Status at HCT/CT/IST

Day 0

Guide to the completion of the EBMT data collection form: Disease_status_HCT_CT_IST_Day0_v1.0

22 August 2023

EBMT Registry
EBMT Clinical Research & Registry Department
Table of Contents

Patient status ........................................................................................................................................... 7

1. Date of HCT/CT/IST .......................................................................................................................... 7

2. Survival status at HCT/CT/IST: ...................................................................................................... 7
   2.1. Date of death: ............................................................................................................................... 7
   2.2. Main cause of death: .................................................................................................................... 7

3. Performance status at initiation of HCT/CT/IST .............................................................................. 8

4. Patient weight at initiation of HCT/CT/IST: ..................................................................................... 9

5. Patient height at initiation of HCT/CT/IST ..................................................................................... 10

Comorbidity Index ..................................................................................................................................... 10

6. Was there any clinically significant co-existing disease or organ impairment as listed below at time
   of patient assessment prior to the preparative regimen? ..................................................................... 10
   6.1. Comorbidity details ..................................................................................................................... 10
   6.2. Was there any additional major clinical abnormality not listed above and present prior to the
        preparative regimen? ..................................................................................................................... 10
   6.3. Were there any autoimmune diseases? ....................................................................................... 10

7. Comorbidity Index (Inborn Errors of Immunity only) ...................................................................... 11

SARS-CoV-2 related questions .................................................................................................................. 11

8. Did the patient have a symptomatic SARS-CoV-2 infection (positive PCR or antigen test) in the 3
   months prior to the day of treatment? .................................................................................................. 11
   8.1. Date .............................................................................................................................................. 11

9. Did the patient have an ongoing SARS-CoV-2 infection (positive PCR or antigen test) at the
   moment of the start of the conditioning regimen? .............................................................................. 11

Diagnosis specific sections ....................................................................................................................... 12

10. Acute leukaemias .............................................................................................................................. 12
   10.1. Status ........................................................................................................................................ 12
   10.2. Number of induction courses ................................................................................................... 12
   10.3. Date of the last relapse before this treatment ......................................................................... 12
<table>
<thead>
<tr>
<th></th>
<th>Description</th>
<th>Page</th>
</tr>
</thead>
<tbody>
<tr>
<td>10.4</td>
<td>CD19 expression at the last relapse</td>
<td>12</td>
</tr>
<tr>
<td>10.5</td>
<td>Bone marrow burden (% blasts) (at time of transplant if patient not in remission)</td>
<td>12</td>
</tr>
<tr>
<td>10.6</td>
<td>Involvement at time of treatment (if patient not in remission)</td>
<td>13</td>
</tr>
<tr>
<td>10.7</td>
<td>Minimal residual disease (MRD) at initiation of treatment:</td>
<td>13</td>
</tr>
<tr>
<td>10.8</td>
<td>Date MRD status evaluated</td>
<td>13</td>
</tr>
<tr>
<td>10.9</td>
<td>Sensitivity of MRD assay</td>
<td>13</td>
</tr>
<tr>
<td>10.10</td>
<td>Method used</td>
<td>13</td>
</tr>
<tr>
<td>11</td>
<td>Chronic Myeloid Leukaemias (CML)</td>
<td>14</td>
</tr>
<tr>
<td>11.1</td>
<td>Status</td>
<td>14</td>
</tr>
<tr>
<td>11.2</td>
<td>Number</td>
<td>14</td>
</tr>
<tr>
<td>11.3</td>
<td>Haematological remission:</td>
<td>14</td>
</tr>
<tr>
<td>11.4</td>
<td>Cytogenetic remission:</td>
<td>15</td>
</tr>
<tr>
<td>11.5</td>
<td>Molecular remission:</td>
<td>15</td>
</tr>
<tr>
<td>12</td>
<td>Chronic Lymphocytic Leukaemias (CLL)</td>
<td>16</td>
</tr>
<tr>
<td>12.1</td>
<td>Status</td>
<td>16</td>
</tr>
<tr>
<td>12.2</td>
<td>Minimal residual disease (MRD) at initiation of treatment:</td>
<td>18</td>
</tr>
<tr>
<td>13</td>
<td>Prolymphocytic (PLL) and Other Chronic Leukaemias</td>
<td>18</td>
</tr>
<tr>
<td>13.1</td>
<td>Status</td>
<td>18</td>
</tr>
<tr>
<td>14</td>
<td>Lymphomas</td>
<td>22</td>
</tr>
<tr>
<td>14.1</td>
<td>Disease status</td>
<td>22</td>
</tr>
<tr>
<td>14.2</td>
<td>Technique used for disease assessment</td>
<td>22</td>
</tr>
<tr>
<td>14.3</td>
<td>Age at diagnosis</td>
<td>22</td>
</tr>
<tr>
<td>14.4</td>
<td>LDH levels elevated</td>
<td>22</td>
</tr>
<tr>
<td>14.5</td>
<td>Ann Arbor staging</td>
<td>23</td>
</tr>
<tr>
<td>14.6</td>
<td>ECOG performance status</td>
<td>23</td>
</tr>
<tr>
<td>14.7</td>
<td>1 extranodal site involved</td>
<td>24</td>
</tr>
<tr>
<td>14.8</td>
<td>4 nodal sites involved</td>
<td>24</td>
</tr>
</tbody>
</table>
14.9. Haemoglobin < 120g/L

14.10. White Blood Cell count

15. MDS

15.1. MDS Classification at diagnosis (WHO 2016):

15.2. Status:

16. MDS/MPN

16.1. MDS/MPN Classification:

16.2. Status:

17. MPN

17.1. MPN Classification (WHO 2016):

17.2. Status:

17.3. Blast count (peripheral blood):

17.4. Spleen size:

17.5. Spleen span in ultrasound or CT scan:

17.6. JAK inhibitor exposure between diagnosis and treatment:

17.7. Was a JAK inhibitor continued during conditioning?

17.8. Response status:

17.9. DIPSS Risk score at treatment: (Myelofibrosis either primary or secondary to ET/PV):

17.10. MIPSS70 score at treatment (Primary Myelofibrosis only):

17.11. MYSEC-PM score at time of secondary MF diagnosis (Secondary myelofibrosis only; post-ET MF, post-PV MF):

18. Plasma cell disorders including MM

18.1. Status

18.2. Number

19. Solid tumours

19.1. Status

19.2. Organ involvement at time of this HCT/CT/IST
19.3. Risk category at disease recurrence (or platinum refractoriness) following first line chemotherapy................................. 34

20. Autoimmune disorders......................................................................................................................................................... 34

20.1. Disease status (Systemic sclerosis only): ......................................................................................................................... 34

20.2. SSc subset:.................................................................................................................................................................................. 34

20.3. Creatinine Clearance (Cockcroft-Gault formula): ................................................................................................................... 35

20.4. Proteinuria: ................................................................................................................................................................................ 35

20.5. Modified Rodnan Skin Score (0-51): ................................................................................................................................. 35

20.6. DLCO (corrected for Hb):......................................................................................................................................................... 35

20.7. Mean Pulmonary Arterial Systolic Pressure [PASP] (from right heart catheterisation): ...... 35

20.8. GI Involvement:........................................................................................................................................................................... 36

20.9. SLEDAI-2K Score:................................................................................................................................................................. 36

20.10. Disease status at time of mobilisation (within 3 months before mobilisation):.................... 38

20.11. EDSS (1-10):............................................................................................................................................................................. 38

20.12. Number of gadolinium enhancing lesions present on MRI brain scan: ............................................ 38

20.13. CDAI (0-700):............................................................................................................................................................................. 38

20.14. Serum albumin................................................................................................................................................................. 39

21. Haemoglobinopathies ............................................................................................................................................................. 40

21.1. Ferritin level ............................................................................................................................................................................. 40

21.2. Number of red blood cell transfusions ................................................................................................................................. 40

21.3. Liver iron concentration.......................................................................................................................................................... 40

21.4. Pre-existing liver disease?....................................................................................................................................................... 40

21.5. Pre-existing cardiac disease .................................................................................................................................................. 40

21.6. Chronic transfusion program.................................................................................................................................................. 41

21.7. Pre-treatment complications................................................................................................................................................ 41

21.8. Cerebrovascular disease: ......................................................................................................................................................... 41

21.9. Renal involvement: ............................................................................................................................................................. 42

21.10. Other SCD related complications.................................................................................................................................. 42
21.11. Endocrinopathies pre-existing to HCT ................................................................. 43

Bibliography ..................................................................................................................... 44
Status at HCT/CT/IST day 0

This form shall be completed after the respective treatment form as part of Day 0 regardless if the treatment took place or not.

Patient status

1. Date of HCT/CT/IST

Report the date the HCT/CT/IST took place. If the patient died before the treatment took place, report the planned treatment date.

When submitting data in the EBMT Registry application, this date should be indicated as the event date for Disease status at HCT/CT/IST.

2. Survival status at HCT/CT/IST:

Indicate the survival status of the recipient at the (planned) date of the treatment. Select Alive if the recipient was still alive at the time of the treatment. If the treatment was initiated by starting the conditioning regimen but the patient died before infusion took place, select Died after conditioning but before HCT/CT/IST: If the patient died after apheresis but before infusion took place, select Died after apheresis but before cell infusion.

2.1. Date of death:

Report the full date of death as stated in the patient documents.

2.2. Main cause of death:

Report only one main cause of death, even if it was considered to be a combination of various causes. If the cause of death is not known, select Unknown. There are identified the following main causes of death:

- Relapse or progression/persistent disease
- Secondary malignancy
- Cellular therapy-related - death caused by complications or infections after cellular therapy
- HCT-related - death caused by complications or infections after transplant.

If none of the suggested options fit, select Other and specify the cause of death in the textbox in English.
In the case of treatment-related cause of death, select all the answer options that apply:

- Graft versus host disease (GvHD)
- Non-infectious complication
- Infectious complication.

In the case of infectious complication, select all the type of infection(s) that apply:

- Bacterial infection
- Viral infection
- Fungal infection
- Parasitic infection
- Infection with an unknown pathogen

3. Performance status at initiation of HCT/CT/IST

Choose one scale system and report the performance status of the patient.

