Activity survey 2025

Manual

April 2026, v2

EBMT Registry
EBMT Clinical Research & Registry Department

Contents

Introduction

Welcome to the EBMT Activity Survey for 2025! The Activity Survey serves as a valuable tool for assessing the real picture of HCT, CT, GT and IST treatments in Europe. This survey delivers a dual purpose: offering insights into current trends and providing essential data for counselling, planning, and decision-making.

The dedicated participation of teams allows EBMT to track changes over time and identify factors influencing the field. This survey is invaluable for individual transplant teams, national organizations, healthcare agencies, the industry, and patients whose lives are impacted by these therapies.

This year we have introduced some updates to the survey's content, separating data collection for different types of treatments and age groups. These changes are essential to ensure the survey evolves to accurately capture the full picture of hematopoietic stem cell transplantation and other cellular and gene therapies across Europe, especially as new treatments and their indications become increasingly common. Despite these adjustments, insights and comparisons from previous years remain relevant and valuable. We encourage all transplant teams to continue contributing to this important initiative.

Activity survey 2025 collects data about centre activities from 1 January 2025 till 31 December 2025.

This manual was developed with significant scientific input from Raffaella Greco (EBMT Secretary) and Isabel Sánchez-Ortega (EBMT Chief Medical Officer and Education Director), whose expertise contributed to the accuracy and integrity of its clinical and methodological content.

We would like to acknowledge the contributions of Helga Neidlinger, Franziska Hanke, Gloria Tridello, Asadullah Sharifi, Ignacio García, Nadiia Dyba, Eden Tsehay Abera for their valuable support in reviewing translations and ensuring the accuracy and consistency of the content across languages.

Access and features of the online survey

The EBMT has provided you with a web link that directs you to the digital platform hosting the online survey. By clicking on this link, you will be taken directly to the survey for your site and can start entering the requested information.

Please note the following technical requirements before starting:

- The online survey can only be completed on a computer and is not suitable for smartphones or tablets.
- The survey is optimised for Google Chrome and Microsoft Edge browsers. If you are using another browser, please switch to one of these supported browsers.
- Before entering any data, we strongly recommend that you read the current user manual in full.

User-friendly features

The digital format of this survey offers several user-friendly features to the participants, including the ability to scroll through the form, zoom in or out, and easily navigate through its

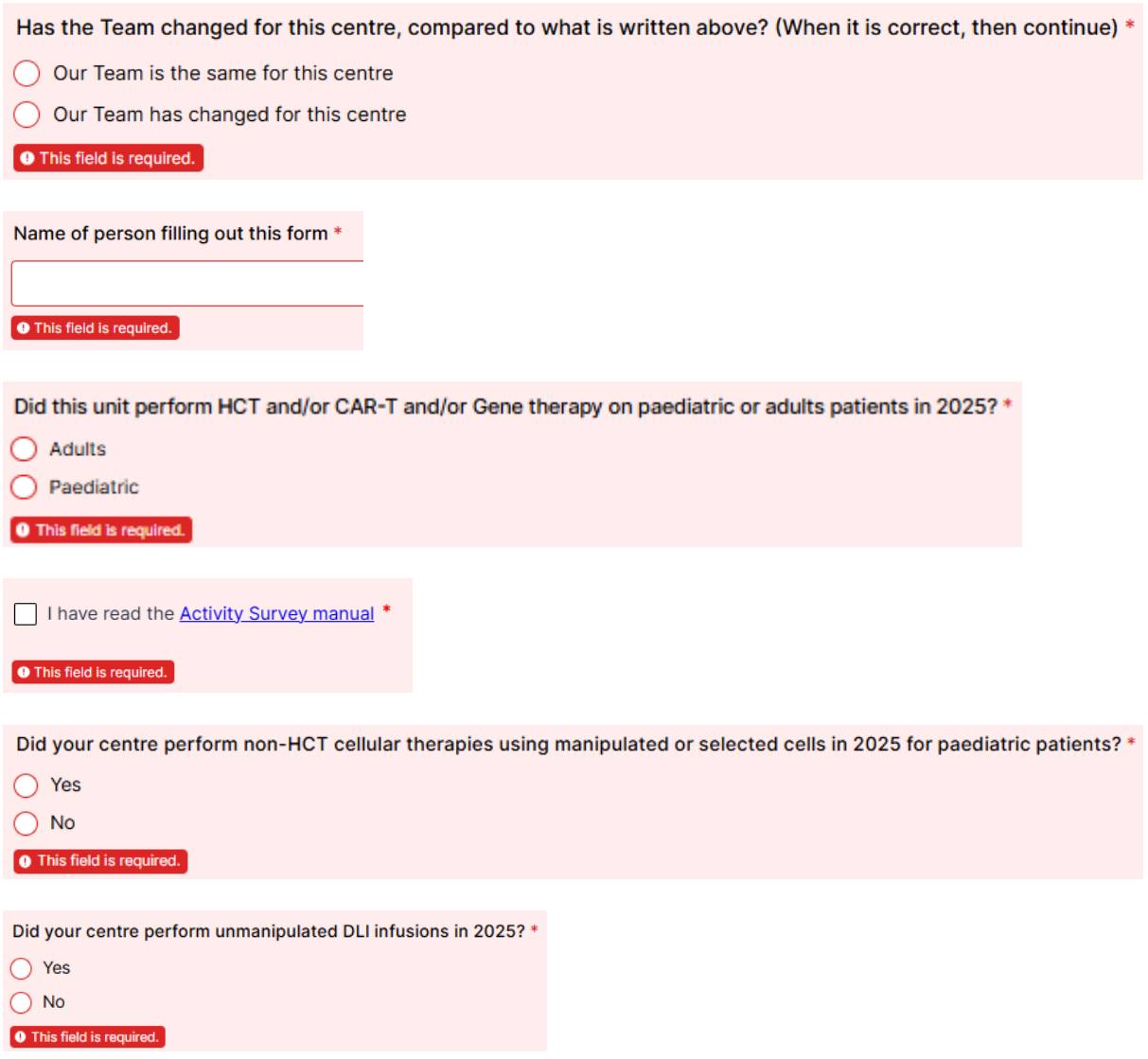
various sections. The survey includes various types of input fields, such as checkboxes, text fields, and dropdown menus. In the case of checkboxes, simply "check" the box if the statement applies to your centre.

