EBMT Registry

# Immunosuppressive treatment (IST) day 100 follow-up

## Guide to the completion v2.2 of the EBMT data collection form:

## IST_FU_D100Core_Extended_v2.2

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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.

# Immunosuppressive treatment day 100 follow-up

IST Day 100 form must be submitted to the EBMT Registry database 100 days after an IST episode or at time of patient death, whichever occurs first.

## Date of follow-up

Report the follow-up date which is the closest to the Day 100 follow-up assessment post-IST. If the patient died or 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**.

## Date of the last IST for this patient

Report the start date of the immunosuppressive treatment episode that this follow-up is reported for.

### Main cause of death (if patient died)

Check only one main cause that applies. In case of doubt, consult a physician. If none of the answers in the table match, tick the box **Other cause of death** and specify it. If the cause of death is not known, select **Unknown**.

The following main causes of death can be reported (check only one):

- **Relapse or progression/persistent disease**;
- **Secondary malignancy**;
- **HCT-related** - death caused by complications or infections after transplant (also indicate the treatment related cause)
- **IST-related –** death caused by complications or infections after an immunosuppressive episode (also indicate the treatment related cause).

#### *Select treatment related cause*

In case of IST- or HCT-related cause of death, specify if the cause of death was related to (select all that apply):

- **Graft versus Host Disease;**

If the main cause of death was IST-related, graft versus host disease can not be selected.

- **Non-infectious complication;**
- **Infectious complication;**
- **Other treatment related cause of death; Please specify.**

If the cause of death was related to an infectious complication, select all types of infections that apply:

- **Bacterial infection;**
- **Viral infection;**
- **Fungal infection;**
- **Parasitic infection;**
- **Infection with unknown pathogen.**

*Extended dataset*

### *Was an autopsy performed?*

Check **No**, if no autopsy has been performed. Check **Yes** if an autopsy was performed. Check the box **Unknown** if it is not known whether or not an autopsy was performed.

## Best response

### Best response after this IST

Report the best response achieved within 100 days of the immunosuppressive treatment but before any subsequent treatment, even if the patient got worse again afterwards. The definitions of the best responses for severe aplastic anaemia can be found in table 1. For congenital bone marrow failures, no formal response criteria are available.

| **Response** | **Definition** | |
|---|---|---|
| | **Severe aplastic anaemia** | **Moderate aplastic anaemia** |
| **Stable disease (no change/no response/loss of response)** | Not meeting any of the response criteria defined below | Not meeting criteria of partial or complete response |

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|---|---|---|
| **Complete Remission (CR)** | All of the following:<br>● No evidence of clonal evolution, by marrow cytogenetic and flow cytometry<br>● Peripheral blood counts: haemoglobin >10 gr/dL, absolute neutrophils >1.0 x $10^9$/L, platelets >100 x $10^9$/L | All of the following:<br>● Haemoglobin normal for age<br>● Neutrophils >= 1.5 x $10^9$/L<br>● Platelets >= 150 x $10^9$/L |
| **Partial Remission (PR):** | All of the following:<br>● No longer meeting criteria for diagnosis of SAA<br>● Transfusion independence (defined as no need of any PRBC or platelet transfusion)<br>● Peripheral blood counts: haemoglobin >8 gr/dL, absolute neutrophils >0.5 x $10^9$/L, platelets >20 x $10^9$/L | At least one of the following:<br>● Transfusion independence (if previously required) doubling or normalisation of at least one cell line<br>● Increase above baseline by: 3 g/dl haemoglobin and 0.5 x $10^9$/L neutrophils and 20 x $10^9$/L platelets |
| **Haematological improvement (HI); NIH partial response:** | No longer meeting criteria for diagnosis of SAA | No longer meeting criteria for diagnosis of MAA |
| **Relapse / Progression:** | Any of the following events after a haematological response (CR or PR):<br>● Meeting again the criteria for SAA<br>● Requirement of transfusion support (if not due to independent medical conditions)<br>● Decrease in any of the peripheral blood counts as follows:<br><br>Decrease to less than 50% of the medium sustained count during | After a haematological response (CR or PR), once again meeting the criteria for MAA |

