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Indications for haematopoietic cell transplantation and CAR-T for haematological diseases, solid tumours and immune disorders: 2025 EBMT practice recommendations

R. Greco[1,23]✉, A. Ruggeri[1,23]✉, D. P. McLornan[2], J. A. Snowden[3,4], T. Alexander[5,6], E. Angelucci[7], D. Averbuch[8], A. Bazarbachi[9], M. D. Hazenberg[10,11], K. Kalwak[12], M. Kenyon[13], H. Mekelenkamp[14], B. Neven[15], P. Pedrazzoli[16,17], Z. Peric[18], A. M. Risitano[19], I. Sánchez-Ortega[20], F. Ciceri[1,21] and A. Sureda[22]

For over two decades, the EBMT has updated recommendations on indications for haematopoietic cell transplantation (HCT) practice based on clinical and scientific developments in the field. This is the ninth special EBMT report on indications for HCT for haematological diseases, solid tumours and immune disorders. Our aim is to provide guidance on HCT indications according to prevailing clinical practice in EBMT countries and centres. In order to inform patient decisions, these recommendations must be considered with the risk of the disease, risk of HCT procedure and non-HCT strategies, including evolving cellular therapies, and their availability on site. HCT techniques are constantly evolving and we make no specific recommendations, but encourage harmonisation of practice, where possible, to ensure experience across indications can be meaningfully aggregated via registry outputs. We also recommend working according to JACIE certification standards to maintain quality in clinical and laboratory practice, including benchmarking of survival outcomes [1–3]. Since the last edition, innovative cellular and gene therapies have entered in activity across indications affecting clinical decision making. As the number and type of regulatory authority-approved cellular therapies grow, recommendations for best practice and quality of patient care were developed to support clinicians and will be regularly updated.

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INTRODUCTION

This is the ninth report from the EBMT covering indications for haematopoietic cell transplantation (HCT) according to prevailing clinical practice in EBMT countries and centres. For over two decades, EBMT has considered changes in HCT practice alongside developments in non-transplant treatments. As in previous editions, these 2025 recommendations are based upon clinical trials, registry data, and the opinion of EBMT experts from the board, scientific council and relevant working parties, but not upon a formal extensive or systematic review of the literature. They aim to provide general guidance on transplant indications to inform individual patient decisions by the multidisciplinary team.

They must be considered in conjunction with the risk of the disease status, the likelihood of the successful outcome of HCT, assessment of patient co-morbidities and estimation of non-relapse mortality (NRM) risk alongside the results of non-transplant strategies and their availability on site. Besides potential survival benefits, assessment must include quality of life, patient-reported outcomes and late effects. The recommendations are not intended to be used to choose a particular transplant protocol, conditioning regimen or stem cell source,

