

# Haematopoietic cell transplantation (HCT) day 100 follow-up

**Guide to the completion of the EBMT data  
collection form: HCT\_FU\_D100\_v2.3**

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EBMT Registry

EBMT Clinical Research & Registry Department



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## Introduction

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](#).

## HCT Day 100 Follow-Up

The follow-up of HCT patients should be recorded using the HCT Day 100 form. The data on this assessment should reflect the patient's status on the day the patient was last seen, closest to 100 days post-HCT. If the patient died within 100 days, the data from the last date the patient was seen alive can be used.

### Date of follow-up

Report the date that the HCT Day 100 follow-up occurred. If the patient died, enter the date of death. If the patient was lost to follow-up, enter the date the patient was last seen alive.

### Survival status

Indicate if the patient is last known to be **Alive** or **Dead** on the date of follow-up previously noted. If the patient is lost to follow-up, tick the box for **Lost to follow-up**.

### 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**

Please note that the category “rejection/poor graft function or failure” as contributory cause of death (previously in MedAB (auto, allo and disease-specific forms)) does not exist since the cause of death following a graft failure is generally an infection.

## Autopsy performed

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

## Best Response

The disease specific options for the best response can be found in [Appendix 1 - Disease specific best response and disease status](#). This section is not applicable for patients receiving HCT for Inborn errors indication diagnosis.

## Best clinical/biological response after HCT

Report the patient’s best response achieved after HCT but before any subsequent treatment, even if the patient got worse again afterwards. If the best response after the HCT has not been evaluated, select **Not evaluated**. If the best response after the HCT is unknown, select **Unknown**.

The best response is often achieved in the first 100 days. However, for some diseases the best response to HCT may take longer and shall be reported in the first annual follow-up form (e.g. PCM). For all indication diagnoses except for inherited disorders, report the best response achieved as per the date of follow-up. In case HCT was performed for inherited disorders, this section can be left blank.

If the patient had a relapse/progression post-HCT and received therapy for the disease relapse/progression, the response to that additional therapy should not be reported in this section. The best response prior to the relapse/progression should be reported here. The response should be captured before the start of unplanned treatment of underlying disease.

## Date best response first observed

Report the date the best response was first observed. The response date is the date that the sample or image was taken for assessing the response. If the patient's best response was already achieved prior to HCT (eg. HCT in CR and best response CR) the first evaluation date after the HCT should be reported. If the date is unknown, select **Unknown**.

## Recovery

### Absolute neutrophil count (ANC) recovery (neutrophils $\geq 0.5 \times 10^9/L$ )

Absolute neutrophil count (ANC) recovery is considered to take place when the number of neutrophils in the patient's peripheral blood rises to at least  $0.5 \times 10^9/L$ . Please note this is regardless of the use of growth factors and neutrophils level should be confirmed by three consecutive laboratory values obtained on different days.

Answer **No** (and proceed to [Date of the last assessment](#)) if:

- The stem cell source is either PB or BM and the ANC  $< 0.5 \times 10^9$  cells/L by Day +28. (both in an allograft and in an autograft setting).
- The stem cell source is CB and the ANC  $< 0.5 \times 10^9$  cells/L by Day +42 (both in an allograft and in an autograft setting).

Answer **Yes** if the absolute count of neutrophils post-HCT is higher or equal to  $0.5 \times 10^9$  cells/L for 3 laboratory values (and proceed to [Date of ANC recovery](#)).

If the absolute count of the patient's neutrophils was never below  $0.5 \times 10^9$  cells/L, the answer **Never below** must be chosen instead of answer **Yes**. This may happen in non-myeloablative transplants.

Mark the ANC as **Unknown** if it was not assessed post-HCT.

### Date of the last assessment

Indicate the date of the last assessment of the patient's neutrophils level.

### Date of ANC recovery

The date to be entered is the first date out of the 3 consecutive neutrophil counts above  $0.5 \times 10^9$  cells/L were recorded on different days. This date must be at least 7 days after the last transfusion containing neutrophils.

## Platelet reconstitution (platelets $\geq 20 \times 10^9$ cells/L)

Indicate whether or not there was platelet reconstitution achieved that is confirmed by 3 consecutive blood tests where absolute count of platelets is  $\geq 20 \times 10^9$  cells/L. All dates should reflect no transfusions in the previous 7 days.

Answer **No** (and proceed to [Date of the last assessment](#)) if the platelet count was  $< 20 \times 10^9$  cells/L or if platelet transfusions were administered in the previous 7 days.

Answer **Yes** (and proceed to [Date of platelet reconstitution](#)) if the platelet count  $\geq 20 \times 10^9$  cells/L was achieved and sustained for 3 consecutive laboratory values, obtained on different days without platelet transfusions administered in the previous 7 days.

Answer **Never below**, if the recipient's platelets never dropped below  $20 \times 10^9$  cells/L at any time post-HCT and a platelet transfusion was never required. If the recipient's platelet count drops below  $20 \times 10^9$  cells/L and/or the recipient received a platelet transfusion even once, do not use this option. This option is only applicable in the 100 day follow-up reporting period.

Answer **Unknown** if recipient's platelets were not assessed post-HCT.

### Date of the last assessment

Indicate the date of the last assessment of the patient's platelets level, or if not known mark the date as **Unknown**.

### Date of platelet reconstitution

The date to be entered is the first date out of the 3 consecutive platelets counts  $\geq 20 \times 10^9$  cells/L checked on different days and after 7 days without platelet transfusion. Mark as **Date unknown** if it is confirmed by medical record that patient achieved platelet reconstitution but the exact date of the first test with platelets counts  $\geq 20 \times 10^9$  cells/L is not known.

### Date of the last platelet transfusion

Indicate the date when the patient received the latest platelet infusion within the 100 day follow-up period.

## Graft function

### Poor graft function

This question is only applicable if the follow-up is being reported for an allogeneic HCT.

Poor graft function is defined as frequent dependence on blood and/or platelet transfusions and/or growth factor support in the absence of other explanations, such as disease relapse, drugs, or infection. If poor graft function was observed - select **Yes**. If poor graft function was not observed, select **No**.

The definition for poor graft function assumes that donor chimaerism is within a desirable target level.

In contrast with graft failure, poor graft function requires some degree of allogeneic graft evidence. However, the “desirable” target level depends on the time point when it’s measured, the indication for HCT (malignant versus non-malignant), intensity of the conditioning regimen, etc.

As an example, a patient with poor haematologic function but confirmation of donor source (proof of at least mixed/partial donor chimaerism), and in the absence of other explanations (disease relapse, drug toxicity, infections, GvHD, etc) represents a case of poor graft function. However, due to the dynamic nature of chimaerism, it is advisable to reassess donor chimaerism levels to confirm the results.

Note: please don’t report graft failure in this section.

- If primary graft failure occurred, please report it in the Recovery section in [Absolute neutrophil count \(ANC\) recovery \(neutrophils  \$\geq 0.5 \times 10^9/L\$ \)](#).
- If secondary graft failure occurred, please report this through the Non-infectious complications section.

### Date of poor graft function

Report the date when the patient started requiring frequent growth factor and/or packed RBC/platelets transfusions (at least weekly transfusions and/or growth factor support for at least 4 weeks) once other causes that could explain the poor graft function such as disease relapse, drugs, or infections have been ruled out.

## Chimaerism

This section is only applicable for patients receiving an allogeneic HCT.

Complete this section for every chimaerism test performed within 100 days since HCT until complete donor chimaerism has been achieved (>95%). If the patient has mixed chimaerism (5%-95% for either one or both myeloid and lymphoid lineages), please, complete the section if the chimaerism results change at least 10% from the previous test.

### Chimaerism test date

Report the chimaerism test date.

### Source of cells tested

Indicate if **Peripheral blood** and/or **Bone marrow** was used as source of cells for the chimaerism test.

## Cell types and test results

Select each cell type that was tested and indicate the percentage of donor cells. In case any other cell types were tested, please select Other and indicate the type of cells in the textbox in English.

## Preventive Therapies

This section is only applicable for patients that received an allogeneic HCT.

### Immunosuppression during this follow-up period?

This question is asked to know if the GvHD prevention initiated at transplant is still ongoing at this follow up.

Select **No** if the patient was not receiving preventive (immunosuppressive) therapy for GvHD post-transplant. Select **Yes** if the patient was receiving preventive therapy post-transplant. Report as **Unknown** if it is unknown if the patient was still receiving immunosuppressive GvHD preventive treatment.

### Immunosuppression stopped?

If immunosuppression for GvHD prevention was stopped, please report **Yes** and provide the end date of the GvHD prevention. If the therapy is ongoing, select **No**. If it is not known if the patient is still on immunosuppressive treatment, select **Unknown**.

### End date

Report the date the preventive treatment for GvHD was stopped. If the patient experiences a GvHD while receiving GvHD prevention, please report the date of onset of GvHD as the end date of GvHD prevention (as this becomes GvHD treatment rather than prevention). If the stop date was not known, select **Unknown**.

### Letermovir used as CMV prophylaxis during this follow-up period?

Indicate whether the patient received letermovir as CMV prophylaxis during the follow-up period. Select **Unknown** if this information is unavailable.

### Start date

If **Yes**, provide the start date of the letermovir regimen. Select **Unknown** if the start date is not known.

### Letermovir treatment stopped

Indicate whether the letermovir regimen has stopped.

### End date

If **Yes**, give the end date of the letermovir treatment. Select **Unknown** if the end date is not known.

#### Extended dataset

### Antimicrobial prophylaxis

#### Did the patient receive prophylaxis for bacterial, viral or fungal infection?

Indicate if the patient received any type of prophylaxis.

#### If yes, what type of prophylaxis?

Check all types of prophylaxis the patient received.

### Antibacterial

#### Antibiotic

Check all types of antibiotics that were administered as prophylaxis.

#### Phase

Select the phases (**pre-engraftment**, **post-engraftment**) during which the antibiotic was administered. If administered during the post-engraftment phase, indicate whether it was given only during post-engraftment, or whether it was a continuation from pre-engraftment into post-engraftment, or started and stopped during pre-engraftment and then restarted during post-engraftment. Select **unknown** if it is not known during what phase(s) the antibiotic was given.

#### Final date the antibacterial prophylaxis was discontinued

Report the date the patient last received any type of antibacterial prophylaxis, or select **unknown** if you do not know the final date antibacterial prophylaxis was administered, or select **ongoing** if the patient is still receiving antibacterial prophylaxis.



## Antiviral

### Did the patient receive CMV prophylaxis other than or in addition to letermovir ?

Choose **yes** if the patient received any type of CMV prophylaxis that is not letermovir. Choose **no** if the patient either received no CMV prophylaxis at all or received only letermovir. Letermovir is not included in this question as letermovir prophylaxis is a separate question in the core dataset.

#### Which drugs were used?

Check all types of drugs that have been administered as CMV prophylaxis. Please note that letermovir is not included here, as letermovir prophylaxis is a separate question in the core dataset. Please also do *not* include letermovir in the 'other drug' category.

#### Final date CMV prophylaxis was discontinued

Report the date the patient last received any type of CMV prophylaxis other than letermovir, or select **unknown** if you do not know the final date CMV prophylaxis other than letermovir was administered, or select **ongoing** if the patient is still receiving any type of CMV prophylaxis other than letermovir.

### Only for allogeneic HCT: Did the patient receive prophylaxis for varicella-zoster virus (VZV) or herpes simplex virus (HSV) with either acyclovir or valacyclovir?

Indicate if either acyclovir or valacyclovir has been administered as VZV or HSV prophylaxis.

#### Final date VZV/HSV prophylaxis was discontinued

Report the date the patient last received either acyclovir or valacyclovir as VZV or HSV prophylaxis, or select **ongoing** if the patient is still receiving either acyclovir or valacyclovir as VZV or HSV prophylaxis, or select **unknown** if you do not know the final date either acyclovir or valacyclovir was last administered as VZV or HSV prophylaxis.

### Only for allogeneic HCT: Did the patient receive rituximab or another anti-CD20 monoclonal drug as prophylaxis for Epstein-Barr virus (EBV) post-transplant lymphoproliferative disorder (EBV-PTLD)?

Indicate if any anti-CD20 monoclonal drug, including rituximab, has been administered as EBV-PTLD prophylaxis.

## Did the patient receive prophylaxis for hepatitis B (HBV)?

Indicate if any type of HBV prophylaxis has been administered.

### Which drugs were used?

Check all types of drugs that have been administered as HBV prophylaxis.

### Final date HBV prophylaxis was discontinued

Report the date the patient last received any type of HBV prophylaxis, or select **ongoing** if the patient is still receiving any type of HBV prophylaxis, or select **unknown** if you do not know the final date HBV prophylaxis was administered.

## Antifungal

### Antifungal

Check all types of antifungals that have been administered as prophylaxis.

### Phase

Select the phases (**pre-engraftment**, **post-engraftment**) during which the antifungal was administered. If administered during the post-engraftment phase, indicate whether it was given only during post-engraftment, or whether it was a continuation from pre-engraftment into post-engraftment, or stopped during pre-engraftment and then restarted during post-engraftment. Select **unknown** if it is not known during what phase(s) the antifungal was given.

