Second organism** **Third organism** **Specify other organism**

54. 1 yes 2 no Pneumonia → 55. 56. 57. 58. _____

59. If pneumonia was present, was it a prominent feature of WAS?
1 yes
2 no

60. 1 yes 2 no Severe or protracted diarrhea → 61. 62. 63. 64. _____

65. If diarrhea was present, was it a prominent feature of WAS?
1 yes
2 no

66. 1 yes 2 no Systemic infection → 67. 68. 69. 70. _____

71. If systemic infection was present, was it a prominent feature of WAS?
1 yes
2 no

72. 1 yes 2 no Other infection → 73. 74. 75. 76. _____

77. Specify other infection site: _____

78. If other infection was present, was it a prominent feature of WAS?
1 yes
2 no

Codes for Commonly Reported Organisms

Bacterial Infections			
121	Acinetobacter	104	Listeria
122	Actinomyces	150	Methylobacterium
123	Bacillus	151	Micrococcus, NOS
124	Bacteroides (gracillis, uniformis, vulgaris, other species)	112	Mycobacterium avium– intracellulare (MAC, MAI)
125	Bordetella pertussis (whooping cough)	174	Mycobacterium species (cheloneae, fortuitum, haemophilum, kansasii, mucogenicum)
126	Borrelia (Lyme disease)	110	Mycobacterium tuberculosis (tuberculosis, Koch bacillus)
127	Branhamella or Moraxella catarrhalis (other species)	175	Other mycobacterium, specify
128	Campylobacter (all species)	176	Mycobacterium, NOS
129	Capnocytophaga	105	Mycoplasma
171	Chlamydia pneumoniae	152	Neisseria (gonorrhoea, meningitidis, other species)
172	Other chlamydia, specify	106	Nocardia
113	Chlamydia, NOS	153	Pasteurella multocida
130	Citrobacter (freundii, other species)	154	Propionibacterium (acnes, avidum, granulosum, other species)
131	Clostridium (all species except difficile)	155	Proteus
132	Clostridium difficile	156	Pseudomonas (all species except cepacia & maltophilia)
173	Corynebacterium jeikeium	157	Pseudomonas or Burkholderia cepacia
133	Corynebacterium (all non-diphtheria species)	158	Pseudomonas or Stenotrophomonas or Xanthomonas maltophilia
101	Coxiella	159	Rhodococcus
134	Enterobacter	107	Rickettsia
177	Enterococcus, vancomycin resistant (VRE)	160	Salmonella (all species)
135	Enterococcus (all species)	161	Serratia marcescens
136	Escherichia (also E. coli)	162	Shigella
137	Flavimonas oryzihabitans	163	Staphylococcus, coagulase negative (not aureus)
138	Flavobacterium	164	Staphylococcus aureus
139	Fusobacterium	165	Staphylococcus, NOS
144	Haemophilus (all species, including influenzae)	166	Stomatococcus mucilaginosus
145	Helicobacter pylori	167	Streptococcus (all species except Enterococcus)
146	Klebsiella	178	Streptococcus pneumoniae
147	Lactobacillus (bulgaricus, acidophilus, other species)		
102	Legionella		
103	Leptospira		
148	Leptotrichia buccalis		
149	Leuconostoc (all species)		
Fungal Infections			
200	Candida, NOS	168	Treponema (syphilis)
201	Candida albicans	169	Vibrio (all species)
206	Candida guilliermondi	197	Multiple bacteria at a single site, specify bacterial codes
202	Candida krusei	198	Other bacteria, specify ‡
207	Candida lusitanae	501	Suspected atypical bacterial infection
203	Candida parapsilosis	502	Suspected bacterial infection
204	Candida tropicalis		
205	Candida (Torulopsis) glabrata		
209	Other Candida, specify ‡		
210	Aspergillus, NOS §		
211	Aspergillus flavus §		
212	Aspergillus fumigatus §		
213	Aspergillus niger §		
219	Other Aspergillus, specify ‡ §		
220	Cryptococcus species		
230	Fusarium species §		
261	Histoplasmosis		
240	Zygomycetes, NOS §		
241	Mucormycosis §		
242	Rhizopus §		
250	Yeast, NOS		
259	Other fungus, specify ‡		
260	Pneumocystis (PCP / PJP)		
503	Suspected fungal infection		
Viral Infections			
301	Herpes simplex (HSV1, HSV2)	304	Adenovirus
302	Varicella (herpes zoster, chicken pox)	305	Enterovirus (coxsackie, echo, polio)
303	Cytomegalovirus (CMV)	306	Hepatitis A (HAV)
		307	Hepatitis B (HBV, Australian antigen) †
		308	Hepatitis C (HCV) †
		309	HIV-1 (HTLV-III) ‡
		310	Influenza, NOS
		323	Influenza A
		324	Influenza B
		311	Measles (rubeola)
		312	Mumps
		313	Progressive multifocal leukoencephalopathy (PML)
		314	Respiratory syncytial virus (RSV)
		315	Rubella (German measles)
		316	Parainfluenza
		317	Human herpesvirus-6 (HHV-6)
		318	Epstein-Barr virus (EBV)
		319	Polyoma virus (BK virus, JC virus)
		320	Rotavirus
		321	Rhinovirus
		322	Human papilloma virus (HPV)
		329	Other virus, specify ‡
		504	Suspected viral infection
Parasitic Infections			
402	Toxoplasma		
403	Giardia		
404	Cryptosporidium		
409	Other parasite, specify ‡		
505	Suspected parasite infection		
Other Infections			
509	No organism identified		

‡ The codes for "other organism, specify" (codes 198, 209, 219, 259, 329 and 409) should rarely be needed; check with your microbiology lab or HSCT physician before using them.