[1]Unit of Hematology and Bone Marrow Transplantation, IRCCS San Raffaele Hospital, Vita-Salute San Raffaele University, Milan, Italy. [2]Department of Haematology and Stem Cell Transplantation, University College London Hospitals NHS Trust, London, UK. [3]Division of Clinical Medicine, School of Medicine and Population Health, University of Sheffield, Sheffield, UK. [4]Department of Haematology, Sheffield Teaching Hospitals NHS Foundation Trust, Sheffield, UK. [5]Department of Rheumatology and Clinical Immunology, Charité - Universitätsmedizin Berlin, corporate member of Freie Universität Berlin, Humboldt-Universität zu Berlin, and Berlin Institute of Health, Berlin, Germany. [6]German Rheumatism Research Centre (DRFZ) Berlin, a Leibniz Institute, Berlin, Germany. [7]Hematology and Cellular Therapy Unit, IRCCS Ospedale Policlinico San Martino, Genova, Italy. [8]Faculty of Medicine, Hebrew University of Jerusalem; Hadassah Medical Center, Jerusalem, Israel. [9]Bone Marrow Transplantation Program, Department of Internal Medicine, American University of Beirut, Beirut, Lebanon. [10]Department of Hematology, Amsterdam UMC, University of Amsterdam, Amsterdam, the Netherlands. [11]Department of Hematopoisis, Sanquin, Amsterdam, the Netherlands. [12]Clinical Department of Pediatric BMT, Hematology and Oncology, Wroclaw Medical University, Wroclaw, Poland. [13]Department of Haematology, King's College Hospital NHS Foundation Trust, London, UK. [14]Willem-Alexander Children's Hospital, Department of Pediatrics, Leiden University Medical Center, Leiden, the Netherlands. [15]Pediatric Immune-Hematology Unit, Necker Children Hospital, Assistance Publique Hôpitaux de Paris, Paris, France. [16]Department of Internal Medicine and Medical Therapy, University of Pavia, Pavia, Italy. [17]Comprehensive Cancer Center, Foundation IRCCS Policlinico San Matteo, Pavia, Italy. [18]University Hospital Centre Rijeka, Rijeka, Croatia. [19]Hematology and Hematopoietic Transplant Unit, Azienda Ospedaliera di Rilievo Nazionale San Giuseppe Moscati (A.O.R.N. Giuseppe Moscati), Avellino, Italy. [20]EBMT Executive Office, Barcelona, Spain. [21]Vita-Salute San Raffaele University, Milan, Italy. [22]Clinical Hematology Department, Institut Català d'Oncologia-Hospitalet, Institut d'Investigació Biomèdica de Bellvitge (IDIBELL), University of Barcelona, Barcelona, Spain. [23]These authors contributed equally: R. Greco, A. Ruggeri. ✉email: greco.raffaella@hsr.it; Ruggeri.Annalisa@hsr.it

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but we encourage harmonisation of practice, where possible, to ensure meaningfully aggregated experience across indications via registry outputs.

Over 30 years numbers of HCT continue to rise [4], alongside a marked growth in chimeric antigen receptor (CAR)-T cell therapies in recent years. Despite a 'dip' related to the COVID pandemic, current registry numbers show a complete recovery from the pandemic dip with increased cellular therapy [5, 6].

TRANSPLANT CATEGORISATION, DEFINITIONS AND FACTORS

Haematopoietic cell transplant (HCT)

HCT refers to any procedure where haematopoietic stem cells (HSC) of any donor type and source are given to a recipient with the intention of repopulating and replacing the haematopoietic system in total or in part. HSC for HCT can be derived from bone marrow (BM), peripheral blood (PB) or cord blood (CB) [7].

Donor categories and stem cell sources

Donor type is categorised as autologous, syngeneic and allogeneic, the latter being either related or unrelated. Beyond HLA-matched related (i.e. sibling) donors (MSD), a well-matched unrelated donor (MUD) is defined as a 10/10 identical unrelated donor (UD) based on HLA high-resolution typing for class I (HLA-A, -B, -C) and II (HLA-DRB1, -DQB1). A mismatched unrelated donor (MMUD) refers to an unrelated donor mismatched in at least one antigen or allele at HLA-A, -B, -C, -DR or -DQ and a haploidentical donor refers to one haplotype mismatched family donor. Criteria for UD selection have been proposed [8, 9], but are beyond the scope of these recommendations and incorporation into clinical practice will depend on the effort from donor registries and transplant centres balanced against strategies to incorporate mismatched alternative donors (MMAD), including haploidentical donors, into practice.

In this current version, we continue to combine the recommendations for MMAD, incorporating CB, haploidentical and MMUD into a single category separated from well-matched related and UD. Beyond this general approach, the relative value of the various modalities is described and addressed in more detail below in sections for the relevant indications.

Since the last update, innovative cellular therapies and gene therapies have entered in activity across indications affecting clinical decision making. Increasingly numbers of advanced therapy medicinal products (ATMPs) are available, including CAR-T for haematological cancers [10] and autoimmune diseases (ADs) [11, 12]: gene therapies are available for rare genetic disorders and significant progress has been made in gene therapy for the hemoglobinopathies [13]. The focus is continually shifting from developing these therapies and proving they can work to refining their effectiveness, reducing side effects and expanding their indications [14, 15]. While this guidance does not primarily intend to cover cellular therapies, it makes reference to CAR-T cells and gene therapies as treatment options alongside HCT where appropriate.