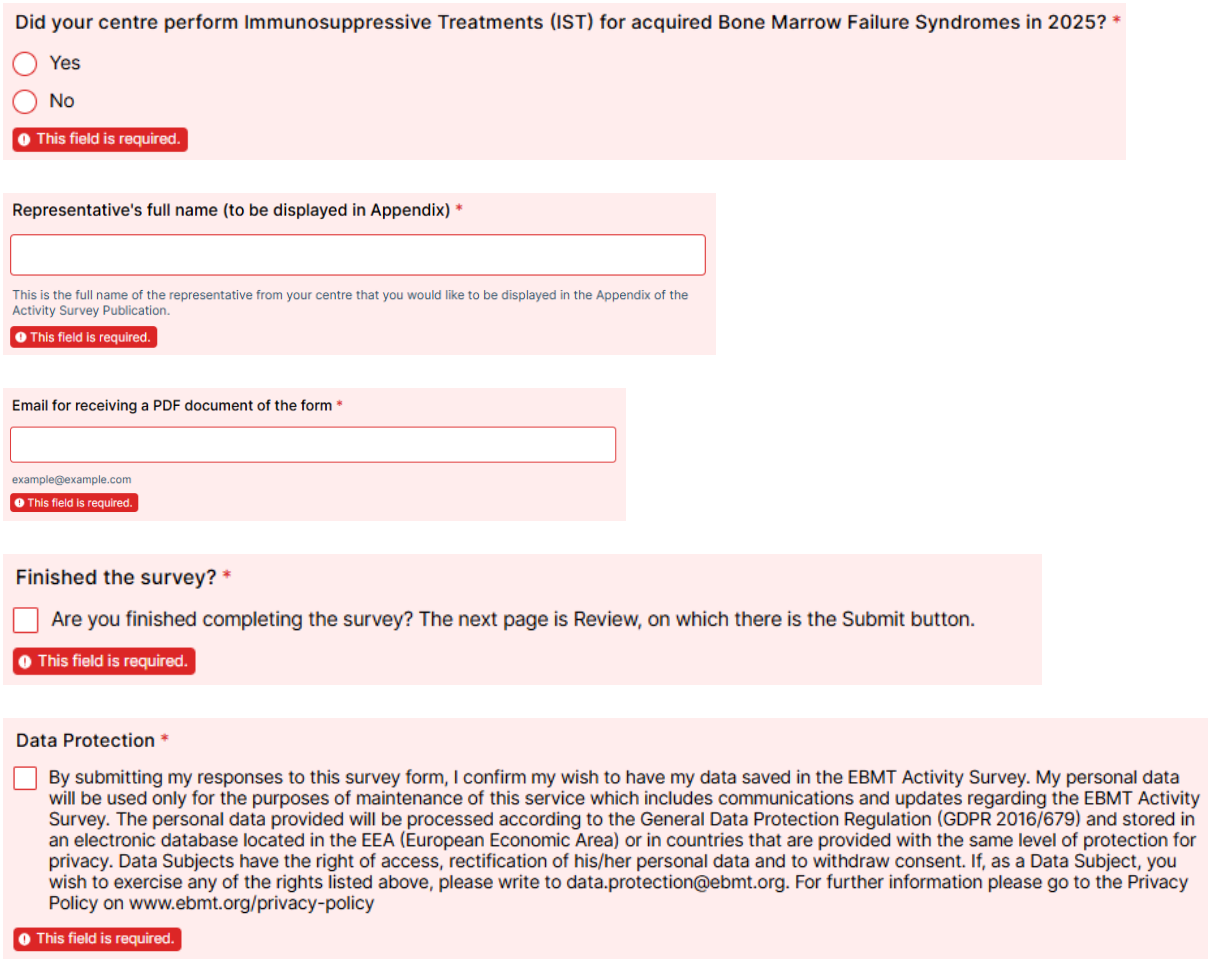
Error messages

The online survey is designed to provide error messages when a participant makes a mistake, helping them correct their input before submission. For instance, if you press “Submit” too soon, an error message will appear if any required fields left empty. However, if all required fields are filled then by pressing “Enter”, the form will be submitted.

The images below show the **mandatory fields** that need to be checked/filled out **before** the submission of the online survey. Mandatory fields can be recognised by the red asterisk (*) that they have at the right end of the statement/question.

Some of those fields, will trigger some parts of the form, depending on your answers. This is to avoid having data entry errors and wrong information reported.



