

INSERT XIII
Wiskott-Aldrich Syndrome (WAS)

FOR REGISTRY USE ONLY:

I.D. - -

Date received: _____

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(Institutional Unique Blood or Marrow Transplant Identification Number)

Registry: IBMTR ABMTR (circle one)

Date of transplant for which this form is being completed:

Month Day Year

Date of report:

Month Day Year

Pretransplant Information

*** If this is a report of a second (or subsequent) transplant, check here and go to Q.9**

1. Date of diagnosis of primary disease:

Month Day Year

Specify the WAS-defining (diagnostic) criteria:

- | | <u>Yes</u> | <u>No</u> | <u>Unknown</u> | |
|----|----------------------------|----------------------------|----------------------------|--|
| 2. | 1 <input type="checkbox"/> | 0 <input type="checkbox"/> | 8 <input type="checkbox"/> | Platelet count (prior to splenectomy) < 150 x 10 ⁹ /L |
| 3. | 1 <input type="checkbox"/> | 0 <input type="checkbox"/> | 8 <input type="checkbox"/> | Small platelet size (average < 2 μ in diameter or mean platelet volume < 8.5 fL) |
| 4. | 1 <input type="checkbox"/> | 0 <input type="checkbox"/> | 8 <input type="checkbox"/> | Eczema |
| 5. | 1 <input type="checkbox"/> | 0 <input type="checkbox"/> | 8 <input type="checkbox"/> | X-linked inheritance demonstrated in family, i.e. affected male but not female relatives |

