

## 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):**

	<b>unknown</b>
1 absolute lymphocyte count <input type="text"/> x 10 <sup>9</sup> /l	<input type="checkbox"/>
2 absolute neutrophil count <input type="text"/> x 10 <sup>9</sup> /l	<input type="checkbox"/>
3 absolute monocyte count <input type="text"/> x 10 <sup>9</sup> /l	<input type="checkbox"/>
4 reticulocytes <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 <sup>8</sup> /kg	<input type="checkbox"/>	<b>unknown</b>
2 CD34:	□□□□.□□	x 10 <sup>6</sup> /kg	<input type="checkbox"/>	
3 CD3:	□□□□.□□	x 10 <sup>4</sup> /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 <sup>2</sup>
4 cyclophosphamide	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/kg
5 fludarabine	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/m <sup>2</sup>
6 thiotepa	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/kg
7 melphalan	<input type="checkbox"/>	<input type="checkbox"/>	□□□□ mg/m <sup>2</sup>
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
<b>Infection:</b>	1 CMV infection	<input type="checkbox"/>	<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"/>	<input type="checkbox"/>
	3 EBV lymphoproliferative disorder	<input type="checkbox"/>	<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"/>	<input type="checkbox"/>
<b>Lungs:</b>	5 oxygen support > 24h	<input type="checkbox"/>	<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"/>	<input type="checkbox"/>
<b>Cardio-vascular:</b>	7 inotropic support	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
<b>Liver:</b>	8 hepatic failure	<input type="checkbox"/>	<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"/>	<input type="checkbox"/>
<b>Kidney:</b>	10 renal insufficiency	<input type="checkbox"/>	<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"/>	<input type="checkbox"/>
<b>Bleeding:</b>	12 hemorrhage site of hemorrhage: _____	<input type="checkbox"/>	<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"/>	<input type="checkbox"/>
<b>CNS:</b>	14 encephalopathy	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
<b>other severe complication:</b>	15 specify: _____	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Patients Initials [ ][ ]	Sex [ ] (m=1, f=2)	Date of birth [ ][ ] . [ ][ ] . [ ][ ][ ][ ]	Institution _____	Local ID [ ][ ][ ][ ][ ][ ][ ][ ][ ][ ]
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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 →

3  died →

4  lost to follow-up / unknown

**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

**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