Form 2033 R3.0: Wiskott-Aldrich Syndrome Pre-HSCT Data

Center: CRID:

Key Fields

Sequence Number: _____

Date Received: __ __ __ __ - __ __- __ __

CIBMTR Center Number: _____

CIBMTR Recipient ID: _____

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

yes no

USIDNET ID: _____

Today's Date: __ __ __ __ - __ __- __ __

Date of HSCT for which this form is being completed: __ __ __ __ - __ __- __ __

HSCT type (check all that apply):

Autologous

Allogeneic, unrelated

Allogeneic, related

Syngeneic (identical twin)

Product type (check all that apply):

Marrow

PBSC

Cord blood

Other product

Specify: _____

If this is a report of a second or subsequent transplant, check here and continue with question 67.

Disease Assessment at Diagnosis

Questions: 1 - 13

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)? __ __ __ __ - __ __- __ __

2 Specify the WAS defining (diagnostic) criteria?

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)

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)

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)

Specify all additional criteria for definitive WAS diagnosis:

3 Mutation in WASp

yes no

4 Absent WASp mRNA on northern blot analysis of lymphocytes

yes no

5 Absent WASp protein in lymphocytes

yes no

6 Maternal cousins, uncles, or nephews with small platelets and thrombocytopenia

yes no

Specify all additional criteria for probable / possible WAS diagnosis:

7 Eczema

yes no

8 Abnormal antibody response to polysaccharide antigens

yes no

9 Autoimmune disease(s)

yes no

10 Lymphoma / Leukemia

yes no

11 Was a WAS gene mutation identified?

yes no

12 Specify gene mutation identified:

nucleotides affected (e.g., 361C>T)

predicted amino acid change (e.g., W14R)

13 Was a WASp protein expressed?

yes no Unknown

Laboratory Studies at Diagnosis

Questions: 14 - 41

14 Date CBC tested: (testing done within 6 weeks of diagnosis) __ __ __ __ - __ __- __ __

15 WBC: _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

WBC not tested

16 Lymphocytes: _____ % Lymphocytes not tested

17 Eosinophils: _____ % Eosinophils not tested

18 Polymorphonuclear leukocytes (PMN): _____ % Polymorphonuclear leukocytes (PMN) not tested

19 Hemoglobin: _____

g/dL g/L mmol/L

Hemoglobin not tested

transfused RBC < 30 days from date of test

20 Platelets: _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

Platelets not tested

transfused platelets < 7 days from date of test

21 Mean platelet volume: _____ fl Mean platelet volume not tested

Immunoglobulin Analysis

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

22 IgG: _____

mg/dL g/dL g/L

IgG not tested

23 Date tested: __ __ __ __ - __ __- __ __

24 IgM: _____

mg/dL g/dL g/L

IgM not tested

25 Date tested: __ __ __ __ - __ __- __ __

26 IgA: _____

mg/dL g/dL g/L

IgA not tested

27 Date tested: __ __ __ __ - __ __- __ __

28 IgE: _____ IU/mL IgE not tested

29 Date tested: __ __ __ __ - __ __- __ __

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

yes no Unknown

31 Was therapy ongoing within one month of immunoglobulin testing?

yes no

Lymphocyte Analysis

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

32 Were lymphocyte analyses performed?

yes no

33 Date of most recent testing performed: __ __ __ __ - __ __- __ __

34 Absolute lymphocyte count: _____ cells /ul (cells / mm^3)

35 CD3 (T cells) % of total lymphocytes _____ %

-- or --

CD3 (T cells) value _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

CD3 (T cells) not tested

36 CD4 (T helper cells) % of total lymphocytes _____ %

-- or --

CD4 (T helper cells) value _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

CD4 (T helper cells) not tested

37 CD8 (cytotoxic T cells) % of total lymphocytes _____ %

-- or --

CD8 (cytotoxic T cells) value _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

CD8 (cytotoxic T cells) not tested

38 CD20 (B lymphocyte cells) % of total lymphocytes _____ %

-- or --

CD20 (B lymphocyte cells) value _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

CD20 (B lymphocyte cells) not tested

39 CD56 (natural killer (NK) cells) % of total lymphocytes _____ %

-- or --

CD56 (natural killer (NK) cells) value _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

CD56 (natural killer (NK) cells) not tested

40 CD4+/CD45RA+ (naive T cells) % of total lymphocytes _____ %

-- or --

CD4+/CD45RA+ (naive T cells) value _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

CD4+ / CD45RA+ (naive T cells) not tested

41 CD4+/CD45RO+ (memory T cells) % of total lymphocytes _____ %

-- or --

CD4+/CD45RO+ (memory T cells) value _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

CD4+/CD45RO+ (memory T cells) not tested

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

Questions: 42 - 143

42 Site of infection: hepatitis

yes no

Hepatitis Infection Organism (1)