| | | |
|---|---|---|
| | remission if: absolute neutrophils <1.0 x $10^9$/L, platelets <50 x $10^9$/L; or<br><br>Or in any case if: absolute neutrophils <0.5 x $10^9$/L , platelets <20 x $10^9$/L<br><br>The peripheral blood count decrease must be:<br>● Not due to any independent concomitant medical condition<br>● Demonstrated in at least 3 tests over a period of 2 weeks<br>● Not responding to re-introduction of low dose cyclosporin A | |

Table 1. Definitions of best responses.

## 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 is in CR, enter the date CR was achieved or assessed. If the date is unknown, select the **Unknown** checkbox.

### Is the date that the PR was achieved/first observed known?

If a CR was achieved within the d100 period after the IST then please indicate if the date that the PR was achieved is known. If there was a PR observed in the patients blood values prior to achieving the CR and the date is known then please select **Yes**. If it is not known which date the patient reached PR prior to the CR then please select **No**. Please note that, although all patients will reach PR prior to achieving CR, this question asks specifically whether or not this PR was observed/measured in the patients blood values.

### *Date PR achieved/first observed*

If the answer to the previous question is **Yes**, indicate the date that the prior PR was achieved/first observed.

# Transfusions

## Red blood cells (RBC) transfusions given since last IST episode

Indicate if any RBC transfusions were given since the last IST episode by selecting **No** or **Yes**. Answer **Unknown** if it is not known whether or not RBC transfusions were given since the last IST episode.

### RBC

Count and indicate the total number of red blood cells (RBC) units transfused since last IST episode by ticking an appropriate checkbox:

- **< 20 units;**
- **20 - 50 units;**
- **> 50 units.**

Answer **Unknown** if there is no information on RBC transfusions in the patient's medical records.

### RBC irradiated

Indicate if RBC were irradiated (answer **Yes**), not irradiated (answer **No**) or if it is unknown (answer **Unknown**).

## Platelet transfusions given since last IST episode

Indicate if any platelets transfusions were given since the last IST episode by selecting **No** or **Yes**. Answer **Unknown** if it is not known whether or not platelets transfusions were given IST episode.

### Platelets

Count and indicate the total number of platelets transfused since the last IST episode by ticking an appropriate checkbox:

- **< 20 units;**
- **20 - 50 units;**
- **> 50 units.**

Answer **Unknown** if there is no information on platelet transfusions in the patient's medical records.

### Platelets irradiated

Indicate if platelets were irradiated (answer **Yes**), not irradiated (answer **No**) or if it is unknown (answer **Unknown**).

*Extended dataset*

# Haematological tests

## Date tests performed

Report the date when the haematological tests were performed.

### Haemoglobin (g/dL)

Report the haemoglobin level in g/dL. Check the box **Not evaluated** if the haemoglobin level was not assessed. If this result is unavailable, check the box **Unknown**.

### *Was haemoglobin transfused within 4 weeks before assessment?*

Indicate if any RBC transfusions were given within 4 weeks before the Day 100 assessment by selecting **No** or **Yes**. Answer **Unknown** if it is not known whether or not RBC transfusions were given during this follow-up.

### Platelets ($10^9$ cells/L)

Indicate the number of platelets x $10^9$/L or make a corresponding mark if it was **Not evaluated**. If this result is unavailable, check the box **Unknown**.

### *Were platelets transfused within 7 days before assessment?*

Indicate if any platelets transfusions were given within 4 weeks before the Day 100 assessment by selecting **No** or **Yes**. Answer **Unknown** if it is not known whether or not platelets transfusions were given during this follow-up.

