

STUDY OSTEOPETROSIS - Transplantation

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Patient Initials (last name/first name) Sex (m=1, f=2) Date of birth . . (DD.MM.YYYY) Local ID

Date of examination: . . (DD.MM.YYYY) Institution: _____

!! Please make sure that the consent form is signed before transmitting these data !!

TREATMENT AND HEMATOLOGY PRIOR TO PREPARATIVE REGIMEN

1. Was any drug given to treat osteopetrosis prior to the preparative regimen:

- 1 no
- 2 yes →
- 3 unknown

2. Specify drug:

- 1 calcitriol
- 2 corticosteroids
- 3 INF-γ
- 4 other: _____

3. Time duration the drug was given:

- 1 known → month(s)
- 2 unknown

4. Date the administration of the drug was stopped:

- 1 known → . . (DD.MM.YYYY)
- 2 unknown

5. Red blood transfusions at any time prior to preparative regimen:

- 1 no
- 2 yes →
- 3 unknown

6. Specify the number of donor exposures (best estimate):

- 1 1 - 5
- 2 6 - 10
- 3 11 - 20
- 4 > 20
- 5 unknown

7. Platelet transfusions at any time prior to preparative regimen:

- 1 no
- 2 yes →
- 3 unknown

8. Specify the number of donor exposures (best estimate):

- 1 1 - 5
- 2 6 - 10
- 3 11 - 20
- 4 > 20
- 5 unknown

9. Hematologic findings prior to preparative regimen:

- 1 known →
- 2 unknown

10. Cell count (untransfused):

	unknown	
1 absolute lymphocyte count	<input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> x 10 ⁹ /l	<input type="checkbox"/>
2 absolute neutrophil count	<input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> x 10 ⁹ /l	<input type="checkbox"/>
3 absolute monocyte count	<input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> x 10 ⁹ /l	<input type="checkbox"/>
4 reticulocytes	<input type="text"/> <input type="text"/> %	<input type="checkbox"/>

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11. Bone biopsy within 2 weeks prior to preparative regimen:

- 1 no
- 2 yes →
- 3 unknown

12. Specify number of osteoclasts in bone biopsy:

- 1 few / one
- 2 normal
- 3 increased
- 4 unknown

TRANSPLANTATION

13. Number of graft: □

14. Date of transplantation: □□.□□.□□□□ (DD.MM.YYYY)

15. Donor Type:
- 1 MSD / MFD (genotypic identical)
 - 2 MMFD (maximum 1 HLA mismatch out of 6)
 - 3 haploidentical donor
 - 4 MUD (maximum 1 HLA mismatch out of 10)
 - 5 MMUD
 - 6 unknown

16. Stem Cell Source:
- 1 bone marrow
 - 2 peripheral blood stem cells
 - 3 cord blood
 - 4 unknown

17. Cell count:

1 NC:	□□□□.□□	x 10 ⁸ /kg	<input type="checkbox"/>	unknown
2 CD34:	□□□□.□□	x 10 ⁶ /kg	<input type="checkbox"/>	
3 CD3:	□□□□.□□	x 10 ⁴ /kg	<input type="checkbox"/>	

18. Graft Manipulation:
- 1 no
 - 2 yes →
 - 3 unknown

19. Type of graft manipulation:

	no	yes
1 CD 34 positive selection	<input type="checkbox"/>	<input type="checkbox"/>
2 CD 2 negative selection	<input type="checkbox"/>	<input type="checkbox"/>
3 CD 3 negative selection	<input type="checkbox"/>	<input type="checkbox"/>
4 CD 19 negative selection	<input type="checkbox"/>	<input type="checkbox"/>
5 red cell depletion	<input type="checkbox"/>	<input type="checkbox"/>
6 other, specify: _____	<input type="checkbox"/>	<input type="checkbox"/>

20. Conditioning:
- 1 no
 - 2 yes →
 - 3 unknown

21. Drugs for conditioning:

	no	yes	total dose
1 busulfan p.o.	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/kg
2 busilfex i.v.	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/kg
3 treosulfan	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ g/m ²
4 cyclophosphamide	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/kg
5 fludarabine	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/m ²
6 thiotepa	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/kg
7 melphalan	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/m ²
8 other, specify: _____	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/kg

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22. Radiation:

- 1 no
- 2 yes →
- 3 unknown

23. Type of radiation:

- 1 total body irradiation (TBI)
- 2 total lymph node irradiation (TLI)
- 3 total abdominal irradiation (TAI)
- 4 other, specify: _____

24. Dose: _____ Gy

25. GvHD prophylaxis:

- 1 no
- 2 yes →
- 3 unknown

26. Drugs for GvHD prophylaxis:

	no	yes
1 ATG	<input type="checkbox"/>	<input type="checkbox"/>
2 ciclosporin A	<input type="checkbox"/>	<input type="checkbox"/>
3 MTX	<input type="checkbox"/>	<input type="checkbox"/>
4 MMF	<input type="checkbox"/>	<input type="checkbox"/>
5 prednisolone	<input type="checkbox"/>	<input type="checkbox"/>
6 campath	<input type="checkbox"/>	<input type="checkbox"/>
7 other, specify: _____	<input type="checkbox"/>	<input type="checkbox"/>

27. VOD prophylaxis:

- 1 no
- 2 yes →
- 3 unknown

28. Drugs for VOD prophylaxis:

	no	yes
1 Defibrotide	<input type="checkbox"/>	<input type="checkbox"/>
2 heparin	<input type="checkbox"/>	<input type="checkbox"/>
3 other, specify: _____	<input type="checkbox"/>	<input type="checkbox"/>