The Karnofsky, Lansky, and ECOG are standard performance scales used to measure the wellbeing of a patient and classify a patient according to their functional impairment, compare the effectiveness of therapies, and assess the prognosis of a patient.

The Karnofsky is used in adults, and the Lansky is used in paediatrics. Their measurements should represent the situation at the start of the conditioning regimen.

<table>
<thead>
<tr>
<th>Score</th>
<th>Performance Status (Karnofsky)</th>
</tr>
</thead>
<tbody>
<tr>
<td>100</td>
<td>Normal, no complaints or evidence of disease</td>
</tr>
<tr>
<td>90</td>
<td>Able to perform normal activity; minor signs and symptoms of disease</td>
</tr>
<tr>
<td>80</td>
<td>Able to perform normal activity with effort; some signs and symptoms of disease</td>
</tr>
<tr>
<td>70</td>
<td>Cares for self, unable to perform normal activity or to do active work</td>
</tr>
<tr>
<td>60</td>
<td>Requires occasional assistance but is able to care for most of own needs</td>
</tr>
<tr>
<td>50</td>
<td>Requires considerable assistance and frequent medical care</td>
</tr>
<tr>
<td>40</td>
<td>Requires special care and assistance; disabled</td>
</tr>
<tr>
<td>30</td>
<td>Hospitalisation indicated, although death not imminent; severely disabled</td>
</tr>
<tr>
<td>20</td>
<td>Hospitalisation necessary; active supportive treatment required, very sick</td>
</tr>
<tr>
<td>10</td>
<td>Fatal processes progressing rapidly; moribund</td>
</tr>
<tr>
<td>0</td>
<td>Dead</td>
</tr>
</tbody>
</table>

Table 1, Karnofsky scale for adult patients
<table>
<thead>
<tr>
<th>Score</th>
<th>Performance Status (Lansky)</th>
</tr>
</thead>
<tbody>
<tr>
<td>100</td>
<td>Fully active, normal</td>
</tr>
<tr>
<td>90</td>
<td>Minor restrictions in physically strenuous activity</td>
</tr>
<tr>
<td>80</td>
<td>Active, but tires more quickly</td>
</tr>
<tr>
<td>70</td>
<td>Both greater restriction of and less time spent in play activity</td>
</tr>
<tr>
<td>60</td>
<td>Up and around, but minimal active play; keeps busy with quieter activities</td>
</tr>
<tr>
<td>50</td>
<td>Gets dressed but lies around much of the day, no active play but able to participate in all quiet play and activities</td>
</tr>
<tr>
<td>40</td>
<td>Mostly in bed; participates in quiet activities</td>
</tr>
<tr>
<td>30</td>
<td>In bed; needs assistance even for quiet play</td>
</tr>
<tr>
<td>20</td>
<td>Often sleeping; play entirely limited to very passive activities</td>
</tr>
<tr>
<td>10</td>
<td>No play; does not get out of bed</td>
</tr>
<tr>
<td>0</td>
<td>Unresponsive</td>
</tr>
</tbody>
</table>

Table 2, Lansky scale for paediatric patients

<table>
<thead>
<tr>
<th>Score</th>
<th>Performance Status (ECOG)</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>Fully active, able to carry on all pre-disease performance without restriction</td>
</tr>
<tr>
<td>1</td>
<td>Restricted in physically strenuous activity but ambulatory and able to carry out work of a light or sedentary nature, e.g., light house work, office work</td>
</tr>
<tr>
<td>2</td>
<td>Ambulatory and capable of all selfcare but unable to carry out any work activities; up and about more than 50% of waking hours</td>
</tr>
<tr>
<td>3</td>
<td>Capable of only limited selfcare; confined to bed or chair more than 50% of waking hours</td>
</tr>
<tr>
<td>4</td>
<td>Completely disabled; cannot carry on any selfcare; totally confined to bed or chair</td>
</tr>
<tr>
<td>5</td>
<td>Dead</td>
</tr>
</tbody>
</table>

Table 3, ECOG performance scale

4. Patient weight at initiation of HCT/CT/IST:

Report the weight of the recipient (patient) at the start of conditioning in kilograms.
5. **Patient height at initiation of HCT/CT/IST**

Report the height of the recipient (patient) at the start of conditioning in centimetres.

### Comorbidity Index

Comorbid conditions are those conditions that are likely to affect the outcome of the treatment but which may not be directly related to the diagnosis indication for transplant.

This table reflects comorbidities as listed in the HCT comorbidity index (1). Ensure an answer is only given if the comorbid condition fits the definition found in the form itself. Do not give a positive answer if the condition exists in a milder way than defined. The answers should represent the situation at the start of conditioning, unless otherwise stated in the definitions.

6. **Was there any clinically significant co-existing disease or organ impairment as listed below at time of patient assessment prior to the preparative regimen?**

Answer **Yes** if the patient had at least one comorbid condition at the time of the patient assessment prior to the preparative (conditioning) regimen. Otherwise, answer **No** to report that there were no co-existing disease or organ impairment as listed in the table. Answer **Unknown** if it is not possible to identify whether the recipient had any comorbid condition at this time point.

**6.1. Comorbidity details**

If a comorbid condition was present at the start of the conditioning regimen, indicate for each comorbidity listed in the table if the recipient has a documented history of any of the conditions listed in the “Definition” column (if asked, specify also the details in the respective text field), check the corresponding **Yes** box in the Comorbidity Index. Otherwise, select **No**. Report **Not evaluated** if comorbidity was not assessed.

**6.2. Was there any additional major clinical abnormality not listed above and present prior to the preparative regimen?**

If the recipient has any major clinical abnormality, significant disease or organ impairment other than listed in the previous question, answer **Yes** and report it in the text field. Otherwise answer **No**.

**6.3. Were there any autoimmune diseases?**

Indicate whether the patient has an autoimmune disease. If the answer is **Yes**, specify the autoimmune disease in the text field and report the date of the autoimmune disease diagnosis. If the answer is **No**, proceed to the next question.
7. Comorbidity Index (Inborn Errors of Immunity only)

For patients with inborn errors of immunity, this section needs to be completed next to the comorbidity index. If the recipient has a documented history of any of the conditions listed in the “Definition” column, check the corresponding Yes box in the Comorbidity Index. Otherwise, select No. Report Not evaluated if comorbidity was not assessed.

SARS-CoV-2 related questions

8. Did the patient have a symptomatic SARS-CoV-2 infection (positive PCR or antigen test) in the 3 months prior to the day of treatment?

Answer Yes to this question if the patient had a symptomatic SARS-CoV-2 infection which was confirmed by PCR or an antigen test in the 3 months before the treatment (start of lymphodepleting/conditioning regimen) took place.

If the infection was asymptomatic, it should not be reported and this question to be answered as No.

8.1. Date

If answered Yes in the previous question, report the date the patient tested positive for SARS-CoV-2.

9. Did the patient have an ongoing SARS-CoV-2 infection (positive PCR or antigen test) at the moment of the start of the conditioning regimen?

Answer Yes to this question if the patient had an ongoing SARS-CoV-2 infection which was confirmed by PCR or an antigen test within one week before starting conditioning and without documentation of negative results prior to the start of treatment, either symptomatic or asymptomatic.

This question is the last in the general section of the disease status part of the status at HCT/CT/IST form. Please find the section specific to the indication diagnosis for which this treatment is given and fill in the applicable diagnose-specific questions attached.
Diagnosis specific sections

10. Acute leukaemias

10.1. Status

Indicate the acute leukaemia disease status or mark as Unknown if it is not possible to identify.

**Primary induction failure:** despite treatment patient has never achieved a complete remission

**Relapse (1st, 2nd, 3rd or higher):** > 5% blasts in the bone marrow after a period of complete remission.

**Complete haematological remission (CR) (1st, 2nd, 3rd or higher):** For at least 4 weeks:

- < 5% blasts in the bone marrow;
- No blasts with Auer rods (applies to AML only);
- Normal maturation of all cellular components in the bone marrow;
- No extramedullary disease (e.g., CNS, soft tissue disease).

10.2. Number of induction courses

Report the number of induction courses the patient had to reach this disease status, or mark as Unknown if the number of induction courses is unknown.

10.3. Date of the last relapse before this treatment

For patients that had relapses, report the date of the last relapse. If the patient never had a relapse, select Not applicable.

10.4. CD19 expression at the last relapse

For patients that had a relapse, if at the time of relapse their leukaemia cells no longer express CD19, mark CD19 expression as **Negative**, if they express CD19 mark it as **Positive**. This may be determined by blood and/or bone marrow tests showing the absence of the tumour antigen. If CD19 was not evaluated, mark it as **Not evaluated**. If the patient did not relapse, leave this field blank.

10.5. Bone marrow burden (% blasts) (at time of transplant if patient not in remission)

Indicate the percentage of blasts in the bone marrow observed at the reported disease status if the patient was not in remission.

Mark as **Not evaluated** if it was not evaluated. Mark as **Unknown** if it is not possible to identify the results of the investigation.
10.6. Involvement at time of treatment (if patient not in remission)

If the patient was not in remission, indicate if acute leukaemia has **medullary, extramedullary** involvement or **both**, or mark if it is **Unknown**.

### 10.6.1. Organs involved at time of treatment, CNS, Skin, Ovaries/testes, Other:

Indicate per each organ in the list if leukaemic cells were found there (answer **Yes**) or not (answer **No**), or if it was **Not evaluated** at time of treatment. If other organs than those from the list were investigated, check the **Other** box and specify the organ, indicating if it is involved (select **Yes**) or not (select **No**).

*Complete the following section only if the disease status is CR*

10.7. Minimal residual disease (MRD) at initiation of treatment:

If the patient is in hematologic CR, but has evidence of disease at initiation of the HCT/CT treatment by more sensitive assessments including molecular, flow cytometry or cytogenetic methods, mark it as **Positive**. If the MRD assay cannot detect leukaemic cells mark it as **Negative**. Mark it as **Not evaluated** if MRD status evaluation was not carried out at initiation of HCT/CT/IST.

10.8. Date MRD status evaluated

Report the date of MRD status evaluation.

