

Non-indication Diagnoses

Guide to the completion v2.3 of the EBMT data

collection form:

Nonindication_Diagnoses_v2.3

September 2026

EBMT Registry

EBMT Clinical Research & Registry Department



**Co-funded by
the European Union**

Co-funded by the European Union. Views and opinions expressed are however those of the author(s) only and do not necessarily reflect those of the European Union or European Health and Digital Executive Agency (HADEA). Neither the European Union nor the granting authority can be held responsible for them.

Table of Contents

Table of Contents.....	2
Introduction	3
Non-indication Diagnosis	3
Date of diagnosis	3
Classification	3
Subclassification.....	3
Acute leukaemia	3
Acute myeloid leukaemias (AML).....	3
AML with myelodysplasia related changes?	3
Was there a previous diagnosis of MDS, MPN or MDS/MPN?	3
Therapy-related myeloid neoplasia (old "secondary acute leukaemia").....	3
Is this a donor cell leukaemia?	4
Autoimmune disorders	4
VEXAS syndrome.....	4
Which form of VEXAS syndrome	4
How was the VEXAS diagnosis made	4
Bone marrow failures	4
Chronic leukaemias.....	4
Haemoglobinopathies	4
Lymphomas.....	4
Myelodysplastic syndrome/myeloproliferative neoplasm (MDS/MPN).....	5
Myelodysplastic neoplasms (MDS).....	5
Therapy-related MDS	5
Is this a donor derived myeloid neoplasm (DDMN)?	5
Myeloproliferative neoplasms (MPNs)	5
Plasma cell neoplasm (PCN)	5
Solid Tumours	5

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.

Non-indication Diagnosis

Date of diagnosis

Enter the date the disease was diagnosed.

Classification

Select the diagnosis classification that is appropriate and check the box next to it.

Subclassification

The subclassification needs to be reported in different levels of detail for different diagnoses. For instructions on selecting the appropriate classification, consult the completion guidelines for the diagnosis you want to enter data for.

Acute leukaemia

Acute leukaemia is a malignant disease that originates either in a lymphopoietic stem cell (Precursor lymphoid neoplasms (PLN), previously ALL) or in a hematopoietic stem cell or progenitor cell (acute myeloid leukaemia, AML);

Acute myeloid leukaemias (AML)

AML with myelodysplasia related changes?

In most cases, this classification applies to AML where an MDS, MPN or an MDS/MPN has been diagnosed beforehand. In a few cases, it applies to what looks as a de novo AML at the time of diagnosis, but which after further analysis of the bone marrow or after treatment for the AML, there is a suggestion that there could have been an undetected history of myelodysplastic syndrome (MDS).

Indicate whether AML with myelodysplasia related changes was diagnosed or not, tick the box Unknown if it is not known. If the answer is Yes, specify also if the patient was previously diagnosed with MDS or MDS/MPN in the subquestion.

Was there a previous diagnosis of MDS, MPN or MDS/MPN?

If the patient had a previous diagnosis of MDS, MPN or MDS/MPN, answer Yes and fill-in and submit respective indication diagnosis form in addition to the current form; otherwise, answer No. If it is unknown whether or not the patient had a previous diagnosis of MDS, MPN or MDS/MPN, check Unknown.

Note: For AML with myelodysplasia-related changes, if MDS, MPN or MDS/MPN was previously diagnosed, besides the Acute leukaemia form, the corresponding indication diagnosis form for MDS, MPN or MDS/MPN must be filled in as well.

Therapy-related myeloid neoplasia (old "secondary acute leukaemia")

Indicate whether therapy-related myeloid neoplasia (therapy-related myeloid neoplasms or t-MN) was diagnosed or not, tick the box Unknown if it is not known.

Therapy-related myeloid neoplasia arises as a late-effect of cytotoxic and/or radiation therapy for a primary diagnosis. Answer Yes if it is related to prior treatment, but not after a previous diagnosis of MDS, MPN or MDS/MPN.

If the patient has therapy-related AML and did not undergo HCT/CT for the previous diagnosis, please complete the non-indication diagnosis form to record the previous diagnosis.

If the patient has AML related to HCT/CT therapy treatment for the previous diagnosis, please complete the appropriate indication diagnosis form for this previous diagnosis.

Is this a donor cell leukaemia?

For Therapy-related myeloid neoplasia, please specify whether this leukaemia is on Donor cell of the previous Allogeneic transplantation, by answering the question “Is this a donor cell leukaemia?”

Autoimmune disorders

Autoimmune disorders (ADs) are disorders where the body’s immune system attacks its own tissue.