§ For fungal infections marked with a section symbol (codes 210, 211, 212, 213, 219, 230, 240, 241, and 242), also complete a Fungal Infection (FNG) form.

† For hepatitis infections marked with a dagger symbol (codes 307 and 308), also complete a Hepatitis (HEP) form.

‡ For HIV infections marked with a currency symbol (code 309), also complete an HIV Infection (HIV) form.

* Do not report fever in the absence of infection. Report the most specific site of infection.

CIBMTR Center Number: [] [] [] [] [] [] [] [] [] []

CIBMTR Recipient ID: [] [] [] [] [] [] [] [] [] [] [] [] [] [] [] []

Clinical Status Between Diagnosis and the Preparative Regimen

79. Did the recipient undergo a splenectomy (between diagnosis and prior to the preparative regimen)?

- 1 yes
- 2 no
- 3 unknown

80. Specify the date the splenectomy was performed:

[] [] / [] [] / 20 [] []
Month Day Year

81. Platelets (after splenectomy):

[] [] [] [] [] [] [] []

- 1 x 10⁹/L (x 10³/mm³)
- 2 x 10⁶/L

- not tested
- transfused platelets < 7 days from date of test

82. Were thrombocytopenia (< 100 x 10⁹/L) and small platelets present without any other symptoms, clinical findings, or laboratory abnormalities attributable to WAS (between diagnosis and prior to the preparative regimen)?

- 1 yes
- 2 no
- 3 unknown

Specify thrombocytopenia in the Form 2000 — Recipient Baseline Data at questions 117–118

83. Was eczema present as a clinical feature (between diagnosis and prior to the preparative regimen)?

- 1 yes
- 2 no
- 3 unknown

84. Specify severity of eczema:

- 1 mild, transient
- 2 persistent but manageable
- 3 difficult to control

85. Was a coexisting malignancy present (between diagnosis and prior to the preparative regimen)?

- 1 yes
- 2 no
- 3 unknown

86. Specify malignancy: _____

Report malignancy in the Form 2000 — Recipient Baseline Data at questions 23–60

87. Did the recipient experience any of the following types of bleeding episodes (between diagnosis and prior to the preparative regimen)?

- 1 yes
- 2 no

Specify types of bleeding:

Bleeding episode(s) present?		If present, is the feature prominent?
88. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Epistaxis	→	89. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
90. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Upper GI hemorrhage	→	91. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
92. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Lower GI hemorrhage / rectal bleeding	→	93. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
94. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Hemarthrosis	→	95. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
96. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Hematuria	→	97. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
98. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Intracranial hemorrhage	→	99. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
100. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Oral	→	101. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
102. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Subcutaneous bleeding	→	103. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
104. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Subdural hematoma	→	105. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
106. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no Other bleeding	→	107. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no
		108. Specify other bleeding: _____

Report GI hemorrhage in the Form 2000 — Recipient Baseline Data at question 63

Report GU hemorrhage in the Form 2000 — Recipient Baseline Data at question 64

Report CNS hemorrhage in the Form 2000 — Recipient Baseline Data at question 65

CIBMTR Center Number:

CIBMTR Recipient ID:

109. Did the recipient experience any of the following autoimmune / inflammatory disorders (between diagnosis and prior to the preparative regimen?)

- 1 yes
2 no

Specify autoimmune / inflammatory disorders:			If present, is the feature prominent?	
Feature present?				
110. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Arthralgia	→	111. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
112. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Arthritis, chronic	→	113. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
114. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Autoimmune hemolytic anemia	→	115. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
116. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Idiopathic thrombocytopenic purpura (ITP)	→	117. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
118. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Inflammatory bowel disease	→	119. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
120. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Juvenile rheumatoid arthritis	→	121. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
122. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Nephritis	→	123. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
124. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Neutropenia	→	125. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
126. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Sclerosing cholangitis	→	127. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
128. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Vasculitis, cerebral	→	129. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
130. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Vasculitis, coronary	→	131. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
132. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Vasculitis, renal	→	133. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
134. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Vasculitis, skin	→	135. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
136. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Vasculitis, other	→	137. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
	138. Specify other vasculitis:	_____		
139. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Other disorder	→	140. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	
	141. Specify other disorder:	_____		

142. Were any biologic specimens collected for this recipient (between the date of diagnosis and the preparative regimen)?

- 1 yes
2 no
3 unknown

Specify if specimen(s) collected <u>and</u> available for future research:		
143. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	DNA	
144. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Epstein-Barr virus (EBV)-transformed B-cell line	
145. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Fibroblast cell line	
146. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Herpes virus saimiri-transformed T-cell line	
147. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Other T-cell line	
148. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Pathological specimen	→ 149. Specify pathological specimen(s): _____
150. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Peripheral blood mononuclear cells (PBMC), frozen	
151. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	RNA	→ 152. Specify RNA source: _____
153. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Serum (pre-IVIG)	
154. 1 <input type="checkbox"/> yes 2 <input type="checkbox"/> no	Other specimen	→ 155. Specify other specimen(s): _____

156. Signed: _____

Person completing form

Please print name: _____

Phone: (_____) _____

Fax: (_____) _____

E-mail address: _____