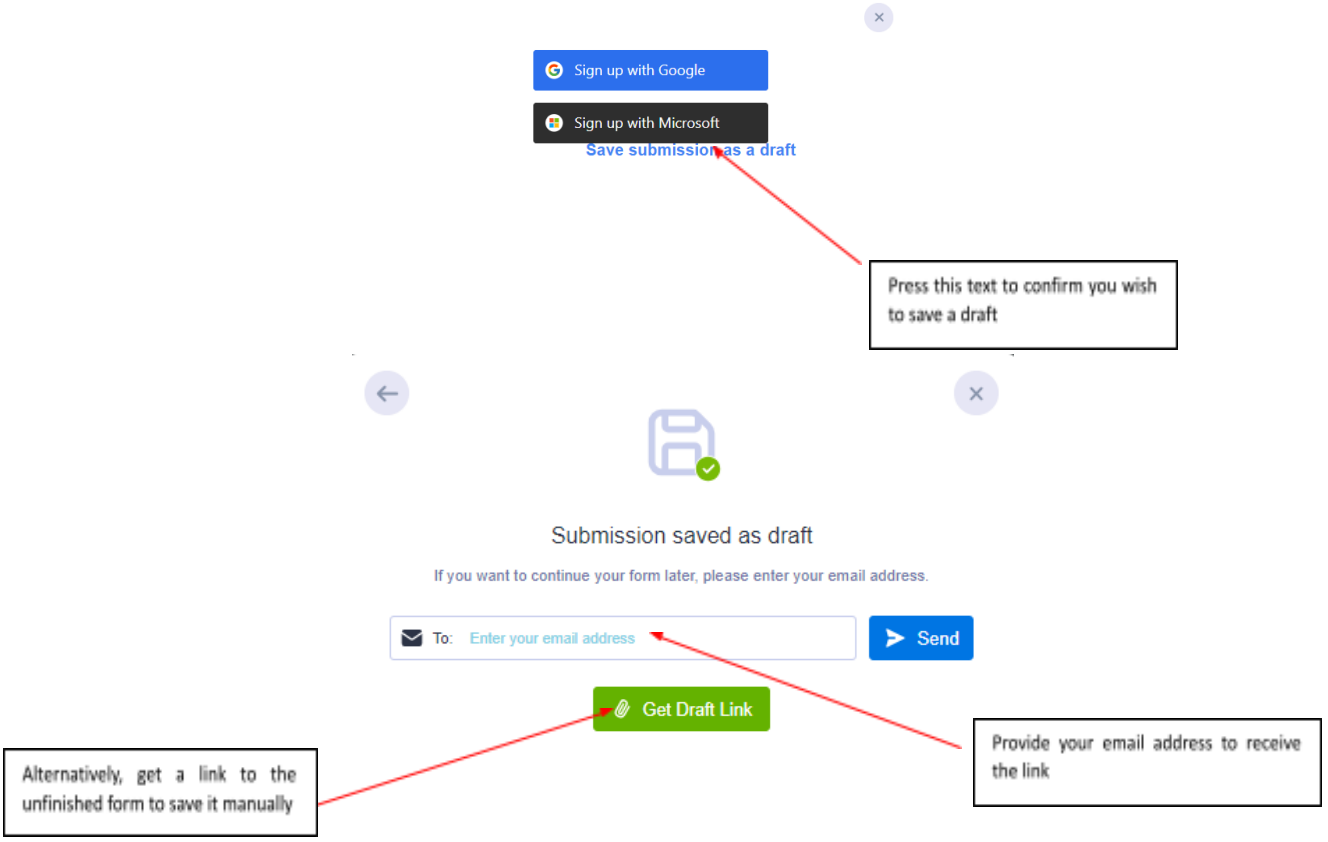


Submission Guidelines

Before submission, please check that all mandatory fields have been completed. This is necessary to successfully submit your response. Please note that only one submission is permitted per centre; multiple submissions from the same centre are not allowed. In the event of a data entry error or a non-intentional submission, please contact us via email at activitysurvey@ebmt.org.

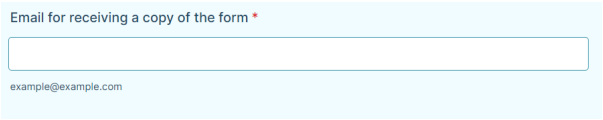
Saving Progress

The online survey also offers the option to save your progress if you don't wish to complete the survey in one session. To do this, click the "Save" button at the end of the form. A window will then appear, asking you to either log in or sign up by creating an account. **It's important to note that logging in or creating an account with Jotform is not mandatory.** You can simply click "Save a submission as draft" and provide your email address. This will allow you to receive a link to the unfinished form. Alternatively, you can select "Get Draft Link." Save this link on your computer, and you can return to it later to complete the survey.

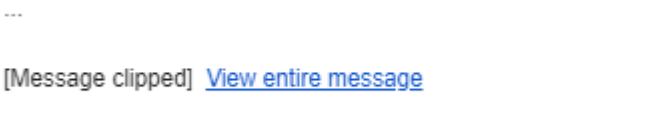


Receive a copy of the submission

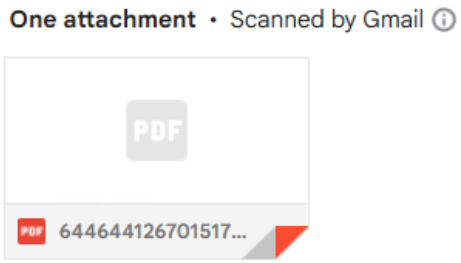
Once you complete the survey and submit it through the online platform you will receive an email with the title “We have received your response for EBMT 2025 ACTIVITY SURVEY ON TRANSPLANT, CELLULAR, AND GENE THERAPY” at the email address you provided in the field shown in the image below (it may take a few minutes for the email to arrive to the specified mailbox).



In this email, you will get a copy of your submission. To view the whole email, please scroll down and click ‘View entire message’.