Questions: 43 - 44

43 Organism _____

44 Specify other organism : _____

45 If hepatitis was present, was it a prominent feature of WAS?

yes no

46 Site of infection: meningitis / encephalitis

yes no

Meningitis / Encephalitis Infection Organism (1)	Questions: 47 - 48

47 Organism _____

48 Specify other organism: _____

49 If meningitis / encephalitis was present, was it a prominent feature of WAS?

yes no

50 Site of infection: pneumonia

yes no

Pneumonia Infection Organism (1)	Questions: 51 - 52

51 Organism _____

52 Specify other organism : _____

53 If pneumonia was present, was it a prominent feature of WAS?

yes no

54 Site of infection: severe or protracted diarrhea

yes no

Severe or Protracted Diarrhea Infection Organism (1)	Questions: 55 - 56

55 Organism _____

56 Specify other organism: _____

57 If diarrhea was present, was it a prominent feature of WAS?

yes no

58 Site of infection: systemic infection

yes no

Systemic Infection Infection Organism (1)	Questions: 59 - 60

59 Organism _____

60 Specify other organism : _____

61 If systemic infection was present, was it a prominent feature of WAS?

yes no

62 Site of infection: other infection

yes no

Other Infection Organism (1)	Questions: 63 - 65

63 Organism _____

64 Specify other organism : _____

65 Specify other infection site: _____

66 If other infection was present, was it a prominent feature of WAS?

yes no

Clinical Status between Diagnosis and the Preparative Regimen

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

yes no Unknown

68 Specify the date the splenectomy was performed: __ __ __ __ - __ __- __ __

69 Platelets (after splenectomy): _____

x 10^9/L (x 10^3/mm^3)

x 10^6/L

Platelets (after splenectomy) not tested

transfused platelets < 7 days from date of test

70 Were thrombocytopenia (<100 x 10^9/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)?

yes **-Specify thrombocytopenia in the Form 2000 Recipient Baseline Data at questions 117-118**

no

Unknown

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

yes no Unknown

72 Specify severity of eczema:

mild, transient

persistent but manageable

difficult to control

73 Was a coexisiting malignancy present (between diagnosis and prior to the preparative regimen)?

yes no Unknown

74 Specify malignancy: _____

Report malignancy in the Form 2000 — Recipient Baseline Data at questions 22–60

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

yes no

76 Is epistaxis present?

yes no

77 Is epistaxis prominent?

yes no

78 Is upper GI hemorrhage present?

yes no

79 Is upper GI hemorrhage prominent?

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

no

80 Is lower GI hemorrhage/rectal bleeding present?

yes no

81 Is lower GI hemorrhage/rectal bleeding prominent?

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

no

82 Is hemarthrosis present?

yes no

83 Is hemarthrosis prominent?

yes no

84 Is hematuria present?

yes no

85 Is hematuria prominent?

yes no

86 Is intracranial hemorrhage present?

yes no

87 Is intracranial hemorrhage prominent?

yes **-Report CNS hemorrhage in the Form 2000 Recipient Baseline Data at question 65**

no

88 Is oral bleeding present?

yes no

89 Is oral bleeding prominent?

yes no

90 Is subcutaneous bleeding present?

yes no

91 Is subcutaneous bleeding prominent?

yes no

92 Is subdural hematoma present?

yes no

93 Is subdural hematoma prominent?

yes no

94 Is other bleeding present?

yes no

95 Is other bleeding prominent?

yes no

96 Specify other bleeding: _____

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

yes no

Specify autoimmune / inflammatory disorders:

98 Is arthralgia present?

yes no

99 Is arthralgia prominent?

yes no

100 Is chronic arthritis present?

yes no

101 Is chronic arthritis prominent?

yes no

102 Is autoimmune hemolytic anemia present?

yes no

103 Is autoimmune hemolytic anemia prominent?

yes no

104 Is idiopathic thrombocytopenic purpura (ITP) present?

yes no

105 Is idiopathic thrombocytopenic purpura (ITP) prominent?

yes no

106 Is inflammatory bowel disease present?

yes no

107 Is inflammatory bowel disease prominent?

yes no

108 Is juvenile rheumatoid arthritis present?

yes no

109 Is juvenille rheumatoid arthritis prominent?

yes no

110 Is nephritis present?

yes no

111 Is nephritis prominent?

yes no

112 Is neutropenia present?

yes no

113 Is neutropenia prominent?

yes no

114 Is sclerosing cholangitis present?

yes no

115 Is sclerosing cholangitis prominent?

yes no

116 Is cerebral vasculitis present?

yes no

117 Is cerebral vasculitis present?

yes no

118 Is coronary vasculitis present?

yes no

119 Is coronary vasculitis prominent?

yes no

120 Is renal vasculitis present?

yes no

121 Is renal vasculitis prominent?

yes no

122 Is skin vasculitis present?

yes no

123 Is skin vasculitis prominent?

yes no

124 Is other vasculitis present?

yes no

125 Is other vasculitis prominent?

yes no

126 Specify other vasculitis: _____

127 Other disorder

yes no

128 Is any other disorder prominent?

yes no

129 Specify other disorder: _____

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

yes no Unknown

Specify if specimen(s) collected and available for future research:

131 DNA

yes no

132 Epstein-Barr virus (EBV)-transformed B-Cell line

yes no

133 Fibroblast cell line

yes no

134 Herpes virus saimiri-transformed T-cell line

yes no

135 Other T-cell line

yes no

136 Pathological specimen

yes no

137 Specify pathological specimen(s): _____

138 Peripheral blood mononuclear cells (PBMC), frozen

yes no

139 RNA

yes no

140 Specify RNA source: _____

141 Serum (pre-IVIG)

yes no

142 Other specimen

yes no

143 Specify other specimen(s): _____

First Name: _____ Last Name: _____

Phone: _____ Fax: _____

E-mail address: _____