10.9. Sensitivity of MRD assay

Report the sensitivity of MRD assay by choosing one of the given answer options, or mark **Other** checkbox and specify it.

10.10. Method used

Indicate if the MRD assessment was performed through **PCR** or **Flow cytometry**. If another method was used, choose the **Other** option and specify it in the textbox.
11. Chronic Myeloid Leukaemias (CML)

11.1. Status

Report the Chronic Myelogenous Leukaemias (CML) status:

- Chronic phase (CP);
- Accelerated phase; or
- Blast crisis.

In order to define the answer, please use WHO 2016 criteria or explanation below.

**Chronic phase (CP):** none of the features of accelerated phase or blast crisis.

**Accelerated phase:** any of the following:

- Blasts 10-19% in peripheral blood and/or nucleated bone marrow cells
- Peripheral blood basophiles >=20%
- Persistent thrombocytopenia (<100 x 10^9/L) unrelated to therapy
- Persistent thrombocytosis (>1000 x 10^9/L) unresponsive to standard therapy
- Increasing spleen size and increasing WBC count unresponsive to standard therapy
- Cytogenetic evidence of clonal evolution

**Blast Crisis:** any one of the following symptoms:

- Blasts >=20% in peripheral blood or nucleated bone marrow cells
- Extramedullary blast proliferation
- Large foci or clusters of blasts in the bone marrow biopsy

11.2. Number

For all disease statuses, report the response number by choosing one of the following check boxes:

- 1st;
- 2nd;
- 3rd or higher;
- Unknown.

**Note:** if a patient presents at diagnosis in accelerated phase or blast crisis, you must assume that prior to the presentation there has been a period of chronic phase which went undetected. Therefore, when a patient presenting in accelerated phase or blast crisis is restored (by whatever means) to chronic phase, this must be CP2.

11.3. Haematological remission:

If the patient was in Chronic phase (CP), report if haematological remission was achieved (answer Yes), or not achieved (answer No). Answer Not evaluated if it was not evaluated or Unknown if it cannot be verified if it was evaluated or not.

Haematological remission is defined by a patient meeting all of the following:

- WBC < 10 x 10^9 /L
- Haemoglobin > 11.0 g/dL
- Platelet Count < 450 x 10^9 /L
• Normal Differential (<1% precursor cells)
• No palpable splenomegaly
• No extramedullary disease

11.4. Cytogenetic remission:

If the patient was in Chronic phase (CP), report if cytogenetic remission achieved (answer Yes), or not achieved (answer No). Answer Not evaluated if it was not evaluated or Unknown if it cannot be verified if it was evaluated or not.

Cytogenetic remission is defined by:

• 0% t(9;22) positive metaphases together with haematological remission
• A minimum of 20 analysable metaphases must be assessed for appropriate evaluation of a cytogenetic remission. Remission should be confirmed with repeated cytogenetic analysis within 4 to 12 weeks

Note: A patient in cytogenetic remission must be in haematological remission but could still present a molecular relapse. This is because the cytogenetic technique has a higher resolution than haematological measurements but lower resolution than molecular methods

11.5. Molecular remission:

If the patient was in Chronic phase (CP), report if molecular remission achieved (answer Yes), or not achieved (answer No). Answer Not evaluated if it was not evaluated or Unknown if it cannot be verified if it was evaluated or not.

Molecular remission is defined by:

• Cells with the BCR-ABL1 fusion protein are not detectable, in the peripheral blood and/or the bone marrow, by an assay with a sensitivity to allow detection of one t(9;22) positive cell in $10^5$ to $10^6$ RT-PCR cells. The result should be confirmed by two consecutive tests done at least 4 weeks apart.

Note: A patient in molecular remission must also be in cytogenetic and haematological remission. This is because molecular techniques have a higher resolution than both haematological and cytogenetic measurements.
12. Chronic Lymphocytic Leukaemias (CLL)

12.1. Status

Report the Chronic Lymphocytic Leukaemias (CLL) status at.

According to 2018 iwCLL criteria (10), the response evaluation will include 2 types of parameters:

Parameters of group A:
- Lymph node size
- Liver and/or Spleen size
- Constitutional symptoms
- Circulating lymphocyte counts

Parameters of group B:
- Platelet count
- Haemoglobin
- Bone marrow assessment

Complete remission (CR): All the following criteria are required:
- Peripheral blood lymphocytes < 4,000/μL
- Absence of significant lymphadenopathy (e.g. lymph nodes should be below 1.5 cm in diameter)
- Absence of hepatomegaly or splenomegaly
- Absence of disease-related constitutional symptoms.

Notes for CR criteria
1) A CT scan (or any other imaging procedure) for assessment of lymphadenopathy, hepatomegaly and splenomegaly is required in clinical trials. For the EBMT Registry, clinical (palpation) evaluation only without CT-scan (or alternate imaging), according to routine practice, is accepted.

2) The formal guidelines for CR definition require adequate blood counts including neutrophiles $\geq 1500$ per $\mu$L, platelets $\geq 100,000$ per $\mu$L, haemoglobin $\geq 11$ g/dL (without blood transfusion). For the EBMT Registry, if a patient has all criteria of a CR but has persistent cytopenia, the patient can be considered as a CR as an adaptation of these guidelines.

3) The formal guidelines for CR definition also require bone marrow evaluation to confirm a CR in clinical trials. For the EBMT Registry, if a patient has all criteria of a CR but bone marrow evaluation has not been performed (even with persistent cytopenia), the patient can be considered as a CR as an adaptation of these guidelines.

Partial remission (PR): To define a PR, at least 2 parameters of group A and 1 parameter of group B (see above) need to improve, if previously abnormal. If only 1 parameter within each group was abnormal before therapy, only 1 needs to improve.
Among group A:

- A decrease in the number of blood lymphocytes to 50% or less from the value prior to therapy;
- A decrease in lymph node size by 50% or more in the sum products of up to 6 lymph nodes, or in one lymph node diameter if only a single lymph node was present prior to therapy, without increase in any lymph node, and no new enlarged lymph node;
- A decrease in the size of the spleen and/or liver by 50% or more.
  - By clinical evaluation (palpation): reduction by 50% or more of the extent of enlargement of the spleen and/or liver below the costal margin or normalization of the size
  - If imaging has been performed: reduction by 50% or more in length beyond normal.

Among group B:

- Polymorphonuclear leukocytes at 1,500/μL or more or 50% improvement over baseline without G-CSF support;
- Platelet counts greater than 100,000/μL or 50% improvement over baseline without transfusion;
- Haemoglobin greater than 11.0 g/dL or 50% improvement over baseline without transfusions or erythropoietin support.

Notes for PR criteria

1) A CT scan (or any other imaging procedure) for assessment of lymphadenopathy, hepatomegaly and splenomegaly is required in clinical trials. For the EBMT Registry, clinical (palpation) evaluation only without CT-scan (or alternate imaging), according to routine practice, is accepted.

2) Caution with liver assessment: Given the impact of numerous medical conditions, liver size should only be counted if hepatomegaly is clearly attributable to lymphoid involvement.

Stable disease (SD): Patients who have not achieved a CR or a PR, and who have not exhibited progression, will be considered to have no change (which is equivalent to a non-response).

Progressive disease (PD): Progressive disease is defined by at least one of the following:

- Progression of lymphadenopathy, occurring if one of the following events is observed:
  - Appearance of any new lesion such as enlarged lymph nodes (≥1.5 cm), splenomegaly, hepatomegaly, or other organ infiltrates.
  - An increase by 50% or more in greatest determined diameter of any previous site.
- An increase in the spleen size by 50% or more or the de novo appearance of splenomegaly
- An increase in the liver by 50% or more or the de novo appearance of hepatomegaly
- An increase in the number of blood lymphocytes by 50% or more with at least 5,000/μL B-cells.
- Transformation to a more aggressive histology (e.g. Richter’s syndrome), established by lymph node or other tissue biopsy.
- Occurrence of cytopenia (neutropenia, anaemia, or thrombocytopenia) attributable to CLL and unrelated to auto-immune cytopenia or treatment toxicity.
Notes for PD criteria

1) Progression of lymphadenopathy, hepatomegaly and/or splenomegaly is often detected by clinical examination but may be also detected by imaging. However sequential imaging is not warranted in CLL outside clinical trials and is not required for the EBMT Registry.

2) Caution with liver assessment: Given the impact of numerous medical conditions, liver size should only be counted if hepatomegaly is clearly attributable to lymphoid involvement.

3) In the setting of splenomegaly, the splenic length must increase by 50% or more of the extent of its prior increase beyond baseline (e.g., a 15-cm spleen must increase to ≥16 cm). If no prior splenomegaly was observed at baseline or if splenomegaly has resolved with treatment, the spleen must increase by at least 2 cm from baseline.

4) Certain therapies (e.g., kinase inhibitors) may cause lymphocytosis. In the setting of therapy with such agents, an increase in blood lymphocyte count by itself does not uniformly indicate an increased tumour burden, but may reflect redistribution of leukaemia cells from lymphoid tissues to the blood. This should be predefined in the protocol of clinical trials for therapies in which redistribution of disease occurs. In such cases, increased lymphocytosis alone is not a sign of treatment failure or PD.

5) A cytopenia can be used as criteria of progression if
   a. Intensity:
      i. decrease of haemoglobin by 2 g/dL or more (or less than 10 g/dL)
      ii. decrease of platelet counts by 50% or more (or less than 100,000/μL)
   b. Occurrence after 3 months post last CLL treatment
   c. Bone marrow assessment is in favour of CLL involvement and not of treatment related toxicity

Relapse (untreated): Relapse is defined as evidence of PD (see above) in a patient who has previously achieved the criteria of a CR or PR (see above) for 6 months or more after the last dose of CLL therapy.

Refractory disease (untreated): Refractory disease is defined as either absence of response (CR/PR) or evidence of PD within 6 months of the last dose of CLL therapy.

Never treated: This situation describes CLL patients submitted to main treatment (cellular therapy) without prior lines of therapy.

Unknown.

12.2. Minimal residual disease (MRD) at initiation of treatment:

If the patient was in Complete remission (CR) or partial remission (PR, the MRD status needs to be reported.