In the same mail, there will be a **PDF attachment** as shown in the image below:



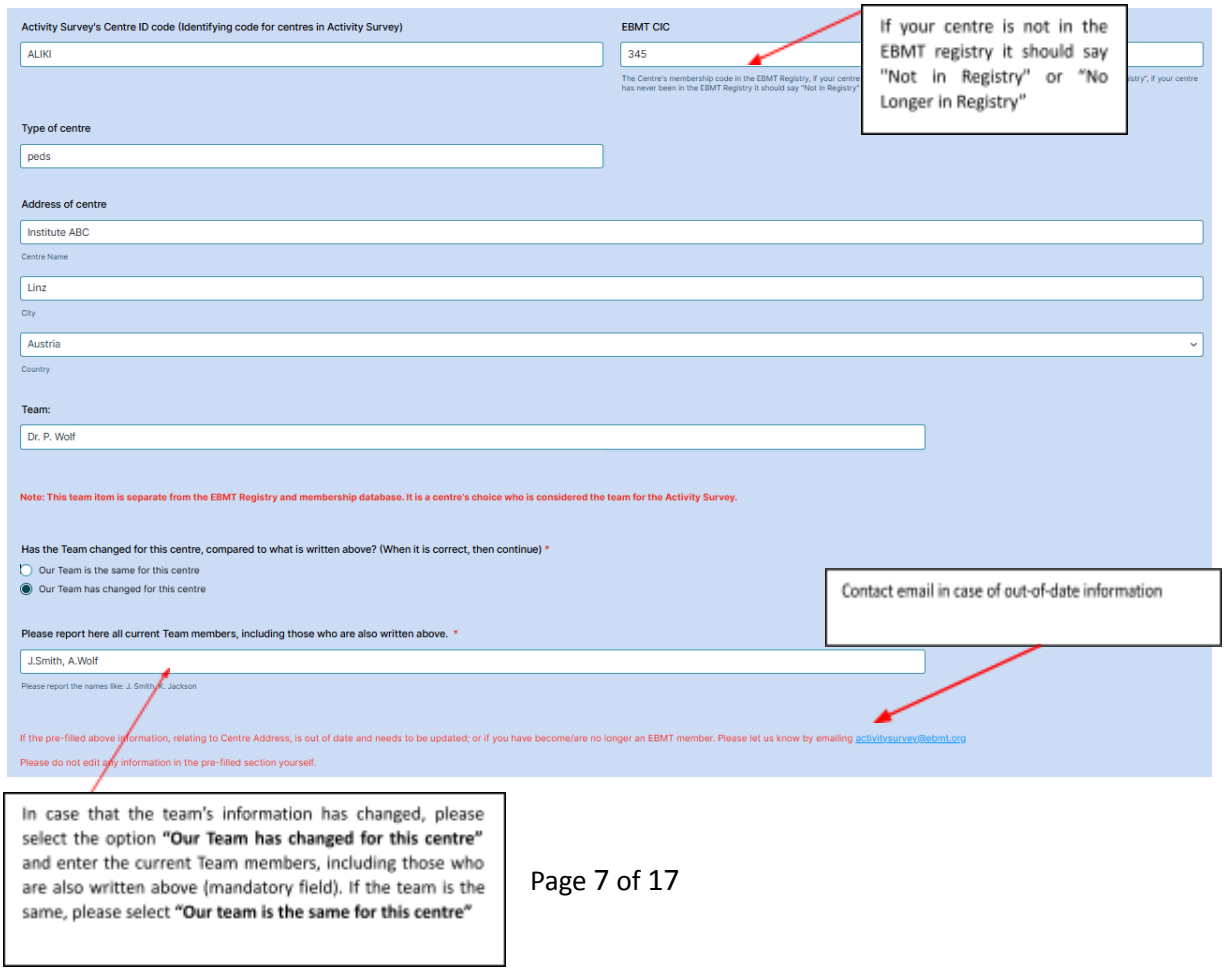
This PDF can be downloaded, saved and printed by the centre. It has all the data entered by the centre in the online form, along with some key information about the centre and the person that filled out the form.

When a centre edits the form, the same mail will be sent again with a PDF containing the most up-to-date data.

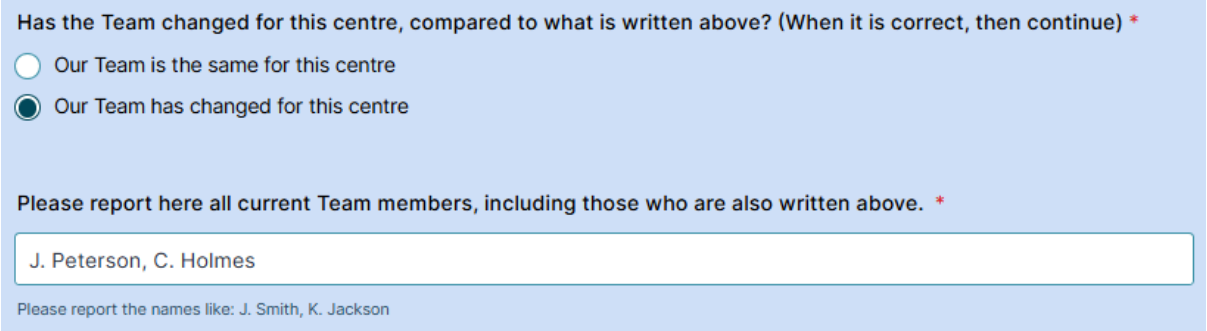
Centre address and team information

The first section of the online survey has been pre-filled by EBMT and contains information about the participant's centre with the most up-to-date centre address and team information that EBMT has.

