

Status at HCT/CT/GT/IST

Day 0

Guide to the completion of the EBMT data collection form:
[Disease_status_HCT_CT_GT_IST_Day0_Core_Extended_v2.2](#)

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EBMT Registry

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Introduction

This form shall be completed after the respective treatment (HCT, CT, GT or IST) form as part of Day 0 regardless if the treatment took place or not.

Please make sure you have already checked the **Introduction to the EBMT Registry Completion Guidelines** document latest version available under *Manuals and Reference Documents* section on [EBMT website](#).

Disease Status at HCT/CT/GT/IST - Day 0

Date of HCT/CT/GT/IST

Report the date the HCT/CT/GT/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, the (planned) treatment date should be indicated as the event date for Disease status at HCT/CT/GT/IST.

Survival status at HCT/CT/GT/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/GT/IST**. If the patient died after apheresis but before infusion took place, select **Died after apheresis but before cell infusion**.

Date of death

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

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**. Please select one of 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
- **Gene therapy-related** - death caused by complications or infections after gene therapy
- **IST-related** - death caused by complications or infections after immunosuppressive treatment

If none of the suggested options fit, select **Other** and specify the cause of death in the textbox in English.

Select treatment related cause

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**

Infectious complication

In the case of an infectious complication, please specify the type of infection. In case of multiple infections with different pathogens. Select all the type of infection(s) that apply:

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

Was an autopsy performed?

Check **No**, if no autopsy has been performed. Check **Yes** if autopsy is performed. Check the box **Unknown** if it is unknown an autopsy was performed

Patient status (All Diagnoses)

Performance status at initiation of HCT/CT/GT/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.

Score	Performance Status (Karnofsky)
100	Normal, no complaints or evidence of disease
90	Able to perform normal activity; minor signs and symptoms of disease
80	Able to perform normal activity with effort; some signs and symptoms of disease
70	Cares for self, unable to perform normal activity or to do active work

60	Requires occasional assistance but is able to care for most of own needs
50	Requires considerable assistance and frequent medical care
40	Requires special care and assistance; disabled
30	Hospitalisation indicated, although death not imminent; severely disabled
20	Hospitalisation necessary; active supportive treatment required, very sick
10	Fatal processes progressing rapidly; moribund
0	Dead

Table 1. Karnofsky scale for adult patients.

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

Table 2. Lansky scale for paediatric patients.

Score	Performance Status (ECOG)
0	Fully active, able to carry on all pre-disease performance without restriction
1	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
2	Ambulatory and capable of all selfcare but unable to carry out any work activities; up and about more than 50% of waking hours

3	Capable of only limited selfcare; confined to bed or chair more than 50% of waking hours
4	Completely disabled; cannot carry on any selfcare; totally confined to bed or chair
5	Dead

Table 3. ECOG performance scale.

Patient weight at initiation of HCT/CT/GT/IST

Report the weight of the patient at the start of conditioning in kilograms.

Patient height at initiation of HCT/CT/GT/IST

Report the height of the patient at the start of conditioning in centimetres.

Patient age at initiation of HCT/CT/GT/IST

Report the age of the patient at the treatment date in years. This field will be automatically calculated by the EBMT Registry application.

Patient age at initiation of HCT/CT/GT/IST

Report the age of the pediatric patient at the treatment date in months, for children less or equal to 24 month olds.

Patient EBV status

Epstein-Barr virus (EBV) is a widespread human herpesvirus (HHV4), infecting the majority of children, that establishes lifelong latent infection in the host memory B cells. This virus accounts for post-transplantation lymphoproliferative disorder (PTLD), one of the most serious allogeneic hematopoietic cell transplantation complications.

Report the laboratory result of the EBV antibody testing of the patient as **Negative** or **Positive** (positive EBV VCA IgG or EBNA assay results, regardless of IgM). If the testing was not performed, select **Not evaluated**. If the results of the testing are not known, report **Unknown**.

Patient CMV status

Human cytomegalovirus (CMV) is a betaherpesvirus in the same family as human herpesvirus-6 and -7. Like the other herpesviruses, CMV remains in the human body after primary infection for life. In allogeneic HCT recipients, the most important risk factors for CMV disease are the serologic status of the

donor and recipient. Approximately 30% of seronegative recipients transplanted from a seropositive donor (D+/R-) develop a primary CMV infection.

Report the laboratory result of the CMV antibody testing of the patient as **Negative** or **Positive** (positive CMV IgG assay result, regardless of IgM). If the testing was not performed, select **Not evaluated**. If the results of the testing are not known, report **Unknown**.

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. Select if there were any clinically significant co-existing disease or organ impairment at time of patient assessment prior to the preparative regimen. If this information is not available, select **Unknown**.

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

The comorbidities are listed as 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. Answer **Not evaluated** if the comorbidities were not assessed. Select **Unknown** if this information is unavailable.

Comorbidity Index (Inborn Errors of Immunity only)

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**. The answers should represent the situation at the start of conditioning, unless otherwise stated in the definitions. Report **Not evaluated** if comorbidity was not assessed.

Patient admitted in ICU

Report if the patient was admitted in ICU within the 3 months before HCT/CT/GT. Select **Unknown** if this information is unavailable.

Was there any additional major clinical abnormality not listed above and present 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.

Are there any autoimmune diseases?

All autoimmune diseases listed on the autoimmune disease form must be considered. However, note that there may be additional diseases not listed on the form. If these additional indications should be reported, it should be based on the clinical judgement of the investigator at the centre.

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.

Date of autoimmune disease diagnosis

Report the date of autoimmune disease diagnosis. If this information cannot be obtained, mark **Unknown**.

Extended dataset

Pre-HCT/CT/GT serology/PCR

Were the serologies and/or PCR performed?

Indicate whether serologies and / or PCR was performed. If the information is not available select **Unknown**.

Were the following pathogens detected at the most recent test performed before HCT/CT/GT?

Indicate for each of these pathogens whether it was detected at the test performed most recently before HCT/CT/GT, or answer **Not evaluated** if a test for this pathogen was not performed. Report the date the test was performed, or indicate that the date is **Unknown**.

Were the following antibodies detected at the most recent test performed before HCT/CT/GT?

Indicate for each of these antibodies whether they were detected at the test performed most recently before HCT/CT/GT, or answer **Not evaluated** if a test for these antibodies was not performed. Report the date the test was performed, or indicate that the date is **Unknown**.

What was the result of Toxoplasma IgG antibody testing? (Only for HCT/GT, not for CT)

For the test performed at the indication diagnosis, indicate whether Toxoplasma IgG antibody testing had a **Positive** or **Negative** result, or answer **Not evaluated** if Toxoplasma IgG antibody testing was not done.

For the test performed most recently before HCT/GT, indicate whether Toxoplasma IgG antibody testing had a **Positive** or **Negative** result, or answer **Not evaluated** if Toxoplasma IgG antibody testing was not done. Report the date the test was performed, or indicate that the date is **Unknown**.

What was the result of Toxoplasma IgM antibody testing? (Only for HCT/GT, not for CT)

For the test performed at the indication diagnosis, indicate whether Toxoplasma IgM antibody testing had a **Positive** or **Negative** result, or answer **Not evaluated** if Toxoplasma IgM antibody testing was not done.

For the test performed most recently before HCT/GT, indicate whether Toxoplasma IgM antibody testing had a **Positive** or **Negative** result, or answer **Not evaluated** if Toxoplasma IgM antibody testing was not done. Report the date the test was performed, or indicate that the date is **Unknown**.

Surveillance

Was the patient screened for colonisation by any resistant bacteria before HCT/CT/GT?

Choose **Yes** when screening for any type of resistant bacteria took place within 3 months before HCT/CT/GT, regardless of the result of screening.

Did screening indicate colonisation by any resistant bacteria within 3 months before HCT/CT/GT?

Choose **Yes** when at least one of the screening tests indicated there was colonisation by any type of resistant bacteria within 3 months before HCT/CT/GT.

Extended spectrum beta-lactamase (ESBL)-producing Enterobacteriaceae

Report whether the patient was colonised or not by ESBL-producing Enterobacteriaceae, and if colonised, indicate whether the colonised site was **Rectal/fecal** or an **Other site**. If the testing was not performed, select **Not screened**.

Carbapenem-resistant Enterobacteriaceae

Report whether the patient was colonised or not by carbapenem-resistant Enterobacteriaceae, and if colonised, indicate whether the colonised site was **Rectal/fecal** or an **Other site**. If the testing was not performed, select **Not screened**.

Carbapenem-resistant *Pseudomonas aeruginosa*

Report whether the patient was colonised or not by carbapenem-resistant *Pseudomonas aeruginosa*, and if colonised, indicate whether the colonised site was **Rectal/fecal**, **Throat** or an **Other site**. If the testing was not performed, select **Not screened**.

Vancomycin-resistant *Enterococcus*

Report whether the patient was colonised or not by vancomycin-resistant *Enterococcus*, and if colonised, indicate whether the colonised site was **Rectal/fecal** or an **Other site**. If the testing was not performed, select **Not screened**.

Methicillin-resistant *Staphylococcus aureus*

Report whether the patient was colonised or not by methicillin-resistant *Staphylococcus aureus*, and if colonised, indicate whether the colonised site was Nasal or an **Other site**. If the testing was not performed, select **Not screened**.

Other resistant bacteria, specify

If you performed screening for any other resistant bacteria, choose the **Other** option and specify the type of bacteria in the textbox in English. Report whether the patient was colonised or not by this bacteria, and if colonised, indicate whether the colonised site was **Rectal/fecal** or an **Other site**.

SARS-CoV-2 related questions

Did the patient have a symptomatic SARS-CoV-2 infection (positive PCR- or antigen test) in the 3 months prior to the day of HCT/CT/GT/IST 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 should be answered as **No**.

Select **Not evaluated** if the patient has not been tested for SARS-CoV-2 in the 3 months before the treatment, and select **Unknown** if it is not known whether the patient has been tested for SARS-CoV-2 during this period, or what the results of the test were, or whether the patient was symptomatic.

Date

If answered **Yes** in the previous question, report the date the patient tested positive for SARS-CoV-2 or indicate that the date is **Unknown**.

Did the patient have an ongoing SARS-CoV-2 infection (positive PCR- or antigen test) at the initiation of HCT/CT/GT/IST (including potential 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.

Select **Not evaluated** if the patient has not been tested for SARS-CoV-2 within one week before starting conditioning, and select **Unknown** if it is not known whether the patient has been tested for SARS-CoV-2 during this period, or what the results were.

End of general section

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

Status at treatment

Acute leukaemias

Status

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

- Primary induction failure;
- Complete haematological remission (CR) (1st, 2nd, 3rd or higher);
- Relapse (1st, 2nd, 3rd or higher);
- Untreated/ Upfront; or
- Non blastic pancytopenia.