### Final date the antifungal prophylaxis was discontinued

Report the date the patient last received any type of antifungal prophylaxis, or select **unknown** if you do not know the final date antifungal prophylaxis was administered, or select **ongoing** if the patient is still receiving antifungal prophylaxis.

## Did the patient receive prophylaxis for *Pneumocystis jirovecii* pneumonia (PJP)?

Indicate if any type of PJP prophylaxis has been administered.

### Which drugs were used?

Check all types of drugs that have been administered as PJP prophylaxis.

### Final date prophylaxis was discontinued

Report the date the patient last received any type of PJP prophylaxis, or select unknown if you do not know the final date PJP prophylaxis was administered, or select ongoing if the patient is still receiving PJP prophylaxis.

## Pre-emptive viral therapy

### Did the patient receive pre-emptive therapy for a viral infection?

Indicate if the patient received pre-emptive therapy for any virus.

#### If yes, for what virus?

Indicate whether the patient has received pre-emptive therapy for CMV and/or EBV.

### Specify the pre-emptive therapy for each CMV episode that occurred

Repeat the questions below for each CMV episode to reflect all episodes that occurred.

#### CMV treatment start date

Report the date the patient first received any type of pre-emptive therapy for CMV, or select unknown if you do not know the date.

#### Antiviral(s) used

Check all types of antivirals that have been administered as pre-emptive therapy for CMV.

#### Was this episode of CMV infection due to a resistant CMV strain?

Indicate if the CMV strain causing this CMV episode was identified to be of a drug-resistant phenotype with viral genetic mutations decreasing susceptibility to one or more antiviral drugs, or select **unknown** if you do not know if it was a resistant CMV strain.

### Specify the pre-emptive therapy for each EBV episode that occurred

Repeat the questions below for each EBV episode to reflect all episodes that occurred.

#### EBV treatment start date

Report the date the patient first received any type of pre-emptive therapy for EBV, or select unknown if you do not know the date.

### Antiviral(s) used

Check all types of antivirals that have been administered as pre-emptive therapy for EBV.

## Complications since the Last Report-GvHD

This section should only be completed if the patient received an allogeneic HCT.

### Did graft versus host disease (GvHD) occur?

If **No** GvHD occurred or if this information is **Unknown**, select the appropriate answer and proceed to the next section: 'Complications since the last report - Non-infectious complications'. Select **Yes** if GvHD occurred and proceed to the next question.

GvHD refers to a clinical syndrome caused by the response of transplanted donor allogeneic cells to histocompatibility antigens expressed on tissues of the transplantation recipient. Acute GvHD (aGvHD) refers to the appearance of an allogeneic inflammatory response in exclusively three organs: the skin (inflammatory maculopapular erythematous skin rash), the liver (hyperbilirubinemia due to cholestatic jaundice), and the gastro-intestinal (GI) tract (upper and/or lower GI tract manifestations). The diagnosis must occur in the absence of manifestations of chronic GvHD (cGvHD) and should ideally be supported by positive histological findings. cGvHD is based on either the presence of specific diagnostic signs or distinctive signs accompanied by additional confirmation (e.g. biopsy or other objective diagnostic test) in at least one target organ (skin & appendages, mouth, eyes, genitalia, oesophagus, lungs and muscles & fascia). Detailed definitions are described in the 2014 NIH Consensus (1) and 2018 EBMT—NIH—CIBMTR Task Force statement on standardised terminology (2).

### Did the patient receive a systemic/immunosuppressive treatment for GvHD?

Indicate if the patient received a systemic immunosuppressive treatment for GvHD. If the answer is **Yes**, specify:

#### *Date treatment started*

Report here the date the systemic immunosuppressive treatment for GvHD started. If immunosuppressive treatment was started before the GvHD (as prevention) and continued as GvHD treatment, please indicate the start date of immunosuppressive treatment before the GvHD.

### Treatment stopped

Indicate whether systemic immunosuppressive treatment for GvHD is still ongoing and if not, report the stop date of this treatment. Mark as **Unknown** if this is not known.

### Did acute GvHD occur?

Indicate if aGvHD occurred within 100 days post HCT. Mark as **Unknown** if this is not known.

Acute graft versus host disease (aGvHD) is a consequence of donor T-cells recognizing the patient's antigens as foreign. It usually consists of dermatitis, hepatitis, and gastroenteritis. Although it usually develops within the first 100 days, it can also appear later on.

### Date of onset

If aGvHD occurred, report the date of onset. Mark as **Unknown** if this is not known.

### Maximum observed organ severity score

Select for each organ listed in the table the observed severity score. If another site was also affected, answer **Yes** in **Other site affected** and specify this site in the text field in English.

The maximum grade for acute graft versus host disease (aGvHD) is defined according to the stages presented by the skin, liver, lower and upper GI tracts and can be found in table 1.

Organ	Stage	Description
Skin	0	No rash attributable to acute GVHD
	1	Skin rash < 25% body surface
	2	Skin rash 25-50% body surface
	3	Skin rash >50% body surface
	4	Generalised erythroderma (> 50% BSA) plus bullous formation and desquamation >5% of BSA

Organ	Stage	Description
Liver	0	Total serum bilirubin < 34 µmole/L (< 2 mg/dL)
	1	Total serum bilirubin 34–50 µmole/L (2 to 3 mg/dL)
	2	Total serum bilirubin 51–102µmole/L (3.1 to 6 mg/dL)
	3	Total serum bilirubin 103–255 µmole/L (6.1 to 15 mg/dL)
	4	Total serum bilirubin >255 µmole/L (> 15 mg/dL)
Lower gut	0	Diarrhoea < 500 mL/day or <3 episodes/day for adults or diarrhoea <10 mL/kg/day or <4 episodes/day for children
	1	Diarrhoea 500–999 mL/day or 3–4 episodes/day for adults or diarrhoea 10–19.9 mL/kg/day or 4–6 episodes/day for children
	2	Diarrhoea 1000–1500mL/day or 5–7 episodes/day for adults diarrhoea 20–30 mL/kg/day or 7–10 episodes/day for children
	3	Diarrhoea >1500 mL/day or >7 episodes/day for adults or diarrhoea > 30 mL/kg/day or >10 episodes/day for children
	4	Severe abdominal pain with or without ileus or grossly bloody stools (regardless of stool volume)
Upper gut	0	No or intermittent anorexia or nausea or vomiting
	1	Persistent anorexia or nausea or vomiting

Table 1. aGvHD grading system per organ (2) <http://dx.doi.org/10.1038/s41409-018-0204-7>.

## Overall maximum grade observed

Select the overall maximum grade that was observed. If it is not known which overall maximum grade was observed, select **Unknown**.

The overall grade (or the stage of skin, liver and gut) should be mentioned in the patients' file. If not clearly stated, ask the treating physician. You should report the maximum grade seen during this follow-up period as calculated from table 2 according to the MAGIC consortium .

Grade							
1	Skin stage 1 or 2	AND	Liver stage 0	AND	Upper gut stage 0	AND	Lower gut stage 0
2	Skin stage 3	AND/ OR	Liver stage 1	AND/ OR	Upper Gut stage 1	AND/ OR	Lower gut stage 1
3	Not skin stage 4	AND	Liver stage 2 or 3	AND/ OR			Lower gut stage 2 or 3
4	Skin stage 4	OR	Liver stage 4	OR			Lower gut stage 4

Table 2. Overall maximum grade for aGvHD (2) <http://dx.doi.org/10.1038/s41409-018-0204-7>.

### Steroid-refractory acute GvHD

Indicate if the patient experienced steroid-refractory acute GvHD or not. Steroid-refractory aGvHD is defined as failure to respond to standard steroid doses (defined as progression within 3–5 days of starting treatment or an incomplete response by 7–14 days) or recurrence after initial dose reduction (steroid dependence) as stated in the EBMT handbook (3). Mark as **Unknown** if this is not known.

### *Date of onset*

If steroid-refractory acute GvHD is observed, report the date of onset. Mark as **Unknown** if this is not known.

### aGvHD resolved?

Please indicate whether the aGvHD was resolved or not. Mark as **Unknown** if this is not known.

### *Date of aGvHD resolution*

If the acute GvHD was resolved, please report the date on which it was thought to have resolved completely. Mark as **Unknown** if this is not known.

*Extended dataset*

## aGvHD first line treatment

## Did the patient receive steroids as first line treatment of aGvHD?

Please indicate here if steroids were used here for treatment of aGvHD in the first line. If steroids were used in prophylaxis only, but not in treatment of aGvHD, please report 'No' here.

## Steroid details

Please report here the steroid used in treatment, start date, initial dose (in mg/kg/day), if the drug was stopped, and stop date if applicable. If you only have the drug in mg units, please divide by the patient weight in kg on the date of start of treatment to calculate in the correct units (mg/kg). If multiple steroids were given in the first line, please report all.

## Were other systemic drugs/strategies used to treat aGvHD in the first line?

Please indicate if other systemic drugs/ strategies used to treat aGvHD in the first line during this follow-up period by answering **Yes** or **No**. Mark as **Unknown** if it is unknown.

## Name of drug/strategy

Please report here all the drugs/strategies that were given in the first line treatment of aGvHD. Do not report drugs that were only given as GvHD prophylaxis.

## How did aGvHD respond to steroids?

Please indicate the response of aGvHD to steroids based on the following definitions below:

## Steroid sensitive (SS)

If a partial or complete response is achieved in aGvHD after undergoing steroid treatment, please report 'Yes' here.

## Steroid refractory (SR)

Refractory: progression in any organ within 3, 4 or 5 days of therapy onset with  $\geq 2$  mg/Kg/day of prednisone equivalent, or failure to improve within 5 to 7 days of treatment initiation, or incomplete response after more than 28 days of immunosuppressive treatment including steroids.



### Steroid dependent (SD)

Dependent: Inability to taper prednisone under 2 mg/Kg/day after an initially successful treatment of at least 7 days or as the recurrence of aGVHD activity during steroid tapering.

### *Date of onset of SD aGVHD*

If steroid dependent, indicate the date of onset of steroid dependence. (Date of onset of steroid refractory aGVHD has already been reported in the Core data form). Please note that by definition, this cannot be the same date as onset of aGVHD. If the date is unavailable, select **Unknown**.

## Steroid refractory/dependent aGVHD

### Did the patient receive treatment for SR/SD aGVHD?

Indicate if the patient receives a treatment for SR/SD aGVHD.

### Overall aGVHD grade at start of SR/SD aGVHD treatment

Please report the overall grade of the aGVHD on the date that SR/SD treatment started. This is the treatment that will be reported below (first line of SR/SD aGVHD, rather than the previously reported first line treatment of aGVHD). If treatment was started before SR/SD was established and continued as the first line treatment, please report the grade of aGVHD on the date that SR/SD was established.

### Organs involved at start of SR/SD aGVHD treatment

As for regular GVHD, please report all involved organ stages according to the Glucksburg scale. The date of start of SR/SD aGVHD treatment applies, as above.

### Drugs given in this line of treatment (after steroid refractoriness/dependence was established)

Please complete the table of drugs used to treat SR/SD aGVHD. Enter drugs line by line (first line for SR/SD aGVHD, then second line and any other subsequent lines). Please note that this is not first line treatment of aGVHD, but line treatment after SR/SD has been established. Enter all start dates, and stop dates, if relevant.

## Organ involved during the course of treatment and response to the line of treatment

Please report if each organ was involved or not at the start of treatment, and if yes, please also report the response to this line of treatment. If organ specific responses are not available, please report the overall response. Responses to each line of treatment should be entered separately, starting with the first line and proceeding to subsequent lines, if applicable.

Note again, that this is the response to SR/SD aGvHD, so the response to treatment after SR/SD was established. This is not asking to report the response to first line steroid treatment of aGvHD (before SR/SD onset).

## Did chronic GvHD occur?

Indicate if chronic GvHD occurred or not within 100 days post-HCT. Mark as **Unknown** if this is not known.

### Date of onset

If cGvHD occurred, report the date of onset. Mark as **Unknown** if this is not known.

### Maximum NIH score during this period

Indicate if the maximum NIH score during this period was **Mild**, **Moderate** or **Severe**. If the score is unknown, select **Unknown**.

The NIH scoring system was first published in 2005 and was updated in 2014 and 2022. As described in the 2014 Diagnosis and Staging Working Group report (1), eight classical organs or sites (skin, mouth, eyes, lungs, musculoskeletal system, gastrointestinal tract, genitourinary tract, and liver) are considered for calculating global score.

Elements included in the proposed global scoring include both the number of organs or sites involved and the severity score within each affected organ. Indicate the maximum NIH score during this period, as per the results of these measurements. Instructions for physicians on assessing the NIH score can be found in the EBMT handbook (1,4) or table 3.