CATEGORISATION OF THE TYPE OF INDICATION FOR TRANSPLANT PROCEDURES

EBMT indications are classified into four categories (Supplementary information Table), to describe the levels of evidence and recommendations for types of transplants and different indications. For certain indications, specific haploidentical categorisations are detailed in the footnote (*). Although we broadly categorise according to adult and paediatric (with age cut-offs also influenced by EBMT registry definitions), the indications should be interpreted with flexibility, particularly in the teenage and young adult age group and some 'paediatric' and teenage and young adult indications may occasionally extend into older adult age groups.

Transplant indications in adults

The updated 2025 classification of HCT procedures in adults is shown in Table 1.

Acute myeloid leukaemia (AML)

As per EBMT's last activity survey, AML accounts for 40% of allo-HCT transplants [5]. The decision to perform allo-HCT in AML patients is guided by the risk/benefit ratio that accounts for both risk of relapse and the predicted NRM of the procedure. Genetic risk factors form the basis of modern prognostication for predicting relapse including cytogenetics [16–18], molecular mutations [19, 20], response to initial therapy and post-induction measurable residual disease (MRD) status [21–23]. Several risk scores are available for evaluating the risk of disease recurrence and mortality of allo-HCT. The risk of NRM is usually predicted by the haematopoietic cell transplantation-specific co-morbidity index (HCT-CI) which scores patients' comorbidities [24] while disease-risk index (DRI) predicts the risk of disease relapse independently of comorbidities [25]. Other factors as donor and transplant characteristics should be considered as well and data-mining-based machine-learning algorithms that include all these variables may improve the prediction of day +100 mortality [26].

Overall, allo-HCT in first complete remission (CR1) is recommended for adverse-risk AML and the majority of intermediate-risk AML defined by the European Leukaemia Net (ELN) risk stratification based on cytogenetics and mutational profile of the disease and the Clinical Practice Guidelines in Oncology (NCCN Guidelines®) [7, 16, 27]. On the contrary, allo-HCT is not recommended for favourable-risk AML, unless there is inadequate clearance of MRD [28]. Specifically, allo-HCT in CR1 is recommended for AML patients with adverse cytogenetics [16] and patients with normal cytogenetics with either both wild-type or both mutated NPM1 and FLT3-ITD or those harbouring ASXL1, BCOR, EZH2, RUNX1, SF3B1, SRSF2, STAG2, U2AF1, ZRSF2 or TP53 mutations [16, 28]. However, allo-HCT is not recommended in CR1 AML patients with core-binding factor (CBF) that are associated with translocations t(8;21), inv (16) or t(16;16), NPM1 mutation plus wild-type FLT3-ITD and mutated CEPBA with normal karyotype [16]. In patients over 60 years, allo-HCT is recommended for patients with high-risk and intermediate-risk AML who are fit enough and willing to undergo intensive induction chemotherapy [16]. Allo-HCT remains a potentially curative treatment modality in fit patients with primary refractory AML (failure to achieve a morphological CR after two courses of intensive induction chemotherapy) providing a suitable donor can be identified rapidly and the predicted TRM is acceptable [28]. Allo- HCT may also be effective in patients with relapsed AML [29, 30], especially in those achieving CR2 [31]. However, patients with resistant disease should be considered for clinical trials. MSD or MUD are the preferred donor options in AML patients but the use of PTCy as GVHD prophylaxis in mismatched unrelated and haplo-identical transplants with encouraging results has widened the donor pool [32, 33] and it may change other aspects of allo-HCT, including the intensity of the conditioning as well as the risk of relapse post-transplantation [28, 34]. Although prospective data are not consistent it is generally considered that reduced intensity conditioning (RIC) regimens decrease NRM, while myeloablative conditioning (MAC) reduce the risk of relapse, with the most benefit seen in MRD-positive patients [35–37].

In favourable prognosis AML, low levels of CBF fusion gene transcripts may persist after treatment without impacting survival. However, failure to achieve a 3-log reduction in CBF fusion transcript after two cycles of chemotherapy is associated with a

Table 1. Proposed classification of transplant indications for adults—2025.