Disease status				
Primary induction failure	Complete remission (CR) (1st, 2nd, 3rd or higher)	Relapse (1st, 2nd, 3rd or higher)	Non blastic pancytopenia	Untreated/upfront
<ul style="list-style-type: none"> • Despite treatment, the patient has never achieved complete remission. 	<p>All of the following response criteria for at least four weeks:</p> <ul style="list-style-type: none"> • <5% leukemic blasts in the bone marrow • No blasts with Auer rods (applies to AML only) • No extramedullary disease (e.g., CNS, soft tissue disease) 	<ul style="list-style-type: none"> • Not in complete haematological remission after a period of complete remission. 	<ul style="list-style-type: none"> • <5% blasts in BM and pancytopenia 	<ul style="list-style-type: none"> • From the diagnosis of the acute Leukemia to the main treatment (HCT/CT) the patient didn't receive any induction therapy. Do not consider the treatment between a previous diagnosis and this acute leukemia.

Table 4. Acute leukaemias disease status.

Number of induction courses

For patients treated (HCT/CT) in Primary Induction Failure or in 1st Complete Remission please report the number of induction courses from diagnosis to 1st complete remission, or mark as **Unknown** if the number of induction courses is unknown.

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 the precise blast count is not available or when it is not possible to identify the results of the investigation.

If the precise blast count is not available, please select whether it is

If the precise blast count is not available, please indicate whether it is **below or equal to 5%, above 5%, Not evaluated, or Unknown**.

If patient was in complete remission

Date of first complete remission

For patients in complete remission, report the date of the first complete remission.

If patient was in relapse

Date of first relapse

For patients in relapse, report the date of the first relapse.

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**.

CD19 expression at the last relapse

Applicable for Cellular Therapy in patients diagnosed with B lymphoblastic leukaemia/lymphoma or Mixed phenotype and 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.

Involvement at time of treatment

Medullary

If the patient was not in remission, indicate whether there was medullary involvement at time of treatment, if there was **No** medullary involvement or if it is **Unknown**.

Extramedullary

If the patient was not in remission, indicate whether there was extramedullary involvement at time of treatment, if there was **No** extramedullary involvement or if it is **Unknown**.

Organs involved at time of treatment

Indicate per 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

Minimal residual disease (MRD) at initiation of treatment

If the patient is in haematologic 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.

Date MRD status evaluated

Report the date of MRD status evaluation.

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.

Method used

Indicate if the MRD assessment was performed through **PCR**, **Flow cytometry** or **NGS** (Next Generation Sequencing). If another method was used, choose the **Other** option and specify it in the textbox.

Chronic Myeloid Leukaemias (CML)

Status

Report the Chronic Myeloid Leukaemias (CML) status:

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

In order to define the answer, please use International Consensus Classification (ICC) (2) criteria as in the table below.

Disease status		
Chronic phase (CP)	Accelerated phase (AP)	Blast crisis (BC)
<ul style="list-style-type: none"> ● None of the features of accelerated phase or blast crisis 	<ul style="list-style-type: none"> ● Bone marrow or peripheral blood blasts 10%-19% ● Peripheral blood basophils $\geq 20\%$ ● Presence of additional clonal cytogenetic abnormality in Ph+ cells (ACA)^a 	<ul style="list-style-type: none"> ● Bone marrow or peripheral blood blasts $\geq 20\%$ ● Extramedullary blast proliferation (myeloid sarcoma) ● Presence of morphologically apparent lymphoblasts ($>5\%$) warrants consideration of lymphoblastic crisis

Table 5. ICC criteria for CML status.

^aSecond Ph, trisomy 8, isochromosome 17q, trisomy 19, complex karyotype, or abnormalities of 3q26.2.

Number

If the disease status was chronic phase (CP), accelerated phase (AP) or blast crisis (BC), select the number.

Number the different disease statuses chronologically. A patient can only be in the next chronic phase after he has experienced a blast crisis or accelerated phase.

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.

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.

Cytogenetic remission

If the patient was in Chronic phase (CP), report if cytogenetic 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.

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.

Molecular remission

If the patient was in Chronic phase (CP), report if molecular 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.

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.

Disease status (only CP)		
Haematological remission	Cytogenetic remission	Molecular remission
<p>Patient meeting all of the following:</p> <ul style="list-style-type: none"> • WBC $< 10 \times 10^9 /L$ • Haemoglobin $> 11.0 \text{ g/dL}$ • Platelet Count $< 450 \times 10^9 /L$ • Normal Differential (<1% precursor cells) • No palpable splenomegaly • No extramedullary disease 	<ul style="list-style-type: none"> • 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 	<ul style="list-style-type: none"> • 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.

Table 6. Definitions of haematological, cytogenetic and molecular remission for patients in chronic phase.

Extended dataset

Chronic Myeloid Leukaemia (CML)

Type of blast crisis

Complete these questions if you are completing the extended dataset for CML if the disease status is blast crisis.

The blast crisis can be **myeloid** or **lymphoid** or **other** (for instance erythroblastic, megakaryoblastic or mixed) depending on the morphology and the immunophenotype. The majority of CML blast crisis cases belong to the myeloid lineage, but up to one-third may transform to lymphoid blast crisis.

Haematological values

Report the values found from blood tests performed at time of HCT. The reported values should indicate the status **before** the preparative (condition) regimen was started.

Peripheral blood

Haemoglobin (g/dL)

Report the haemoglobin in grams per deciliter (g/dL). If the haemoglobin was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Platelets (10⁹/L)

Report the platelets in 10⁹ cells per litre (10⁹/L). If the platelets were not tested, select **not evaluated**. If the value is not known, select **unknown**.

White blood cells (10⁹/L)

Report the white blood cells in 10⁹ cells per litre (10⁹/L). If the white blood cells were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Absolute basophils (10⁹/L)

Report the basophils in 10⁹ cells per litre (10⁹/L). If the basophils were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% basophils

Report the basophils as a percentage. If the basophils were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% blasts

Report the blasts as a percentage. If the blasts were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Bone marrow

Report the findings of the bone marrow investigation at HCT.

% blasts

Report the blasts as a percentage. If the blasts were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Precise blast count not available

If the precise blast count is not available, please indicate whether it was **below or equal to 5%, above 5%, Not evaluated, or Unknown**.

Extramedullary blast proliferation

Extramedullary blast proliferation is defined as cytologically and/or histologically proven growth of blast cells, in tissue other than peripheral blood or bone marrow.

Please report if extramedullary blast proliferation was found or not. If it was not tested, select **not evaluated**. If it is not known whether it was tested or what the results were, select **unknown**.

Chronic Lymphocytic Leukaemias (CLL)

Status

Report the Chronic Lymphocytic Leukaemias (CLL) status:

- Complete Remission (CR);
- Partial Remission (PR);
- Stable Disease (no change, no response/loss of response);
- Relapse (untreated);
- Progressive disease (PD);
- Never treated;
- Unknown.

See table 7 for the response evaluation according to 2018 iwCLL criteria (3).

Group	Parameter	Complete Remission (CR)	Partial Remission (PR)	Stable Disease (SD)	Progressive Disease (PD)
A	Lymph nodes	None ≥ 1.5 cm	Decrease $\geq 50\%$ (from baseline)*	Change of -49% to $+49\%$	Increase $\geq 50\%$ from baseline or from response
	Liver and/or spleen size†	Spleen size < 13 cm; liver size normal	Decrease $\geq 50\%$ (from baseline)	Change of -49% to $+49\%$	Increase $\geq 50\%$ from baseline or from response
	Constitutional symptoms	None	Any	Any	Any

	Circulating lymphocyte count	Normal	Decrease $\geq 50\%$ from baseline	Change of -49% to $+49\%$	Increase $\geq 50\%$ over baseline
B	Platelet count	$\geq 100 \times 10^9/L$	$\geq 100 \times 10^9/L$ or increase $\geq 50\%$ over baseline	Change of -49 to $+49\%$	Decrease of $\geq 50\%$ from baseline secondary to CLL
	Haemoglobin	$\geq 11.0 \text{ g/dL}$ (untransfused and without erythropoietin)	$\geq 11 \text{ g/dL}$ or increase $\geq 50\%$ over baseline	Increase $< 11.0 \text{ g/dL}$ or $< 50\%$ over baseline, or decrease $< 2 \text{ g/dL}$	Decrease of $\geq 2 \text{ g/dL}$ from baseline secondary to CLL
	Marrow	Normocellular, no CLL cells, no B-lymphoid nodules	Presence of CLL cells, or of B-lymphoid nodules, or not done	No change in marrow infiltrate	Increase of CLL cells by $\geq 50\%$ on successive biopsies

Table 7. Response evaluation according to 2018 iwCLL criteria.

*Sum of the products of 6 or fewer lymph nodes (as evaluated by CT scans and physical examination in clinical trials or by physical examination in general practice).

†Spleen size is considered normal if $< 13 \text{ cm}$. There is not a firmly established international consensus of the size of a normal liver; therefore, liver size should be evaluated by imaging and manual palpation. For the EBMT Registry, clinical (palpation) evaluation only without CT-scan (or alternate imaging), according to routine practice, is accepted.

Disease status	
Complete Remission (CR)	<p>See table 7 for detailed criteria. All of the criteria have to be met. But:</p> <ul style="list-style-type: none"> • If a patient has all CR criteria but has persistent cytopenia, the patient can be considered as a CR as an adaptation of these guidelines. • 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)	<p>See table 7 for detailed criteria. At least 2 of the parameters of group A and 1 parameter of group B need to improve if previously abnormal; if only 1 parameter of both groups A and B is abnormal before therapy, only 1 needs to improve.</p> <p>Clinical (palpation) evaluation only without CT-scan (or alternate</p>

	imaging), according to routine practice, is accepted.
Stable Disease (no change, no response/loss of response)	See table 7 for detailed criteria. All of the criteria have to be met. Constitutional symptoms alone do not define PD.
Relapse (untreated)	Evidence of PD in a patient who has previously achieved the criteria of a CR or PR for 6 months or more after the last dose of CLL therapy.
Progressive disease (PD)	At least 1 of the criteria of group A or group B has to be met. Sequential imaging is not warranted in CLL outside clinical trials and is not required for the EBMT Registry.
Never treated	No treatment was given.

Table 8. Additional clarifications for Chronic lymphocytic leukaemias disease status classification.

If progressive disease, sensitivity to last chemotherapy regimen

If the disease status or best response was progression, indicate if the progression was **resistant** to the last chemotherapy regimen the patient received, or if it was **sensitive**. If this is not known, select **unknown**.

Minimal residual disease (MRD) at initiation of treatment

If the patient was in Complete remission (CR), 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.

If the patient is in 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.

Date MRD status evaluated

Report the date of MRD status evaluation.

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.

Method used

Indicate if the MRD assessment was performed through **PCR**, **Flow cytometry** or **NGS** (Next Generation Sequencing). If another method was used, choose the **Other** option and specify it in the textbox.

Prolymphocytic (PLL) and Other Chronic Leukaemias

Status

Report the Prolymphocytic Leukaemias (PLL) status:

- Complete Remission (CR);
- Partial Remission (PR);
- Stable Disease (no change, no response/loss of response);
- Relapse (untreated);
- Progressive disease (PD);
- Never treated;
- Unknown.

For T-cell prolymphocytic leukaemia (T-PLL) according to the T-PLL consensus criteria (4), the response evaluation should be classified as follows (for other Chronic Leukaemias the response should be reported according to local evaluation).