Mild cGvHD	1 or 2 organs involved with no more than score 1 AND Lung score 0
Moderate cGvHD	3 or more organs involved with no more than score 1 OR At least 1 organ (not lung) with a score of 2 OR

	Lung score 1
Severe cGvHD	At least 1 organ with a score of 3 OR Lung score of 2 or 3

Table 3. Assessing the maximum NIH score (1).

In 2022 the NIH consensus (5) recognized atypical manifestations of chronic GvHD, which should be reported in the section '**Other site affected**' below the list of organs involved. Atypical manifestations do not contribute to the global severity score.

### Date maximum NIH score

Report the date the maximum NIH score was observed.

### Maximum observed organ severity score

Select for each organ in the table the observed severity score. If another site was affected, answer **Yes** in **Other site affected** and specify this site in the text field in English.

Use the NIH scoring system as described in 6.3.2.

### Steroid-refractory chronic GvHD

Indicate if the patient experienced steroid-refractory chronic GvHD. Steroid refractory cGvHD is defined as progression of cGvHD while on prednisone at  $\geq 1$  mg/kg/day for 1 to 2 weeks or stable GvHD on  $\geq 0.5$  mg/kg/day (or 1 mg/kg every other day) of prednisone for 1 to 2 months (2).

### Date of onset

If steroid-refractory chronic GvHD was observed, report the date of onset. Mark as **Unknown** if this is not known.

### cGvHD resolved?

Please indicate whether the cGvHD was resolved or not. Mark as **Unknown** if this is not known.

### Date of cGvHD resolution

If the chronic GvHD was resolved, please report the date on which this occurred. Mark as **Unknown** if this is not known.

### Was overlap syndrome observed (features of both chronic and acute GvHD)

If overlap syndrome was observed, select **Yes**. If overlap syndrome was not observed, select **No**. Mark as **Unknown** if this is not known.

*Extended dataset*

## cGvHD first line treatment

## Did the patient receive steroids as first line treatment of cGvHD?

Please indicate here if steroids were used here for treatment of cGvHD in the first line. If steroids were used in prophylaxis only, but not in treatment of cGvHD, please report 'No' here. If steroids were given for aGvHD treatment only, but not for cGvHD treatment, please report 'No' here.

## Steroid details

Please report here the steroid used in treatment of cGvHD, start date, initial dose (in mg/kg/day), if the drug was stopped, and stop date if applicable. If you only have the drug in mg units, please divide by the patient weight in kg on the date of start of treatment to calculate in the correct units (mg/kg). If multiple steroids were given in the first line, please report all.

## Were other systemic drugs/strategies used to treat cGvHD in the first line?

Please report here all the drugs/strategies that were given in the first line treatment of cGvHD. Do not report drugs that were only given as GvHD prophylaxis.

## How did cGvHD respond to steroids?

Please indicate the response of cGvHD to steroids based on the following definitions below:

## Steroid sensitive (SS)

If a partial or complete response is achieved in aGvHD after undergoing steroid treatment, please report 'Yes' here.

## Steroid refractory (SR)

Refractory: progression of GvHD while on prednisone at  $\geq 1$  mg/Kg/day for 1-2 weeks or stable GvHD while on  $\geq 0.5$  mg/Kg/day (or 1 mg/Kg every other day) of prednisone for 1-2 months.

## Steroid dependent (SD)

Dependent: inability to control GVHD symptoms while tapering prednisone below 0.25 mg/Kg/day (or 0.5 mg/Kg every other day) in at least two individual attempts, separated by at least 8 weeks.

### Steroid intolerant (SI)

Intolerant: Includes avascular necrosis, severe myopathy, uncontrolled diabetes mellitus, systemic viral or fungal infections.

### *Date of onset of SD/SI cGvHD*

If steroid dependent/intolerant, indicate the date of onset of steroid dependence/intolerance. (Date of onset of steroid refractory cGvHD has already been reported in the Core data form). Please note that by definition, this cannot be the same date as onset of cGvHD. If the date is unavailable, select **Unknown**.

## Steroid refractory/dependent/intolerant cGvHD

### Did the patient receive treatment for SR/SD/SI cGvHD?

Indicate if the patient receives a treatment for SR/SD cGvHD.

### Overall cGvHD grade at start of SR/SD/SI treatment

Please report the overall grade of the cGvHD on the date that SR/SD/SI treatment started. This is the treatment that will be reported below (first line of SR/SD/SI cGvHD, rather than the previously reported first line treatment of cGvHD). If treatment was started before SR/SD/SI was established and continued as the first line treatment, please report the grade of cGvHD on the date that SR/SD/SI was established.

### Organs involved at start of SR/SD/SI treatment

As for regular GvHD, please report all involved organ stages according to the NIH scoring system. The date of start of SR/SD/SI cGvHD treatment applies, as above.

### Drugs given in this line of treatment (after steroid refractoriness/dependence/intolerant was established)

Please complete the table of drugs used to treat SR/SD/SI cGvHD. Enter drugs line by line (first line for SR/SD/SI cGvHD, then second line and any other subsequent lines). Please note that this is not first line treatment of aGvHD, but lines of treatment after SR/SD/SI has been established. Enter all start dates and stop dates, if relevant.

## Organ involvement during the course of treatment and response to the line of treatment

Please report if each organ was involved or not at the start of treatment, and if yes, please also report the response to this line of treatment. If organ specific responses are not available, please report the overall response. Responses to each line of treatment should be entered separately, starting with the first line and proceeding to subsequent lines, if applicable.

Note again, that this is the response to SR/SD/SI cGvHD, so the response to treatment after SR/SD/SI was established. This is not asking to report the response to first line steroid treatment of aGvHD (before SR/SD/SI onset).

## Complications since the Last Report Non-infectious complications

### Did non-infectious complications occur during the follow-up period?

If no other non-infectious complication than GvHD occurred during the follow-up period or if the complication was grade 1 or 2, select **No** and proceed to the next section. If non-infectious complications with a CTCAE grade of at least 3 occurred or graft failure, pure red cell aplasia, posterior reversible encephalopathy syndrome or VOD of any grade occurred, select **Yes** and report in the table below.

For adverse events not listed in the table, specify them in the **Other** text field. Consult with Appendix 3 in the paper form which non-infectious complications should not be reported even for grades 3 and 4.

### Adverse event observed

Specify for each adverse event listed whether it was observed or not. The CTCAE gradings (v5) can be found on the website of the NIH (6). Please note that if an event can be reported in more than one type of adverse event, it should be reported only once in the most precise category (eg. a cerebral thrombosis should be reported only as a cerebral thrombosis and not also as a vascular event).

If a grading is dependent on hospitalisation but the patient was an inpatient at the time of onset, the centre will make the interpretation. If the patient had been an out-patient and the severity was such that the patient would have been hospitalised, grading will be selected accordingly.

### Maximum CTCAE grade observed

Select for each adverse event the maximum CTCAE grade that was observed. If the grade is unknown, select **unknown**.

Note, for the following complications there are different grading systems to be used:

- Graft failure (fatal/non-fatal)
- Pure red cell aplasia (fatal/non-fatal)
- Posterior reversible encephalopathy syndrome (considered severe if a patient was re-hospitalised, transferred to the ICU, experiences severe mental impairment or severe cerebral edema)
- Venous-occlusive disease (VOD)/ Sinusoidal obstruction syndrome (SOS) is diagnosed based on clinical criteria. The most recently proposed EBMT criteria can be found in table 4.

Adults	Children			
<p>Classical SOS/VOD <i>In the first 21 days after HSCT:</i> Bilirubin <math>\geq 2</math> mg/dL and two of the following criteria must be present: Painful hepatomegaly Weight gain <math>&gt;5\%</math> Ascites</p> <p>Late onset SOS/VOD <math>&gt;21</math> Days after HSCT: Classical VOD/SOS beyond day 21 Or Histologically proven SOS/VOD OR Two or more of the following criteria must be present: Bilirubin <math>\geq 2</math> mg/dL (or <math>34 \mu\text{mol/L}</math>) Painful hepatomegaly Weight gain <math>&gt;5\%</math> Ascites AND Hemodynamic or/and ultrasound evidence of SOS/VOD</p>	<p>No limitation for time of onset of SOS/VOD The presence of two or more of the following:</p> <ul style="list-style-type: none"><li>• Unexplained consumptive and transfusion-refractory thrombocytopenia</li><li>• Otherwise unexplained weight gain on three consecutive days despite the use of diuretics or a weight gain <math>&gt;5\%</math> above baseline value</li><li>• Hepatomegaly (best if confirmed by imaging) above baseline value</li><li>• Ascites (best if confirmed by imaging) above baseline value</li><li>• Rising bilirubin from a baseline value on 3 consecutive days or bilirubin <math>\geq 2</math> mg/dL within 72 h</li></ul>			
Severity definition				
	Mild	Moderate	Severe	Very severe
Time since first clinical symptoms of SOS/VOD	$>7$ Days	5–7 Days	$\leq 4$ Days	Any time
Bilirubin ( $\mu\text{mole/L}$ )	$\geq 34$ and $<51$	$\geq 51$ and $<85$	$\geq 85$ and $<136$	$\geq 136$
Bilirubin kinetics			Doubling within 48 h	
Transaminases	$\geq 2 \times$ normal	$> 2$ and $\leq 5 \times$ normal	$>5$ and $\leq 8 \times$ normal	$>8 \times$ Normal
Weight increase	$< 5\%$	$\geq 5\%$ and $<10\%$	$\geq 5\%$ and $<10\%$	$\geq 10\%$
Renal function	$<1.2 \times$ baseline at transplant	$\geq 1.2$ and $<1.5 \times$ baseline at transplant	$\geq 1.5$ and $<2 \times$ baseline at transplant	$\geq 2 \times$ baseline at transplant or others signs of MOD/MOF

Table 4. Diagnostic criteria for SOS and VOD (8).

No grading needs to be marked for Pure red cell aplasia.

### Onset date

Report the onset date when the adverse event was observed.

### Resolved

Answer **Yes** if the non-infectious complication has been resolved within the follow-up period. If the complication was resolved in the observed period, report the date the complication was resolved/stopped.

#### *Extended dataset*

### Treatment of early complications

#### Was TA-TMA treatment given?

Indicate if treatment was given.

#### Line of TA-TMA treatment given

Please complete the table of drugs used to treat TA-TMA. Enter drugs line by line, including the start date and the stop date if applicable. .

#### Other TA-TMA treatments given in this line of treatment

Please report any of the other requested procedures or therapies that were carried out as treatment of TA-TMA here, including renal replacement therapy, mechanical ventilation or exchange plasmapheresis. If applicable, please report the first date that each of these treatments was performed.

#### Response to this line of TA-TMA treatment

#### Did the patient achieve complete/partial response?

Please report whether the patient achieved a complete response (CR) or not per line of treatment for TA-TMA , and if so, please provide the date of this response. CR can be defined as normal LDH, no organ manifestations, high-risk TA-TMA harmonisation criteria not fulfilled anymore.

If the patient did not achieve a complete response, please report whether the patient achieved a partial response (PR) or not , and if so, please provide the date of this response. PR can be defined as



LDH decreased, residual organ manifestations, high-risk TA-TMA harmonisation criteria not fulfilled anymore.

### Was VOD treatment given?

Indicate if treatment was given.

### Line of VOD treatment given

Please complete the table of drugs used to treat VOD . Enter drugs line by line, including the start date and the stop date if applicable.

### Other VOD treatments given in this line of treatment

Please report any of the other requested procedures or therapies that were carried out as treatment of VOD here, including renal replacement therapy, mechanical ventilation or extracorporeal membrane oxygenation support. If applicable, please report the first date that each of these treatments was performed.

### Response to this line of VOD treatment

#### Did the patient achieve complete/partial response?

Please report whether the patient achieved a complete response (CR) or not per line of treatment for VOD, and if so, please provide the date of this response. CR can be defined as serum bilirubin <2 mg/dL, no oxygen support, eGFR >50% from baseline before VOD and no renal replacement therapy.

If the patient did not achieve a complete response, please report whether the patient achieved a partial response (PR) or not , and if so, please provide the date of this response. PR can be defined as serum bilirubin increased, but >2 mg/dL, or pulmonary dysfunction, or eGFR <=50% from baseline before VOD.

## Complications since the last report-Infectious complications

### Did infectious complications occur during the follow-up period?

Answer **Yes** if any infectious complications occurred during this follow-up period.

Infections that were resolved before the HCT do not need to be reported, unless a reactivation occurred after HCT.