Disease	Disease status	MSD allo	MUD allo	MMAD allo	Auto	CAR-T
Haematological Malignancies						
AML[a^]	CR1 (favourable risk and MRD–)[b]	GNR/II	GNR/II	GNR/II	CO/I	
	CR1 (favourable risk and MRD+)[b]	S/II	CO/II	CO/II	GNR/II	
	CR1 (intermediate risk)[b]	S/II	CO/II	CO/II	CO/I	
	CR1 (adverse risk)[b]	S/II	S/II	S/II	GNR/I	
	CR2	S/II	S/II	S/II	CO/II	
	APL Molecular CR2	S/II	CO/II	GNR/III	S/II	
	Relapse or refractory	CO/II	CO/II	CO/II	GNR/III	
ALL[a]	Ph (–), CR1 (standard risk and MRD–)[b]	GNR/II	GNR/II	GNR/III	CO/III	
	Ph (–), CR1 (standard risk and MRD+)[b]	S/II	CO/II	CO/II	GNR/II	CO/II
	Ph (–), CR1 (high risk)[b]	S/II	S/II	CO/II	GNR/III	
	Ph (+), CR1 (MRD–)	S/II	S/II	CO/II	CO/III	
	Ph (+), CR1 (MRD+)	S/II	S/II	S/II	GNR/II	
	CR2	S/II	S/II	S/II	GNR/II	
	Relapse or refractory	CO/II	CO/II	CO/II	GNR/III	
CML	CP1, failing ≥ 3 TKI	S/II	S/II	CO/II	GNR/II	
	Advanced Phase, BP-CML or >CP1	S/II	S/II	S/II	GNR/III	
Myelofibrosis (MF)	Primary MF with an intermediate-2 or high-risk DIPSS or high-risk MIPSS70/MIPSS70-plus score. Secondary MF with an intermediate-2 or high risk MYSEC-PM score [88]	S/II	S/II	S/III	GNR/III	
MDS	Very low and low-risk (IPSS-R or IPSS-M)	GNR/II	GNR/II	GNR/II	GNR/III	
	Intermediate-risk without additional factors[c] (IPSS-R) or moderate-low (IPSS-M)	D/II	D/II	D/II	CO/II	
	Intermediate-risk (IPSS-R) or moderate low (IPSS-M) with additional factors[c]	S/II	S/II	S/II	GNR/III	
	High-, very high-risk (IPSS-R) or moderate-high, high or very high-risk (IPSS-M)	S/II	S/II	S/II		
	sAML in CR1 or CR2	S/II	S/II			
CMML	CPSS-Mol High	S/II	S/II	S/II	GNR/III	
	CPSS-Mol Intermediate-2 with one additional risk factor[d]	S/II	S/II	S/II	GNR/III	
CLL	Poor risk disease refractory or relapsing after treatment with BTKi- and BCL2 inhibitor-based regimens (Richter's transformation excluded)	CO/II	CO/II	CO/III	GNR/III	CO/II
	Richter transformation (clonally related)	S/II	S/II	S/II	GNR/III	CO/II
LBCL	CR1 (intermediate/high IPI at diagnosis)	GNR/III	GNR/III	GNR/III	CO/II	D/II
	Primary refractory disease	GNR/III	GNR/III	GNR/III	GNR/III	S/I
	Early relapse with untested or chemoresistant disease	GNR/III	GNR/III	GNR/III	GNR/I	S/I
	Early chemosensitive relapse ≥CR2	GNR/III	GNR/III	GNR/III	CO/II	S/I
	Late relapse ≥CR2	GNR/III	GNR/III	GNR/III	S/II	CO/II
	Relapse or progressive disease after CAR-T	CO/II	CO/II	CO/II	GNR/III	GNR/III
	Relapse after auto-HCT	CO/II	CO/II	CO/II	GNR/III	S/II
	Primary CNS lymphoma, first-line therapy	GNR/III	GNR/III	GNR/III	S/II	GNR/III
	Primary CNS lymphoma, relapse and progressive disease	GNR/III	GNR/III	GNR/III	CO/II	CO/II
FL	First line, untransformed	GNR/III	GNR/III	GNR/III	GNR/II	GNR/III
	First line chemosensitive, transformed	GNR/III	GNR/III	GNR/III	CO/III	GNR/II
	Chemosensitive relapse ≥2nd line, POD24	CO/III	CO/III	GNR/III	S/II	D/III
	Chemosensitive relapse ≥3rd line, after auto-HCT failure	S/II	S/II	CO/II	GNR/III	S/II
	Refractory relapse	GNR/III	GNR/III	GNR/III	GNR/III	S/II
	After CAR-T failure	CO/II	CO/II	CO/III	GNR/III	D/II