Group	Parameter	CR (all met)	PR (≥ 2 in A and ≥ 1 in B)	SD (all met)	PD (≥ 1 in A or B met)
A	Lymph nodes	long-axis diameters to <1.0 cm	Decrease $\geq 30\%$ in SLD	Change of -<30% to + $\leq 20\%$	Increase >20% in SLD
	Spleen†	Spleen size <13 cm	Decrease $\geq 50\%$ in vertical length beyond normal from baseline	Change of -49% to +49% beyond normal from baseline	Increase $\geq 50\%$ in vertical length beyond normal from baseline

	Constitutional symptoms	None	Any	Any	Any
	Circulating lymphocyte count	$<4 \times 10^9/L$	$\leq 30 \times 10^9/L$ and decrease $\geq 50\%$ from baseline	$>30 \times 10^9/L$ or change of -49% to $+49\%$	Increase $\geq 50\%$ from baseline
	Marrow	T-PLL cells $<5\%$ of mononuclear cells	Any	Any	Any
	Any other specific site involvement*	None	Any	Any	Any
B	Platelet count	$\geq 100 \times 10^9/L$	$\geq 100 \times 10^9/L$ or increase $\geq 50\%$ from baseline	Change of -49% to $+49\%$	Decrease of $\geq 50\%$ from baseline
	Haemoglobin	$\geq 11.0 \text{ g/dL}$ (untransfused)	$\geq 11 \text{ g/dL}$ or increase $\geq 50\%$ from baseline	$<11.0 \text{ g/dL}$ or $<50\%$ from baseline, or change $<2 \text{ g/dL}$	Decrease of $\geq 2 \text{ g/dL}$ from baseline
	Neutrophils	$\geq 1.5 \times 10^9/L$	$\geq 1.5 \times 10^9/L$ or increase $\geq 50\%$ from baseline	Change of -49% to $+49\%$	Decrease of $\geq 50\%$ from baseline

Table 9. T-PLL response evaluation according to the T-PLL consensus criteria.

SLD: sum of long-axis diameters of up to 3 target lesions

*Pleural or peritoneal effusion, skin infiltration, central nervous system involvement.

† For the EBMT Registry, clinical (palpation) evaluation only without CT-scan or alternate imaging, according to routine practice, is accepted.

Disease status: additional clarifications	
Complete Remission (CR)	<p>See table 9 for detailed criteria. All of the criteria have to be met, however a few exceptions are possible:</p> <ul style="list-style-type: none"> • If a patient has all CR criteria but has persistent cytopenia, the patient can be considered as being in CR as an adaptation of these guidelines. • If a patient has all criteria of CR but bone marrow evaluation has not been performed (even with persistent cytopenia), the patient can be considered as being in CR as an adaptation of these guidelines.

Partial Remission (PR)	See table 9 for detailed criteria. At least 2 of the parameters of group A and 1 parameter of group B need to improve if previously abnormal; if only 1 parameter of both groups A and B is abnormal before therapy, only 1 needs to improve. Clinical (palpation) evaluation only without CT-scan (or alternate imaging), according to routine practice, is accepted.
Stable Disease (no change, no response/loss of response)	See table 9 for detailed criteria. All of the criteria have to be met.
Relapse (untreated)	Evidence of PD in a patient who has previously achieved the criteria of a CR or PR for 6 months or more after the last dose of CLL therapy.
Progressive Disease (PD)	At least 1 of the criteria of group A or group B has to be met. Sequential imaging is not warranted in CLL outside clinical trials and is not required for the EBMT Registry. Constitutional symptoms alone do not define PD.

Table 10. Additional clarifications for T-PLL disease status classification.

If progressive disease, sensitivity to last chemotherapy regimen

If the patient has progressive disease (PD), report whether the patient was sensitive or resistant to the last regimen. If the sensitivity to the last chemotherapy is unknown, tick checkbox **Unknown**.

Lymphomas

Status

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

- **Chemorefractory relapse or progression, including primary refractory disease;**
- **Complete remission (CR);**
- **Partial response (PR) with or without prior CR;**
- **Stable disease (no change, no response/loss of response);**
- **Untreated relapse from previous CR/untreated progression from previous PR;**

- Unknown; or
- Not evaluated.

Disease status		
Chemorefractory relapse or progression, including primary refractory disease	Does not present any of the features of any type of remission after treatment.	
Complete remission (CR)	Complete absence of disease, no signs or symptoms of the original disease.	Confirmed (Only applicable if the Complete Remission was evaluated by CT-scan or MRI methods.)
		Unconfirmed Only applicable if the Complete Remission was evaluated by CT-scan or MRI methods.
Partial response (PR) with or without prior CR	Reduction in the disease of 50% or more	
Stable disease (no change, no response/loss of response)	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.	

Table 11. Lymphomas disease status.

Number of CR

Please indicate the number of Complete remissions (either confirmed or unconfirmed) achieved by the patient prior to this treatment. Count the current CR if the patient treated (HCT, CT) is in CR.

Complete remission confirmed

If the patient was in CR, indicate if the complete remission was confirmed or not, by selecting either **Confirmed** or **Unconfirmed (CRU)**. CRU is defined as a complete response with persistent scan abnormalities of unknown significance. Mark **Unknown** if this information is unavailable.

Number of PR

Please indicate the number of Partial remissions achieved by the patient prior to this treatment. Count the current CR if the patient treated (HCT, CT) is in PR.

Technique used for disease assessment

Select the technique that was used for the assessment of the disease status. Mark **Unknown** if this information cannot be obtained.

- **CT scan**
- **PET**
- **MRI**

PET technique is not valid for CR status confirmation.

Parameters for prognostic indices at HCT/CT/GT

Age at treatment

The patient's age at the time of treatment is calculated automatically.

LDH levels elevated

Indicate if serum lactate dehydrogenase (LDH) level is elevated at the start of preparatory regimen (answer **Yes**) as per the reference laboratory's ranges, not elevated (answer **No**) or it was **Not evaluated** by clicking the correspondent answer box .

Haemoglobin < 120g/L

Indicate if the haemoglobin (haemoglobin) level was lower than 120g/L at the start of preparatory regimen (answer **Yes**), otherwise answer **No**.. Check the box **Not evaluated** if the haemoglobin level was not assessed.

White Blood Cell count

Indicate the number of white blood cells $\times 10^9/L$ at the start of preparatory regimen or make a corresponding mark if it was **Not evaluated**.

Ann Arbor staging

For patients not in complete remission at main treatment, please indicate the Ann Arbor staging. 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.

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

Table 12. Ann Arbor stage definitions (5).

>1 extranodal site involved

For patients not in complete remission at main treatment, please 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 treatment (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.

>4 nodal sites involved

For patients not in complete remission at main treatment, please indicate if more than 4 nodal sites were involved at the time of treatment (answer **Yes**), otherwise answer **No**. Check the box **Not evaluated** if the index was not assessed.

CNS involvement

For patients not in complete remission at main treatment, please indicate whether the CNS was involved or not.

Final score

If the separate items to calculate the prognostic scores are not available and the patient is not in complete remission, complete the **final score**. This should be completed for LBCL, mantle cell lymphoma, follicular lymphoma or Waldenstrom macroglobulinaemia only.

- For LBCL, please complete the final score according to the **IPI score** is applicable for all “Large B-cell lymphomas (LBCL)” except for “Primary large B-cell lymphoma of immune-privileged sites”. IPI is also applicable for “Follicular Large B cell lymphoma (FLBL)” that is now treated as LBCL .
- For mantle cell lymphoma, please complete the final score according to the **MIPI score**.
- For follicular lymphoma, please complete the final score according to the **FLIPI score**; except for “Follicular Large B cell lymphoma (FLBL)” for which IPI is more appropriate.
- For Waldenstrom macroglobulinaemia, please complete the final score according to the **ISSWM score**.

For other lymphoma, final score is not applicable.

Myelodysplastic Neoplasms (MDS)

Classification at treatment (WHO 2022)

Please see the tables 13, 14 and 15 below for definitions of the MDS sub-classifications according to WHO 2022 (6).

Classification	Blasts	Cytogenetics	Mutations
MDS with low blasts and isolated 5q deletion (MDS-5q)	<5% BM and <2% PB	5q deletion alone, or with 1 other abnormality other than monosomy 7 or 7q deletion	
MDS with low blasts and SF3B1 mutation ^a (MDS-SF3B1)	<5% BM and <2% PB	Absence of 5q deletion, monosomy 7, or complex karyotype	SF3B1

MDS with biallelic TP53 inactivation (MDS-biTP53)	<20% BM and PB	Usually complex	Two or more TP53 mutations, or 1 mutation with evidence of TP53 copy number loss or cnLOH
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Table 13. MDS with defining genetic abnormalities (WHO 2022).

^a Detection of ≥15% ring sideroblasts may substitute for SF3B1 mutation

Classification	Blasts
MDS with low blasts (MDS-LB)	<5% BM and <2% PB
MDS, hypoplastic ^a (MDS-h)	
MDS with increased blasts (MDS-IB1)	5–9% BM or 2–4% PB
MDS with increased blasts (MDS-IB2)	10–9% BM or 5–19% PB or Auer rods
MDS with fibrosis (MDS-f)	5–19% BM; 2–19% PB

Table 14. MDS, morphologically defined (WHO 2022).

^a By definition, ≤25% bone marrow cellularity, age adjusted

Classification	Blasts
Childhood MDS ^a with low blasts	<5% BM; <2% PB
Childhood MDS ^a with increased blasts	5–19% BM; 2–19% PB

Table 15. Childhood MDS (WHO 2022).

^a A clonal haematopoietic stem cell neoplasm arising in children and adolescents (<18 years of age)

Status

Indicate the disease status at the time of HCT/CT/IST. Please find below the definitions for the

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

- **Complete remission (CR);**
- **Improvement but no CR;**
- **Primary refractory phase (no change);**
- **Relapse;**
- **Progression/Worsening; or**
- **Never treated (supportive care or treatment without chemotherapy)**

MDS Disease status	
Complete remission (CR) 1st, 2nd, 3rd or higher	For patients with MDS with increased blasts: 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.
Improvement but no CR	<p>1) Haematological response (in patients with cytopenia)</p> <ul style="list-style-type: none"> ● 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) <p>2) Marrow blast response (in patients with increased marrow blasts): A decrease of 50% in marrow blasts, but still >5% marrow blasts.</p>
Primary refractory phase (no change)	Treatment with the intent to achieve remission was given, but no remission was achieved.
Relapse 1st, 2nd, 3rd or higher	Loss of complete remission.
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).

Table 16. MDS disease status or best response.

Number

If the disease status was complete remission (CR) or relapse, please report the number (if it is **1st, 2nd, 3rd or higher** or if it is **Unknown**).

Each different status has their own sequential count.

For example, a patient received a non-graft treatment and is CR1 in response to this treatment, after that there is a (1st) relapse, another treatment, and response CR2.

The count doesn't reflect the different disease statuses (eg. in the example above it should not be CR1, 2nd Relapse, CR3), but within that status the sequential count (so CR1, 1st Relapse, CR2).