VEXAS syndrome

VEXAS syndrome is an adult-onset autoinflammatory disease primarily affecting males, caused by a somatic mutation of the UBA1 gene in hematopoietic progenitor cells. The disease arises in late adulthood (typically after the age of 50) and causes both autoinflammatory and hematologic symptoms.

Which form of VEXAS syndrome

Please indicate the presence or absence of concurrent myelodysplastic neoplasms (MDS) at the time of VEXAS diagnosis or during the disease course.

If VEXAS syndrome occurs with concurrent MDS, please complete the MDS diagnosis form.

How was the VEXAS diagnosis made

Select the option that best describes how the diagnosis of VEXAS syndrome was made. If multiple diagnostic elements contributed, select the option that represents the most definitive method used.

Select Clinical diagnosis only if the diagnosis was made based on clinical presentation and supportive laboratory or imaging findings, without molecular confirmation of a UBA1 mutation and without definitive bone marrow diagnostic features.

Select Confirmed UBA1 mutation (e.g. Met41) if genetic testing confirmed a somatic mutation in the UBA1 gene, such as a mutation affecting Met41 or another pathogenic variant associated with VEXAS syndrome.

Select Bone marrow features (e.g. vacuoles, dysplasia) if the diagnosis was supported primarily by characteristic bone marrow findings, such as cytoplasmic vacuoles in myeloid and erythroid precursor cells, with or without dysplastic features, suggestive of VEXAS syndrome.

If the diagnosis was made using another diagnostic approach not listed above. Provide a brief description of the method used.

Bone marrow failures

Bone marrow failure syndromes are disorders of the hematopoietic stem cells leading to cytopenia that can involve one or more cell lineages. They can be acquired (non-constitutional) or genetic (constitutional).

Chronic leukaemias

Chronic leukaemia is a malignant disease that originates from either the bone marrow, lymphocytes or prolymphocytes. It can be divided into chronic myeloid or chronic lymphocytic leukaemia. For the purposes of reporting anonymous events, prolymphocytic leukaemia can be reported as ‘other’.

Haemoglobinopathies

Haemoglobinopathies are a heterogeneous group of inherited diseases characterised by alteration of haemoglobin production.

Lymphomas

Lymphomas are malignant neoplasms of the lymphatic system, which includes lymph nodes, spleen, thymus, Waldeyer’s ring, appendix, and Peyer’s patches. Lymphomas are divided into two subgroups: Hodgkin lymphoma (HL) and Non-Hodgkin lymphomas (NHL).

Myelodysplastic syndrome/myeloproliferative neoplasm (MDS/MPN)

Myelodysplastic syndrome/myeloproliferative neoplasm (MDS/MPN) are a group of chronic clonal myeloid malignancies in which there are features of both MDS and MPN at the time of presentation. This category was originally composed of the following major myeloid disorders: chronic myelomonocytic leukaemia (CMML), juvenile myelomonocytic leukaemia (JMML), MPN/MDS with ring sideroblasts and atypical chronic myeloid leukaemia (aCML). Myeloid disease that shows features of both MDS and MPN but does not meet the criteria for any of the major MDS/MPN entities is designated as myelodysplastic/myeloproliferative neoplasm, unclassifiable (MDS/MPN-U).

Myelodysplastic neoplasms (MDS)

Myelodysplastic neoplasms (MDS) is a heterogeneous group of clonal haematopoietic stem cell disorders characterised by ineffective, dysplastic haematopoiesis, peripheral cytopenia and a variable rate of progression to acute myelogenous leukaemia (AML).

Therapy-related MDS

Indicate if MDS developed due to medical treatment (therapeutic agents or radiation).

Is this a donor derived myeloid neoplasm (DDMN)?

If therapy-related MDS is Yes, please indicate if it was a donor derived myeloid neoplasm (DDMN). If there was no prior transplant please select Not applicable (no previous allo HCT). If it is not known whether or not it was a donor derived myeloid neoplasm (DDMN), select unknown.

Myeloproliferative neoplasms (MPNs)

Myeloproliferative neoplasms (MPNs) include a group of haematological disorders originating from a pluripotent stem cell of haematopoiesis that typically present with a hypercellular bone marrow with fibrosis, hepatomegaly, splenomegaly, and increased blood cell counts (cytopenias are possible).

Plasma cell neoplasm (PCN)

Plasma cell neoplasms are related to an overproduction of plasma cells in the body and subsequently a possible overproduction of immunoglobulins;

Solid Tumours

Solid Tumours are a group of malignancies presenting with masses internal or external to organs such as breast, ovarian or lung carcinoma.