Table 1. continued

Disease	Disease status	MSD allo	MUD allo	MMAD allo	Auto	CAR-T
MCL	CR1	GNR/III	GNR/III	GNR/III	S/I	D/III
	CR/PR > 1, no prior BTKi	CO/III	CO/III	D/III	CO/II	D/III
	CR/PR 2, after 1st-line BTKi	CO/III	CO/III	D/III	D/III	CO/III
	CR/PR > 2, after prior BTKi	CO/II	CO/II	CO/III	GNR/II	S/II
	Refractory	CO/II	CO/II	CO/III	GNR/II	S/II
WM	CR1	GNR/III	GNR/III	GNR/III	GNR/III	GNR/III
	Chemosensitive relapse, ≥CR2	GNR/III	GNR/III	GNR/III	CO/II	GNR/III
	Poor risk, multiply relapse, refractory disease	CO/II	CO/II	D/III	CO/II	GNR/III
PTCL	CR1				CO/II	GNR/III
	Chemosensitive relapse, ≥CR2/PR2	S/II	S/II	CO/III*	CO/II	GNR/III
	Refractory	CO/II	CO/II	CO/III	GNR/II	GNR/III
Primary CTCL	EORTC/ISCL Stages I–IIA (early)	GNR/III	GNR/III	GNR/III	GNR/III	GNR/III
	EORTC/ISCL Stages IIB–IV (advanced)	CO/III	CO/III	D/III**	GNR/III	GNR/III
HL	CR1	GNR/III	GNR/III	GNR/III	GNR/I	GNR/III
	Chemosensitive relapse, no prior auto-HCT	D/III	D/III	GNR/III	S/I	GNR/III
	Chemosensitive relapse, after prior auto-HCT	S/II	S/II	S/II	CO/III	GNR/III
	Refractory	D/II	D/II	D/III	CO/III	GNR/III
MM	Upfront standard risk	CO/II	CO/II	GNR/III	S/I	
	Upfront high risk	CO/III	CO/III	GNR/II	S/I	
	Chemosensitive relapse, prior auto-HCT	CO/II	CO/II	CO/II	S/II	GNR/III
	Refractory/relapse after three lines of prior therapy, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38					S/II
AL		GNR/III	GNR/III	GNR/III	CO/II	
Other Diseases						
Acquired SAA[#] (+/- asymptomatic PNH clone)	Newly diagnosed	S/II	CO/III	GNR/III***	NA	
	Relapsed/refractory	S/II	S/II	CO/II***	NA	
PNH	Classical (haemolysis, ±thrombosis)	GNR/II	GNR/II	GNR/II****	NA	
	AA/PNH syndrome (severe cytopenia)	CO/II	CO/II	CO/III****	NA	
Constitutional BMF syndromes/SAA[e]		S/II	S/II	CO/II*****	NA	
Breast Cancer	Adjuvant high risk, selected population	NA	NA	NA	D/CO/I	NA
	Metastatic	GNR/II	NA	NA	CO/I	D
Germ cell tumours	Second line, high risk	NA	NA	NA	CO/II	NA
	Primary refractory, second and further relapse	NA	NA	NA	S/II	D/II
	First line mediastinal non seminoma	NA	NA	NA	CO/II	D
Ovarian Carcinoma	High risk/recurrent	GNR/II	NA	NA	GNR/I	D/II
Medulloblastoma	Post-surgery, high risk/recurrent disease	NA	NA	NA	CO/III	D/II
Gliomas	Recurrent disease	NA	NA	NA	GNR	D/II
Small cell lung Cancer	Limited	NA	NA	NA	GNR/I	NA
Soft tissue Sarcoma/ Bone sarcoma	Advanced	GNR/III	NA	NA	GNR/D/II	D/II
Ewing Sarcoma	Locally advanced/metastatic	GNR/III	NA	NA	CO/II	D
Renal cell Carcinoma	Refractory to conventional treatments	GNR/II	NA	NA	NA	D
Colorectal Ca, Pancreatic Ca, other selected solid tumours	Refractory to conventional treatments	GNR/III	NA	NA	NA	D/II
Multiple sclerosis	Highly active RR-MS failing DMT	D/III	GNR/III	GNR/III	S/I	D/II
	Progressive MS with AIC, and Aggressive MS[f]	D/III	GNR/III	GNR/III	CO/II	D/II
	Progressive MS without AIC	GNR/III	GNR/III	GNR/III	GNR/III	D/II
Systemic sclerosis		D/III	GNR/III	GNR/III	S/I	D/II
SLE		D/III	GNR/III	GNR/III	CO/II	D/II