The techniques for assessing MRD include, six-colour (or more) flow cytometry (MRD flow/MRD FACS), Allele-specific oligonucleotide PCR or next generation sequencing. Using such techniques patients will be defined as having undetectable MRD (MRD-neg) remission if they have blood or marrow with <1 CLL cell per 10,000 leukocytes.

Indicate if the MRD status was positive or negative, or not evaluated.

13. Prolymphocytic (PLL) and Other Chronic Leukaemias

13.1. Status

A. For T-cell prolymphocytic leukaemia (T-PLL) according to the T-PLL consensus criteria (11), the response evaluation should be classified as follows:
Complete remission (CR): All the following criteria are required:

- Absence of lymphadenopathy.
- Absence of splenomegaly and hepatomegaly.
- Absence of disease-related constitutional symptoms.
- Lymphocyte count < 4,000 /μL in peripheral blood.
- Clearance of phenotypic T-PLL cells in bone marrow (<5% of mononuclear cells).
- Absence of other specific site involvement if previously involved.
- Complete recovery of bone marrow function.

Notes for CR criteria

1) For previously enlarged lymph nodes (≥1.5 cm long-axis diameter), regression of the long-axis diameters to <1.0 cm. A CT scan (or any other imaging procedure) for assessment of lymphadenopathy, hepatomegaly and splenomegaly is required in clinical trials. For the EBMT Registry, clinical (palpation) evaluation only without CT-scan or alternate imaging, according to routine practice, is accepted.

2) In analogy to criteria for CLL a cut-off of 13 cm in craniocaudal length defines splenomegaly. Currently, there are no clear-cut criteria for hepatomegaly.

3) The formal guidelines for CR definition require bone marrow aspirate and biopsy to confirm a CR in clinical trials. The cytological or histological evaluation needs to document a bone marrow with T-PLL less than 5% of nucleated cells (assessed by immunohistochemistry or flow cytometry). For the EBMT Registry, if a patient has all criteria of a CR but bone marrow evaluation has not been performed (even with persistent cytopenia), the patient can be considered as a CR as an adaptation of these guidelines. In this case, the absence of bone marrow evaluation (not performed or not available) should be recorded.

4) If previously involved, absence of pleural or peritoneal effusions, skin infiltrates, and involvement of central nervous system, or other extra-medullary sites.

5) The formal guidelines for CR definition require adequate blood count including:
   a. neutrophils ≥1,500/μL
   b. platelets ≥100,000/μL (without blood transfusion).
   c. haemoglobin ≥11 g/dL (without blood transfusion).

Complete remission with incomplete marrow recovery (CRI): Patients with all CR criteria but without complete recovery of bone marrow function (i.e. persistent anaemia (haemoglobin <11 g/dL) and/or thrombocytopenia (platelets < 100,000/μL) and/or neutropenia (neutrophils ≥1,500/μL) that are unrelated to T-PLL are classified CRI.

Partial remission (PR): To define a PR, at least 2 parameters of group A and 1 parameter of group B (see above) need to improve, if previously abnormal. If only 1 parameter within each group was abnormal before therapy, only 1 needs to improve.

Among group A:

- A blood lymphocyte count below 30,000/μL and a decrease of 50% or more from the baseline value (prior to therapy)
- For previously enlarged lymph nodes (≥ 1.5 cm long axis diameter), at least a 30% decrease in lymph node size in the sum of long-axis diameters of up to 3 lymph nodes.
- Regression of splenomegaly by 50% or less in vertical length beyond normal (13 cm) from baseline compared with baseline.
- Any result other than CR on bone marrow evaluation, if performed.
Among group B:

- Polymorphonuclear leukocytes ≥1,500/μL or 50% improvement over baseline without G-CSF support;
- Platelet counts ≥100,000/μL or 50% improvement over baseline without transfusion.
- Haemoglobin ≥11.0 g/dL or 50% improvement over baseline without transfusions or erythropoietin support.

**Notes for PR criteria**

1) A CT scan (or any other imaging procedure) for assessment of lymphadenopathy, hepatomegaly and splenomegaly is required in clinical trials. For the EBMT Registry, clinical (palpation) evaluation only without CT-scan (or alternate imaging), according to routine practice, is accepted.

**Stable disease (SD):** Patients who have not achieved a CR, a CRi or a PR, and who have not exhibited progression, will be considered to have no change (which is equivalent to a non-response).^1

**Notes for SD criteria**

1) According to T-PLL consensus a SD should be sustained for at least 3 months. However, for the EBMT Registry, if the patient received the main treatment (transplantation or other cellular therapy) before 3 months duration of SD, the reported status at main treatment remains SD.

**Progressive disease (PD):** Progressive disease is defined by at least one of the following:

- Progressive lymphadenopathy: indicated by a more than 20% increase in the sum of long-axis diameters of up to 3 target lesions; for small lymph nodes measuring less than 1.5 cm after therapy, a minimum increase of 5 mm and a long-axis diameter of more than 1.5 cm, or appearance of a new lesion
- An increase in the spleen size by 50% or more in vertical length beyond normal from baseline or the de novo appearance of splenomegaly
- Presence of disease-related constitutional symptoms
- An increase in the number of blood lymphocytes by 50% or more from baseline.
- Demonstration of T-PLL involvement by more than 5% on bone marrow evaluation
- Progression or apparition of any other site of involvement including pleural or peritoneal effusion, skin infiltration, central nervous system
- Occurrence of cytopenia (neutropenia, anaemia or thrombocytopenia) attributable to T-PLL and unrelated to auto-immune cytopenia or treatment toxicity.

**Notes for PD criteria**

1) Progression of lymphadenopathy, hepatomegaly and/or splenomegaly is often detected by clinical examination, but may be also detected by imaging. However sequential imaging is not warranted in CLL outside clinical trial and is not required for the EBMT Registry.

2) A cytopenia can be used as criteria if:
   - decrease of haemoglobin by 2 g/dL or more from baseline
   - decrease of platelet counts by 50% or more from baseline
   - decrease of neutrophils counts by 50% or more from baseline
**Relapse (untreated):** Relapse is defined as evidence of PD (see above) in a patient who has previously achieved the criteria of a CR, CRi, PR or SD (see above).

**Refractory disease (untreated):** Refractory disease is defined as disease progression in the absence of previous response or disease control (CR, CRi, PR or SD) therapy.

**Never treated:** This situation describe T-PLL patients submitted to main treatment (cellular therapy) without prior lines of therapy.

**Unknown.**

B. For B-PLL: use the iwCLL CLL criteria (see above).

C. For other Chronic Leukaemias attribute status at treatment by choosing the corresponding check box, according to local evaluation:

- **Complete remission (CR):**
- **Partial remission (PR):**
- **Stable disease (SD):**
- **Relapse (untreated):**
- **Progressive disease (PD):**
- **Never treated:**
- **Unknown.**
14. Lymphomas

14.1. Disease status

Select the appropriate disease status for the patient at the time of treatment.

- **Complete remission** (CR): Complete absence of disease, no signs or symptoms of the original disease.
- **Partial response** (PR) with or without prior CR: Reduction in the disease of 50% or more.
- **Stable disease**: Less than 50% reduction in the disease burden.
- **Untreated relapse from previous CR/untreated progression from previous PR**: Worsening of the disease status in patients in PR or re-appearance of the lymphoma in patients in CR, such as: recurrence of disease or systemic symptoms (B-symptoms), patient remains untreated after the relapse or progression.
- **Chemorefractory relapse or progression, including primary refractory disease**: Does not present any of the features of any type of remission after treatment.
- **Disease status: unknown**.

14.1.1. Complete remission confirmed:

If the patient was in CR, indicate if the complete remission was confirmed or not, by selecting either Confirmed or Unconfirmed.

14.1.2. Histopathological verification of relapse

If the patient was in a relapse or progression, report if the relapse or progression was histopathologically verified by selecting answer Yes, or not verified - select No.

14.2. Technique used for disease assessment

Select the technique that was used for the assessment of the disease status:

- **CT scan**;
- **PET**;
- **MRI**.

*Parameters for prognostic indices:*

14.3. Age at diagnosis

Indicate the patient’s age at the time of treatment.

The EBMT Registry will automatically complete this item.

14.4. LDH levels elevated

Indicate if serum lactate dehydrogenase (LDH) level is elevated (answer Yes) as per the reference laboratory’s ranges, not elevated (answer No) or it was Not evaluated by clicking the correspondent answer box.
14.5. Ann Arbor staging

The Ann Arbor staging system is widely used for anatomic staging of lymphoma, both Hodgkin and non-Hodgkin lymphomas. The definition of these stages can be found in the AJCC Cancer Staging Manual (7th edition) or Union for International Cancer Control (UICC) staging manual. Check the box Not evaluated if it was not assessed.

<table>
<thead>
<tr>
<th>Stage</th>
<th>Definition</th>
</tr>
</thead>
<tbody>
<tr>
<td>I</td>
<td>Involvement of a single lymph node region (I), or localized involvement of</td>
</tr>
<tr>
<td></td>
<td>a single extralymphatic organ or site in the absence of any lymph node</td>
</tr>
<tr>
<td></td>
<td>involvement (IE).</td>
</tr>
<tr>
<td>II</td>
<td>Involvement of two or more lymph node regions on the same side of the</td>
</tr>
<tr>
<td></td>
<td>diaphragm (II), or localized involvement of a single extralymphatic organ</td>
</tr>
<tr>
<td></td>
<td>or site in association with regional lymph node involvement with or</td>
</tr>
<tr>
<td></td>
<td>without the involvement of other lymph node regions on the same side of</td>
</tr>
<tr>
<td></td>
<td>the diaphragm (IIE). The number of regions involved may be indicated by</td>
</tr>
<tr>
<td></td>
<td>a subscript, for example, II3.</td>
</tr>
<tr>
<td>III</td>
<td>Involvement of lymph node regions on both sides of the diaphragm (III),</td>
</tr>
<tr>
<td></td>
<td>which also may be accompanied by extralymphatic extension in association</td>
</tr>
<tr>
<td></td>
<td>with adjacent lymph node involvement (IIIE) or by the involvement of the</td>
</tr>
<tr>
<td></td>
<td>spleen (IIIS) or both (IIIE,S).</td>
</tr>
<tr>
<td>IV</td>
<td>Diffuse or disseminated involvement of one or more extralymphatic organs,</td>
</tr>
<tr>
<td></td>
<td>with or without associated lymph node involvement; or isolated extralymphatic</td>
</tr>
<tr>
<td></td>
<td>organ involvement in the absence of adjacent regional lymph node</td>
</tr>
<tr>
<td></td>
<td>involvement, but in conjunction with the disease in distant site(s). Any</td>
</tr>
<tr>
<td></td>
<td>involvement of the liver or bone marrow or nodular involvement of the</td>
</tr>
<tr>
<td></td>
<td>lung(s) is always Stage IV. Stage IV disease is identified further by</td>
</tr>
<tr>
<td></td>
<td>specifying the site according to the notations listed for Stage III.</td>
</tr>
</tbody>
</table>

Table 4, Ann Arbor stage definitions (2,3)

14.6. ECOG performance status

The ECOG performance status scale describes a patient’s level of functioning in terms of their ability to care for themselves, daily activity, and physical ability (walking, working, etc.). It is published here. Check the box Not evaluated if it was not assessed.