IPSS-R

The Revised International Prognostic Scoring System (IPSS-R) (7) consists of the following:

- Haemoglobin value
- Absolute Neutrophil Count (ANC)
- Platelet count
- Bone marrow blasts (%)
- Cytogenetic risk group

Please see tables 17, 18 and 19 how to calculate this score. There are online calculators available, one example is: <https://www.mds-foundation.org/ipss-r-calculator/>

Cytogenetic risk groups	Cytogenetic abnormalities
Very good	-Y, del(11q)
Good	Normal, del(5q), del(12p), del(20q), double including del(5q)
Intermediate	del(7q), +8, +19, i(17q), any other single or double independent clones
Poor	-7, inv(3)/t(3q)/del(3q), double including -7/del(7q), Complex: 3 abnormalities
Very poor	Complex: >3 abnormalities

Table 17. Cytogenetic risk groups.

Prognostic variable	0	0.5	1	1.5	2	3	4
Cytogenetics	Very Good		Good		Intermediate	Poor	Very Poor
BM Blast (%)	<=2		>2-<5%		5-10%	>10%	
Haemoglobin (g/dL)	=>10		8-<10	<8			
Platelets (10^9/L)	=>100	50-<100	<50				
ANC (10^9/L)	=>0.8	<0.8					

Table 18. IPSS-R points.

Risk category	Risk score
Very Low	<=1.5
Low	>1.5 – 3
Intermediate	>3 – 4.5
High	>4.5 – 6
Very High	>6

Table 19. IPSS-R risk categories.

IPSS-M

The Molecular International Prognostic Scoring System (IPSS-M) (8) combines genomic risk factors with haematological and cytogenetic risk factors and consists of the following:

- Haemoglobin value
- Platelet count
- Bone marrow blasts
- IPSS-R cytogenetic risk groups (see IPSS-R section above, table 18)
- Molecular information on 31 genes (see table 20)

There are online calculators available, one example is: <https://mds-risk-model.com/>

Prognostic genes	Additional genes
ASXL1	BCOR
CBL	BCORL1
DNMT3A	CEBPA
ETV6	ETNK1
EZH2	GATA2
FLT3	GNB1
IDH2	IDH1
KRAS	NF1
MLL PTD	PHF6
NPM1	PPM1D
NRAS	PRPF8
RUNX1	PTPN11
SF3B15q/SF3B1 α	SETBP1
SRSF2	STAG2
TP53multihit	WT1
U2AF1	

Table 20. Molecular information for IPSS-M.

Risk category	Risk score
Very Low	<=-1.5
Low	>-1.5 – -0.5
Moderate Low	>-0.5 – 0
Moderate High	>0 – 0.5
High	>0.5 – 1.5
Very High	>1.5

Table 21. IPSS-M risk categories.

Extended dataset

MYELODYSPLASTIC NEOPLASMS (MDS)

Haematological values

Report the values from the blood tests performed at time of HCT. The reported values should indicate the status **before** the preparative (condition) regimen was started.

*Peripheral blood**Haemoglobin (g/dL)*

Report the haemoglobin in grams per deciliter (g/dL). If the haemoglobin was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Platelets (10^9 /L)

Report the platelets in 10^9 cells per litre (10^9 /L). If the platelets were not tested, select **not evaluated**. If the value is not known, select **unknown**.

White blood cells (10^9 /L)

Report the white blood cells in 10^9 cells per litre (10^9 /L). If the white blood cells were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% blasts

Report the blasts as a percentage. If the blasts were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% monocytes

Report the monocytes as a percentage. If the monocytes were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% neutrophils

Report the neutrophils as a percentage. If the neutrophils were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Bone marrow

Report the findings of the bone marrow investigation at HCT.

% blasts

Report the blasts as a percentage. If the blasts were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Precise blast count not available

If the precise blast count is not available, please indicate whether it is **below or equal to 5%, above 5%, Not evaluated, or Unknown**.

Bone marrow investigation

Report the findings of the bone marrow investigation at HCT. The reported data should indicate the status **before** the preparative (condition) regimen was started.

Hypocellularity

Indicate if there was hypocellularity at time of HCT. Hypocellularity means there were fewer haematopoietic cells in the bone marrow than usual. If this was not checked, select **not evaluated**. If it is not known if there was hypocellularity, select **unknown**.

Fibrosis

Indicate if the bone marrow biopsy revealed any signs of fibrosis at time of HCT. If this was not investigated, select **not evaluated**. If it is not known if there was bone marrow fibrosis, select **unknown**.

Transfusions (within 4 months prior to HCT)

If the patient received red blood cell transfusions, indicate if the transfusion burden was either:

- Low transfusion burden (LTB) (3-7 RBCs in 16 wk in at least 2 transfusion episodes, maximum 3 in 8 wk);
- High transfusion burden (HTB) (≥ 8 RBCs in 16 wk, ≥ 4 in 8 wk);
- Unknown.

The definitions used in this section are can be found in the 2019 Blood article from Platzbecker et al (#).

MDS/MPN Overlap Syndromes

Classification (WHO 2022)

According to the WHO 2022 (6) classification there are five subclassifications of the MDS/MPN overlapping syndrome:

Chronic myelomonocytic leukaemia (CMMoL, CMML):

Prerequisite criteria

1. Persistent absolute ($\geq 0.5 \times 10^9/L$) and relative ($\geq 10\%$) peripheral blood monocytosis.
2. Blasts constitute $< 20\%$ of the cells in the peripheral blood and bone marrow.^a
3. Not meeting diagnostic criteria of chronic myeloid leukaemia or other myeloproliferative neoplasms.^b
4. Not meeting diagnostic criteria of myeloid/lymphoid neoplasms with tyrosine kinase fusions.^c

Supporting criteria

1. Dysplasia involving ≥ 1 myeloid lineages.^d
2. Acquired clonal cytogenetic or molecular abnormality.
3. Abnormal partitioning of peripheral blood monocyte subsets.^e

Requirements for diagnosis

- Pre-requisite criteria must be present in all cases.
- If monocytosis is $\geq 1 \times 10^9/L$: one or more supporting criteria must be met.
- If monocytosis is ≥ 0.5 and $< 1 \times 10^9/L$: supporting criteria 1 and 2 must be met.

^aBlasts and blast equivalents include myeloblasts, monoblasts and promonocytes.

^bMyeloproliferative neoplasms (MPN) can be associated with monocytosis at presentation or during the course of the disease; such cases can mimic CMML. In these instances, a documented history of MPN excludes CMML. The presence of MPN features in the bone marrow and/or high burden of MPN-associated mutations (JAK2, CALR or MPL) tends to support MPN with monocytosis rather than CMML.

^cCriteria for myeloid/lymphoid neoplasms with tyrosine kinase fusions should be specifically excluded in cases with eosinophilia.

^dMorphologic dysplasia should be present in $\geq 10\%$ of cells of a haematopoietic lineage in the bone marrow.

^eBased on detection of increased classical monocytes ($> 94\%$) in the absence of known active autoimmune diseases and/or systemic inflammatory syndromes.

MDS/MPN with SF3B1 mutation and thrombocytosis:

- Platelet count $\geq 450 \times 10^9/L$.
- 15% ring sideroblasts in the BM or $> 5\%$ with SF3B1 mutation.

Presence of megakaryocytic atypia resembling ET or MF.

MDS/MPN with neutrophilia (Atypical CML (t(9;22) negative and BCR::ABL1 negative):

- WBC count $> 13 \times 10^9 / \text{L}$ with increased and dysplastic neutrophils (immature myeloid cells $\geq 10\%$).
- No or minimal absolute basophils and monocytosis.
- Hypercellular BM with granulocytic proliferation and dysplasia.

MDS/MPN with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T):

- Platelet count $\geq 450 \times 10^9 / \text{L}$.
- 15% ring sideroblasts in the BM or $> 5\%$ with wild-type SF3B1.
- Presence of megakaryocytic atypia resembling ET or MF.

MDS/MPN-NOS (not otherwise specified):

Myeloid neoplasm with mixed MDS and MPN features, not meeting WHO criteria for other MDS/MPN overlap neoplasms, MDS or MPN.

CMMI subtype

The prototype and most common MDS/MPN is chronic myelomonocytic leukaemia (CMMI), which is characterised by sustained peripheral blood monocytosis and various combinations of somatic mutations involving epigenetic regulation, spliceosome, and signal transduction genes.

Two main phenotypic types of CMMI can be distinguished:

CMMI subtype	Subtyping criteria
Myelodysplastic (MD-CMMI)	WBC $< 13 \times 10^9 / \text{L}$
Myeloproliferative (MP-CMMI)	WBC $> 13 \times 10^9 / \text{L}$

Table 22. WHO 2022 CMMI subtypes.

Patients with myeloproliferative type tend to have bulkier splenomegaly and more often have extramedullary infiltrations. MP-CMMI 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-CMMI than in MD-CMMI.

CMMI subgroup

According to the WHO 2022, CMMI can be further subclassified according to the percentage of blasts in peripheral blood and in bone marrow into CMMI-1 and CMMI-2:

CMMML subgroup	Subgrouping criteria
CMMML-1	<5% blasts in the blood and <10% blasts in the bone marrow
CMMML-2	5-19% blasts in the blood and 10-19% blasts in the bone marrow

Table 23. WHO 2022 CMMML subgroups.

Status

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

- **Complete remission (CR);**
- **Improvement but no CR;**
- **Primary refractory phase (no change);**
- **Relapse;**
- **Progression/Worsening; or**
- **Never treated (supportive care or treatment without chemotherapy)** - No treatment was given (blood transfusions are not considered treatment in this context).

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

MDS/MPN Disease status	
Complete remission (CR) 1st, 2nd, 3rd or higher	Marrow blast count < 5% and a normalisation of peripheral blood counts was observed for at least 4 weeks.
Improvement but no CR	Bone marrow blasts decreased by $\geq 50\%$ 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 CMMML.

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

Table 24. MDS/MPN disease status or best response.

Number

If the disease status was complete remission (CR) or relapse, please report the number (if it is **1st, 2nd, 3rd or higher** or if it is **Unknown**).

Each different status has their own sequential count.

For example, a patient received a non-graft treatment and is CR1 in response to this treatment, after that there is a (1st) relapse, another treatment, and response CR2.

The count doesn't reflect the different disease statuses (eg. in the example above it should not be CR1, 2nd Relapse, CR3), but within that status the sequential count (so CR1, 1st Relapse, CR2).

CPSS (for CMML only)

The CMML-specific prognostic scoring system (CPSS) combines clinical and cytogenetic data. Patients can be categorised into 4 risk groups according to following points:

- CMML-2 according to WHO 2022 (1 point)
- WBC $\geq 13 \times 10^9 / \text{L}$ (1 point)
- RBC transfusion dependency (1 point)
- Cytogenetic risk group:
 - Low (normal and -Y) (0 points)
 - Intermediate (other abnormalities) (1 point)
 - High (trisomy 8, complex and abnormalities of chromosome 7) (2 points)

Risk category	Risk score
Low	0
Intermediate-1	1
Intermediate-2	2-3
High	4-5

Table 25. CMML specific prognostic system risk categories and scores.