Table 1. continued

Disease	Disease status	MSD allo	MUD allo	MMAD allo	Auto	CAR-T
Crohn's disease		D/III	D/III	D/III	CO/II	
Rheumatoid arthritis		D/III	GNR/III	GNR/III	CO/II	D/II
JIA		CO/II	CO/II	CO/III	CO/II	
Monogenic AD		CO/II	CO/II	CO/III	GNR/II	
Vasculitis	ANCA+ve, BD, Takayasu, others	GNR/III	GNR/III	GNR/III	CO/II	
Idiopathic inflammatory myopathy		GNR/III	GNR/III	GNR/III	CO/II	D/II
Autoimmune cytopenias		CO/II	CO/II	CO/III	CO/II	
Neuromyelitis optica		D/III	D/III	D/III	CO/II	D/II
CIDP, MG and SPS		GNR/III	GNR/III	GNR/III	CO/II	D/II
Type 1 diabetes		GNR/III	GNR/III	GNR/III	D/II	
RCD type II		GNR/III	GNR/III	GNR/III	CO/II	
Primary ID		CO/II	CO/II	CO/II	NA	
IEM		CO/III	CO/III	CO/III	NA	
TDT	Absence of iron severe tissue/organ damage	CO/II	CO/II	D/II[##]	NA	
SCD	Severe clinical disease and absence of severe organ/tissue damage	S/II	D/II	CO/II[##]	NA	