<table>
<thead>
<tr>
<th>Grade</th>
<th>ECOG performance status</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>Fully active, able to carry on all predisease performance without restriction</td>
</tr>
<tr>
<td>1</td>
<td>Restricted in physically strenuous activity but ambulatory and able to carry out</td>
</tr>
<tr>
<td></td>
<td>work of a light or sedentary nature, e.g., light housework, office work</td>
</tr>
<tr>
<td>2</td>
<td>Ambulatory and capable of all self-care but unable to carry out any work activities;</td>
</tr>
<tr>
<td></td>
<td>up and about more than 50% of waking hours</td>
</tr>
</tbody>
</table>
3
|   | capable of only limited self-care; confined to bed or chair more than 50% of waking hours |

4
|   | completely disabled; cannot carry on any self-care; totally confined to bed or chair |

Table 5, definitions of ECOG scores (4)

14.7. 1 extranodal site involved
Indicate if more than 1 extranodal site (area or organ outside of the lymph nodes, spleen, thymus, and the pharyngeal lymphatic ring) was involved at the time of diagnosis (answer Yes). Answer No if only 1 or no extranodal sites were involved at the time of diagnosis. Check the box Not evaluated if the index was not assessed.

14.8. 4 nodal sites involved
Indicate if more than 4 nodal sites were involved at the time of diagnosis (answer Yes), otherwise answer No. Check the box Not evaluated if the index was not assessed.

14.9. Haemoglobin < 120g/L
Indicate if the haemoglobin (haemoglobin) level was lower than 120g/L at the time of diagnosis (answer Yes), otherwise answer No. Check the box Not evaluated if the haemoglobin level was not assessed.

14.10. White Blood Cell count
Indicate the number of white blood cells x 10⁹ cells/L at the time of diagnosis or make a corresponding mark if it was Not evaluated.

15. MDS

15.1. MDS Classification at diagnosis (WHO 2016):
Select the sub-class that is appropriate for MDS and check the box next to it.

15.2. Status:
Indicate the disease status at the time of HCT/CT/IST by choosing one of the following answer options:
Select Unknown, if the status is not known.

Complete remission (CR)
- For patients with MDS – EB: Complete remission was achieved if marrow blast count was below 5% and normalisation of peripheral blood counts was observed for at least 4 weeks.
- For patients with other types of MDS: normalisation of PB counts.
Report also the number of the complete remission.
Improvement but no CR:

1) Haematological response (in patients with cytopenia)
   - If haemoglobin < 11g/dl, erythroid response (>11 g/dl);
   - If platelets <100g/l, platelet response (>100 g/l);
   - If neutrophils < 1g/l, neutrophil response (>1g/l);
   - If >0% peripheral blasts, response when 0% peripheral blood blasts;
   - If transfusion dependant (red blood cells), independence of transfusion achieved (8 weeks without transfusions);
   - If transfusion dependant (platelets), independence of transfusion achieved (8 weeks without transfusions)

2) Marrow blast response (in patients with marrow EB): A decrease of 50% in marrow blasts, but still >5% marrow blasts.

Primary refractory phase (no change) - Treatment with the intent to achieve remission was given, but no remission was achieved.

Relapse - Loss of complete remission.

Report also the number of the relapse.

Progression/Worsening - More blasts in BM than before treatment.

Never treated (supportive care or treatment without chemotherapy) - No treatment was given (blood transfusions are not considered a treatment in this context).

16. MDS/MPN

16.1. MDS/MPN Classification:

Select the subclassification that is appropriate for the MDS/MPN and check the box next to it. For definitions, see the MDS/MPN manual.

16.1.1. CMML type:

Report if CMML type is Myelodysplastic or Myeloproliferative by choosing corresponding answer option. Two main phenotypic types of CMML can be distinguished: Myelodysplastic (MD-CMML, WBC < 13×10^9/L) and Myeloproliferative (MP-CMML, WBC > 13×10^9/L). Patients with myeloproliferative type tend to have bulkier splenomegaly and more often have extramedullary infiltrations. MP-CMML is commonly associated with activating RAS pathway mutations and adverse clinical outcomes. Even though no difference exists with regard to the AML transformation rate, patient life expectancy is generally shorter in MP-CMML than in MD-CMML.

16.1.2. CMML WHO subclassification (2016):

Report the CMM subclass based on WHO 2016 classification.
According to the WHO, CMML can be further subclassified according to the percentage of blasts in peripheral blood and in bone marrow into CMML-0, CMML-1 and CMML-2 (2):

<table>
<thead>
<tr>
<th>CMML WHO subclassification (2016)</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>CMML-0</td>
<td>&lt;2% blasts in the blood and &lt;5% blasts in the bone marrow</td>
</tr>
<tr>
<td>CMML-1</td>
<td>2-4% blasts in the blood and/or 5-9% blasts in the bone marrow</td>
</tr>
<tr>
<td>CMML-2</td>
<td>5-19% blasts in the blood and 10-19% blasts in the bone marrow or presence of Auer rods</td>
</tr>
</tbody>
</table>

Table 6, WHO CMML subclassifications

If the exact CMML subclassification is unknown, select Unknown.

16.2. Status:

Indicate the disease status at the time of HCT/CT/IST by choosing one of the following answer options:

- **Complete remission (CR)** - Complete remission was achieved if marrow blast count is < 5% and a normalisation of peripheral blood counts was observed for at least 4 weeks.
- **Improvement but no CR** - Bone marrow blasts decreased by ≥ 50% after pre-treatment but still > 5%. All CR criteria were abnormal before treatment.
- **Primary refractory phase (no change)** - Treatment with intent to achieve remission was given, but no remission was achieved.
- **Relapse** - Loss of complete remission.
- **Progression/Worsening** - Higher blast count in the BM and/or PB than before treatment. Worsening of cytopenias (anaemia and/or thrombocytopenia). Progression from the MD- to the MP-variant of CMML.
- **Never treated (supportive care or treatment without chemotherapy)** - No treatment was given (blood transfusions are not considered treatment in this context).

In addition, report the response number for Complete remission (CR) and Relapse.

Select Unknown, if the status is not known.

17. MPN

17.1. MPN Classification (WHO 2016):

Select the subclassification that is appropriate for the Myeloproliferative neoplasm, and check the box next to it. For definitions, see the MPN manual.
If the subclassification is not listed, check the box **Other** and specify the MPN classification (for example, Myeloid and lymphoid neoplasms with FLT3 rearrangement).

### 17.2. Status:

Indicate the disease status at the time of HCT/CT/IST by choosing one of the answer options. For both primary and secondary myelofibrosis, which is the most common indication for transplant within the MPN disorders, disease status should be defined as follows:

**Complete remission (CR)** - the 4 following criteria must be true:

1. Resolution of disease-related symptoms and signs including palpable hepatosplenomegaly.
2. Haemoglobin (Hb) ≥ 10g/dL, platelet ≥ 100 × 10^9/L and neutrophils ≥ 1 × 10^9/L.
3. <2% immature myeloid cells (<5% in splenectomized patients)
4. Normal bone marrow histology and fibrosis grade no higher than 1

**Improvement but no CR** - requires one criterion in absence of progression:

1. Hb increase of 2g/dL or transfusion independence
2. Spleen reduction of 50%
3. 100% increase in platelet count and an absolute platelet count of at least 50 × 10^9/L
4. 100% increase in absolute neutrophil count (ANC) and an ANC of at least 0.5 × 10^9/L

**Primary refractory phase (no change)** - Treatment with intent to achieve remission was given, but no remission was achieved.

**Relapse** - loss of complete remission.

**Progression/Worsening** - requires one of the following:

1. Progressive splenomegaly
2. Leukaemic transformation (increase of peripheral blood blast percentage of at least 20%)

**Never treated (supportive care or treatment without chemotherapy)** - No treatment was given (blood transfusions are not considered a treatment in this context).

Note: If transformed to Acute Leukaemia at HCT, report the status of the Acute Leukaemia.

In addition, report the response number for **Complete remission (CR)** and **Relapse**.

Select **Unknown**, if the status is not known.

### 17.3. Blast count (peripheral blood):

Indicate peripheral blood blasts count in %. Select **Not evaluated** if the blast count was not assessed. If the value is unavailable, check **Unknown**.
17.4. Spleen size:
Indicate the size of the spleen in centimetres, measured below the costal margin as assessed by physical exam. Select Not evaluated if the spleen size was not assessed. If the value is unavailable, check Unknown.

17.5. Spleen span in ultrasound or CT scan:
Indicate the maximum diameter of the spleen in centimetres, as assessed by ultrasound or CT scan. Select Not evaluated if the spleen span was not assessed. If the value is unavailable, check Unknown.

17.6. JAK inhibitor exposure between diagnosis and treatment:
JAK inhibitor therapy, when given before a transplant, may help in (1) reducing splenomegaly; (2) decreasing symptoms due to proinflammatory cytokines; and (3) improving performance status before HCT.
Indicate if the patient was treated with a JAK inhibitor after diagnosis and prior to the treatment by checking either Yes or No. If it is not known whether the patient was treated with a JAK inhibitor or not, select Unknown.