RECONSTITUTION

29. Engraftment:

- 1 no
- 2 yes →
- 3 died shortly after transplantation
- 4 unknown

30. Engraftment: (first of three consecutive days)

	no	yes	at day+
1 white blood cells > 1000 /μl	<input type="checkbox"/>	<input type="checkbox"/>	□□□□
2 neutrophils > 500 /μl	<input type="checkbox"/>	<input type="checkbox"/>	□□□□
3 neutrophils > 1000 /μl	<input type="checkbox"/>	<input type="checkbox"/>	□□□□
4 platelets > 50 G/l	<input type="checkbox"/>	<input type="checkbox"/>	□□□□
5 platelets > 100 G/l	<input type="checkbox"/>	<input type="checkbox"/>	□□□□
6 reticulocytes > 10%o	<input type="checkbox"/>	<input type="checkbox"/>	□□□□
7 G-CSF	<input type="checkbox"/>	<input type="checkbox"/>	until day+ □□□□
8 erythrocyte transfusion	<input type="checkbox"/>	<input type="checkbox"/>	last at day+ □□□□
9 platelet transfusion	<input type="checkbox"/>	<input type="checkbox"/>	last at day+ □□□□

31. Graft failure:

- 1 no
- 2 yes →
- 3 unknown

32. Type of graft failure:

- 1 non-engraftment (neutrophils never > 500 /μl)
- 2 complete autologous reconstitution
- 3 rejection
- 4 other, specify: _____

33. Treatment of graft failure:

- 1 no treatment
- 2 subsequent transplant (please add new transplantation form)
- 3 other, specify: _____

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COMPLICATIONS

- 34. aGvHD:**
- 1 no
 - 2 yes →
 - 3 unknown

35. aGvHD grading:

	no	yes	unknown	grade
1 skin	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
2 liver	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
3 gut	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

- 36. cGvHD:**
- 1 no
 - 2 yes →
 - 3 unknown

37. cGvHD grading:

- 1 mild
- 2 moderate
- 3 severe
- 4 unknown

38. Progression from aGvHD:

- 1 no
- 2 yes
- 3 unknown

39. Type of onset:

- 1 de novo
- 2 quiescent
- 3 progressive
- 4 unknown

40. Severe complications:

	no	yes	unknown	<d+100	>d+100
Infection:	1 CMV infection	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	2 CMV disease	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	3 EBV lymphoproliferative disorder	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	4 other severe infections, please specify: _____	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Lungs:	5 oxygen support > 24h	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	6 artificial ventilation	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Cardio-vascular:	7 inotropic support	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Liver:	8 hepatic failure	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	9 veno-occlusive disease (VOD)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Kidney:	10 renal insufficiency	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	11 hemodialysis / hemofiltration	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Bleeding:	12 hemorrhage site of hemorrhage: _____	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	13 hemorrhagic cystitis	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
CNS:	14 encephalopathy	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
other severe complication:	15 specify: _____	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

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STATUS AT DAY 100

41. Performance Status Criteria:

Karnofsky / Lansky	Karnofsky Description (Patients elder than 16 years)	Lansky Description (Patients younger than 16 years)
100 <input type="checkbox"/>	Normal, no complaints, no evidence of disease.	Fully active, normal.
90 <input type="checkbox"/>	Able to carry on normal activity; minor signs or symptoms of disease.	Minor restrictions in physically strenuous activity.
80 <input type="checkbox"/>	Normal activity with effort; some signs or symptoms of disease.	Active, but tires more quickly.
70 <input type="checkbox"/>	Cares for self, unable to carry on normal activity or do active work.	Both greater restriction of and less time spent in play activity.
60 <input type="checkbox"/>	Requires occasional assistance, but is able to care for most of his/her needs	Up and around, but minimal active play, keeps busy with quieter activities.
50 <input type="checkbox"/>	Requires considerable assistance and frequent medical care.	Gets dressed, but lies around much of the day; no active play; able to participate in all quiet play and activities.
40 <input type="checkbox"/>	Disabled, requires special care and assistance.	Mostly in bed; participates in quiet activities.
30 <input type="checkbox"/>	Severely disabled, hospitalization indicated. Death not imminent.	In bed; needs assistance even for quiet play.
20 <input type="checkbox"/>	Very sick, hospitalization indicated. Death not imminent.	Often sleeping; play entirely limited to very passive activities.
10 <input type="checkbox"/>	Moribund, fatal processes progressing rapidly.	No play; does not get out of bed.

42. Status:

- 1 alive without disease
- 2 alive with disease →

43. Treatment:

- 1 new transplantation in preparation
- 2 supportive therapy
- 3 cell therapy
- 4 other therapy:

	no	yes	unknown
a calcitriol	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
b corticosteroids	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
c IFN-γ	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
- 5 unknown

- 3 died →
- 4 lost to follow-up / unknown

44. Date of death: . (DD.MM.YYYY)

45. Main cause of death:

- 1 progression of disease
- 2 transplant related cause: (tick all that apply)
 - a GvHD
 - b graft failure
 - c pulmonary toxicity
 - d cardiac toxicity
 - e infection, please specify: _____
 - f VOD
 - g post-transplant lymphoproliferative disorder
 - h other: _____
- 3 other: _____
- 4 unknown

Date _____ Clinic Stamp _____ Name _____ Signature _____