6. Was the diagnosis confirmed by molecular identification of the presence of a defect in the WAS gene?

1 Yes
0 No
8 Unknown

7. Did B cell lymphoproliferative disorder (BLPD) develop pretransplant?

1 Yes
0 No
8 Unknown

8. Was BLPD associated with EBV?

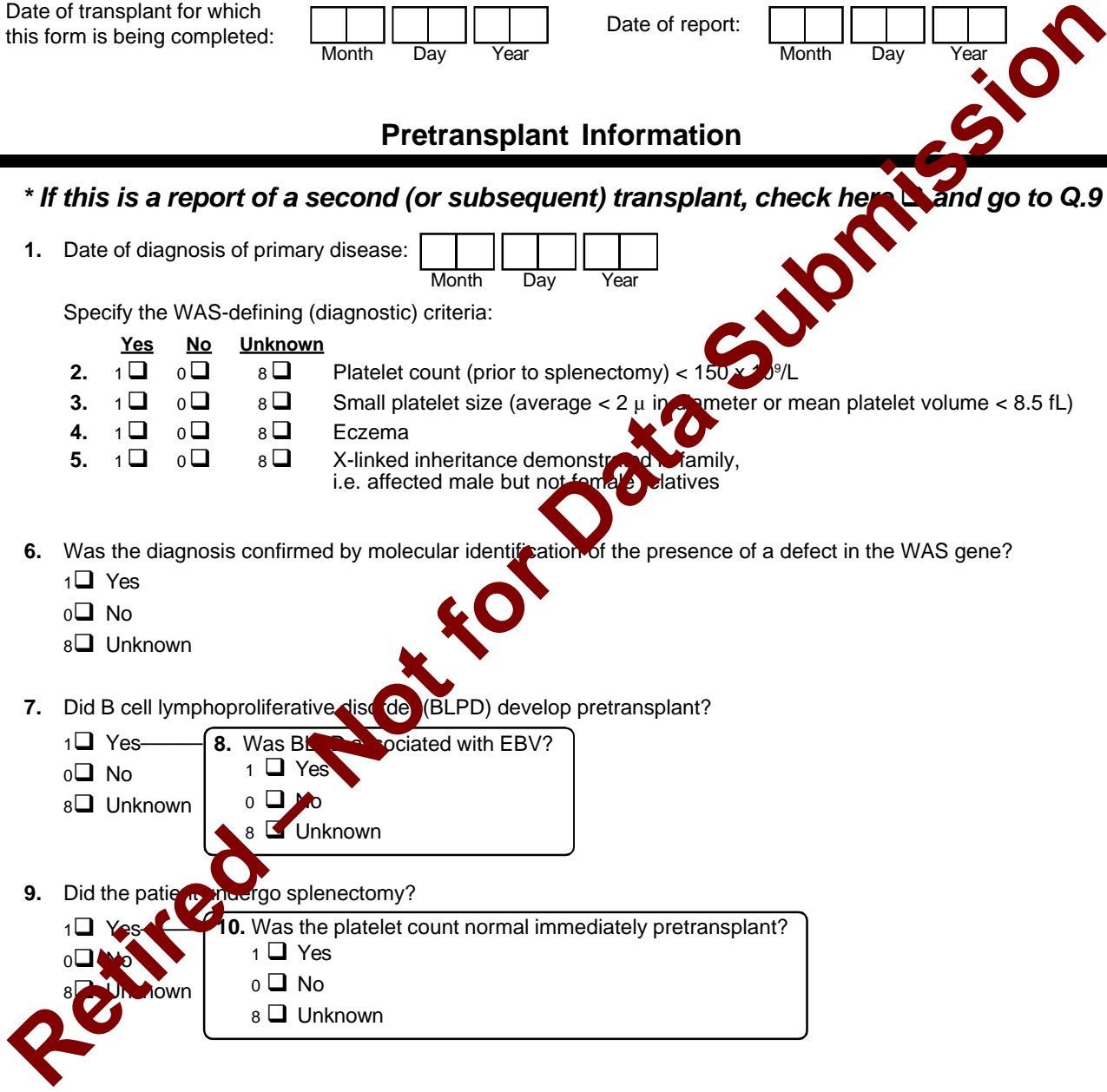
1 Yes
0 No
8 Unknown

9. Did the patient undergo splenectomy?

1 Yes
0 No
8 Unknown

10. Was the platelet count normal immediately pretransplant?

1 Yes
0 No
8 Unknown



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Hematologic Findings Prior to Transplant (or prior to conditioning, if used)

11. WBC x 10⁹/L
12. Lymphocytes % of WBC
13. T cells (CD3 or equivalent) % of Lymphocytes
14. CD4+ cells % of Lymphocytes
15. CD8+ cells % of Lymphocytes
16. B cells (CD19+, CD20+, sIg+) % of Lymphocytes
17. NK cells (CD16+ or equivalent) % of Lymphocytes

Immunologic Findings Prior to Conditioning

18. Specify the mitogen proliferation response (*response of lymphocytes to phytohemagglutinin, concavalin A or pokeweed mitogen*):
1 Absent ($\leq 10\%$ normal) 2 Decreased (11-60% Normal) 3 Normal 4 Not tested or Unknown
19. Natural killer cell function (specific cytolysis of NK-sensitive target cells, e.g. K562):
1 Absent ($\leq 10\%$ normal) 2 Decreased (11-50% Normal Mean) 3 Normal 8 Not tested or Unknown
20. IgG:
1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown
21. IgM:
1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown
22. IgA:
1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown
23. IgE:
1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown
24. Specific antibody response to any antigen against which recipient has been adequately immunized (e.g. > 3 DPT):
1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown

TEAM IUBMID **Clinical Status of Recipient Prior to Conditioning**

25. Did the patient have failure to thrive (*decrease of 0.5 standard deviation in weight on standard growth curve or weight < 5th percentile for age*)?
- 1 Yes
0 No
8 Unknown
26. Did the patient experience chronic (protracted) diarrhea in year before transplant (*> 6 weeks in duration*)?
- 1 Yes
0 No
8 Unknown
27. Did the patient have respiratory impairment (*need for chronic or intermittent support with O₂ or artificial ventilation and/or presence of persistent interstitial, nodular or lobar pneumonia*)?
- 1 Yes
0 No
8 Unknown

Posttransplant Information

**To be completed >100 days post transplant or
at time of death if death occurred less than 100 days post transplant**

28. Specify platelet count at most recent follow up:
- 1 Normal
3 Decreased (<150 x 10⁹/L)
8 Unknown
29. Specify platelet size at most recent follow up:
- 1 Normal
3 Decreased (average <2 μ in diameter or mean platelet volume <8.5 fL)
8 Unknown

Status of Hematologic Engraftment

(refers to quantitative analyses utilizing discriminating DNA markers. Peripheral blood cells must undergo separation or sorting into T, B or lymphoid vs. myeloid populations to perform this determination. If chimerism studies (i.e., RFLP/ANTR etc.) on blood or bone marrow post transplant indicate only donor type hematopoiesis mark T-cell, B-cell and myeloid as "predominantly or completely donor")

30. Status of T cell engraftment:
- 1 Predominantly or completely donor ($\geq 80\%$ donor chimerism)
2 Mixed chimerism (5-80% donor)
3 Only host T cells detected
8 Unknown or Not tested
31. Status of B cell engraftment:
- 1 Predominantly or completely donor ($\geq 80\%$ donor chimerism)
2 Mixed chimerism (5-80% donor)
3 Only host B cells detected
8 Unknown or Not tested
32. Status of myeloid engraftment:
- 1 Completely donor
2 Mixed chimerism
3 Host only
8 Unknown or Not tested

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Status of Immunologic Reconstitution

33. Specify the mitogen proliferation response (*response of lymphocytes to phytohemagglutinin, concavalin A or pokeweed mitogen*):

1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 8 Not tested or Unknown
(11-60% Normal)

34. Natural killer cell function (*specific cytolysis of NK-sensitive target cells, e.g. K562*):

1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 8 Not tested or Unknown
(11-50% Normal Mean)

35. IgG:

1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown

36. IgM:

1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown

37. IgA:

1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown

38. IgE:

1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown

39. Specific antibody response to any antigen against which recipient has been adequately immunized (e.g. > 3 DPT):

1 Absent ($\leq 10\%$ normal) 2 Decreased 3 Normal 4 Increased 8 Not tested or Unknown

Retired – Not for Data Submission