17.7. Was a JAK inhibitor continued during conditioning?
Answer this question only if you selected Yes in the previous question. Select Yes if the patient was still treated with a JAK inhibitor during conditioning. Otherwise, choose No. If answered Yes, also specify the Dose of the inhibitor in mg/day and the Start date and End date of the treatment.

17.8. Response status:
Specify the type of response achieved by the time of treatment.

- **Spleen response** - It is achieved when a baseline splenomegaly that is palpable at 5-10 cm below the left costal margin (LCM) becomes not palpable or a baseline splenomegaly that is palpable at >10 cm below the LCM decreases by ≥ 50%. A baseline splenomegaly that is palpable at <5 cm below the LCM, is not eligible for spleen response. A spleen response requires confirmation by MRI or computed tomography showing ≥ 35% spleen volume reduction.

- **No response/loss of response** - No apparent change or worsening.

- **Primary resistance** - absence or minor reduction in spleen size and constitutional symptoms.

If the response status is not known, choose Unknown.
17.9. DIPSS Risk score at treatment: (Myelofibrosis either primary or secondary to ET/PV):

The Dynamic International Prognostic Scoring System (DIPSS) score places a time-dependent risk evaluation over the original IPSS evaluation, generating a new prognostic score.

<table>
<thead>
<tr>
<th>Prognostic factors</th>
<th>Points</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>0</td>
</tr>
<tr>
<td>Age (years)</td>
<td>≤ 65</td>
</tr>
<tr>
<td>WBC (x 10^9/L)</td>
<td>≤ 25</td>
</tr>
<tr>
<td>Haemoglobin (g/dL)</td>
<td>≥ 10</td>
</tr>
<tr>
<td>% Peripheral blood blasts</td>
<td>&lt; 1</td>
</tr>
<tr>
<td>Constitutional symptoms</td>
<td>No</td>
</tr>
</tbody>
</table>

Table 7, prognostic factors for DIPSS calculation

<table>
<thead>
<tr>
<th>DIPSS score</th>
<th>Total number of points</th>
<th>Median OS¹ (years)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Low risk</td>
<td>0</td>
<td>Not reached</td>
</tr>
<tr>
<td>Intermediate-1:</td>
<td>1-2</td>
<td>14.2</td>
</tr>
<tr>
<td>Intermediate-2</td>
<td>3-4</td>
<td>4</td>
</tr>
<tr>
<td>High risk</td>
<td>5-6</td>
<td>1.5</td>
</tr>
</tbody>
</table>

Table 8, DIPSS scores based on prognostic factors

If the DIPSS score was not assessed, select Not evaluated. If the score is unavailable, check Unknown.

17.10. MIPSS70 score at treatment (Primary Myelofibrosis only):

The Mutation-Enhanced International Prognostic Scoring System (MIPSS70) is based on three genetic variables and six clinical risk factors present at diagnosis:

- Haemoglobin (Hb) < 10 g/dL
- WBC > 25×10^9/L

¹ Overall survival
• Platelets < $100 \times 10^9$/L
• Peripheral blood blasts ≥ 2%
• Bone marrow fibrosis grade ≥ 2
• Constitutional symptoms
• Absence of CALR type 1/like mutation
• Presence of any high molecular risk [HMR] mutation, specifically ASXL1, SRSF2, EZH2, IDH1, or IDH2
• Presence of ≥2 HMR mutations

<table>
<thead>
<tr>
<th>MIPSS70 score</th>
<th>Total number of points</th>
<th>Median OS¹² (years)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Low risk</td>
<td>0–1</td>
<td>27.7</td>
</tr>
<tr>
<td>Intermediate</td>
<td>2–4</td>
<td>7.1</td>
</tr>
<tr>
<td>High risk</td>
<td>≥ 5</td>
<td>2.3</td>
</tr>
</tbody>
</table>

Table 9, MIPSS70 scores

You can visit [http://www.mipss70score.it/](http://www.mipss70score.it/) for the MIPSS70 score calculation.

If the MIPSS70 score was not assessed, select **Not evaluated**. If the score is unavailable, check **Unknown**.

17.11. **MYSEC-PM score at time of secondary MF diagnosis (Secondary myelofibrosis only; post-ET MF, post-PV MF):**

Myelofibrosis Secondary to PV and ET-Prognostic Model (MYSEC-PM) is a prognostic risk score for patients with secondary myelofibrosis. It identified six variables associated with poor outcome and subgroups patients into four risk levels accordingly. The variables are:

• Age (0.15 points per year);
• Hb < 11g/dL (2 points);
• Platelets < $150 \times 10^9$/L (1 point);
• Peripheral blood blasts ≥ 3% (2 points);
• Constitutional symptoms (1 point);
• Lack of CALR mutation (2 points).

<table>
<thead>
<tr>
<th>MYSEC-PM score</th>
<th>Total number of points</th>
<th>Median OS² (years)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Low risk</td>
<td>&lt; 11</td>
<td>NA</td>
</tr>
</tbody>
</table>

² Overall Survival
### Table 10, MYSEC-PM scores

| Intermediate-1: | 11 to <14 | 9.3 |
| Intermediate-2 | 14 to <16 | 4.5 |
| High risk      | ≥16       | 2   |

Table 10, MYSEC-PM scores

You can visit [http://www.mysec-pm.eu/](http://www.mysec-pm.eu/) for the MYSEC-PM score calculation.

If the MYSEC-PM score was not assessed, select **Not evaluated**. If the score is unavailable, check **Unknown**.

### 18. Plasma cell disorders including MM

#### 18.1. Status

Report response status at HCT/CT/IST by choosing the corresponding check box.

**MRD negative CR**

According to the International Myeloma Working Group (IMWG) 2016 recommendations, a CR is MRD negative when no myeloma cells are detected in a bone marrow aspirate sample to a sensitivity of at least $10^5$ normal cells by multi-parameter flow cytometry (MFC) or Next Generation Sequencing (NGS) technology in MM patients who have achieved CR.

**Complete remission (CR)** – All of the following:

Absence of detectable monoclonal immunoglobulin in serum and monoclonal light chains in the urine by immunofixation. The detection of monoclonal immunoglobulin, even at low levels which are too weak to quantitate, is not a CR.

- <5% of plasma cells in bone marrow aspirate
- Disappearance of any soft tissue plasmacytomas.
- No increase in size or number of lytic lesions if assessed (radiographic studies are not mandatory)

If any of the above investigations have not been done, even if the others are indicative of a CR, the status should be registered as VGPR.

Where CR has already been attained (bone marrow evaluation included) it may not be necessary to do a bone marrow evaluation again to confirm that the patient is still in CR (all other criteria confirming CR). Therefore, the patient is still in CR.

**Stringent complete remission (sCR)** – All of the following:

- CR as defined above
- normal free light (FLC) chain ratio
- Absence of clonal cells in bone marrow by immunohistochemistry or immunofluorescence

**MRD (Minimal Residual Disease) Negative CR** – All of the following:

- Stringent complete remission (sCR) as defined above
- No myeloma cells are detected in a bone marrow aspirate sample to a sensitivity of at least 10^5 normal cells by multiparameter flow cytometry (MFC) or Next Generation Sequencing (NGS) technology
- As per International Myeloma Working Group (IMWG) 2016 recommendations

**Very good partial remission (VGPR)** – One or more of the following:

- Serum and urine M-protein detectable by immunofixation but not on electrophoresis
- >=90% reduction in serum M-protein plus urine M-protein level <0.1g/ per 24h

In addition, there must be no increase in size or number of lytic lesions if assessed (radiographic studies are not mandatory)

If any of the above investigations have not been done, even if the others are indicative of a VGPR, the status should be registered as PR.

**Partial remission (PR)** – All of the following:

- >50% reduction in serum M-protein plus reduction in 24h urinary M-protein by >=90% or to <0.2g/ per 24h
In the absence of measurable serum and urine M-protein, the following criteria applies:
  - A decrease in the difference between involved and uninvolved free light chain (FLC) of more than 50%
  - If the FLC assay cannot be measured, the following criteria applies:
    - >=50% reduction in plasma cells provided baseline bone marrow plasma cell percentage was >=30%
    - A reduction of more than 50% in the size of soft tissue plasmacytomas if present at pre-treatment
- A reduction of more than 50% in the size of soft tissue plasmacytomas if present at pre-treatment
- No increase in size or number of lytic lesions if assessed (radiographic studies are not mandatory)

**Stable disease / No change**

Does not meet the criteria for CR, VGPR, PR or progressive disease (includes the old Minimal response (MR) criteria)

**Progression** - One or more of the following:

- Increase of 25% or more in measurable monoclonal immunoglobulin in serum and urine (absolute increase must be >=0.5g/dL). This is not applicable to light chain myelomas
- Increase of 25% or more in urinary light chains (absolute increase must be >=0.2g/ per 24h)
In the absence of measurable serum and urine M-protein, the following criteria applies:

- An increase of 25% or more in the difference between involved and uninvolved free light chain (absolute increase must be >0.01g/dL from nadir)
- An increase of 25% or more in bone marrow plasma cells (absolute % must be >=10%)
- Increase of old/appearance of new osteolytic bone lesions on x-ray
- Appearance of soft tissue plasmacytoma
- Development of hypercalcemia (corrected serum calcium >11.5 mg/dL or 2.65 mmol/L) that can be attributed solely to the plasma cell disorder

Never treated

No treatment was given.

Unknown

Select unknown if the disease status was not known.

18.2. Number

In addition, report the response number (if it is 1st, 2nd, 3rd or higher or if it is Unknown) if any of the following disease statuses was chosen:

- Stringent complete remission (sCR);
- Complete remission (CR);
- Very good partial remission (VGPR);
- Partial remission (PR);
- Stable disease / No change;
- Progression.

19. Solid tumours

19.1. Status

Report disease status at treatment by choosing the corresponding check box (5).