Please note that the following infections do NOT need to be reported:

- Minor ophthalmologic bacterial infections (e.g. conjunctivitis treated topically; blepharitis treated topically; styne treated topically)
- External otitis treated topically
- Otitis media treated with oral antibiotics
- Isolated lip herpes simplex
- Bacterial tonsillitis or pharyngitis treated orally
- Laryngitis without viral identification managed at home by inhalations or without any intervention
- Upper respiratory tract infection (URTI) without viral/bacterial identification managed at home
- Bilateral cervical lymph node enlargement concurrent with URTI that resolved without specific treatment, together with the resolution of URTI
- Local superficial wound infection resolved under topical antibiotics (including impetigo)
- Minor skin bacterial infections (e.g. folliculitis; acne)
- Minor fungal skin infection (e.g. candidal intertrigo treated topically)
- Diaper rash treated with local antifungals
- Candidal balanitis treated topically
- Vaginal candidiasis treated topically or with a single oral dose
- Asymptomatic bacteriuria due to a pathogen not multi-resistant
- Single low urinary tract infection treated orally without need for hospitalisation
- Phlebitis following peripheral intravascular infusion that resolved after intravascular removal without treatment with antibiotics
- Any isolate that is considered part of the normal flora of the place (oral cavity, vagina, skin, stools) except if it carries an antimicrobial resistance that has clinical implications (induce isolation precautions or a pathogen-directed therapy)
- Positive culture without clinical implications (i.e. symptoms/signs of disease; administration of pathogen-directed therapy; isolation precautions or surveillance)

## Bacterial infection

Indicate if the patient had a bacterial infection in the follow-up period after the treatment (after lymphodepleting/conditioning regimen) took place. Report here only bacterial infections with microbiological documentation, otherwise they shall be reported as infection with unknown pathogen.

### *Start date*

Report the date a first positive blood or other relevant culture or diagnostic sample was obtained. In case a diagnostic sample was obtained with a delay since the symptoms of infection started – report here the date when symptoms attributable to this infection started (e.g. patient with pneumonia, urine test for legionella was sent after a few days and the test result was positive).

In case an infection was found for the first time at autopsy, use the date of death, unless you can identify the date when the symptoms attributable to this infection were reported for the first time.

### *Type of bacteria*

Select the type of bacteria by marking if it is 'Gram-positive', 'Gram-negative' or 'Other' (see the list in Appendix 2 of the form or available in the database).

### *Pathogen*

Select the bacterium that caused the infection from the list in Appendix 2 of the form or available in the database. Choose the most specific option. If the pathogen cannot be found, choose the 'Gram-positive bacteria other spp', 'Gram-negative bacteria other spp' or 'Bacteria other' option and enter its name in a textbox. Always report the full name of the bacterium.

Please note that some bacteria appear several times but with the emphasis on their resistance pattern. If relevant susceptibility data is unavailable, 'Gram-positive bacteria other spp' or 'Gram-negative bacteria other spp' can be selected (e.g. in case of *Pseudomonas* without information on carbapenem susceptibility (meropenem, imipenem or doripenem) choose 'Gram-negative bacteria other spp'). For *Staphylococcus aureus*: if vancomycin susceptibility is unavailable, but it is methicillin-susceptible (can appear as "oxacillin"), it should be reported as '*Staphylococcus aureus* MSSA (methicillin-susceptible)'. For '*Staphylococcus aureus* MRSA (methicillin-resistant)', indicate whether the vancomycin susceptibility was not tested, or whether it was VISA (vancomycin-intermediate) or VRSA (vancomycin-resistant) based on the minimum inhibitory concentration (MIC) for vancomycin falling within the range noted in the Appendix.

Common commensals (most commonly coagulase-negative Staphylococci, *Micrococcus* spp., *Bacillus* spp., *Propionibacterium* spp.) should be reported only if there are at least two positive blood cultures.

### *Infection with clinical implications*

Indicate if the infection had clinical implications or not, or mark **Unknown** if it is not possible to identify. Infection with clinical implications is at least one of the following: symptomatic infection in the relevant organ/system, or infection that requires pathogen-directed therapy.

#### *Infection with clinical implications, yes*

Select all clinical implications of the infection that apply from suggested answer options:

- Symptoms/signs of disease;
- Administration of pathogen-directed therapy;

### *Localisation (adapted from CTCAE terms)*

Select the localisation for the infection from the list in Appendix 3 of the form or available in the database.

In the appendix, we include both **general localisation** as part of the core dataset and **detailed localisation** as part of the extended dataset. If the more specific localisation is known, please report the **general localisation** and, if requested in the extended dataset, further specify by providing the **detailed localisation**. If only the higher-level localisation is known, report only the core dataset general localisation.

Please note that the impact of an infection can differ greatly depending on its localisation, and therefore, localisation is essential and at least 1 location involved during this follow-up period must be reported.

The localisations used in the current form are adapted from CTCAE version 5.0. Additional information on the CTCAE scoring can be found on the NIH website (6).

If the clinical information available does not specify the localisation of the infection, probably the infection was asymptomatic and will not have to be reported. Otherwise, the symptoms should guide the choice.

### *Intravascular catheter-related infection*

Indicate if the infection was related to a central venous catheter (CVC). Please note that bacteraemia can be CVC-related or not CVC-related. The definition for the CVC-related bacteraemia requires one of the following:

- The same organism (genus, species and susceptibility pattern) growing from at least 1 percutaneous blood sample culture and from the catheter tip (e.g. two coagulase-negative Staphylococci, but different species, such as *Staphylococcus capitis* and *Staphylococcus epidermidis*, or two *Staphylococcus epidermidis* with completely different susceptibilities are not the same).
- 2 blood samples for culture are obtained (1 from a catheter hub and 1 from a peripheral vein) that meet CVC-related bacteraemia criteria for differential time to positivity (DTP): growth of microbes from blood obtained through the catheter hub being detected at least 2 hours before microbial growth is detected in blood samples obtained from a peripheral vein (9).

### *Specify*

If the infection was related to an intravascular catheter, select the type of CVC infection from the list in Appendix 4 or available in the database.

### *Infection resolved*

Indicate if the infection was resolved during the follow-up period. By resolved it is understood that the patient is free of symptoms/signs of this infection or symptoms/signs of this infection are under control, and negative diagnostic tests were obtained in case the investigation was repeated, and the relevant imaging is compatible with expected improvement.

### *Contributory cause of death*

In case the patient is deceased, indicate if the infection contributed to death.

If there was more than one bacterial infectious complication during the follow-up period, repeat these questions for the subsequent infection. Copy and fill-in the table as many times as necessary.

### *Viral infection*

Indicate if the patient had a viral infection in the follow-up period after the treatment (after lymphodepleting/conditioning regimen) took place. Report here only infections with microbiological documentation, otherwise they shall be reported as infection with unknown pathogen.

### *Start date*

Report the date a first positive viral test (usually PCR or antigen) was obtained. In case a diagnostic sample was obtained with a delay since the symptoms of infection started – report here the date when symptoms attributable to this infection started (e.g. patient with encephalitis, with a positive PCR in cerebrospinal fluid done 10 days after symptoms started).

In case an infection was found for the first time at autopsy, use the date of death, unless you can identify the date when the symptoms attributable to this infection were reported for the first time.

### *Pathogen*

Select the virus that caused the infection from the list in Appendix 2 of the form or available in the database. Choose the most specific option. If the pathogen cannot be found, choose the 'Viruses other' option and enter its name in a textbox. Always report the full name of the virus.

### *If the pathogen was CMV/EBV: was this infection a reactivation?*

Answer yes, if the patient's serology tests (CMV IgG, EBNA, EBV IgG) were positive before the treatment (start of lymphodepleting/conditioning regimen) took place or if the patient has been reported to have previously had an active CMV/EBV infection.

### *Infection with clinical implications*

Indicate if the infection had clinical implications or not, or mark unknown if it is not possible to identify. Infection with clinical implications is at least one of the following: symptomatic infection in the relevant organ/system, or infection that requires pathogen-directed therapy.

#### *Infection with clinical implications, yes:*

Select all clinical implications of the infection that apply from suggested answer options:

- Symptoms/signs of disease;
- Administration of pathogen-directed therapy;

### *Localisation (adapted from CTCAE terms)*

Select the localisation for the infection from the list in Appendix 3 of the form or available in the database.

In the appendix, we include both **general localisation** as part of the core dataset and **detailed localisation** as part of the extended dataset. If the more specific localisation is known, please report the **general localisation** and, if requested in the extended dataset, further specify by providing the **detailed localisation**. If only the higher-level localisation is known, report only the core dataset general localisation.

Please note that the impact of an infection can differ greatly depending on its localisation, and therefore, localisation is essential and at least 1 location involved during this follow-up period must be reported.

When EBV DNA is detected in the blood—which constitutes the vast majority of EBV detections—you can report the localization as viremia/DNAemia by selecting from the blood infections group.

The localisations used in the current form are adapted from CTCAE version 5.0. Additional information on the CTCAE scoring can be found on the NIH website (6).

If the clinical information available does not specify the localisation of the infection, probably the infection was asymptomatic and will not have to be reported. Otherwise, the symptoms should guide the choice.

### *Infection resolved*

Indicate if the infection was resolved during the follow-up period. By resolved it is understood that the patient is free of symptoms/signs of this infection or symptoms/signs of this infection are under control, and negative diagnostic tests were obtained in case the investigation was repeated, and the relevant imaging is compatible with expected improvement.

### *Contributory cause of death*

In case the patient is deceased, indicate if the infection contributed to death.

If there was more than one viral infectious complication during the follow-up period, repeat these questions for the subsequent infection. Copy and fill-in this table as many times as necessary.

### *Fungal infection*

Indicate if the patient had a fungal infection in the follow-up period after the treatment (after lymphodepleting/conditioning regimen) took place. Report here only infections with microbiological documentation, otherwise they shall be reported as infection with unknown pathogen.

### *Start date*

Report the date a first positive culture, PCR test or galactomannan test was obtained, or the pathogen was first identified by its typical appearance in the tissue/specimen material. In case a diagnostic sample was obtained with a delay since the symptoms of infection started – report here the date when symptoms attributable to this infection started, or when this is not known, the date of the first imaging (e.g. CNS, lungs, or liver/spleen imaging for instance in hepatosplenic candidiasis in a patient with persistent fever and negative blood cultures).

In case an infection was found for the first time at autopsy, use the date of death, unless you can identify the date when the symptoms attributable to this infection were reported for the first time.

### *Type of fungi*

Select the type of fungal infection by marking if it is 'Yeasts' or 'Moulds'.

### *Pathogen*

Select the fungus that caused the infection from the list in Appendix 2 of the form or available in the database. Choose the most specific option. If the pathogen cannot be found, choose the 'Yeasts other' or 'Moulds other spp' option and enter its name in a textbox. Always report the full name of the fungus. Please note that there is an option for mould infection diagnosed based on positive galactomannan only without additional microbiological confirmation.

### *Infection with clinical implications*

Indicate if the infection had clinical implications or not, or mark unknown if it is not possible to identify. Infection with clinical implications is at least one of the following: symptomatic infection in the relevant organ/system, or infection that requires pathogen-directed therapy.

### *Infection with clinical implications, yes*

Select all clinical implications of the infection that apply from suggested answer options:

- Symptoms/signs of disease;
- Administration of pathogen-directed therapy;

### *Localisation (adapted from CTCAE terms)*

Select the localisation for the infection from the list in Appendix 3 of the form or available in the database.

In the appendix, we include both **general localisation** as part of the core dataset and **detailed localisation** as part of the extended dataset. If the more specific localisation is known, please report the **general localisation** and, if requested in the extended dataset, further specify by providing the **detailed localisation**. If only the higher-level localisation is known, report only the core dataset general localisation.

Please note that the impact of an infection can differ greatly depending on its localisation, and therefore, localisation is essential and at least 1 location involved during this follow-up period must be reported.

The localisations used in the current form are adapted from CTCAE version 5.0. Additional information on the CTCAE scoring can be found on the NIH website (6).

If the clinical information available does not specify the localisation of the infection, probably the infection was asymptomatic and will not have to be reported. Otherwise, the symptoms should guide the choice.

### *Intravascular catheter-related infection*

Indicate if the infection was related to a central venous catheter (CVC). Please note that fungemia can be CVC-related or not CVC-related. The definition for the CVC-related fungemia requires one of the following:

- The same organism (genus, species and susceptibility pattern) growing from at least 1 percutaneous blood sample culture and from the catheter tip
- 2 blood samples for culture are obtained (1 from a catheter hub and 1 from a peripheral vein) that meet CVC-related fungemia criteria for differential time to positivity (DTP): growth of fungi from blood obtained through the catheter hub being detected at least 2 hours before fungal growth is detected in blood samples obtained from a peripheral vein (9).

### *Specify*

If the infection was related to an intravascular catheter, select the type of CVC infection from the list in Appendix 4 or available in the database.



### *Infection resolved*

Indicate if the infection was resolved during the follow-up period. By resolved it is understood that the patient is free of symptoms/signs of this infection or symptoms/signs of this infection are under control, and negative diagnostic tests were obtained in case the investigation was repeated, and the relevant imaging is compatible with expected improvement.

### *Contributory cause of death*

In case the patient is deceased, indicate if the infection contributed to death.

If there was more than one fungal infectious complication during the follow-up period, repeat these questions for the subsequent infection. Copy and fill-in this table as many times as necessary.

### *Parasitic infection*

Indicate if the patient had a parasitic infection in the follow-up period after the treatment (after lymphodepleting/conditioning regimen) took place. Report here only infections with microbiological documentation, otherwise they shall be reported as infection with unknown pathogen.