### Neutrophils ($10^9$ cells/L)

Indicate the number of neutrophils x $10^9$/L or make a corresponding mark if it was **Not evaluated**. If this result is unavailable, check the box **Unknown**.

### Reticulocytes ($10^9$ cells/L)

Indicate the number of reticulocytes x $10^9$/L or make a corresponding mark if it was **Not evaluated**. If this result is unavailable, check the box **Unknown**.

### Ferritin (ng/mL)

Report the ferritin level in ng/mL. Check the box **Not evaluated** if the ferritin level was not assessed. If this result is unavailable, check the box **Unknown**.

## 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 IST episode. Answer **Yes** if secondary malignancy or autoimmune disorder occurred and specify:

#### Was it a secondary malignancy or autoimmune disorder?

Indicate whether it is a secondary malignancy or autoimmune disorder.

#### Date of diagnosis

Indicate the date of diagnosis. In case the date is not known, report **Unknown**.

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

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

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

## PNH Tests at this Follow-Up

### PNH test done

Report if PNH test was done in the follow up period or not. If it is not known if the PNH test was performed please select **Unknown.**

#### Date of PNH test

If the answer to the previous question is **Yes**, indicate the date of the PNH test. In case the date is not known, report **Unknown**.

## PNH diagnostics by flow cytometry

Blood cells that have been affected by PNH are known as PNH clone cells. Indicate if the clone was absent or present by selecting the respective check box. If it is not known whether or not the PNH clone is absent or present, report **Unknown.**

### Size of PNH clone in percentage

If the **Clone present** option is selected, specify also the **Size of the clone in percentage (%)** (PNH clone size refers to the proportion of PNH-affected cells versus normal cells within the total cell population). This information can be found in the haematology report.

## Flow cytometry assessment done on

Indicate the type of cells used for the PNH test by selecting one of the options from the list: Red blood cells (RBC) or Granulocytes. If both RBC and granulocytes were tested please select **Both.** If flow cytometry was done on other cells, select **Other** and specify the cell type in English.

## Clinical manifestation of PNH

If there are any clinical manifestations of PNH, select **Yes**, if not, select **No**.

Clinical manifestations of PNH include cytopenias, thrombocytopenia, neutropenia or anaemia, thrombotic complications such as the Budd Chiari syndrome (hepatic vein thrombosis) or thromboses in different locations and active haemolysis which may manifest by dark urine, flank pain, elevated LDH. PNH patients may also exhibit cramps of the bowel, oesophagus, or other muscles, as well as erectile dysfunction.

### Date of clinical manifestation of PNH

If the answer to the previous question is Yes, report the date when the clinical manifestation was first reported. In case the date is not known, report **Unknown**.

### Anti-complement treatment given?

Indicate if any anti-complement treatment was given to the patient by selecting either **No** or **Yes**. Some examples of compounds that inhibit the complement system include: C-5 inhibitors (Eculizumab & Ravulizumab) and C-3 inhibitors (Pegcetacoplan). If the anti-complement treatment that has been given is not listed please select: **Other; specify**.

If anti-complement treatment was given, please provide details.

## *Drug*

Select the drug name from the list of options or use the **Other** field and report the generic drug/agent name(s) in the textbox in English.

Please consult the List of Chemotherapy Drugs, Agents and Regimens on the EBMT website for drug/agent names. This document provides alternative names for many of the drugs/agents. Once you have found the drug/agent of interest on the list, add its database name to the table.

## *Start date*

Indicate the date when the treatment was started. In case the date is not known, report **Unknown**.

## *Treatment stopped*

Indicate if anti-complement treatment stopped. In case this information is unavailable, report **Unknown**.

### Stop date

If the answer to the previous question is **Yes**, indicate the date when anti-complement treatment stopped. In case the date is not known, report **Unknown**.

If there were more drugs given during one line of treatment use copies of the page for the paper form. It is also possible to add additional fields in the online EBMT Registry application to report multiple drugs here.