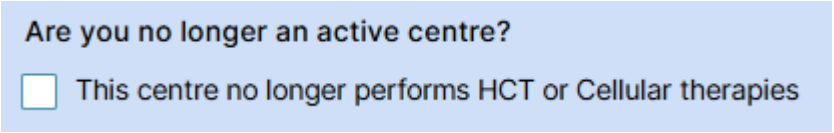
The image below shows an example of this section with pre-filled dummy data. In case the information about your centre is out of date and needs to be updated, please contact us by email at activitysurvey@ebmt.org. Also, if your centre has recently become an EBMT member or if it is no longer an EBMT member, please contact us at activitysurvey@ebmt.org.



If the team of your centre has changed, please check the box “Our Team has changed for this centre”. Then in the white field, please provide the names of the people who are currently in the team (including any names that existed in the team previously).



In case your centre is no longer an active centre, please check the box as shown in the image below “This centre no longer performs HCT or Cellular Therapies”.



Moreover, in case your centre was closed, merged or split with another centre, please let us know by emailing activitysurvey@ebmt.org.

Tables

In the image below, you can see a portion of table 1a. The names of rows and columns are highlighted in light blue. Please use Appendix 1 for abbreviations used. Note that on the left side of the table, the rows are numbered, and this numbering is used in the next section to provide more in-depth details on how to report the data.

The white text fields (or light blue fields) should be filled by the participant using numbers only. If you have zero patients to report, you can leave the field empty. In the grey shaded areas, the sum of each row is automatically calculated. Therefore, there's no need to manually calculate the number of patients yourself.

It is possible to use ‘Tab’ button on your keyboard to go through the row when filling in a particular indication. However, do not use the tab when going from one line to another, this will cause the table to grow and the bottom part of the table will no longer be visible.

Table 1a: Report the number of adult *patients* receiving their 1st allogeneic and/or 1st autologous transplant or non 1st HCT in 2025:

	Indication	HLA-id sibling BM	HLA-id sibling PBSC	HLA-id sibling Cord	Twin BM	Twin PBSC	Haplo and other HLA mismatched related donors BM	Haplo and other HLA mismatched related donors PBSC	Haplo and other HLA mismatched related donors Cord	Unrelated BM	Unrelated PBSC	Unrelated Cord	Auto BM	Auto PBSC	Auto Cord	Total Allo	Total Auto	Total HCT
1	AML 1st CR															0	0	0
2	AML in CR > 1st CR															0	0	0
3	AML not in CR															0	0	0
4	AML therapy - related															0	0	0
5	AML with MDS - related changes															0	0	0
6	ALL 1st CR															0	0	0
7	ALL non 1st CR															0	0	0
	Indication	HLA-id sibling BM	HLA-id sibling PBSC	HLA-id sibling Cord	Twin BM	Twin PBSC	Haplo and other HLA mismatched related donors BM	Haplo and other HLA mismatched related donors PBSC	Haplo and other HLA mismatched related donors Cord	Unrelated BM	Unrelated PBSC	Unrelated Cord	Auto BM	Auto PBSC	Auto Cord	Total Allo	Total Auto	Total HCT
8	CML 1st CP															0	0	0
9	CML non 1st CP															0	0	0

Queries on the survey

After we receive your submission it is possible that will contact you again to update (a part) of your response. This may occur in case we noticed some strange counts in your submission or in case we noticed some inconsistencies with the previous year's submissions. In any case, a link will be send to you along with some indication on what to correct.

Data protection

By submitting your response as a participant to this survey form, you confirm your wish to have your data saved in the EBMT Activity Survey. The participant's personal data will be used only for the purposes of maintenance of this service which includes communications and updates regarding the EBMT Activity Survey.

The personal data provided will be processed according to the General Data Protection Regulation (GDPR 2016/679) and stored in an electronic database located in the EEA (European Economic Area) or in countries that are provided with the same level of protection for privacy.

Data Subjects have the right of access, rectification of his/her personal data and to withdraw consent. If, as a Data Subject, you wish to exercise any of the rights listed above, please write to data.protection@ebmt.org. For further information please go to the Privacy Policy on www.ebmt.org/privacy-policy

Questions and technical support

In our website https://www.ebmt.org/registry/ebmt-transplant-activity-survey, you can find the most important information about the Activity Survey as well as past publications.

In case of any questions or technical issues regarding the online survey, please contact us via email at activitysurvey@ebmt.org. To be able to assist you faster, you can save the link of your unfinished form and forward it to us via email along with your question.

Other ways to participate

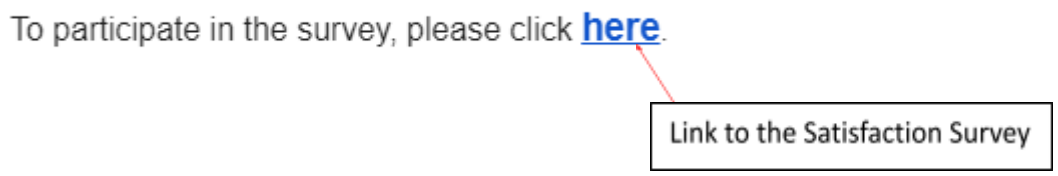
In the unfortunate case that you are unable to fill the online Activity survey for a reason, we would appreciate it if you could submit your centre's activity using the excel or pdf version that we have in our website https://www.ebmt.org/registry/ebmt-transplant-activity-survey. To do so, please follow the next steps:

1. Download the EBMT 2025 Activity survey PDF form that is published on the website.
2. Complete the relevant data fields and total counts at the bottom rows.
3. Name and save your file with your CIC number and/or centre details. If you do not have a CIC number, name the file after the name of your institution.
4. Email the form to activitysurvey@ebmt.org.