CPSS-Mol (for CMML only)

The CMML-specific prognostic scoring system Molecular (CPSS-Mol) combines clinical, cytogenetic and molecular data. Patients can be categorised into 4 risk groups according to following points:

- WBC $\geq 13 \times 10^9 / \text{L}$ (1 point)
- Bone marrow blasts (%) $\geq 5\%$ (1 point)
- RBC transfusion dependency (1 point)
- Cytogenetic risk group:
 - Low (normal and -Y) (0 points)
 - Intermediate (other abnormalities) (1 point)
 - High (trisomy 8, complex and abnormalities of chromosome 7) (2 points)
- ASXL1 mutation (1 point)
- NRAS mutation (1 point)
- RUNX1 mutation (2 points)
- SETBP1 mutation (1 point)

Please see the table below for the risk groups. The score can be calculated with an online tool, such as:

https://qxmd.com/calculate/calculator_609/cmml-cpss-mol

Risk category	Risk score
Low	0
Intermediate-1	1
Intermediate-2	2-3
High	≥ 4

Table 26. CMML specific molecular prognostic system risk categories and scores.

Extended dataset

MDS/MPN overlap syndromes

Haematological values

Report the values from the blood tests performed at time of HCT. The reported values should indicate the status **before** the preparative (condition) regimen was started.

Peripheral blood

Haemoglobin (g/dL)

Report the haemoglobin in grams per deciliter (g/dL). If the haemoglobin was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Platelets

Report the platelets in 10^9 cells per litre ($10^9/L$). If the platelets were not tested, select **not evaluated**. If the value is not known, select **unknown**.

White blood cells

Report the white blood cells in 10^9 cells per litre ($10^9/L$). If the white blood cells were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% blasts

Report the blasts as a percentage. If the blasts were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% monocytes

Report the monocytes as a percentage. If the monocytes were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% neutrophils

Report the neutrophils as a percentage. If the neutrophils were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Bone marrow

Report the findings of the bone marrow investigation at HCT.

% blasts

Report the blasts as a percentage. If the blasts were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Precise blast count not available

If the precise blast count is not available, please indicate whether it is **below or equal to 5%**, **above 5%**, **Not evaluated**, or **Unknown**.

Auer rods present

Indicate if auer rods were present at the time of HCT. If auer rods presence was not tested, select **not evaluated**. If it is not known if auer rods were present, select **unknown**.

Bone marrow investigation

Report the findings of the bone marrow investigation at HCT. The reported data should indicate the status **before** the preparative (condition) regimen was started.

Fibrosis

Indicate if the bone marrow biopsy revealed any signs of fibrosis at time of HCT. If this was not checked, select **not evaluated**. If it is not known if there was bone marrow fibrosis, select **unknown**.

Transfusions (within 4 months prior to HCT)

If the patient received red blood cell transfusions, indicate if the transfusion burden was either:

- Low transfusion burden (LTB) (3-7 RBCs in 16 wk in at least 2 transfusion episodes, maximum 3 in 8 wk);
- High transfusion burden (HTB) (≥ 8 RBCs in 16 wk, ≥ 4 in 8 wk);
- Unknown.

The definitions used in this section are can be found in the 2019 Blood article from Platzbecker et al (#).

Myeloproliferative Neoplasms (MPN)

Classification at treatment (WHO 2022)

Select the subclassification that is appropriate for the MPN.

Please see table 27 below for definitions of the MPN sub classifications according to WHO 2022 (6).

Name	Diagnostic criteria
Primary myelofibrosis (overt PMF)	<i>Meeting all three major criteria and at least one minor criterion</i>

	<p>Major criteria:</p> <ol style="list-style-type: none"> 1. Megakaryocyte proliferation and atypia¹ and ≥ grade 2 reticulin/collagen fibrosis 2. Not meeting WHO criteria for other myeloid neoplasms 3. Presence of JAK2, CALR, <u>or</u> MPL mutation <u>or</u> presence of another clonal marker or absence of evidence for reactive bone marrow fibrosis <p>Minor criteria:</p> <ol style="list-style-type: none"> 1. Anaemia not otherwise attributed 2. Leukocytosis ≥ 11 × 10⁹/L 3. Palpable splenomegaly 4. Increased lactate dehydrogenase (LDH), above upper limit 5. Leukoerythroblastosis
Primary myelofibrosis (prePMF)	<p><i>Meeting all 3 major criteria, and at least 1 minor criterion</i></p> <p>Major criteria:</p> <ol style="list-style-type: none"> 1. Megakaryocytic proliferation and atypia, without reticulin fibrosis > grade 1 (MF-1), accompanied by increased age-adjusted BM cellularity, granulocytic proliferation, and often decreased erythropoiesis 2. Not meeting the WHO criteria for BCR::ABL1⁺ CML, PV, ET, myelodysplastic syndromes, or other myeloid neoplasms 3. Presence of JAK2, CALR, or MPL mutation or in the absence of these mutations, presence of another clonal marker,² or absence of minor reactive BM reticulin fibrosis³ <p>Minor criteria:</p> <p>Presence of at least 1 of the following, confirmed in 2 consecutive determinations:</p> <ol style="list-style-type: none"> a. Anaemia not attributed to a comorbid condition b. Leukocytosis ≥ 11 × 10⁹/L c. Palpable splenomegaly d. LDH increased to above the upper normal limit of institutional reference range

¹ Megakaryocytes with aberrant nuclear/cytoplasmic ratio and hyperchromatic and irregularly folded nuclei and dense clustering

² In the absence of any of the 3 major clonal mutations, the search for the most frequent accompanying mutations (eg, ASXL1, EZH2, TET2, IDH1/IDH2, SRSF2, SF3B1) are of help in determining the clonal nature of the disease.

³ Minor (grade 1) reticulin fibrosis secondary to infection, autoimmune disorder or other chronic inflammatory conditions, hairy cell leukaemia or other lymphoid neoplasm, metastatic malignancy, or toxic (chronic) myelopathies.

Secondary myelofibrosis (Transformed to myelofibrosis from PV/ET)	In some cases, MF develops from another type of blood cancer: essential thrombocythaemia (ET) or polycythaemia vera (PV). The general term for this is secondary MF, or post-ET myelofibrosis or post-PV myelofibrosis.
Polycythaemia vera (PV)	<p><i>Meeting all three major criteria or the first two major criteria and one minor criterion</i></p> <p>Major criteria:</p> <ol style="list-style-type: none"> 1. Haemoglobin (Hb) > 16.5 g/dL/16 g/dL (men/women) <u>and/or</u> Haematocrit (Hct) > 49%/48% (men/women) Bone marrow (BM) tri-lineage hyperplasia (panmyelosis) with pleomorphic mature megakaryocytes⁴ 2. Presence of JAK2 mutation (JAK2 p.V617F or JAK2 exon 12 mutations) <p>Minor criterion:</p> <ol style="list-style-type: none"> 1. Subnormal serum erythropoietin level
Essential or primary thrombocythaemia (ET)	<p><i>Meeting all four major criteria or first three major criteria and one minor criterion</i></p> <p>Major criteria:</p> <ol style="list-style-type: none"> 1. Platelet count $\geq 450 \times 10^9/L$ 2. BM megakaryocyte proliferation with large and mature morphology and hyper-lobulated nuclei, Reticulin fibrosis grade should be ≤ 1 3. Not meeting WHO criteria for other myeloid neoplasms 4. Presence of JAK2, CALR or MPL mutation <p>Minor criteria:</p> <ol style="list-style-type: none"> 1. Presence of a clonal marker or absence of evidence for reactive thrombocytosis
Juvenile myelomonocytic leukaemia (JMML)	<p>I. Clinical and hematologic features (all 4 features mandatory)</p> <ol style="list-style-type: none"> 1. Peripheral blood monocyte count $\geq 1 \times 10^9/L$ 2. Blast percentage in peripheral blood and bone marrow $< 20\%$ 3. Splenomegaly 4. Absence of BCR::ABL1 rearrangement <p>II. Genetic studies (1 finding sufficient)</p>

⁴ BM biopsy may not be required if Hb > 18.5 g/dL in men or 16.5 in women (Hct > 55.5 in men and 49.5 in women).

	<ul style="list-style-type: none"> • Somatic mutation in PTPN11 or KRAS or NRAS • Clinical diagnosis of neurofibromatosis type 1 (NF1) or NF1 mutation • Germ line CBL mutation and loss of heterozygosity of CBL <p>III. If none of the category II criteria are met, 2 of the following criteria must be fulfilled:</p> <ul style="list-style-type: none"> • Any clonal cytogenetic abnormality • Fetal haemoglobin increased for age • Circulating myeloid precursors • GM-CSF hypersensitivity • White blood cell count $>10 \times 10^9/L$
Hyper eosinophilic syndrome (HES)	<ol style="list-style-type: none"> 1. Peripheral blood hypereosinophilia – defined as $> 1.5 \text{ eosinophils} \times 10^9/L$ blood ($[>1500/\text{mcl}]$) on two examinations at an interval of 1 month or greater – and/or – <ul style="list-style-type: none"> • Tissue hypereosinophilia defined by the following: • Percentage of eosinophils in BM section exceeds 20% of all nucleated cells – and/or – • Pathologist is of the opinion that tissue infiltration by eosinophils is extensive – and/or – • Marked deposition of eosinophil granule proteins is found in the absence or presence of major tissue infiltration by eosinophils 2. Organ damage and/or dysfunction attributable to tissue hypereosinophilia 3. Exclusion of other disorders or conditions as a major reason for organ damage
Chronic eosinophilic leukaemia (CEL)	<ol style="list-style-type: none"> 1. Eosinophilia $\geq 1.5 \times 10^9/L$ 2. Absence of the Ph chromosome, BCR::ABL1 fusion gene, and exclusion of other myeloproliferative (polycythaemia vera, essential thrombocythosis, primary myelofibrosis) or myelodysplastic-myeloproliferative (chronic myelomonocytic leukaemia, atypical chronic myelogenous leukaemia) neoplasms. 3. Absence of t(5;12)(q31-35;p13) or other PDGFRB gene rearrangements 4. Absence of the FIP1L1-PDGFRB fusion gene or other PDGFRB gene rearrangements 5. Absence of FGFR1 gene rearrangements 6. Less than 20% blasts in peripheral blood and BM, absence of inv(16)(p13q22), t(16;16)(p13;q22), or other features that warrant the diagnosis of AML 7. Presence of a clonal or cytogenetic abnormality, $> 2\%$ blasts in peripheral blood, or $> 5\%$ blasts in BM
Chronic neutrophilic	<ol style="list-style-type: none"> 1. PB WBC $\geq 25 \times 10^9/L$:

leukaemia (CNL)	<p>Segmented neutrophils plus band forms $\geq 80\%$ of WBCs</p> <p>Neutrophil precursors (promyelocytes, myelocytes, and metamyelocytes) $< 10\%$ of WBC</p> <p>Myeloblasts rarely observed</p> <p>Monocyte count $< 1 \times 10^9/L$</p> <p>No dysgranulopoiesis</p> <p>2. Hypercellular BM:</p> <p>Neutrophil granulocytes increased in percentage and number</p> <p>Neutrophil maturation appears normal</p> <p>Myeloblasts $< 5\%$ of nucleated cells</p> <p>3. Not meeting WHO criteria for <i>BCR::ABL1</i>⁺ CML, PV, ET, or PMF</p> <p>4. No rearrangement of <i>PDGFRA</i>, <i>PDGFRB</i>, or <i>FGFR1</i>, or <i>PCM1-JAK2</i></p> <p>5. Presence of <i>CSF3R</i> T618I or other activating <i>CSF3R</i> mutation or In the absence of a <i>CSF3R</i> mutation, persistent neutrophilia (at least 3 months), splenomegaly, and no identifiable cause of reactive neutrophilia including the absence of a plasma cell neoplasm or, if present, demonstration of clonality of myeloid cells by cytogenetic or molecular studies</p>
Aggressive systemic mastocytosis	<p>SM diagnostic criteria plus "C" findings; no features of mast cell leukaemia</p> <p>Major criterion plus one minor criterion OR three minor criteria</p> <p>Major criterion:</p> <p>Multifocal, dense infiltrates of mast cells (≥ 15 mast cells in aggregates) detected in sections of bone marrow and/or other extracutaneous organ(s)</p> <p>Minor criteria:</p> <ul style="list-style-type: none"> • In biopsy sections of bone marrow or other extracutaneous organs, $> 25\%$ of the mast cells in the infiltrate are spindle-shaped or have atypical morphology, or of all mast cells in bone marrow aspirate smears, $> 25\%$ are immature or atypical • Detection of an activating point mutation at codon 816 of KIT in bone marrow, blood, or another extracutaneous organ • Mast cells in bone marrow, blood, or other extracutaneous organs express CD25, with or without CD2, in addition to normal mast cell markers • Serum total tryptase persistently exceeds 20 ng/mL (unless there is an associated clonal myeloid disorder, in which case this parameter is not valid) <p>"C" findings:</p> <ul style="list-style-type: none"> • Bone marrow dysfunction manifested by one or more cytopenia ($ANC < 1 \times 10^9/L$, $Hb < 10 \text{ g/dL}$, or platelets $< 100 \times 10^9/L$) but no obvious nonmast cell hematopoietic malignancy