### *Start date*

Report the date a first positive antigen or DNA test was obtained or the first positive microscopic examination was performed. In case a diagnostic sample was obtained with a delay since the symptoms of infection started – report here the date when symptoms attributable to this infection started, or when this is not known, the date of the first imaging (e.g. CNS imaging for instance in Toxoplasmosis).

In case an infection was found for the first time at autopsy, use the date of death, unless you can identify the date when the symptoms attributable to this infection were reported for the first time.

### *Type of parasite*

Select the type of parasitic infection by marking if it is 'Protozoa' or 'Helminths'.

### *Pathogen*

Select the parasite that caused the infection from the list in Appendix 2 of the form or available in the database. Choose the most specific option. If the pathogen cannot be found, choose the 'Protozoa other spp' or 'Other helminths' option and enter its name in a textbox. Always report the full name of the parasite.

### *Infection with clinical implications*

Indicate if the infection had clinical implications or not, or mark unknown if it is not possible to identify. Infection with clinical implications is at least one of the following: symptomatic infection in the relevant organ/system, or infection that requires pathogen-directed therapy.

### *Infection with clinical implications, yes*

Select all clinical implications of the infection that apply from suggested answer options:

- Symptoms/signs of disease;
- Administration of pathogen-directed therapy;

### *Localisation (adapted from CTCAE terms)*

Select the localisation for the infection from the list in Appendix 3 of the form or available in the database.

In the appendix, we include both **general localisation** as part of the core dataset and **detailed localisation** as part of the extended dataset. If the more specific localisation is known, please report the **general localisation** and, if requested in the extended dataset, further specify by providing the **detailed localisation**. If only the higher-level localisation is known, report only the core dataset general localisation.

Please note that the impact of an infection can differ greatly depending on its localisation, and therefore, localisation is essential and at least 1 location involved during this follow-up period must be reported.

The localisations used in the current form are adapted from CTCAE version 5.0. Additional information on the CTCAE scoring can be found on the NIH website (6).

If the clinical information available does not specify the localisation of the infection, probably the infection was asymptomatic and will not have to be reported. Otherwise, the symptoms should guide the choice.

### *Infection resolved*

Indicate if the infection was resolved during the follow-up period. By resolved it is understood that the patient is free of symptoms/signs of this infection or symptoms/signs of this infection are under control, and negative diagnostic tests were obtained in case the investigation was repeated, and the relevant imaging is compatible with expected improvement.

### *Contributory cause of death*

In case the patient is deceased, indicate if the infection contributed to death.

If there was more than one parasitic infectious complication during the follow-up period, repeat these questions for the subsequent infection. Copy and fill-in the table as many times as necessary.

## Infection with unknown pathogen

Indicate if the patient had an infection with unknown pathogen in the follow-up period after the treatment (after lymphodepleting/conditioning regimen) took place.

Use this section to report clinical infections without microbiological documentation, like pneumonia, cellulitis, typhlitis, etc.

### *Start date*

Report the date the first signs or complaints were recorded or the first positive radiology was obtained. In case an infection was found for the first time at autopsy, use the date of death, unless you can identify the date when the symptoms attributable to this infection were reported for the first time.

### *Infection with clinical implications*

Infection with clinical implications is at least one of the following: symptomatic infection in the relevant organ/system, or infection that requires pathogen-directed therapy. Since an infection with an unknown pathogen always has clinical implications to be reported, the 'Infection with clinical implications, yes' field always has to be filled in.

### *Infection with clinical implications, yes*

Select all clinical implications of the infection that apply from suggested answer options:

- Symptoms/signs of disease;
- Administration of pathogen-directed therapy;

### *Localisation (adapted from CTCAE terms)*

Select the localisation for the infection from the list in Appendix 3 of the form or available in the database.

In the appendix, we include both **general localisation** as part of the core dataset and **detailed localisation** as part of the extended dataset. If the more specific localisation is known, please report the **general localisation** and, if requested in the extended dataset, further specify by providing the **detailed localisation**. If only the higher-level localisation is known, report only the core dataset general localisation.

Please note that the impact of an infection can differ greatly depending on its localisation, and therefore, localisation is essential and at least 1 location involved during this follow-up period must be reported.

The localisations used in the current form are adapted from CTCAE version 5.0. Additional information on the CTCAE scoring can be found on the NIH website (6).

If the clinical information available does not specify the localisation of the infection, probably the infection was asymptomatic and will not have to be reported. Otherwise, the symptoms should guide the choice.

### *Intravascular catheter-related infection*

Indicate if the infection was related to a central venous catheter (CVC). For example, a purulent infection of the exit site or tunnel, without isolation of pathogen.

#### *Specify*

If the infection was related to an intravascular catheter, select the type of CVC infection from the list in Appendix 4 or available in the database.

### *Infection resolved*

Indicate if the infection was resolved during the follow-up period. By resolved it is understood that the patient is free of symptoms/signs of this infection or symptoms/signs of this infection are under control, and the relevant imaging is compatible with expected improvement.

### *Contributory cause of death*

In case the patient is deceased, indicate if the infection contributed to death.

If there was more than one infectious complication with an unknown pathogen during the follow-up period, repeat these questions for the subsequent infection. Copy and fill-in the table as many times as necessary.

#### *Extended dataset*

### SARS-CoV-2 related question

#### Did the patient receive a vaccination against SARS-CoV-2 during this period?

Indicate if the patient received a vaccination against SARS-CoV-2 in the follow-up period after the HCT treatment took place.

#### Number of doses

Report how many doses of the SARS-CoV-2 vaccine the patient has received.

#### Date of last dose

Report the date on which the patient received their last dose of the SARS-CoV-2 vaccine.

## Secondary Malignancies and Autoimmune Disorders

### Did a secondary malignancy or autoimmune disorder occur?

Answer **No** if neither secondary malignancy nor autoimmune disorder has been observed after this HCT.

Mark as **Unknown** if this information is unavailable. Answer **Yes** if secondary malignancy or autoimmune disorder occurred and specify:

### Was this disease an indication for a subsequent HCT/CT/IST/GT?

If the answer is **No**, complete the respective non-indication diagnosis form.

If the answer is **Yes**, complete the relevant indication diagnosis form.

Mark as **Unknown** if this is not known.

## Additional treatments

### Did the patient receive any additional disease treatment since the last follow-up?

If the patient received additional disease treatment (excluding additional cell infusions) since the last follow-up, select **Yes** and complete the Treatment - non-HCT/CT/GT/IST form. If the patient did not receive additional disease treatment, select **No**. Mark as **Unknown** if this is not known.

Please leave this field empty if such additional disease treatment was reported as part of the HCT follow-up form v.1.

## Additional cell infusions

### Did the patient receive additional cell infusions?

If the patient received additional cell infusions, excluding a new HCT and/or CT treatment, select **Yes** and proceed to the next question. If the patient did not receive additional cell infusions, select **No**.

For this question, it does not matter if it was a boost or DLI. This can be clarified in the next questions.

If answered Yes, the following should be taken into account:

If the cells were infused with the aim of improving chimaerism, or preventing or treating relapses, it most likely was a DLI. The treating physician knows the aim of the infusion. In rare cases, the aim can be as for a boost and a DLI. In this case boost should be selected and the cell infusion sheet for DLI should be completed.

### Is this cell infusion an allogeneic boost?

Indicate whether the cell infusion was an allogeneic boost or not.

An allogeneic boost is an infusion of cells from the same donor without conditioning, with no evidence of graft rejection. A boost is infused with the aim of providing enough hematopoietic cells to have an effect on engraftment.

If cells are not from the same donor OR there is conditioning (chemotherapy and/or TBI), then it is considered to be an HCT and not a boost.

#### *Date of the allogeneic boost*

If applicable, report the date the boost took place.

### Is this cell infusion an autologous boost?

If the cell infusion was an autologous boost answer **Yes**. If it was not an autologous boost, select **No**.

An autologous boost is an infusion of pre-collected and stored autologous stem cells without conditioning.

#### *Date of the autologous boost*

If applicable, report here the date the boost took place.

Note: If this cell infusion is not a boost, attach the Cell Infusion (CI) sheet available in Appendix 6, completing as many sheets as episodes of cell infusion that took place during this follow-up period; then continue with questions below.

### Did the patient receive subsequent HCT/CT?

If the patient received subsequent HCT/CT, either at your or another centre, select **Yes** and make sure that this subsequent treatment is registered using the appropriate HCT/CT form before proceeding.

If the patient did not receive subsequent HCT/CT, select **No**.

## Relapse, Progression, Recurrence of disease or Significant Worsening

### Was there a relapse, progression, recurrence of disease or significant worsening of organ function related to the primary disease after HCT?

Indicate if there was a relapse, progression, recurrence or significant worsening of organ function related to the primary disease after CT detected by any method. If multiple instances of relapse, progression, recurrence of disease or significant worsening took place in this follow-up period, report all instances.

When filling this question in the EBMT registry, click the **add** button as many times as necessary for reporting all instances. ('+ Add' on the EBMT Registry.) If the answer is **No**, proceed to the next section.

### Type

Report if a **relapse or recurrence of disease** is reported, or a **(continuous) progression or significant worsening**.

### Date of relapse/progression/recurrence/significant worsening

Report the date of the relapse/progression/recurrence/significant worsening since HCT. If the date is not known, select **unknown**.

#### Extended dataset

#### In case of relapse or progression

#### Type of relapse (MPN and CML only)

Indicate if there was a relapse related to the primary disease after HCT.

Select from the list the worst disease status detected at this time point:

- Haematological
- Cytogenetic
- Molecular

If the disease status at relapse is not known, select **Unknown**.

#### What was the disease status? (CML only)

If haematological relapse occurred, also indicate the disease status:

- Chronic phase
- Accelerated phase
- Blast crisis

Disease status		
Chronic phase (CP)	Accelerated phase (AP)	Blast crisis (BC)
<ul style="list-style-type: none"> <li>• None of the features of accelerated phase or blast crisis</li> </ul>	<ul style="list-style-type: none"> <li>• Bone marrow or peripheral blood blasts 10%-19%</li> <li>• Peripheral blood basophils <math>\geq 20\%</math></li> </ul>	<ul style="list-style-type: none"> <li>• Bone marrow or peripheral blood blasts <math>\geq 20\%</math></li> <li>• Extramedullary blast proliferation (myeloid)</li> </ul>

	<ul style="list-style-type: none"> <li>• Presence of additional clonal cytogenetic abnormality in Ph+ cells (ACA)<sup>a</sup></li> </ul>	<ul style="list-style-type: none"> <li>• Presence of morphologically apparent lymphoblasts (&gt;5%) warrants consideration of lymphoblastic crisis</li> </ul>
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Table 5. International Consensus Classification (ICC) criteria for Chronic Myeloid Leukaemias.

### Medullary involvement

Indicate if the marrow or blood were affected by the disease. Please be aware that although the vast majority of acute leukaemias involve the invasion of the bone marrow by blasts, there are cases where blast invasion is only found in organs other than the bone marrow (e.g. choromas).

Medullary relapse is when malignant cells are only found in the bone marrow. In case of extramedullary relapse, malignant cells are found in sites other than the bone marrow, such as soft tissues or organs.

### Extramedullary involvement

Extramedullary involvement (EMI) refers to disease cells found in organs or tissue outside the blood or bone marrow. The most common sites of extramedullary disease are the central nervous system (CNS), skin and ovaries/testes.

### Involvement at time of relapse (If the relapse was extramedullary or both medullary and extramedullary)

Report if **Skin**, **CNS** (central nervous system) or **Testes/Ovaries** were involved at time of relapse. Also indicate if any other site was involved. If yes, please specify this in the textfield in English.

## Disease status

### Disease status at this follow-up or at time of death

Indicate the disease status at this follow-up or at time of death corresponding to indication diagnosis by selecting from the list provided in [Appendix 1](#).



## Appendix 1 - Disease specific best response and disease status

The disease status and best response are split into disease specific sections which can be found in appendix 1 of the follow-up form. This section is separated into disease status for:

- Acute leukaemias;
- Chronic leukemias;
- Plasma cell neoplasms;
- MPN, MDS, MDS/MPN overlap syndromes;
- Lymphomas;
- Solid tumours;
- Bone marrow failure syndromes (BMF) including aplastic anaemia (AA);
- Autoimmune disorders;
- Haemoglobinopathies;
- Other diagnoses;
- Inborn errors.

### Acute leukaemias

This section is applicable to acute myeloid leukaemias (AML), precursor lymphoid neoplasms (PLN) and other acute leukaemias.

#### Acute leukaemias disease status or best response

Select the disease status or best response from the following list, consult with criteria described in table5:

- Complete remission (CR);
- Not in complete remission.

If the disease status or best response is not known or was not evaluated, select **unknown** or **not evaluated**, respectively. If reporting the disease status, also indicate the minimal residual disease (MRD) status.