Participate in Satisfaction Survey

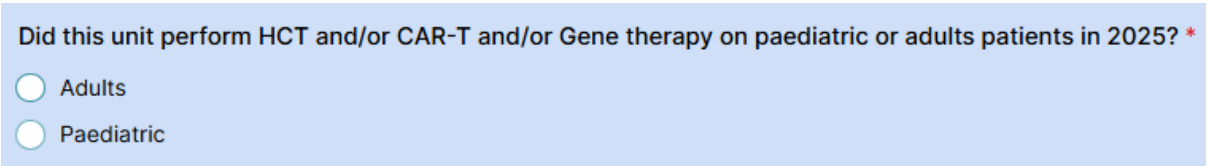
Approximately 7-14 days after the submission of your form, you will receive a mail from Jotform with title "Participate in Satisfaction Survey - EBMT Activity Survey 2025". With this mail we invite you to participate in the Satisfaction Survey. The Satisfaction Survey was launched to assess the experience of the participants with the online platform and to collect feedback regarding the medical content of the survey. It is crucial that you participate in this short survey to help us improve the EBMT Activity Survey.

The link to the online survey will be available in the mail. To access the survey click here:



How to report data

Since 2025, the EBMT provides separate reporting tables for adult and paediatric patients. The appropriate table must be completed according to the population treated. The population treated by the reporting team/unit in the centre should be marked in the question shown on the image below.



For clarification, age alone does not determine form selection. For example, a patient aged 17 years treated in an adult unit should be reported using the adult form, while a patient aged 18 years treated in a paediatric unit should be reported using the paediatric form.

Both forms follow the same structure; however, specific indications for paediatrics and adult populations are included in each form.

Section 1: HCT (autologous and allogeneic)

Includes a table to report the number of patients receiving allogeneic and/or autologous HCT in 2025. One of the following tables is displayed according to the treated population marked at the beginning of the form:

- Table 1a: Report the number of adult *patients* receiving their 1st allogeneic and/or 1st autologous transplant or non 1st HCT in 2025.
- Table 1b: Report the number of *paediatric patients* receiving their 1st allogeneic and/or 1st autologous transplant or non 1st HCT in 2025.

The transplant procedure is considered to start at the initiation of the conditioning regimen. If a patient dies after starting conditioning but before infusion, the procedure should still be reported.

Disease classification follow the WHO classification of tumours:
https://tumourclassification.iarc.who.int/welcome/ and the EBMT disease classification dictionary, https://www.ebmt.org/registry/ebmt-data-collection.

Table 1a for adult patients:

Rows 1–38, report the first allogeneic HCT and/or first autologous HCT for adult patients:

- Report according to disease indication, donor type, and stem cell source.
- The same patient may be reported twice if the first occurrence of each HCT type (allogeneic and autologous) took place in 2025.
- Since all data are collected anonymously, patients without consent for data sharing must still be reported.

Row 39: Total number of adult patients receiving their first allo-HCT or auto-HCT in 2025 (i.e., the total of rows 1–38).
Row 40: Total number of additional transplants (non-first autologous and allogeneic HCT) performed for any reason in 2025 in adult patients, including transplants performed as part of planned multiple transplant protocols.
Row 41: Total number of transplants performed in 2025, including first allo-HCT and first auto-HCT (rows 1–38) plus additional non-first transplants (row 40).

Table 1b: for paediatric patients:

Rows 1–52, report the first allogeneic HCT and/or first autologous HCT for paediatric patients:

- Report according to disease indication, donor type, and stem cell source.
- The same paediatric patient may be reported twice if the first occurrence of each HCT type (allogeneic and autologous) took place in 2025.
- Since all data are collected anonymously, paediatric patients without consent for data sharing must still be reported.

Row 53: Total number of paediatric patients receiving their first allo-HCT or auto-HCT in 2025 (i.e., the total of rows 1–52).
Row 54: Total number of additional transplants (non-first autologous and allogeneic HCT) performed for any reason in 2025, including transplants performed as part of planned multiple transplant protocols.

Row 55: Total number of transplants performed in 2025, including first allo-HCT and first auto-HCT (rows 1–52) plus additional non-first transplants (row 54).

Definitions for tables 1a and 1b

- **First HCT:** Patient who has never previously undergone an HCT.
- **First Allo-HCT:** First allogeneic HCT performed regardless of prior auto-HCT or other cellular therapy.
- **First Auto-HCT:** First autologous HCT performed regardless of prior allo-HCT or other cellular therapy.
- **HLA-identical sibling:** sibling with identical HLA.
- **Haploidentical donor:** One haplotype mismatched family donor.
- **Other HLA-mismatched related donor**: Related donors that are mismatched to a lesser degree than a full haplotype.
- **Unrelated Donor:** Matched or mismatched unrelated donor.

Stem cell source Combination	How to Report
Bone Marrow + Peripheral Blood	Report as PBSC (peripheral blood stem cell transplant)
Bone Marrow + Cord Blood	Report as Cord (cord blood transplant)
Peripheral Blood + Cord Blood	Report as Cord (cord blood transplant)
Bone Marrow + Peripheral Blood + Cord Blood	Report as Cord (cord blood transplant)