- **Adjuvant**: the patient has no residual disease after surgery and the HCT/CT/IST is part of the consolidation treatment; metastatic patients can never be considered adjuvant.
- **Never treated (upfront)**: the patient has never been treated for this disease and the high dose chemotherapy (HDC) is part of the overall treatment strategy. It is possible that high-dose therapy is preceded by some courses of standard-dose therapy. In this continuum, high-dose therapy is considered as upfront.
- **Stable disease/no response**: neither sufficient shrinkage to qualify for PR nor sufficient increase to qualify for PD.
- **Complete remission (CR)**: the patient has achieved complete absence of disease prior to the treatment and the treatment is not part of any adjuvant therapy. Additionally, indicate:
  - if it is “Unconfirmed” (UCR – complete response with persistent scan abnormalities of unknown significance) or “Confirmed” (CR with No abnormalities detected in scan)
  - the number (“1st”, “2nd”, “3rd or higher” or if it is “Unknown”)

Unknown
19.1. Disease status (HCT/CT/IST Day 0):

- **1st partial response (PR1):** patient achieved a reduction in disease of 30% or more for the first time ever, but did not achieve complete remission.
- **Relapse:** reappearance of disease in patients whose last disease status was complete remission. Additionally, indicate:
  - number of this relapse ("1st", "2nd", "3rd or higher" or if it is "Unknown")
  - sensitivity to chemotherapy:
    - **Sensitive:** patient achieves a reduction of >50% in the disease burden after treatment for this relapse.
    - **Resistant:** patient has not achieved a reduction of more than 50% in the disease burden after treatment for this relapse.
    - **Untreated:** patient has not been treated for this relapse
- **Progressive disease (PD):** pay attention as it can overlap with relapse - both can be indicated. At least a 20% increase in dimension of lesions
- **Unknown**

19.2. Organ involvement at time of this HCT/CT/IST

If the disease status was **not Complete remission (CR),** report all organs involved at time of this HCT/CT/IST:

- Nodes below diaphragm;
- Nodes above diaphragm;
- CNS;
- Liver;
- Bone;
- Lung;
- Soft tissue.

If the organ is not listed, check the **Other** and specify it in the text field.

19.3. Risk category at disease recurrence (or platinum refractoriness) following first line chemotherapy

If the patient was treated for a germ cell tumour, report the risk category according to International Prognostic Factors Study Group classification published in 2010 (6).

20. Autoimmune disorders

Complete the questions relevant for the autoimmune disease the patient received an HCT/CT/IST for.

20.1. Disease status (Systemic sclerosis only):

There is no universally accepted disease activity score for SSc, which is why skin and organ involvement are assessed separately.

20.2. SSc subset:

For patients with Systemic sclerosis only, report the SSc subset:
Disease_status_HCT_CT_IST_Day0_v1.0

- Diffuse cutaneous;
- Limited cutaneous;
- Sine scleroderma.

If no answer option is applicable, select Other and specify it.

Assessments at time of mobilisation (within 3 months before mobilisation):

The results of the tests assessing disease activity at the time of mobilisation should be reported here.

20.3. Creatinine Clearance (Cockcroft-Gault formula):

Creatinine is one of the clinical markers of renal dysfunction, observed in patients with SSc. Report the Creatinine Clearance value in ml/min as calculated according to the Cockcroft-Gault formula. If the value is not known, select Unknown.

20.4. Proteinuria:

Proteinuria is another marker of renal dysfunction observed in patients with SSc. Indicate urine total protein value in g/24hrs. If the value is not known, select Unknown.

20.5. Modified Rodnan Skin Score (0-51):

The modified Rodnan skin score is a validated score to assess the extent of skin thickening with prognostic value: (persistently) high scores are associated with a worse outcome. Seventeen bodily areas (face, anterior chest, abdomen, upper arms, forearms, hands, fingers, thighs, lower legs, feet) are each scored for skin thickness on a scale from 0 to 3 (0=normal, 1=mild, 2=moderate, 3=severe), resulting in a maximum score of 51. The scoring requires training, and serial scores should preferably be done by one assessor to avoid interobserver variability.

Indicate the score value here. If the value is not known, select Unknown.

20.6. DLCO (corrected for Hb):

In systemic sclerosis, impaired diffusing capacity for carbon monoxide (DLCO) can indicate interstitial lung disease, pulmonary hypertension, and/or other disease manifestations, including anaemia.

Indicate DLCO value corrected for measured haemoglobin in percentage. If the value is not known, select Unknown.

20.7. Mean Pulmonary Arterial Systolic Pressure [PASP] (from right heart catheterisation):

Elevated mean Pulmonary Arterial Systolic Pressure is a marker of pulmonary hypertension, a common complication of SSc.

Indicate the value as measured by right heart catheterisation in mmHg.
20.8. GI Involvement:

The gastrointestinal tract (GI) is the second most affected organ system in individuals suffering from SSc. SSc can affect any part of the GI, between the oral cavity and anorectum.

Indicate if the GI was involved by selecting No or Yes. If the GI involvement was not assessed, select Not evaluated. In case it was not known, please report Unknown.

Status (Systemic lupus erythematosus only):

SLE clinical manifestations may vary in a single patient and among various types of patients from mild to moderate or severe, and therefore account for either isolated skin or arthritis manifestations with a few significant biological abnormalities or a multi-systemic aggressive form with major organ involvement predominantly affecting the kidneys (various types of glomerulonephritis), the heart (polyserositis), the brain (psychological manifestations, seizures, and encephalitis) which can hamper the vital prognosis.

The results of the tests assessing disease activity at the time of mobilisation should be reported here.

20.9. SLEDAI-2K Score:

SLEDAI-2K is the most commonly used score system for measuring global disease activity.

The SLEDAI-2K relies on the presence of several criteria corresponding to various clinical manifestations, each of which with its own scoring and a global score between 0 and 105.
**Disease status (Multiple sclerosis only):**

Disease activity in multiple sclerosis (MS) has traditionally been defined by the occurrence of new neurological symptoms and the rate of relapses. The definition of disease activity has become more refined with the use of clinical markers, evaluating ambulation, dexterity, and cognition. Magnetic resonance imaging (MRI) has become an important tool in the investigation of disease activity. The number of lesions as well as brain atrophy have been used as surrogate outcome markers in several clinical trials, for which a reduction in these measures is appreciated in most treatment studies.

---

<table>
<thead>
<tr>
<th>Weight</th>
<th>SLEDALI Score</th>
<th>Descriptor</th>
<th>Definition</th>
</tr>
</thead>
<tbody>
<tr>
<td>8</td>
<td>Seizure</td>
<td></td>
<td>Recent onset, exclude metabolic, infectious or drug causes.</td>
</tr>
<tr>
<td>8</td>
<td>Psychosis</td>
<td></td>
<td>Altered ability to function in normal activity due to severe disturbance in the perception of reality. Include hallucinations, incoherence, marked loose associations, impoverished thought content, marked illogical thinking, bizarre, disorganized, or catatonic behavior. Exclude urtica and drug causes.</td>
</tr>
<tr>
<td>8</td>
<td>Organic brain syndrome</td>
<td></td>
<td>Altered mental function with impaired orientation, memory, or other intellectual function, with rapid onset and fluctuating clinical features, inability to sustain attention to environment, plus at least 2 of the following: perceptual disturbance, incoherent speech, insomnia or daytime drowsiness, or increased or decreased psychomotor activity. Exclude metabolic, infectious, or drug causes.</td>
</tr>
<tr>
<td>8</td>
<td>Visual disturbance</td>
<td></td>
<td>Retinal changes of SLE. Include cyanotic bodies, retinal hemorrhages, serous exudate or hemorrhages in the choroid, or optic neuritis. Exclude hypertension, infection, or drug causes.</td>
</tr>
<tr>
<td>8</td>
<td>Cranial nerve disorder</td>
<td></td>
<td>New onset of sensory or motor neuropathy involving cranial nerves.</td>
</tr>
<tr>
<td>8</td>
<td>Lupus headache</td>
<td></td>
<td>Severe, persistent headache; may be migrainous, but must be unresponsive to narcotic analgesia.</td>
</tr>
<tr>
<td>8</td>
<td>CVA</td>
<td></td>
<td>New onset of cerebrovascular accident(s). Exclude arteriosclerosis.</td>
</tr>
<tr>
<td>8</td>
<td>Vasculitis</td>
<td></td>
<td>Ulceration, gangrene, tender finger nodules, periangual infarction, splinter hemorrhages, or biopsy or angiogram proof of vasculitis.</td>
</tr>
<tr>
<td>4</td>
<td>Arthritis</td>
<td></td>
<td>Proximal muscle ache/weakness, associated with elevated creatine phosphokinase/ aldolase or electromyogram changes or a biopsy showing myositis.</td>
</tr>
<tr>
<td>4</td>
<td>Myositis</td>
<td></td>
<td>Hemi-granular or red blood cell cases.</td>
</tr>
<tr>
<td>4</td>
<td>Urinary casts</td>
<td></td>
<td>&gt;3 red blood cells/high power field. Exclude sepsis, infection or other cause.</td>
</tr>
<tr>
<td>4</td>
<td>Hematuria</td>
<td></td>
<td>&gt;3 red blood cells/high power field. Exclude infection.</td>
</tr>
<tr>
<td>4</td>
<td>Proteinuria</td>
<td></td>
<td>&gt;0.5 gram/24 hours</td>
</tr>
<tr>
<td>4</td>
<td>Pyuria</td>
<td></td>
<td>&gt;3 white blood cells/high power field. Exclude infection.</td>
</tr>
<tr>
<td>2</td>
<td>Rash</td>
<td></td>
<td>Inflammatory type rash.</td>
</tr>
<tr>
<td>2</td>
<td>Alopecia</td>
<td></td>
<td>Abnormal, patchy or diffuse loss of hair.</td>
</tr>
<tr>
<td>2</td>
<td>Mucosal ulcers</td>
<td></td>
<td>Oral or nasal ulcerations.</td>
</tr>
<tr>
<td>2</td>
<td>Pleural</td>
<td></td>
<td>Pleuritic chest pain with pleural rub or effusion, or pleural thickening.</td>
</tr>
<tr>
<td>2</td>
<td>Pericarditis</td>
<td></td>
<td>Pericardial pain with at least 1 of the following: rub, effusion, or electrocardiogram or echocardiogram confirmation.</td>
</tr>
<tr>
<td>2</td>
<td>Low complement</td>
<td></td>
<td>Decrease in CH50, C3, or C4 below the lower limit of normal for testing laboratory.</td>
</tr>
<tr>
<td>2</td>
<td>Increased DNA binding</td>
<td></td>
<td>Increased DNA binding by Farr assay above normal range for testing laboratory.</td>
</tr>
<tr>
<td>1</td>
<td>Fever</td>
<td></td>
<td>&gt;38°C. Exclude infectious cause.</td>
</tr>
<tr>
<td>1</td>
<td>Thrombocytopenia</td>
<td></td>
<td>&lt;100,000 platelets x 10^9/L, exclude drug causes.</td>
</tr>
<tr>
<td>1</td>
<td>Leukopenia</td>
<td></td>
<td>&lt;3,000 white blood cells x 10^9/L, exclude drug causes.</td>
</tr>
</tbody>
</table>

**Figure 1, SLEDALI-2K score (7)**

Indicate the score value here. If the score was not assessed, select **Not evaluated**. In case it was not known, please report **Unknown**.