	<ul style="list-style-type: none"> ● Palpable hepatomegaly with impairment of liver function, ascites, and/or portal hypertension ● Skeletal involvement with large osteolytic lesions and/or pathologic fractures ● Palpable splenomegaly with hypersplenism ● Malabsorption with weight loss due to gastrointestinal mast cell infiltrates
Systemic mastocytosis with an associated haematological neoplasm	<p>SM diagnostic criteria plus clonal haematologic disorder (eg, MDS, MPN, AML)</p> <p>Major criterion plus one minor criterion OR three minor criteria.</p> <p>Major criterion:</p> <p>Multifocal, dense infiltrates of mast cells (≥ 15 mast cells in aggregates) detected in sections of bone marrow and/or other extracutaneous organ(s).</p> <p>Minor criteria:</p> <ul style="list-style-type: none"> ● In biopsy sections of bone marrow or other extracutaneous organs, $> 25\%$ of the mast cells in the infiltrate are spindle-shaped or have atypical morphology, or of all mast cells in bone marrow aspirate smears, $> 25\%$ are immature or atypical ● Detection of an activating point mutation at codon 816 of KIT in bone marrow, blood, or another extracutaneous organ ● Mast cells in bone marrow, blood, or other extracutaneous organs express CD25, with or without CD2, in addition to normal mast cell markers ● Serum total tryptase persistently exceeds 20 ng/mL (unless there is an associated clonal myeloid disorder, in which case this parameter is not valid)
Mast cell leukaemia	Meets criteria for Systemic mastocytosis (SM). BM biopsy shows a diffuse infiltration, usually compact, by atypical, immature MCs. BM aspirate smears show 20% or more MCs.
Mast cell sarcoma	Local mast cell tumour with immature atypical mast cells and aggressive (invasive) growth pattern Cutaneous mastocytosis (CM) and SM criteria not fulfilled (CM and SM/Mast cell leukaemia excluded). High rate of recurrence/relapse. Resistance to therapy.
MLN-TK with FGFR1 rearrangement	Evidence of myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions (MLN-TK) defined by FGFR1 rearrangement
MLN-TK with PDGFRA rearrangement	Evidence of myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions (MLN-TK) defined by PDGFRA rearrangement
MLN-TK with PDGFRB	Evidence of myeloid/lymphoid neoplasms with eosinophilia and tyrosine

rearrangement	kinase gene fusions (MLN-TK) defined by PDGFRB rearrangement
MLN-TK with JAK2 rearrangement	Evidence of myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions (MLN-TK) defined by JAK2 rearrangement
MLN-TK with FLT3 rearrangement	Evidence of myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions (MLN-TK) defined by FLT3 rearrangement
MLN-TK with ETV6::ABL1 fusion	Evidence of myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions (MLN-TK) defined by ETV6::ABL1 fusion
Transformed to AML	Leukaemic transformation of MPN, also referred to as MPN blast-phase (MPN-BP). This is defined by the presence of $\geq 20\%$ circulating peripheral or bone marrow blasts.
MPN not otherwise specified (NOS)	Includes MPN -like neoplasms that cannot be clearly classified as one of the other subcategories of MPN

Table 27. WHO 2022 diagnostic criteria for MPN subclassification.

Extended dataset

Subclassification at HCT

Date of MF transformation

If myelofibrosis transformed from PV/ET, report the date of transformation. If the date is unavailable, select **unknown**.

Date of AML transformation

If myelofibrosis transformed to AML, report the date of transformation. If the date is unavailable, select **unknown**.

Status

Indicate the disease status at the time of HCT/CT/GT/IST. Disease status should be defined as follows:

- **Complete remission (CR);**
- **Improvement but no CR;**

- Primary refractory phase (no change);
- Relapse;
- Progression/Worsening; or
- Never treated (supportive care or treatment without chemotherapy).

Note: If transformed to Acute Leukaemia at HCT, report the status of the Acute Leukaemia in this MPN section.

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

MPN Disease status	
Complete remission (CR) 1st, 2nd, 3rd or higher	<p>The 4 following criteria must be true:</p> <ol style="list-style-type: none"> 1. Resolution of disease-related symptoms and signs including palpable hepatosplenomegaly 2. Haemoglobin (Hb) $\geq 10\text{g/dL}$, platelet $\geq 100 \times 10^9/\text{L}$ and neutrophils $\geq 1 \times 10^9/\text{L}$ 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	<p>Requires one criterion in absence of progression:</p> <ol style="list-style-type: none"> 1. Hb increase of 2g/dL or transfusion independence 2. Spleen reduction of 50% 3. 100% increase in platelet count and absolute platelet count of at least $50 \times 10^9/\text{L}$ 4. 100% increase in absolute neutrophil count (ANC) and an ANC of at least $0.5 \times 10^9/\text{L}$
Primary refractory phase (no change)	<p>Treatment with intent to achieve remission was given, but no remission was achieved.</p>
Relapse (1st, 2nd, 3rd or higher)	<p>Loss of complete remission.</p>
Progression/Worsening	<p>Requires one of the following:</p> <ol style="list-style-type: none"> 1. Progressive splenomegaly 2. Leukaemic transformation (increase of peripheral blood blast percentage of at least 20%)
Never treated (supportive care or treatment without chemotherapy)	<p>No treatment was given (blood transfusions are not considered a treatment in this context).</p>

Table 28. MPN disease status.

Number

If the disease status was complete remission (CR) or relapse, please report the number (if it is **1st, 2nd, 3rd or higher** or if it is **Unknown**).

Each different status has their own sequential count.

For example, a patient received a non-graft treatment and is CR1 in response to this treatment, after that there is a (1st) relapse, another treatment, and response CR2.

The count doesn't reflect the different disease statuses (eg. in the example above it should not be CR1, 2nd Relapse, CR3), but within that status the sequential count (so CR1, 1st Relapse, CR2).

Blast count (peripheral blood)

Indicate blast count in peripheral blood in percentage (%). Select **Not evaluated** if the blast count was not assessed. If the value is unavailable, check **Unknown**.

(Palpable) 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**.

Spleen span on 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**.

JAK inhibitor exposure between diagnosis and HCT/CT/GT/IST

JAK inhibitor therapy, when given before a HCT, may help in:

1. reducing splenomegaly;
2. decreasing symptoms due to proinflammatory cytokines;
3. improving performance status before HCT.

Indicate if the patient was treated with a JAK inhibitor after diagnosis and prior to the HCT/CT/GT/IST by checking either **Yes** or **No**. If it is not known whether the patient was treated with a JAK inhibitor or not, select **Unknown**.

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.

Response status

Answer this question only if you selected **Yes** in the JAK inhibitor exposure question. Specify the type of response achieved by the time of HCT/CT/GT/IST.

- **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 $\geq 50\%$. 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 $\geq 35\%$ spleen volume reduction.
- **Symptoms response** - $\geq 50\%$ reduction in the MPN-SAF TSS. The MPN-SAF TSS is assessed by the patients themselves and this includes fatigue, concentration, early satiety, inactivity, night sweats, itching, bone pain, abdominal discomfort, weight loss, and fevers. Scoring is from 0 (absent/as good as it can be) to 10 (worst imaginable/as bad as it can be) for each item. The MPN-SAF TSS is the summation of all the individual scores (0-100 scale).
- **Stable disease (no change, 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 evaluated or not known, please report accordingly.

DIPSS at HCT/CT/GT/IST (Myelofibrosis only)

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

Prognostic factors	Points		
	0	1	2
Age (years)	≤ 65	> 65	
WBC ($\times 10^9/L$)	≤ 25	> 25	
Haemoglobin (g/dL)	≥ 10		< 10
% Peripheral blood blasts	< 1	≥ 1	
Constitutional symptoms	No	Yes	

Table 29. DIPSS Prognostic Factors in MF.

DIPSS risk category	Total number of points	Median OS (years)
Low risk	0	Not reached
Intermediate-1:	1-2	14.2
Intermediate-2	3-4	4
High risk	5-6	1.5

Table 30. DIPSS risk assessment in MF.

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

MIPSS70 at HCT/CT/GT/IST (Myelofibrosis only)

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

- Haemoglobin (Hb) < 10 g/dL
- WBC > 25×10⁹/L
- Platelets < 100×10⁹/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

MIPSS70 risk category	Total number of points	Median OS (years)
Low risk	0-1	27.7
Intermediate	2-4	7.1
High risk	≥ 5	2.3

Table 31. MIPSS70 risk assessment in MF.

You can visit <http://www.mipss70score.it/> for the MIPSS70 calculation.

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

MYSEC-PM 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 $\geq 3\%$ (2 points)
- Constitutional symptoms (1 point)
- Lack of CALR mutation (2 points)

MYSEC-PM risk category	Total number of points	Median OS (years)
Low risk	<11	NA
Intermediate-1:	11 to <14	9.3
Intermediate-2	14 to <16	4.5
High risk	≥ 16	2

Table 32. MYSEC-PM risk assessment in secondary MF.

You can visit <http://www.mysec-pm.eu/> for the MYSEC-PM calculation.

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

Extended dataset

Myeloproliferative Neoplasms (MPN)

Haematological values

Report the values from the blood tests performed at time of HCT. The reported values should indicate the status **before** the preparative (condition) regimen was started.

Peripheral blood**Haemoglobin (g/dL)**

Report the haemoglobin in grams per deciliter (g/dL). If the haemoglobin was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Platelets (10^9 /L)

Report the platelets in 10^9 cells per litre (10^9 /L). If the platelets were not tested, select **not evaluated**. If the value is not known, select **unknown**.

White blood cells (10^9 /L)

Report the white blood cells in 10^9 cells per litre (10^9 /L). If the white blood cells were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% monocytes

Report the monocytes as a percentage. If the monocytes were not tested, select **not evaluated**. If the value is not known, select **unknown**.