Additional Information

- **Pre-planned multiple transplants:** Double or triple allogeneic or autologous HCT, each preceded by its own conditioning regimen, must be classified as **additional transplants** and reported in **row 40 for adult** and in **row 54 for paediatric patients**. Allogeneic haematopoietic stem cells (HSC) administered after a prior allo-HCT for any indication, following conditioning therapy (chemotherapy and/or TBI), should also be classified as a **second transplant**, irrespective of donor type or stem cell source, and reported in **row 40 for adult (table 1a)** and in **row 54 for paediatric patients (table 1b)**.
- **Allogeneic "boost":** Reinfusion of allogeneic HSC from the same donor **without conditioning** and with no evidence of graft failure is considered an **allo boost** and **NOT a transplant**. These cases should be reported under **Graft Enhancement – Other Therapies**, **table 3a** and/or **table 3b**, row 2.
- **Autologous "boost" or "top-up":** Reinfusion of autologous HSC as rescue following graft failure is considered an **auto boost** or **top-up** and **NOT a transplant**. Report these cases under **Graft Enhancement – Other Therapies, table 3a** and/or **table 3b,** row 2.
- **Multiple infusions of the same product:** Multiple infusions of the same stem cell product (e.g., double cord or multiple PBSC infusions) administered over several days **after the same conditioning regimen** are considered a **single transplant**.

- **Concurrent autologous and allogeneic HCT:** Autologous stem cells administered concurrently with an allogeneic transplant within a few days are considered part of a **single allogeneic transplant**.

Section 2: CAR-T and/or Gene Therapies

This section includes a table to report all ex vivo genetically modified therapies for therapeutic purposes: CAR-T, other genetically modified T cells, Autologous HSC Gene Therapy and other gene therapies. One of the following tables is displayed according to the treated population marked at the beginning of the form:

- Table 2a: Report the number of **adult patients** receiving their 1st CAR-T and/or 1st Gene Therapy or non 1st CAR-T/Gene Therapy in 2025:
- Table 2b: Report the number of **paediatric patients** receiving their 1st CAR-T and/or 1st Gene Therapy or non 1st CAR-T/Gene Therapy in 2025:

Definitions for tables 2a and 2b

- **CAR-T cells**: T cells genetically modified in vitro to express a chimeric antigen receptor.
- **Other genetically modified T cells:** Genetically modified T cells other than CAR-T cells.
- **Autologous haematopoietic stem cell gene therapy (autologous HSC Gene Therapy):** Ex vivo genetic modification of autologous CD34+ haematopoietic stem cells, followed by reinfusion after a conditioning regimen.
- **Other gene therapies:** Autologous or allogeneic gene therapies not included in the categories above.

Additional information:

- **Additional indications** have been included in Section 2, including ALL relapse post-HCT, myositis, myasthenia gravis, GVHD, graft failure/enhancement, infection, and other.
- **Virus-specific T cells** should be reported in Section 3 under Other genetically modified T cells with the indication Infection.

Rows 1-45 in the adult table 2a and rows 1-59 in the paediatric table 2b: Report the number of patients receiving their first CAR-T therapy (autologous or allogeneic), other genetically modified T-cell therapies, autologous HSC gene therapy and/or other autologous or allogeneic gene therapies in 2025 at your centre.

- Report by indication and donor type.
- Include patients with or without a prior HCT.
- Include patients enrolled in clinical trials.

Row 48 adult table 2a and row 61 paediatric table 2b: Report the total number of additional (non-first) CAR-T and gene therapies procedures administered for any reason during 2025.

Row 49 adult table 2a and row 62 paediatric table 2b: Total number of CAR-T and gene therapies performed in 2025, including first plus additional therapies (For adults table 2a: (rows1-45 plus row 48, for paediatric table 2b: rows 1-59 plus row 62).

Academic/Investigational. There is a separate data field below the table to indicate the number of **CAR-T** (autologous and allogeneic) and **autologous HSC gene therapies** reported by your centre in 2025 that were **academic or investigational**. The fields are titled as follows:

- How many CAR-T (autologous and allogeneic) and Autologous HSC Gene Therapies reported in your centre in 2025 were academic? (adult patients)
- How many CAR-T (autologous and allogeneic) and Autologous HSC Gene Therapies reported in your centre in 2025 were academic? (paediatric patients)

Academic CAR-T cell therapies refer to CAR-T cell therapies that are developed, manufactured, and clinically tested within academic institutions (such as universities, academic hospitals, or publicly funded research centres) rather than by pharmaceutical or biotech companies.

Section 3: Other Cellular Therapies (excluding HCT, gene therapy and DLI) in 2025

This section includes a table to report other cellular therapies. One of the following tables is displayed according to the treated population marked at the beginning of the form:

- Table 3a: Report the number of adult patients receiving non-HCT cellular therapies or cell infusions in 2025 by indication and cell type
- Table 3b: Report the number of paediatric patients receiving non-HCT cellular therapies or cell infusions in 2025 by indication and cell type

Report here the number of patients receiving other cellular therapies at your centre in 2025, by indication and cell type for which the therapy is given.

- **Exclude HCT (reported in Section1), therapies with genetically modified cells (reported in Section 2) and DLI (reported in Section 4).**
- Include patients with or without prior HCT.
- Patients enrolled in clinical trials should also be reported.

Definitions for tables 3a and 3b**:**

- **Selected/expanded T cells or Cytokine Induced Killer cells (CIK)**: Non-genetically modified T cells that have been selected and expanded in vitro or activated using cytokines or other stimuli.
- **Regulatory T cells (TRegs)**: Subset of $CD4^+$ T cells with immunosuppressive properties, essential for immune homeostasis and self-tolerance.
- **NK cells:** Natural Killer cells, subset of lymphocytes characterized by the expression of CD56 and/or CD16 and the absence of CD3 and part of the innate immune system.
- **Dendritic cells**: Antigen-presenting cells that regulate T-cell mediated immune responses, influence graft rejection, tolerance and GVHD.
- **Mesenchymal stromal cells (MSC):** Multipotent stromal cells with immunomodulatory and regenerative properties.