Disease status or best response	
<p><b>Complete remission (CR)</b> is defined as meeting all of the following response criteria for at least four weeks:</p> <ul style="list-style-type: none"> <li>• &lt;5% leukemic blasts in the bone marrow</li> <li>• No blasts with Auer rods (applies to AML only)</li> <li>• No extramedullary disease (e.g., CNS, soft</li> </ul>	<p><b>Not in complete remission:</b> In accordance with the defined criteria for complete remission (CR), a patient would not attain complete remission if they do not fulfil at least one of the complete remission criteria.</p>

tissue disease)	
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Table 6. Acute leukaemias disease status or best response.

### Minimal residual disease (MRD)

Only for disease status section report the MRD status of acute leukaemia according to the guidelines provided [below](#).

## Chronic leukaemias

The chronic leukaemias section is split into chronic myeloid leukaemia (CML) and chronic lymphocytic leukaemia (CLL), prolymphocytic leukaemia (PLL) and other chronic leukaemias.

### Chronic myeloid leukaemia disease status or best response

Select the disease status or best response from the list:

- Chronic phase (CP) and type of remission (haematological, cytogenetic, molecular)
- Accelerated phase
- Blast crisis

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

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

Table 7. International Consensus Classification (ICC) criteria for Chronic Myeloid Leukaemias.

<sup>a</sup>Second Ph, trisomy 8, isochromosome 17q, trisomy 19, complex karyotype, or abnormalities of 3q26.2.

## Number

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

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.

If the disease status or best response was chronic phase (CP) also indicate:

## Haematological remission

If the patient was in Chronic phase (CP), report if haematological remission was achieved (answer Yes), or not achieved (answer No) according to the criteria provided in table 8. 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) according to the criteria provided in table 8. 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.

### *Extended dataset*

#### Cytogenetic detection

Please answer these questions when the patient is in accelerated phase, blast crisis, or chronic phase without cytogenetic remission.

#### t(9;22) positive metaphases

Please report here the percentage (%) of the metaphases with the t(9;22) translocation. If the test was not done, select **not evaluated**. If the value is not known, select **unknown**.

### t(9;22) positive cells detected by FISH

FISH (Fluorescent In Situ Hybridisation) is another cytogenetic technique that is frequently used. Where “conventional” cytogenetic investigation is performed on dividing cells (metaphases), FISH can also analyse non-dividing bone marrow cells (cells in interphase) and investigates about 400 cells, thus many more than the cells that are analysed with “conventional” cytogenetic analysis.

### Molecular remission

If the patient was in Chronic phase (CP), report if molecular remission was achieved (answer Yes), or not achieved (answer No) according to the criteria provided in table 8. 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.

#### *Extended dataset*

### BCR::ABL1 variant allele frequency (VAF)

Please answer this question when the patient is in an accelerated phase, blast crisis, or chronic phase without molecular remission.

Please report the variant allele frequency of BCR::ABL1.

Disease status or best response (only CP)		
Haematological remission	Cytogenetic remission	Molecular remission
<p>Haematological remission is defined by a patient meeting all of the following:</p> <ul style="list-style-type: none"> <li>• WBC &lt; 10 x 10<sup>9</sup> /L</li> <li>• Haemoglobin &gt; 11.0 g/dL</li> <li>• Platelet Count &lt; 450 x 10<sup>9</sup> /L</li> <li>• Normal Differential (&lt;1% precursor cells)</li> <li>• No palpable splenomegaly</li> <li>• No extramedullary disease</li> </ul>	<p>Cytogenetic remission is defined by:</p> <ul style="list-style-type: none"> <li>• 0% t(9;22) positive metaphases together with haematological remission</li> <li>• 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</li> </ul>	<p>Molecular remission is defined by:</p> <ul style="list-style-type: none"> <li>• 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<sup>5</sup> to 10<sup>6</sup> RT-PCR cells. The result should be confirmed by two consecutive tests done at least 4 weeks apart.</li> </ul>

Table 8. Definitions of haematological, cytogenetic and molecular remission for patients in chronic phase.

## Minimal residual disease (MRD)

Only for disease status section report the MRD status of chronic myeloid leukaemia according to the guidelines provided [below](#).

## Chronic lymphocytic leukaemia (CLL), prolymphocytic leukaemia (PLL) and other chronic leukaemias disease status or best response

Select the disease status or best response from the list according to the criteria provided in tables 9-12:

- Complete Remission (CR)
- Partial Remission (PR)
- Progression
- Stable Disease (SD)
  - RelapseRelapse is defined as evidence of disease progression (see table. 9) in a patient who has previously achieved the criteria of a CR or PR see table. 9) for ≥6 months.

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

Group	Parameter	Complete Remission (CR)	Partial Remission (PR)	Stable Disease (SD)	Progressive Disease (PD)
A	Lymph nodes	None $\geq 1.5$ 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	$\geq 11.0$ g/dL (untransfused and without erythropoietin)	$\geq 11$ g/dL or increase $\geq 50\%$ over baseline	Increase $< 11.0$ g/dL or $< 50\%$ over baseline, or decrease $< 2$ g/dL	Decrease of $\geq 2$ 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 9. 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  $< 13$  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 or best response	
<b>Complete Remission (CR)</b>	<p>See table 9 for detailed criteria. All of the criteria have to be met. But:</p> <ul style="list-style-type: none"> <li>• 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.</li> <li>• 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.</li> </ul>
<b>Partial Remission (PR)</b>	<p>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.</p> <p>Clinical (palpation) evaluation only without CT-scan (or alternate imaging), according to routine practice, is accepted.</p>
<b>Stable Disease (SD)</b>	<p>See table 9 for detailed criteria. All of the criteria have to be met.</p> <p>Constitutional symptoms alone do not define PD.</p>
<b>Progression</b>	<p>At least 1 of the criteria of group A or group B has to be met.</p> <p>Sequential imaging is not warranted in CLL outside clinical trials and is not required for the EBMT Registry.</p>

Table 10. Additional clarifications for Chronic lymphocytic leukaemias disease status classification.

Group	Parameter	CR (all met)	PR ( $\geq 2$ in A and $\geq 1$ in B)	SD (all met)	PD ( $\geq 1$ 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 ≥50% in vertical length beyond normal from baseline	Change of -49% to +49% beyond normal from baseline	Increase ≥50% in vertical length beyond normal from baseline
	Constitutional symptoms	None	Any	Any	Any
	Circulating lymphocyte count	<4 × 10 <sup>9</sup> /L	≤30 × 10 <sup>9</sup> /L and decrease ≥50% from baseline	>30 × 10 <sup>9</sup> /L or change of -49% to +49%	Increase ≥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	≥100 × 10 <sup>9</sup> /L	≥100 × 10 <sup>9</sup> /L or increase ≥50% from baseline	Change of -49% to +49%	Decrease of ≥50% from baseline
	Haemoglobin	≥11.0 g/dL (untransfused)	≥11 g/dL or increase ≥50% from baseline	<11.0 g/dL or <50% from baseline, or change <2 g/dL	Decrease of ≥2 g/dL from baseline
	Neutrophils	≥1.5 × 10 <sup>9</sup> /L	≥1.5 × 10 <sup>9</sup> /L or increase ≥50% from baseline	Change of -49% to +49%	Decrease of ≥50% from baseline

Table 11. 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	
<b>Complete Remission (CR)</b>	<p>See table 11 for detailed criteria. All of the criteria have to be met, however a few exceptions are possible:</p> <ul style="list-style-type: none"> <li>• 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.</li> <li>• 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.</li> </ul>
<b>Partial Remission (PR)</b>	<p>See table 11 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 imaging), according to routine practice, is accepted.</p>
<b>Stable Disease (no change, no response/loss of response)</b>	See table 11 for detailed criteria. All of the criteria have to be met.
<b>Relapse (untreated)</b>	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.
<b>Progressive Disease (PD)</b>	<p>At least 1 of the criteria of group A or group B has to be met.</p> <p>Sequential imaging is not warranted in CLL outside clinical trials and is not required for the EBMT Registry.</p> <p>Constitutional symptoms alone do not define PD.</p>

Table 12. Additional clarifications for T-PLL disease status classification.

### Progression sensitivity

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)

Only for disease status section report the MRD status of chronic lymphocytic leukaemia according to the guidelines provided [below](#).



## Plasma cell neoplasms

### Disease status or best response

Select the disease status or best response from the list according to the criteria provided in the tables below (13 and 14):

- 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

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

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

Disease status PCN (except Immunoglobulin-related (AL) Amyloidosis)	
Complete remission (CR)	<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"> <li>• &lt;5% of plasma cells in bone marrow aspirate</li> <li>• Disappearance of any soft tissue plasmacytomas.</li> <li>• No increase in size or number of lytic lesions if assessed (radiographic studies are not mandatory)</li> </ul> <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>

<b>Stringent complete remission (sCR)</b>	<p>All of the following:</p> <ul style="list-style-type: none"> <li>• CR as defined above</li> <li>• normal free light (FLC) chain ratio</li> <li>• Absence of clonal cells in bone marrow by immunohistochemistry or immunofluorescence</li> </ul>
<b>Very good partial remission (VGPR)</b>	<p>One or more of the following:</p> <ul style="list-style-type: none"> <li>• Serum and urine M-protein detectable by immunofixation but not on electrophoresis</li> <li>• <math>\geq 90\%</math> reduction in serum M-protein plus urine M-protein level <math>&lt; 0.1\text{g/ per } 24\text{h}</math></li> </ul> <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>
<b>Partial remission (PR)</b>	<p>All of the following:</p> <ul style="list-style-type: none"> <li>• <math>&gt; 50\%</math> reduction in serum M-protein plus reduction in 24h urinary M-protein by <math>\geq 90\%</math> or to <math>&lt; 0.2\text{g/ per } 24\text{h}</math>.</li> <li>• A reduction of more than 50% in the size of soft tissue plasmacytomas if present at pre-treatment</li> <li>• No increase in size or number of lytic lesions if assessed (radiographic studies are not mandatory)</li> </ul> <p>In the absence of measurable serum and urine M-protein, the following criteria applies:</p> <ul style="list-style-type: none"> <li>• A decrease in the difference between involved and uninvolved free light chain (FLC) of more than 50%</li> </ul> <p>If the FLC assay cannot be measured, the following criteria apply:</p> <ul style="list-style-type: none"> <li>• <math>\geq 50\%</math> reduction in plasma cells provided baseline bone marrow plasma cell percentage was <math>\geq 30\%</math></li> <li>• A reduction of more than 50% in the size of soft tissue plasmacytomas if present at pre-treatment</li> </ul>
<b>Stable disease(no change, no response/loss of response)</b>	<p>Does not meet the criteria for CR, VGPR, PR or progressive disease (includes the old Minimal response (MR) criteria)</p>

<b>Progression</b>	<p>One or more of the following:</p> <ul style="list-style-type: none"> <li>• Increase of 25% or more in measurable monoclonal immunoglobulin in serum and urine (absolute increase must be <math>\geq 0.5</math>g/dL). This is not applicable to light chain myelomas</li> <li>• Increase of 25% or more in urinary light chains (absolute increase must be <math>\geq 0.2</math>g/ per 24h)</li> <li>• An increase of 25% or more in bone marrow plasma cells (absolute % must be <math>\geq 10\%</math>)</li> <li>• Increase of old/appearance of new osteolytic bone lesions on x-ray</li> <li>• Appearance of soft tissue plasmacytoma</li> <li>• Development of hypercalcemia (corrected serum calcium <math>&gt; 11.5</math> mg/dL or <math>2.65</math> mmol/L) that can be attributed solely to the plasma cell disorder</li> </ul> <p>In the absence of measurable serum and urine M-protein, the following criterium applies:</p> <ul style="list-style-type: none"> <li>• An increase of 25% or more in the difference between involved and uninvolved free light chain (absolute increase must be <math>&gt; 0.01</math>g/dL from nadir)</li> </ul>
<b>Relapse</b>	<p>Clinical relapse requires one or more of the following criteria:</p> <ul style="list-style-type: none"> <li>• Direct indicators of increasing disease and/or end organ dysfunction (CRAB features) related to the underlying clonal plasma-cell proliferative disorder.</li> <li>• Development of new soft tissue plasmacytomas or bone lesions</li> <li>• Increase of 50% (and at least 1 cm) in the size of existing plasmacytomas or bone lesions.</li> <li>• Hypercalcemia (<math>&gt; 11.5</math> mg/dL)</li> <li>• Decrease in haemoglobin of <math>&gt; 2</math> g/dL</li> <li>• Rise in serum creatinine by 2 mg/dL or more</li> </ul>

Table 13. Plasma cell neoplasms disease status or best response.