% neutrophils

Report the neutrophils as a percentage. If the neutrophils were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Bone marrow

Report the findings of the bone marrow investigation at HCT.

% blasts

Report the blasts as a percentage. If the blasts were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Precise blast count not available

If the precise blast count is not available, please indicate whether it is **below or equal to 5%, above 5%, Not evaluated, or Unknown**.

Constitutional symptoms

Indicate if constitutional symptoms were present or not. The constitutional symptoms should be evaluated **before** the preparative (condition) regimen was started. Constitutional symptoms include for example pruritus (itching), night sweats, fever, weight loss, or fatigue.

Transfusions (within 4 months prior to HCT)

If the patient received red blood cell transfusions, indicate if the transfusion burden was either:

- Low transfusion burden (LTB) (3-7 RBCs in 16 wk in at least 2 transfusion episodes, maximum 3 in 8 wk);
- High transfusion burden (HTB) (≥ 8 RBCs in 16 wk, ≥ 4 in 8 wk);
- Unknown.

The definitions used in this section are can be found in the 2019 Blood article from Platzbecker et al (#).

Plasma cell neoplasms (PCN)

Status

Report the response status at HCT/CT/GT/IST.

- **Complete remission (CR);**
- **Stringent complete remission (sCR);**
- **Very good partial remission (VGPR);**
- **Partial remission (PR);**
- **Stable disease (no change, no response/loss of response);**
- **Progression;**
- **Relapse; or**
- **Never treated.**

If the disease status is not known or was not evaluated, select **unknown** or **not evaluated**, respectively.

Please find below a table with the criteria for each response category for plasma cell neoplasms and a separate table with criteria for Immunoglobulin-related (AL) Amyloidosis.

Disease status PCN	
Complete remission (CR) 1st, 2nd, 3rd or higher	<p>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.</p> <ul style="list-style-type: none"> • <5% of plasma cells in bone marrow aspirate • Disappearance of any soft tissue plasmacytomas.

	<ul style="list-style-type: none"> • No increase in size or number of lytic lesions if assessed (radiographic studies are not mandatory) <p>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.</p> <p>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.</p>
Stringent complete remission (sCR) 1st, 2nd, 3rd or higher	All of the following: <ul style="list-style-type: none"> • CR as defined above • normal free light (FLC) chain ratio • Absence of clonal cells in bone marrow by immunohistochemistry or immunofluorescence
Very good partial remission (VGPR) 1st, 2nd, 3rd or higher	One or more of the following: <ul style="list-style-type: none"> • Serum and urine M-protein detectable by immunofixation but not on electrophoresis • $\geq 90\%$ reduction in serum M-protein plus urine M-protein level $< 0.1\text{g}/\text{per 24h}$ <p>In addition, there must be no increase in size or number of lytic lesions if assessed (radiographic studies are not mandatory).</p> <p>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.</p>

Partial remission (PR) 1st, 2nd, 3rd or higher	<p>All of the following:</p> <ul style="list-style-type: none"> • >50% reduction in serum M-protein plus reduction in 24h urinary M-protein by >=90% or to <0.2g/ per 24h. • 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) <p>In the absence of measurable serum and urine M-protein, the following criteria applies:</p> <ul style="list-style-type: none"> • A decrease in the difference between involved and uninvolved free light chain (FLC) of more than 50% <p>If the FLC assay cannot be measured, the following criteria apply:</p> <ul style="list-style-type: none"> • >=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
Stable disease(no change, no response/loss of response)	Does not meet the criteria for CR, VGPR, PR or progressive disease (includes the old Minimal response (MR) criteria)
Progression	<p>One or more of the following:</p> <ul style="list-style-type: none"> • 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) • 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 <p>In the absence of measurable serum and urine M-protein, the following criteria applies:</p> <ul style="list-style-type: none"> • 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)

Relapse 1st, 2nd, 3rd or higher	<p>Clinical relapse requires one or more of the following criteria:</p> <ul style="list-style-type: none"> • Direct indicators of increasing disease and/or end organ dysfunction (CRAB features) related to the underlying clonal plasma-cell proliferative disorder. • Development of new soft tissue plasmacytomas or bone lesions • Increase of 50% (and at least 1 cm) in the size of existing plasmacytomas or bone lesions. • Hypercalcemia ($> 11.5 \text{ mg/dL}$) • Decrease in haemoglobin of $> 2 \text{ g/dL}$ • Rise in serum creatinine by 2 mg/dL or more
Never treated	No treatment was given.

Table 33. Plasma cell neoplasms disease status.

Disease status Immunoglobulin-related (AL) amyloidosis	
Complete remission (CR) 1st, 2nd, 3rd or higher	Normalisation of the free light chain (FLC) levels and ratio, negative serum and urine immunofixation
Stringent complete remission (sCR) 1st, 2nd, 3rd or higher	Not applicable
Very good partial remission (VGPR) 1st, 2nd, 3rd or higher	Reduction in the dFLC (difference between involved FLC and unininvolved FLC) to $<40 \text{ mg/L}$
Partial remission (PR) 1st, 2nd, 3rd or higher	A greater than 50% reduction in the dFLC
Stable disease(no change, no response/loss of response)	Less than a PR

Progression	<ul style="list-style-type: none"> • 50% increase in serum M protein to >0.5 g/dL or 50% increase in urine M protein to >200 mg/day (a visible peak must be present) • Free light chain increase of 50% to >100 mg/L
Relapse 1st, 2nd, 3rd or higher	<ul style="list-style-type: none"> • Any detectable monoclonal protein or abnormal free light chain ratio (light chain must double) • Free light chain increase of 50% to >100 mg/L
Never treated	No treatment was given.

Table 34: response for Immunoglobulin-related (AL) amyloidosis

Number

-

Please report the number (if it is **1st, 2nd, 3rd or higher** or if it is **Unknown**) for the following disease statuses:

- Complete remission (CR)
- Stringent complete remission (sCR)
- Very good partial remission (VGPR)
- Partial remission (PR)
- Relapse

Each different status has their own sequential count.

For example, a patient received a non-graft treatment and is in PR1 as response to this treatment. After that there is a progression, another treatment, and response to this treatment is CR1, relapse, another treatment, response CR2.

The count doesn't reflect the different disease statuses (eg. in the example above it should not be PR1, CR2, CR3), but within that status the sequential count (so PR1, CR1, CR2).

Minimal residual disease (MRD) at initiation of treatment

If the patient is in CR or sCR, 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.

Date MRD status evaluated

Report the date of MRD status evaluation.

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.

Method used

Indicate if the MRD assessment was performed through **PCR**, **Flow cytometry** or **NGS** (Next Generation Sequencing). If another method was used, choose the **Other** option and specify it in the textbox.

Extramedullary disease (EMD)

EMD is an aggressive form of PCM, characterised by the ability of a clone and/or subclone to thrive and grow independently of the bone marrow microenvironment.

Please indicate if extramedullary involvement was diagnosed or not, or mark as unknown by ticking the corresponding box.

If EMD was diagnosed, please fill out the corresponding sub questions and indicate the method of diagnosis, the location of EMD, the number of sites, and specify the organs involved.

Extended dataset

Clinical and laboratory data

Report the values found from clinical and laboratory tests performed at time of HCT. The reported values should indicate the status **before** the preparative (condition) regimen was started.

Haemoglobin (g/dL)

Report the haemoglobin in grams per deciliter (g/dL). If the haemoglobin was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Serum creatinine (μmol/L)

To monitor kidney function, serum creatinine should be reported. Creatinine is a substance produced by the muscle which is filtered, reabsorbed and secreted by the tubular kidney. Therefore, the serum creatinine level in a patient is a good indication of renal function. Mean serum creatinine values differ

between males and females (due to differences in muscle mass and, therefore, creatinine generation) as well as other factors.

Please provide the amount of creatinine present in the serum in micromol/litre ($\mu\text{mol/l}$). If the serum creatinine was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Serum calcium (mmol/L)

Serum calcium levels are normally tightly controlled within a narrow range, usually 2.2 to 2.6 mmol/L. In multiple myeloma patients, these levels are often abnormally high (hypercalcemia).

Please provide the amount of calcium present in the serum in millimol/litre (mmol/l). If the serum calcium was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Serum albumin (g/L)

Albumin is synthesised in the liver and serum albumin tests therefore reflect the condition of the liver. The normal range is 34 to 54 g/L.

Please provide the amount of albumin present in the serum in grams/litre (g/l). If the serum albumin was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Serum β 2 microglobulin (mg/L)

β 2 microglobulin is one of the most important independent prognostic factors in multiple myeloma.

Please provide the amount of β 2 microglobulin present in the serum in milligram/litre (mg/l). If the serum β 2 microglobulin was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Was the patient on dialysis at any time before HCT/CT?

Please indicate whether the patient was on dialysis or not. Select **Unknown** if this information is not available.

Start date

If **Yes**, enter the start date. Select **Unknown** if this information is not available.

Did dialysis stop?

If dialysis stopped before the treatment select **Yes**. Select **No** if the patient is still on dialysis. Select **Unknown** if this information is not available.

End date

If dialysis stopped before the treatment please provide the End date. Select **Unknown** if this information is not available.

Solid tumours

Status

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

- **Adjuvant;**
- **Complete remission (CR);**
- **First partial remission;**
- **Partial remission (PR);**
- **Progressive disease (PD);**
- **Relapse;**
- **Stable disease;**
- **Never treated;**
- **Unknown;**
- **Not evaluated**

Disease status		
Adjuvant	The patient has no residual disease after surgery and the HCT/CT is part of the consolidation treatment. Tumour markers in germ cell tumours have to be in the normal range. Metastatic patients can never be considered adjuvant.	
Complete remission (CR)	Disappearance of all target lesions and all non-target lesions and normalisation of tumour marker level.	Unconfirmed complete response with persistent scan abnormalities of unknown significance
		Confirmed CR with No abnormalities detected in scan

Disease status		
		Unknown if it is not known if the complete remission was confirmed, select unknown
First partial remission	The patient achieved a reduction in disease of > 30% or more for the first time ever, but did not achieve complete remission ^a	
Partial remission (PR)	The patient achieved partial remission not for the first time.	
Progressive disease (PD)	At least 20% increase in the sum of diameters of target lesions, in addition to the relative increase of 20%, the sum must also demonstrate an absolute increase of at least 5 mm. (Note: the appearance of one or more new lesions is also considered progression).	
Relapse	Reappearance of disease in patients whose last disease status was complete remission.	Sensitive: patient achieves a reduction of >30% in the disease burden after treatment for this relapse.
		Resistant: patient has not achieved a reduction of more than 30% in the disease burden after treatment for this relapse.
		Unknown: if it is not known if the relapse was resistant or sensitive, select unknown.
Stable disease (no change, no response/loss of response)	<u>Target Lesions:</u> Neither sufficient shrinkage to qualify for PR nor sufficient increase to qualify for PD, taking as reference the smallest sum length diameters since the treatment started. <u>Non-Target Lesions:</u> Persistence of one or more non-target lesion(s) or/and maintenance of tumour marker level above the normal limits	
Never treated (upfront)	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.	

Table 35. Solid tumours disease status.

a. As per RECIST 1.1 guidelines <https://pubmed.ncbi.nlm.nih.gov/19097774/>

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.