- **Expanded CD34+ cells:** Ex vivo–cultured haematopoietic stem cells expressing CD34+, expanded to increase cell dose while maintaining engraftment potential.
- **Other therapies:** Allogeneic or autologous boosts and any other cellular therapies not listed above.
    - **Allogeneic "boost":** Reinfusion of allogeneic HSC from the same donor **without conditioning** and with no evidence of graft failure is considered an allo boost and NOT a transplant. These cases should be reported in section 3 under Graft Enhancement – Other Therapies, row 2.
    - **Autologous "boost" or "top-up":** Reinfusion of autologous HSC as rescue following graft failure is considered an auto boost or autologous top-up and NOT a transplant. Report these cases in Section 3 under Graft Enhancement – Other Therapies, row 2.

Section 4: Donor Lymphocyte infusion (DLI):

Report the total number of patients for the selected age group (separately for adults and paediatrics) who received one or more unmanipulated donor lymphocyte infusion (DLI) in 2025, irrespective of the year of HCT.

Centres should specify the primary indication for DLI administration. This section includes one of the following tables (in line with the treated population marked at the beginning of the form) to report the breakdown of DLI in columns 2-5:

- Table 4a: Report the number of **adult patients** receiving their 1st unmanipulated DLI (donor lymphocyte infusion) in 2025.
- Table 4b: Report the number of **paediatric patients** receiving their 1st unmanipulated DLI (donor lymphocyte infusion) in 2025.

If the indication is unknown, answer No to the question 'EBMT would like to collect the number of patients receiving unmanipulated DLI , categorised by graft enhancement/ failure, residual disease, relapse and protocol. Is your centre able to provide the number of unmanipulated DLI with this breakdown?' and report only the total number of patients receiving DLI for the selected age group.

Section 5: Immunosuppressive Treatments (IST) for acquired Bone Marrow Failure Syndromes

Report the number of patients who received immunosuppressive treatment (IST) for acquired bone marrow failure syndromes at your centre in 2025.

Centres should report separately the number of patients treated for aplastic anaemia (AA) and for other acquired bone marrow failure syndromes. This section includes one of the following tables, displayed according to the treated population marked at the beginning of the form:

- Table 5a: Report the number of **adult patients** receiving Immunosuppressive Treatments (IST) for acquired Bone Marrow Failure Syndromes in 2025.
- Table 5b: Report the number of **paediatric patients** receiving Immunosuppressive Treatments (IST) for acquired Bone Marrow Failure Syndromes in 2025.

If the specific indication is unknown, answer No to the question 'EBMT would like to collect the number of patients receiving IST, categorised by Aplastic Anaemia and Other Bone Marrow Failure Syndromes. Is your centre able to provide the number of IST patients with a breakdown by Aplastic Anaemia and Other Bone Marrow Failure Syndromes? ' and report only the total number of patients treated with IST separately for the selected age group.

Appendix 1

Abbreviations used in the Activity survey form:

1. AA – Aplastic Anaemia
2. ALL – Acute Lymphoblastic Leukaemia
3. AML – Acute Myeloid Leukaemia
4. BM – Bone Marrow
5. BMF – Bone Marrow Failure
6. CAR-T – Chimeric Antigen Receptor T-cell Therapy
7. CGD – Chronic Granulomatous Disease
8. CIK – Cytokine-Induced Killer cell
9. CLL – Chronic Lymphocytic Leukaemia
10. CML – Chronic Myeloid Leukaemia
11. CR – Complete Remission
12. CP – Chronic Phase
13. D9L – SAMD9L Mutation Disorder
14. DADA2 – Deficiency of Adenosine Deaminase 2
15. DBA – Diamond-Blackfan Anaemia
16. DLBCL – Diffuse Large B-Cell Lymphoma
17. DLI – Donor Lymphocyte Infusion
18. GVHD – Graft-Versus-Host Disease
19. Haplo – Haploidentical
20. HCT - Hematopoietic Cell Transplantation
21. HLA – Human Leukocyte Antigens
22. HLA-id – Human Leukocyte Antigen identical
23. HLH – Hemophagocytic Lymphohistiocytosis
24. IST - Immunosuppressive Therapy
25. JIA – Juvenile Idiopathic Arthritis

26. JMML – Juvenile Myelomonocytic Leukaemia
27. MDS – Myelodysplastic Syndrome
28. MDS/MPN – Myelodysplastic Syndrome/Myeloproliferative Neoplasm overlap syndrome
29. MLD – Metachromatic Leukodystrophy
30. MM – Multiple Myeloma
31. MPN – Myeloproliferative Neoplasm
32. MPS I – Mucopolysaccharidosis Type I
33. MS – Multiple Sclerosis
34. NHL – Non-Hodgkin Lymphoma
35. NK – Natural Killer cells
36. PBSC – Peripheral Blood Stem Cells
37. PCN – Plasma Cell Neoplasm
38. PLL – Prolymphocytic Leukaemia
39. PNH – Paroxysmal Nocturnal Haemoglobinuria
40. PNH-AA – Paroxysmal Nocturnal Haemoglobinuria Aplastic Anemia
41. SAA – Severe Aplastic Anaemia
42. SAMD9 – SAMD9 Mutation Disorder
43. SCID – Severe Combined Immunodeficiency
44. SDS – Shwachman-Diamond Syndrome
45. SLE – Systemic Lupus Erythematosus
46. SSC – Systemic Sclerosis
47. TBD – Telomere Biology Diseases
48. TREG – Regulatory T-cell
49. WAS – Wiskott-Aldrich Syndrome
50. X-ALD – X-linked Adrenoleukodystrophy