<b>Disease status</b> Immunoglobulin-related (AL) amyloidosis	
<b>Complete remission (CR) 1st, 2nd, 3rd or higher</b>	Normalisation of the free light chain (FLC) levels and ratio, negative serum and urine immunofixation

<b>Stringent complete remission (sCR) 1st, 2nd, 3rd or higher</b>	Not applicable
<b>Very good partial remission (VGPR) 1st, 2nd, 3rd or higher</b>	Reduction in the dFLC (difference between involved FLC and uninvolved FLC) to <40 mg/L
<b>Partial remission (PR) 1st, 2nd, 3rd or higher</b>	A greater than 50% reduction in the dFLC
<b>Stable disease(no change, no response/loss of response)</b>	Less than a PR
<b>Progression</b>	<ul style="list-style-type: none"> <li>• 50% increase in serum M protein to &gt;0.5 g/dL or 50% increase in urine M protein to &gt;200 mg/day (a visible peak must be present)</li> <li>• Free light chain increase of 50% to &gt;100 mg/L</li> </ul>
<b>Relapse 1st, 2nd, 3rd or higher</b>	<ul style="list-style-type: none"> <li>• Any detectable monoclonal protein or abnormal free light chain ratio (light chain must double)</li> <li>• Free light chain increase of 50% to &gt;100 mg/L</li> </ul>
<b>Never treated</b>	No treatment was given.

Table 14 : 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 or responses:

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

- Relapse

Each different status/response 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/responses (eg. in the example above it should not be PR1, CR2, CR3), but within that status/response the sequential count (so PR1, CR1, CR2).

### Extended dataset

## Immunoglobulin-related (AL) amyloidosis

### Organ response

Please see the table below for definitions of organ response and progression.

Organ	Response	Progression
Heart	<ul style="list-style-type: none"> <li>• NT-proBNP response (&gt;30% and &gt;300 ng/L decrease in patients with baseline NT-proBNP <math>\geq</math>650 ng/L) or</li> <li>• NYHA class response (<math>\geq</math>2 class decrease in subjects with baseline NYHA class 3 or 4).</li> </ul>	<ul style="list-style-type: none"> <li>• NT-proBNP progression (&gt;30% and &gt;300 ng/L increase)* or</li> <li>• cTn progression (<math>\geq</math>33% increase) or</li> <li>• ejection fraction progression (<math>\geq</math>10% decrease)</li> </ul>
Kidney	<ul style="list-style-type: none"> <li>• <math>\geq</math>30% decrease (at least 0.5 g/day) of 24-hr urine protein (urine protein must be &gt; 0.5 g/day pretreatment)</li> <li>• Creatinine and creatinine clearance must not worsen by 25% over baseline</li> </ul>	<ul style="list-style-type: none"> <li>• <math>\geq</math>25% decrease in eGFR</li> </ul>
Liver	<ul style="list-style-type: none"> <li>• 50% decrease in abnormal alkaline phosphatase value</li> <li>• Decrease in liver size radiographically at least</li> </ul>	<ul style="list-style-type: none"> <li>• 50% increase of alkaline phosphatase above the lowest value</li> </ul>

	2 cm	
Peripheral nervous system	<ul style="list-style-type: none"> <li>Improvement in electromyogram nerve conduction velocity</li> </ul>	<ul style="list-style-type: none"> <li>Progressive neuropathy by electromyography or nerve conduction velocity</li> </ul>

Table 15. Definitions of organ response and progression.

\* Patients with progressively worsening renal function cannot be scored for NT-proBNP progression.[\(11\)](#)

## Was the patient on dialysis during this follow-up period?

Report whether the patient was on dialysis during this follow-up period. Select **Unknown** if this information is unavailable.

### *Start date*

If the answer to the previous question was **Yes**, report the start date of dialysis. If the start date is not known, select **Unknown**.

### *Did dialysis stop?*

Report whether dialysis was stopped during this follow-up. Select **Unknown** if this information is unavailable.

### *End date*

Report the dialysis end date. If the dialysis stopped but the end date is not known, select **Unknown**.

## Minimal residual disease

Complete this section only if the patient disease status is reported for an AL, CLL diagnosis, or a plasma cell neoplasm.

If the patient is in haematologic CR, but has evidence of disease 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.

Indicate if there was a minimal residual disease by selecting positive, no minimal residual disease by selecting negative, or if the minimal residual disease was not evaluated or unknown.



### Positive minimal residual disease

If there was positive minimal residual disease, indicate if this was **increasing**, **stable** or **decreasing**. If the level is not known, select **unknown**. A change in MRD should be confirmed within 4 weeks, in a second consecutive sample, preferably with a BM sample. The definitions are as follows:

- **Increasing** ( $>1 \log_{10}$  increase between any 2 positive samples measured in the same tissue (PB or BM));
- **Stable** ( $<1 \log_{10}$  between any 2 positive samples measured in the same tissue (PB or BM));
- **Decreasing** ( $>1 \log_{10}$  decrease between any 2 positive samples measured in the same tissue (PB or BM)).

### Date MRD status evaluated

Report the date that the MRD status was evaluated. If the date is not known, select **unknown**.

### Sensitivity of MRD assay

Select the appropriate sensitivity of the MRD assay from the list.

### Method used

Select the most sensitive method that was used to assess the MRD status.

## Myeloproliferative neoplasms (MPN), Myelodysplastic neoplasms (MDS), MDS/MPN overlap syndromes

### Disease status or best response

Select the disease status or best response from the list according to the criteria provided in the corresponding tables 16-18:

- Complete remission (CR).
- Improvement but no CR
- Primary refractory phase (no change)
- Relapse
- Progression/worsening

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

MPN Disease status or best response	
<b>Complete remission (CR)</b>	<p>The 4 following criteria must be true:</p> <ol style="list-style-type: none"> <li>1. Resolution of disease-related symptoms and signs including palpable hepatosplenomegaly</li> <li>2. Haemoglobin (Hb) <math>\geq 10\text{g/dL}</math>, platelet <math>\geq 100 \times 10^9/\text{L}</math> and neutrophils <math>\geq 1 \times 10^9/\text{L}</math></li> <li>3. <math>&lt;2\%</math> immature myeloid cells (<math>&lt;5\%</math> in splenectomized patients)</li> <li>4. Normal bone marrow histology and fibrosis grade no higher than 1</li> </ol>
<b>Improvement but no CR</b>	<p>Requires one criterion in absence of progression:</p> <ol style="list-style-type: none"> <li>1. Hb increase of <math>2\text{g/dL}</math> or transfusion independence</li> <li>2. Spleen reduction of <math>50\%</math></li> <li>3. <math>100\%</math> increase in platelet count and absolute platelet count of at least <math>50 \times 10^9/\text{L}</math></li> <li>4. <math>100\%</math> increase in absolute neutrophil count (ANC) and an ANC of at least <math>0.5 \times 10^9/\text{L}</math></li> </ol>
<b>Primary refractory phase (no change)</b>	Treatment with intent to achieve remission was given, but no remission was achieved.
<b>Relapse</b>	Loss of complete remission.
<b>Progression/Worsening</b>	<p>Requires one of the following:</p> <ol style="list-style-type: none"> <li>1. Progressive splenomegaly</li> <li>2. Leukaemic transformation (increase of peripheral blood blast percentage of at least <math>20\%</math>)</li> </ol>

Table 16. MPN disease status or best response.

MDS Disease status or best response	
<b>Complete remission (CR)</b>	<p>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.</p> <p>For patients with other types of MDS: normalisation of PB counts.</p>
<b>Improvement but no CR</b>	<p><b>1) Haematological response (in patients with cytopenia)</b></p> <ul style="list-style-type: none"> <li>• If haemoglobin &lt; 11g/dl, erythroid response (&gt;11 g/dl);</li> <li>• If platelets &lt;100g/l, platelet response (&gt;100 g/l);</li> <li>• If neutrophils &lt; 1g/l, neutrophil response (&gt;1g/l);</li> <li>• If &gt;0% peripheral blasts, response when 0% peripheral blood blasts;</li> <li>• If transfusion dependant (red blood cells), independence of transfusion achieved (8 weeks without transfusions);</li> <li>• If transfusion dependant (platelets), independence of transfusion achieved (8 weeks without transfusions)</li> </ul> <p><b>2) Marrow blast response (in patients with increased marrow blasts):</b> A decrease of 50% in marrow blasts, but still &gt;5% marrow blasts.</p>
<b>Primary refractory phase (no change)</b>	Treatment with the intent to achieve remission was given, but no remission was achieved.
<b>Relapse</b>	Loss of complete remission.
<b>Progression/ Worsening</b>	More blasts in BM than before treatment.

Table 17. MDS disease status or best response.

MDS/MPN Disease status or best response	
<b>Complete remission (CR)</b>	Marrow blast count < 5% and a normalisation of peripheral blood counts was observed for at least 4 weeks.
<b>Improvement but no CR</b>	Bone marrow blasts decreased by $\geq 50\%$ after pre-treatment but still > 5%. All CR criteria were abnormal before treatment.
<b>Primary refractory phase (no change)</b>	Treatment with intent to achieve remission was given, but no remission was achieved.
<b>Relapse</b>	Loss of complete remission.
<b>Progression/Worsening</b>	Higher blast count in the BM and/or PB than before treatment. Worsening of cytopenias (anaemia and/or thrombocytopenia). Progression from the MD- to the MP-variant of CMML.

Table 18. MDS/MPN disease status or best response.

## Number

If the disease status or best response 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/response 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/responses (eg. in the example above it should not be CR1, 2nd Relapse, CR3), but within that status/response the sequential count (so CR1, 1st Relapse, CR2).

## Lymphomas

### Disease status or best response

Select the disease status or best response from the list according to the criteria provided in table 19:

- Chemorefractory relapse or progression, including primary refractory disease
- Complete remission (CR)

- Partial remission
- Stable disease (no change, no response/loss of response)
- Untreated relapse (from a previous CR) or progression (from a previous PR)

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

Disease status or best response		
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.	

<b>Chemorefractory relapse or progression, including primary refractory disease</b>	Does not present any of the features of any type of remission after treatment.
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Table 19. Lymphomas disease status or best response.

### Complete remission: confirmed

Indicate if the complete remission was **confirmed** or **unconfirmed**. Unconfirmed means a complete response with persistent scan abnormalities of unknown significance. If it is not known if the complete remission was confirmed, select **unknown**.

## Solid tumours

### Disease status or best response

Select the disease status or best response from the list according to the criteria provided in table 20:

- Complete remission (CR)
- First partial remission
- Partial remission (PR)
- Progressive disease
- Relapse
- Stable disease (no change, no response/loss of response)

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

Disease status or best response		
<b>Complete remission (CR)</b>	Disappearance of all target lesions and all non-target lesions and normalisation of tumour marker level.	<b>Unconfirmed</b> complete response with persistent scan abnormalities of unknown significance
		<b>Confirmed</b> CR with No abnormalities detected in scan
		<b>Unknown</b> if it is not known if the complete remission was confirmed, select unknown

Disease status or best response		
<b>First partial remission</b>	The patient achieved a reduction in disease of > 30% or more for the first time ever, but did not achieve complete remission <sup>a</sup>	
<b>Partial remission (PR)</b>	The patient achieved partial remission not for the first time.	
<b>Progressive disease (PD)</b>	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).	
<b>Relapse</b>	Reappearance of disease in patients whose last disease status was complete remission.	<b>Sensitive:</b> patient achieves a reduction of >30% in the disease burden after treatment for this relapse.
		<b>Resistant:</b> patient has not achieved a reduction of more than 30% in the disease burden after treatment for this relapse.
		<b>Unknown:</b> if it is not known if the relapse was resistant or sensitive, select unknown.
<b>Stable disease (no change, no response/loss of response)</b>	<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	

Table 20. Solid tumours disease status or best response.