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 (9).

Autoimmune diseases: Status at Mobilisation

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

Status (Systemic sclerosis only)

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

SSc subset

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

- **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.

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**.

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**.

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**.

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**.

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.

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.

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.

Weight	SLEDAI	Descriptor	Definition
SCORE			
8	_____	Seizure	Recent onset, exclude metabolic, infectious or drug causes.
8	_____	Psychosis	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 uremia and drug causes.
8	_____	Organic brain syndrome	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.
8	_____	Visual disturbance	Retinal changes of SLE. Include cytoid bodies, retinal hemorrhages, serous exudate or hemorrhages in the choroid, or optic neuritis. Exclude hypertension, infection, or drug causes.
8	_____	Cranial nerve disorder	New onset of sensory or motor neuropathy involving cranial nerves.
8	_____	Lupus headache	Severe, persistent headache; may be migraineous, but must be nonresponsive to narcotic analgesia.
8	_____	CVA	New onset of cerebrovascular accident(s). Exclude arteriosclerosis.
8	_____	Vasculitis	Ulceration, gangrene, tender finger nodules, perungual infarction, splinter hemorrhages, or biopsy or angiogram proof of vasculitis.
4	_____	Arthritis	≥ 2 joints with pain and signs of inflammation (i.e., tenderness, swelling or effusion).
4	_____	Myositis	Proximal muscle aching/weakness, associated with elevated creatine phosphokinase/aldolase or electromyogram changes or a biopsy showing myositis.
4	_____	Urinary casts	Heme-granular or red blood cell casts.
4	_____	Hematuria	>5 red blood cells/high power field. Exclude stone, infection or other cause.
4	_____	Proteinuria	>0.5 gram/24 hours
4	_____	Pyuria	>5 white blood cells/high power field. Exclude infection.
2	_____	Rash	Inflammatory type rash.
2	_____	Alopecia	Abnormal, patchy or diffuse loss of hair.
2	_____	Mucosal ulcers	Oral or nasal ulcerations.
2	_____	Pleurisy	Pleuritic chest pain with pleural rub or effusion, or pleural thickening.
2	_____	Pericarditis	Pericardial pain with at least 1 of the following: rub, effusion, or electrocardiogram or echocardiogram confirmation.
2	_____	Low complement	Decrease in CH50, C3, or C4 below the lower limit of normal for testing laboratory.
2	_____	Increased DNA binding	Increased DNA binding by Farr assay above normal range for testing laboratory.
1	_____	Fever	>38°C. Exclude infectious cause.
1	_____	Thrombocytopenia	<100,000 platelets / x10 ⁹ /L, exclude drug causes.
1	_____	Leukopenia	< 3,000 white blood cells / x10 ⁹ /L, exclude drug causes.
TOTAL			
SLEDAI			
SCORE _____			

Figure 1. SLEDAI-2K score (10).

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

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.

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.

Assessments at time of mobilisation

Report the status of MS within 3 months before mobilisation.

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 (11).

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

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.

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) (12).

Item (daily sum per week)	Weighting factor
Number of liquid or very soft stools	2
Abdominal pain score in one week (rating, 0-3)	5
General well-being (rating, 1-4)	7
Sum of physical findings per week: - Arthritis/arthralgia - Mucocutaneous lesions (e.g. erythema nodosum, aphthous ulcers)	20
Iritis/uveitis - Anal disease (fissure, fistula, etc) - External fistula (enterocutaneous, vesicle, vaginal, etc) - Fever over 37.8°C	
Antidiarrheal use (e.g. diphenoxylate)	30
Abdominal mass (no = 0, equivocal = 2, yes = 5)	10
47 minus haematocrit (males) or 42 minus haematocrit (females)	6
1-x (1-body weight divided by a standard weight)	1

Table 36. CDAI.

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

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**.

Haemoglobinopathies (Thalassemia and Sickle Cell Disease only)

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

Ferritin level

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

Total number of red blood cell transfusions

Report the number of red blood cell transfusions the patient was receiving before the main treatment since the diagnosis. If a patient had multiple main treatments, report the number of units transfused since previous main treatment and not since diagnosis. Choose one of the answer options:

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

Liver study?

Indicate whether the liver status was assessed. If **Yes**, please indicate if a liver biopsy was performed.

Liver biopsy performed?

If **Yes**, please indicate the Ishak staging.

Ishak staging

If liver biopsy was performed, indicate the Ishak staging. The Ishak staging is a scoring system that assesses the status of liver fibrosis. Further information on the Ishak staging can be found in table 36 (13).

Stage	Definition
F0	No fibrosis
F1	Fibrous expansion of some portal areas, with or without short fibrous septa
F2	Fibrous expansion of most portal areas, with or without short fibrous septa
F3	Fibrous expansion of most portal areas with occasional portal to portal (P-P) bridging
F4	Fibrous expansion of portal areas with marked bridging (portal to portal (P-P) as well as portal to central (P-C))

F5	Marked bridging (P-P and/or P-C) with occasional nodules (incomplete cirrhosis)
F6	Cirrhosis, probable or definite

Table 37. Ishak staging for liver fibrosis (13).

Chronic hepatitis?

Indicate whether the patient has chronic hepatitis (**Yes/No**).

Liver iron concentration assessed?

Report whether the liver iron concentration was assessed and if **Yes**, indicate the concentration in mg/g of dry weight

MRI (fibroscan) performed?

If **Yes**, report whether liver fibrosis was: Absent, Moderate or Severe (bridging cirrhosis).

Liver fibrosis

If a liver was analysed by MRI, select **absent** if there was no fibrosis, **moderate** if there was moderate fibrosis and **severe** if the fibrosis was near cirrhosis.

Liver iron concentration assessed?

Report whether the liver iron concentration was assessed and if **Yes**, indicate the concentration in mg/g of dry weight.

Was chelation performed regularly?

Start date of chelation therapy

If **Yes**, report the start date of chelation therapy.

Estimate the completeness of the chelation therapy administered

If **No**, estimate (in %) to what extent you can assess the completeness and regularity of the chelation therapy administered.

*Extended dataset***Iron chelators:**

For each iron chelator, **Deferoxamine, Deferiprone, Deferasirox**, please indicate if they were used at any time before transplant or gene therapy by ticking **Yes** or **No**. If you don't know, please select **Unknown**.

Cardiac evaluation - Cardiac Study

Report if a cardiac evaluation took place by answering **Yes**, otherwise answer **No**.

Cardiac echography: ejection fraction

Report if a cardiac echography (echocardiogram) was performed. If yes, answer **Yes** and add the left ventricle **ejection fraction (%)**, otherwise answer **No**.

Cardiovascular magnetic resonance (CMR) T2

If cardiovascular magnetic resonance imaging (also known as cardiovascular MRI) was performed, select **Yes** and report the cardiovascular magnetic resonance (CMR) T2 in milliseconds (ms). If it was not performed, select **no**.

Chronic transfusion program

For the patients diagnosed with sickle cell disease, report if they were on a chronic red blood cell (RBC) transfusion program. (**Yes/ No**)

Did the patient receive hydroxyurea?

If **Yes**, please specify if the patient received hydroxyurea. If **Yes**, please specify the duration of hydroxyurea therapy in months.

Endocrinopathies pre-existing to HCT/CT/GT

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

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

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**.

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

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

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

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.

Cerebrovascular disease

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**.

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

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

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

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

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

Renal involvement

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

Glomerular filtration rate: Report the glomerular filtration rate in mL/min/1.73m² measured prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

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

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**. If the answer is Yes, indicate also if it was: **Hyperhaemolysis or Autoimmune haemolytic anaemia**.

Other SCD related complications

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

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

Priapism: Indicate whether priapism was observed in the patient prior to HCT/CT/IST. If not evaluated, select **Not evaluated**.

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

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**.

Extended dataset

Liver status

Hepatomegaly

Please indicate whether hepatomegaly was **present**, **absent** or **unknown** at time of transplant/gene therapy

Costal arch

For patients with hepatomegaly at time of transplant/gene therapy, please Indicate the size of the liver in centimetres, measured below the costal margin as assessed by physical exam.

Spleen status

Splenomegaly

Please indicate whether splenomegaly was **present**, **absent** or **unknown** at time of transplant/gene therapy

Costal arch

For patients with splenomegaly at time of transplant/gene therapy, please indicate the size of the spleen in centimetres, measured below the costal margin as assessed by physical exam.

Ultrasound done

Please indicate whether the size of the Spleen has been assessed by ultrasound or CT scan or not, If you don't know, please select Unknown.

If **Yes**, please indicate the maximum diameter of the spleen in centimetres, as assessed by ultrasound or CT scan.

Splenectomy

Indicate if the patient underwent a splenectomy before HCT. If this is not known, select **unknown**.

Date of Splenectomy

If a splenectomy was performed, also enter the date.

Complimentary treatment and complications

For each other clinical features and complication:

Substitutional hormonal therapy

Red blood cell immunization

Central nervous system haemorrhage

Osteonecrosis of multiple joints

Sickle cell nephropathy

Bilateral proliferative retinopathy and/or visual impairment

Impaired neuropsychological function and abnormal MRI scan

Please indicate if they are Absent or present at time of transplant/gene therapy. If you don't know, please select Unknown.

Inborn Errors

Immune profiling

Test date

Enter the test date of the immune profile of the patient (within 3 months prior to HCT/CT/GT).

Cell type and test results

Report the values for tested cells. If the cell type was not checked, mark as **Not evaluated**.

Immunomodulatory treatments

Report treatments administered in the 3 months before this HCT/CT. If other treatments that are not included in the list were administered, please specify.

Bone marrow failures

Serology

Ferritin level

Indicate the patient's ferritin level (in **ng/ml**) before the start of this IST episode. If the ferritin level was not assessed, report **Not evaluated**. Select **Unknown** in case it is not known if the ferritin level was measured or not.

Extended dataset

Haematological tests - Bone Marrow Failure only

The questions should only be completed for aplastic anaemia patients.

Date tests performed

Report the date these tests were completed. The reported data should represent the values of these items at the treatment (HCT or IST) you are reporting. For HCT these values should be evaluated just before starting the preparative conditioning regimen.

Haemoglobin (g/dL)

Report the haemoglobin in grams per deciliter (g/dL). If the haemoglobin was not tested, select **not evaluated**. If the value is not known, select **unknown**.

Was haemoglobin transfused within 4 weeks before assessment?

Indicate if the patient received haemoglobin transfusions in the 4 weeks before this value was measured. If this information is unavailable, select **unknown**.

Platelets (10^9 cells/L)

Report the platelets in 10^9 cells per litre ($10^9/L$). If the platelets were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Were platelets transfused within 7 days before assessment?

Indicate if the patient received platelet transfusions in the 7 days before this value was measured.

If this information is unavailable, select **unknown**.

Neutrophils (10^9 cells/L)

Report the neutrophils in 10^9 cells per litre ($10^9/L$). If the neutrophils were not tested, select **not evaluated**. If the value is not known, select **unknown**.

Reticulocytes (10^9 cells/L)

Report the reticulocytes in 10^9 cells per litre ($10^9/L$). If the reticulocytes were not tested, select **not evaluated**. If the value is not known, select **unknown**.

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