---

37
20.10. Disease status at time of mobilisation (within 3 months before mobilisation):

Report the status of MS at the time of mobilisation by selecting one of the options from the list:

- **Primary progressive** (PPMS) is characterised by continuous disease progression without distinct acute disease exacerbations.
- **Secondary progressive** (SPMS) is characterised by acute disease exacerbations periods where there is disease progression after acute disease exacerbations.
- **Relapsing/remitting** (RRMS) disease course is characterised by a series of periods with acute disease exacerbations that resolve completely without worsening the neurological functions.

If the status is not available on the list, select **Other** and report the status name in the textbox in English.

20.11. EDSS (1-10):

The EDSS is a composite assessment, performed by the neurologist that illustrates the degree of disability associated with MS. It provides a useful snapshot of the disease status of a patient at a given time and a composite picture of the disease course over time. The EDSS is universally used in clinical trials (8).

Indicate the score value here. If the score was not assessed, select **Not evaluated**.

20.12. Number of gadolinium enhancing lesions present on MRI brain scan:

Gadolinium (Gd) enhancement is a marker for blood-brain barrier breakdown and histologically correlates with the inflammatory phase of lesion development.

Indicate the number of lesions present on the MRI brain scan here. If the number of lesions is not known, select **Unknown**.

**Status (Crohn’s disease only):**

The primary characteristic of Crohn’s disease is inflammation. Therefore, the measurement of gastrointestinal inflammation is a key component of disease and treatment monitoring, and there are various biochemical, imaging, and scoring methods to determine inflammatory disease activity.

The results of the tests assessing disease activity at the time of mobilisation should be reported here.

20.13. CDAI (0-700):

The Crohn’s disease activity index (CDAI) is a numerical calculation derived from the sum of products from a list of 8 items, and multiplied by weighting factors for each item to define the severity of “disease activity” in patients with Crohn’s disease (CD) (9).
<table>
<thead>
<tr>
<th>Item (daily sum per week)</th>
<th>Weighting factor</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of liquid or very soft stools</td>
<td>2</td>
</tr>
<tr>
<td>Abdominal pain score in one week (rating, 0-3)</td>
<td>5</td>
</tr>
<tr>
<td>General well-being (rating, 1-4)</td>
<td>7</td>
</tr>
<tr>
<td>Sum of physical findings per week:</td>
<td></td>
</tr>
<tr>
<td>- Arthritis/arthralgia</td>
<td></td>
</tr>
<tr>
<td>- Mucocutaneous lesions (e.g. erythema nodosum, aphthous ulcers)</td>
<td></td>
</tr>
<tr>
<td>Iritis/uveitis</td>
<td>20</td>
</tr>
<tr>
<td>- Anal disease (fissure, fistula, etc)</td>
<td></td>
</tr>
<tr>
<td>- External fistula (enterocutaneous, vesicle, vaginal, etc)</td>
<td></td>
</tr>
<tr>
<td>- Fever over 37.8°C</td>
<td></td>
</tr>
<tr>
<td>Antidiarrheal use (e.g. diphenoxylate)</td>
<td>30</td>
</tr>
<tr>
<td>Abdominal mass (no = 0, equivocal = 2, yes = 5)</td>
<td>10</td>
</tr>
<tr>
<td>47 minus haematocrit (males) or 42 minus haematocrit (females)</td>
<td>6</td>
</tr>
<tr>
<td>1-x (1-body weight divided by a standard weight)</td>
<td>1</td>
</tr>
</tbody>
</table>

Table 11, CDAI

Indicate the score value here. If the score was not assessed, select Not evaluated. In case it was not known, please report Unknown.

20.14. Serum albumin

Serum albumin concentration is a very sensitive marker of inflammatory activity in CD. Indicate the concentration of serum albumin in g/L here. In case it was not known, please report Unknown.
21. Haemoglobinopathies

The haemoglobinopathies section refers to the disease status at the time of indication for a curative treatment option, either HCT or gene editing.

21.1. Ferritin level

Report the ferritin level in ng/mL. Or mark as Not evaluated or Unknown.

21.2. Number of red blood cell transfusions

Report the number of red blood cell transfusions the patient was receiving before. Chose one of the answer options:

- <20 units;
- 20 to 50 units;
- >50 units;
- None;
- Unknown.

21.3. Liver iron concentration

Report the liver iron concentration in mg/g of the dry weight.

21.4. Pre-existing liver disease?

Report if there was pre-existing liver disease by answering Yes, otherwise answer No.

21.4.1. Hepatitis

If the patient had pre-existing liver disease (answered Yes to previous question), indicate if hepatitis was Absent or whether it was Chronic persistent hepatitis or Chronic active hepatitis.

21.4.2. Liver biopsy performed?

If the patient had pre-existing liver disease, report if a biopsy was performed (answer Yes) or not (answer No).

21.4.3. Liver fibrosis (Ishak staging)

If a liver biopsy was performed, select the relevant fibrosis stage according to Ishak staging that was found in the liver.

Further information on the Ishak staging can be found here: https://doi.org/10.1016/0168-8278(95)80226-6.

21.5. Pre-existing cardiac disease

Report if there was pre-existing cardiac disease by answering Yes, otherwise answer No.
21.5.1. Cardiac echography: ejection fraction

If the patient had pre-existing cardiac disease, report if there was cardiac echography ejection fraction (answer Yes), otherwise answer No.

21.5.2. Cardiovascular magnetic resonance (CMR) T2

If the patient had pre-existing cardiac disease, report the cardiovascular magnetic resonance (CMR) T2 in mg/g of the dry weight.

21.6. Chronic transfusion program

For the patients diagnosed with sickle cell disease, report if they were on a chronic red blood cell (RBC) transfusion program.

21.7. Pre-treatment complications

For patients diagnosed with sickle cell disease only, report if the patient had any pre-treatment complications and select all the overarching categories that apply and complete the subsequent questions of the section.

21.8. Cerebrovascular disease:

21.8.1. Abnormal Doppler

Indicate whether the Doppler test gave abnormal results prior to the HCT/CT/IST. Abnormal Doppler test results mean the transcranial Doppler ultrasonography velocity is 200 cm/sec or higher. If no Doppler test was performed, select Not evaluated.

21.8.2. Stroke

Indicate if the patient had a stroke prior to HCT/CT/IST. If not evaluated, select Not evaluated.

21.8.3. Haemorrhage

Indicate if any cerebrovascular haemorrhages (not strokes) were found prior to HCT/CT/IST. If not evaluated, select Not evaluated.

21.8.4. Arteriopathy

Indicate if the patient had any arteriopathies prior to HCT/CT/IST. If not evaluated, select Not evaluated.

21.8.5. Moyamoya disease

Indicate if the patient was diagnosed with moyamoya disease prior to HCT/CT/IST. If not evaluated, select Not evaluated.
21.8.6. Silent infarcts

Indicate if silent infarcts were diagnosed in the patient prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.9. Renal involvement:

21.9.1. Microalbumin level

Report the microalbumin level in mg/g measured prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.9.2. Glomerular filtration rate

Report the glomerular filtration rate in mL/min/1.73^2 measured prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.9.3. Avascular necrosis

Indicate if avascular necrosis was diagnosed in the patient prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.9.4. Hyperhaemolysis or autoimmune haemolytic anaemia

Indicate if the patient was diagnosed with hyperhaemolysis or autoimmune haemolytic anaemia prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.9.5. Hyperhaemolysis or autoimmune haemolytic anaemia; specify

Report if the patient had hyperhaemolysis or autoimmune haemolytic anaemia (answer Yes, No or Not evaluated). If the answer is Yes, indicate also if it was: **Hyperhaemolysis** or **Autoimmune haemolytic anaemia**.

21.10. Other SCD related complications

21.10.1. Acute chest syndrome

Indicate whether the patient was diagnosed with acute chest syndrome prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.10.2. Vaso-occlusive crisis

Indicate whether the patient had any vaso-occlusive crises prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.10.3. Priapism

Indicate whether priapism was observed in the patient prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.
21.10.4. Pulmonary Artery Pressure

Indicate if abnormal pulmonary artery pressure was observed in the patient prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.10.5. Chronic lung disease

Indicate whether the patient was diagnosed with a chronic lung disease prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.11. Endocrinopathies pre-existing to HCT

This section only needs to be completed for patients diagnosed with any type of thalassemia.

21.11.1. Hypothyroidism

Indicate whether the patient was diagnosed with hypothyroidism prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.11.2. Hypoparathyroidism

Indicate whether the patient was diagnosed with hypoparathyroidism prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.11.3. Diabetes mellitus

Indicate whether the patient was diagnosed with any type of diabetes mellitus prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.11.4. Osteoporosis

Indicate if the patient was diagnosed with osteoporosis prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.11.5. Gonadal dysfunction

Indicate if the patient was diagnosed with gonadal dysfunction prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

21.11.6. Growth impairment

Diagnose if a growth impairment was observed in the patient prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.
Bibliography