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

## Bone marrow failures (incl. AA)

### Disease status or best response

Select the disease status or best response from the list according to the criteria provided in table 21:

- Complete remission (CR)
- Partial remission (PR)
- Haematological improvement (HI); NIH partial response
- Stable disease (no change, no response/loss of response)
- Relapse/Progression

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

Disease status or best response	Severe aplastic anaemia	Moderate aplastic anaemia	Genetic BMF
<b>Complete Remission (CR)</b>	<p>All of the following:</p> <ul style="list-style-type: none"> <li>No evidence of clonal evolution, by marrow cytogenetic and flow cytometry</li> <li>Peripheral blood counts: haemoglobin &gt;10 gr/dL, absolute neutrophils &gt;1.0 x 10<sup>9</sup>/L, platelets &gt;100 x 10<sup>9</sup>/L</li> </ul>	<p>All of the following:</p> <ul style="list-style-type: none"> <li>No evidence of clonal evolution, by marrow cytogenetic and flow cytometry</li> <li>Peripheral blood counts: haemoglobin normal for age, absolute neutrophils &gt;1.5 x 10<sup>9</sup>/L, platelets &gt;150 x 10<sup>9</sup>/L</li> </ul>	<p>All of the following:</p> <ul style="list-style-type: none"> <li>Haemoglobin higher than the inferior limit according to age, transfusion independence. For age-related reference values</li> <li>Absolute neutrophils ≥1.5 x 10<sup>9</sup>/L up to age 18 years, ≥1.8 x 10<sup>9</sup>/L, in adults from 18 years</li> <li>Platelets &gt;150 x 10<sup>9</sup>/L, transfusion independence</li> </ul>
<b>Partial Remission (PR):</b>	<p>All of the following:</p> <ul style="list-style-type: none"> <li>No evidence of clonal evolution, by marrow cytogenetic and flow cytometry</li> <li>No longer meeting criteria for diagnosis of SAA</li> <li>Transfusion independence (defined as no need of any PRBC or platelet transfusion)</li> <li>Peripheral blood counts:</li> </ul>	<p>At least one of the following:</p> <ul style="list-style-type: none"> <li>No evidence of clonal evolution, by marrow cytogenetic and flow cytometry</li> <li>Transfusion independence (if previously required) doubling or normalisation of at least one cell line</li> <li>Peripheral blood counts:</li> </ul>	<p>All of the following:</p> <ul style="list-style-type: none"> <li>Haemoglobin ≥8 &lt;10 gr/dL, transfusion independence</li> <li>Absolute neutrophils ≥0.5 &lt;1.0 x 10<sup>9</sup>/L</li> <li>Platelets ≥20 &lt;50 x 10<sup>9</sup>/L, transfusion independence</li> </ul>



	haemoglobin >8 gr/dL, absolute neutrophils >0.5 x 10 <sup>9</sup> /L, platelets >20 x 10 <sup>9</sup> /	haemoglobin >10 gr/dL, absolute neutrophils >1.0 x 10 <sup>9</sup> /L, platelets >100 x 10 <sup>9</sup> /L	
<b>Haematological improvement (HI); NIH partial response:</b>	No longer meeting criteria for diagnosis of SAA, in absence of CR or PR	No longer meeting criteria for diagnosis of MAA or genetic BMF, in absence of CR or PR	One or two of the following: <ul style="list-style-type: none"> <li>● Haemoglobin ≥8 &lt;10 gr/dL, transfusion independence; or</li> <li>● Absolute neutrophils ≥0.5 &lt;1.0 x 10<sup>9</sup>/L; or</li> <li>● Platelets ≥20 &lt;50 x 10<sup>9</sup>/L, transfusion independence</li> </ul>
<b>Stable disease (no change, no response/loss of response)</b>	Patients who have not achieved a CR, PR, HI, relapse or progression will be considered to have a stable disease.		

<b>Relapse / Progression:</b>	<p>Any of the following events after a haematological response (CR or PR):</p> <ul style="list-style-type: none"> <li>• Meeting again the criteria for SAA</li> <li>• Requirement of transfusion support (if not due to independent medical conditions)</li> <li>• Decrease in any of the peripheral blood counts as follows:</li> </ul> <p>Decrease to less than 50% of the medium sustained count during remission if: absolute neutrophils <math>&lt;1.0 \times 10^9/L</math>, platelets <math>&lt;50 \times 10^9/L</math>; or</p> <p>Or in any case if: absolute neutrophils <math>&lt;0.5 \times 10^9/L</math>, platelets <math>&lt;20 \times 10^9/L</math></p> <p>The peripheral blood count decrease must be:</p> <ul style="list-style-type: none"> <li>• Not due to any independent concomitant medical condition</li> <li>• Demonstrated in at least 3 tests over a period of 2 weeks</li> <li>• Not responding to re-introduction of low dose cyclosporin A</li> </ul>	<p>After a haematological response (CR or PR), once again meeting the criteria for MAA</p>	<p>All of the following:</p> <ul style="list-style-type: none"> <li>• Haemoglobin <math>&lt;8 \text{ gr/dL}</math> or transfusion dependence</li> <li>• Absolute neutrophils <math>&lt;0.5 \times 10^9/L</math></li> <li>• Platelets <math>&lt;20 \times 10^9/L</math> or transfusion dependence</li> </ul>
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Table 21. BMF (incl. AA) disease status or best response.

### Did transfusions stop during the follow-up period?

If the disease status is reported for bone marrow failures, indicate if transfusions stopped since the last follow-up. If the patient was never transfusion dependent, select **patient was never transfusion dependent**.

If the transfusions are ongoing, select **no**. If the transfusions did stop, select **Yes** and complete the next questions. If the transfusion status is not known, select **unknown**.

### Did the patient return to transfusion dependency afterwards?

If the patient was transfusion independent after HCT but is back to needing transfusions within this follow-up period, select **yes**. If they continue to be independent of transfusions after stopping in this follow-up period, select **no**. If it is not known if the patient went back to needing transfusions, select **unknown**.

### *First transfusion date*

If the patient stopped transfusions during the follow-up period but went back to being transfusion dependent, report the first transfusion date after the transfusion free period. If the date is not known, select **unknown**.

## Autoimmune disorders

### Disease status or best response

Select the disease status or best response from the list:

- **No evidence of disease** - the patient has achieved complete absence of disease, there are no signs or symptoms of the original disease described.
- **Improved**
- **Unchanged** - Patients who have not demonstrated complete absence of disease, improvement in symptoms, or deterioration of symptoms will be classified as **Unchanged**.
- **Worse**

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

## Haemoglobinopathies

The haemoglobinopathies section is split into thalassemia and sickle cell disease.

### Thalassemia best response

Select the best response that was achieved since the HCT:

- Transfusion independent
- Transfusions required

For clarification, transfusion independence is typically defined as going 8-12 weeks without needing transfusions, without a specific haemoglobin threshold.

If the best response is not known or was not evaluated, select **unknown** or **not evaluated**, respectively.

### Date of last transfusion

If a patient reached transfusion independence, report the date of the last transfusion after main treatment the patient received. If the date is not known, select **unknown**.

### Date of first transfusion

If a patient still requires transfusions, report the date of the first transfusion the patient received after main treatment due to Haemoglobin deficiency (recurrence of disease). If the date is not known, select **Unknown**.

### Thalassemia disease status

#### Patient requires transfusions during follow-up period

Indicate if the patient requires transfusions during follow-up period after haematopoietic recovery by selecting **No** or **Yes**.

### Date of first transfusion

If a patient has returned to transfusion dependence after main treatment, report the date of the first transfusion after main treatment. If the date is not known, select **Unknown**.

### Number of units

Report the number of transfusion units patient received during this follow-up period. If the exact number is not known, select **unknown**.

### Did transfusions stop?

Indicate if the patient stopped receiving the transfusions. If the patient stopped transfusions and did not require more during this follow-up period, select **yes**.

### *Date of last transfusion*

If the patient stopped transfusions during the reporting period, provide the date when the last transfusion was administered. If it is not known, select **unknown**.

### Sickle cell disease best response

Select the best response that was achieved since the main treatment:

- No return of sickling episodes. Patients are considered to have no return of sickling episodes when they have shown an absence of recurrent sickle cell crises.
- Return of sickling episodes. When recurrent sickle cell crises reoccur.

If the best response is not known or was not evaluated, select **unknown** or **not evaluated**, respectively.

### Date of first episode

If a patient has returned to sickling episodes, report the date of the first episode after main treatment. If the date is not known, select **unknown**.

### Sickle cell disease best response

Select the best response that was achieved since the main treatment:

- No return of sickling episodes. Patients are considered to have no return of sickling episodes when they have shown an absence of recurrent sickle cell crises.
- Return of sickling episodes. When recurrent sickle cell crises reoccur.

If the best response is not known or was not evaluated, select **Unknown** or **Not evaluated**, respectively.

### Sickling episodes occur during follow-up period

This should only be completed when reporting the disease status. Indicate if there were no more recurrent sickle cell episodes after HCT by selecting **no**. If recurrent sickling episodes were present after HCT, select **yes**. If it is not known if the sickling episodes returned, select **Unknown**.

### Sickling episodes occur during follow-up period, Yes

Indicate whether the sickling episodes first return after gene therapy or there was an ongoing presence of sickling episodes since last follow-up assessment.

### *Date of first episode*

If the sickling episodes reoccurred for the first time since the main treatment, report the date of the first sickling episode. If the date is not known, select **Unknown**.

### *Number of SCD episodes (during follow-up)*

If the sickling episodes first returned after main treatment, report the number of sickling episodes which occurred during this follow-up period. If the number is not known, select **Unknown**.

## Other diagnosis

### Disease status or best response

Select the disease status or best response from the list:

- **No evidence of disease**- the patient has achieved complete absence of disease, there are no signs or symptoms of the original disease described.
- **Improved**
- **Unchanged**- Patients who have not demonstrated complete absence of disease, improvement in symptoms, or deterioration of symptoms will be classified as **Unchanged**.
- **Worse**

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

### *Extended dataset*

## Inborn errors

### Patient height

Report the patient height within 100 days after HCT in centimetres.

### Patient weight

Report the patient weight within 100 days after HCT in kilograms.

## Patient is attending

Please select whether one of the situation below applies to the patient:

- **Regular school/work:** If the patient attending regular school or work;
- **Alternative school/adapted work:** If the patient attending school or working that is adapted to their inabilities;
- **Patient is not able to attend work/school:** if the patient does not attend school or work.

If the patient is not known whether he/she is attending school or working, please select **Unknown**.

## Immune profiling

Indicate if immune profiling was done.

### Test date

Report the date of the most recent immune reconstitution was tested

## Cell type and test results

Please report the value of the cell types below from the most recent test performed. For Naive CD4 T-cells and CD8 T-cells please select the unit of measurements.

Cell type	Unit of measurement
T-cells (CD3)	Cells/ $\mu$ l
CD4 T-cells (CD4)	Cells/ $\mu$ l
CD4 T-cells (CD8)	Cells/ $\mu$ l
B-cells (CD19)	Cells/ $\mu$ l
NK-cells (CD16/CD56)	Cells/ $\mu$ l
Naive CD4 T-cells (CD4/CD45RA)	Please select the unit of measurement
Naive CD8 T-cells (CD8/CD45RA)	Please select the unit of measurement
IgG	Gram/l
IgA	Gram/l
IgM	Gram/l

## Select the immunomodulatory treatments the patient received within 100 days post HCT

Only report treatments administered within 100 days post HCT for the underlying disease. Do not report treatments for GvHD or other HCT/CT related complications.

### Comorbidities after HCT (Inborn errors of immunity only)

Indicate if the comorbidities de novo, resolved, improved, stabilised or worsened since the last treatment took place.

#### Was the patient admitted to ICU after HCT

Indicate if the patient was admitted to ICU after HCT. If the information is not available mark as **Unknown**.

## Cell Infusion Sheet

The following completion guidelines refer to the completion of appendix 4 of the day 100 form, the cell infusion sheet.

Please report each cell infusion episode performed during the follow-up period in a separate cell infusion sheet, completing as many sheets as episodes of cell infusion that took place. Cell infusion treatment is often given as sequential cell infusions through a series of days or even weeks. In order to make the data comparable, one episode of cell infusion treatment (one "CI") is defined as any number of cell infusions that take place for the same indication within 10 weeks from first to last infusion. If the indication for the treatment changes within the 10 weeks, that would be considered as 2 separate episodes of cell infusion (2 "CI"), with the 2nd episode starting on the 1st day infusions were given after the change in indication.

Do not use this cell infusion sheet for any boost. All boosts shall be registered inside the HCT follow-up form.

### Chronological number of CI episode for this patient

Report the chronological number of this cell infusion episode for this patient.

### Date of the first infusion

Report the date of the first infusion within this episode.

### Number of infusions within this episode (10 weeks)

Report the number of infusions within 10 weeks. Count only infusions that are part of the same regimen and given for the same indication.

### Source of cells

Indicate if the source of cells are **allogeneic** or **autologous**.



## Type of cells

Select the type of cells:

- Lymphocytes (DLI);
- Mesenchymal;
- Fibroblasts;
- Dendritic cells;
- NK cells;
- Regulatory T-cells;
- Gamma/delta cells;
- Virus-specific T-cells.

If the type of cells is **virus specific T-cells**, also specify the virus the T-cells were directed against in the textbox in English.

If the type of cells is not listed, select **Other** and specify the type of cells in the text field in English.

## Disease status at time of this cell infusion

Report the disease status at the time of this cell infusion. If the disease status has not been evaluated, select **Not evaluated**. Select **Unknown** if the disease status at the time of this cell infusion is not known.

## Indication

Select all the indications for this cell infusion episode that apply:

- Planned/protocol;
- Prophylactic;
- Treatment of acute GvHD;
- Treatment of chronic GvHD;
- Treatment PTLD, EBV lymphoma;
- Treatment for primary disease;
- Mixed chimaerism;
- Loss/decreased donor chimaerism;
- Treatment of viral infection other than EBV;
- Poor graft function;
- Infection prophylaxis.

If the indication is not listed, select **Other** and specify it in the text field in English.

## Acute GvHD - maximum grade (*after this infusion episode but before any subsequent cell infusion/HCT/CT*)

Indicate the maximum grade (Grade scale 0 - 4) of acute GvHD. If the grade is unknown but aGvHD is present, select **Present but grade unknown**. The grades are as in question [Maximum observed organ severity score](#) of the main questions.

### Date Acute GvHD onset after cell infusion

Report the aGvHD onset date after the cell infusion. If the date is not known, select **unknown**.

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