This classification does not cover patients for whom a syngeneic donor is available.
AA aplastic anaemia, *AD* autoimmune disorders, *AIC* active inflammatory component, *AL* amyloidosis, *ALL* acute lymphoblastic leukaemia, *Allo* allogeneic transplantation, *AML* acute myeloid leukaemia, *APL* acute promyelocytic leukaemia, *Auto* autologous transplantation, *BF-CML* blast phase CML, *BMF* bone marrow failure, *BTKi* Bruton's tyrosine kinase inhibitor, *Ca* cancer or carcinoma, *CAR-T* chimeric antigen receptor T cells, *CIDP* chronic inflammatory demyelinating polyneuropathy, *CLL* chronic lymphocytic leukaemia, *CML* chronic myelogenous leukaemia, *CMML* chronic myelomonocytic leukaemia, *CO* clinical option (can be carried after careful assessment of risks and benefits), *CP* chronic phase, *CPSS* CMML-specific Prognostic Scoring System, *CR1, 2, 3* first, second, third complete remission, *CTCL* cutaneous T-cell lymphoma, *D* developmental (further trials are needed), DIPSS dynamic international prognostic score system, DMT disease-modifying treatments, FL follicular lymphoma, GNR generally not recommended, HL Hodgkin lymphoma, *HCT* haematopoietic cell transplantation, *IEM* inborn error of metabolism, *ID* immunodeficiency, *IPI* international prognostic index, *IPSS-R* revised International Scoring System, *JIA* juvenile idiopathic arthritis, *LBCL* large B-cell lymphoma, *MCL* mantle cell lymphoma, *MDS* myelodysplastic syndromes, *MG* myasthenia gravis, *MIPSS70 or MIPSS70-plus* Mutation-Enhanced International Prognostic Score Systems, MM multiple myeloma, MMAD mismatched alternative donors (cord blood, haploidentical and mismatched unrelated donors), MP-CMML myeloproliferative CMML, *MRD* measurable residual disease, *MS* multiple sclerosis, *MSD* matched sibling donor, *MUD* well-matched unrelated donor (8/8, 10/10, or 9/10 if mismatched is in DQB1), MYSEC-PM for secondary MF, NA not applicable, PM-DM polymyositis-dermatomyositis, PNH paroxysmal nocturnal haemoglobinuria, PR partial remission, PTCL Peripheral T-cell lymphomas, RA refractory anaemia, *RAEB* refractory anaemia with excess blasts, *RCD* refractory coeliac disease, *RCMD* refractory cytopenia with multilineage dysplasia, *RR-MS* relapsing-remitting multiple sclerosis, *S* standard of care (generally indicated in suitable patients), *Sa* sarcoma, *SAA* severe aplastic anaemia, *sAML* secondary acute myeloid leukaemia, *SCD* sickle cell disease, *SLE* systemic lupus erythematosus, *SPS* stiff person syndrome, *TCL* T-cell lymphoma, *TDT* Transfusion dependent Thalassaemia, *TKI* tyrosine kinase inhibitors, *WM* Waldenström macroglobulinemia.
^Haploidentical allo-HCT for secondary AML: S/II.
*Haploidentical allo-HCT for PTCL and primary CTCL: S/II for chemosensitive relapse, ≥CR2/PR2.
**Haploidentical allo-HCT for primary CTCL: CO for Refractory and EORTC/ISCL Stages IIB–IV (advanced).
***Haploidentical allo-HCT for Acquired SAA (±asymptomatic PNH clone): Newly diagnosed: D/III and Relapsed/refractory: CO/III.
**** Haploidentical allo-HCT for PNH Classical (haemolysis, ±thrombosis): GNR/II and AA/PNH syndrome (severe cytopenia): CO/III.
***** Haploidentical allo-HCT for Constitutional BMF syndromes/SAA: CO/III.
# Here adult patients are defined as aged up to 40–50 years old; in patients >50 yo, allo-HCT should always be considered at the best as CO, even in refractory/relapsed patients.
For thalassaemia and SCD, MMAD indication applies ONLY to haploidentical allo-HCT.
[a]Transplant indications, maintenance therapy after transplant is being increasingly used with the aim of improving survival outcomes (e.g. FLT3 inhibitors in FLT3-ITD AML or TKI in Ph+ALL).
[b]Categories are based on number of white blood cells, cytogenetics and molecular markers at diagnosis and time to achieve remission (see text).
[c]Additional factors include >5% marrow blasts, poor karyotype, profound cytopenias (i.e. Hb <80 g/L, ANC $< 0.8 \times 10^9$/L, platelets $<50 \times 10^9$/L), or severe BM fibrosis.
[d]e.g. extramedullary involvement, significant B symptoms, (hyper)leukocytosis, iron overload, red blood cell transfusion dependence (≥2 per month), platelet transfusion dependence (≥1 per week) splenomegaly, PB or BM blasts ≥10%, as well as adverse cytogenetics and/or gene mutations e.g. *TP53*.
[e]Constitutional SAA includes Fanconi anaemia, dyskeratosis congenita, Blackfan–Diamond anaemia and other inborn bone marrow failure syndromes (see also the section and table for paediatric indications).
[f]Aggressive MS as per Menon et al. Menon S, Shirani A, Zhao Y, et al. Characterising aggressive multiple sclerosis. J Neurol Neurosurg Psychiatry. 2013;84:1192–8.

high risk of relapse and these patients may benefit from allo-HCT [38, 39]. Similarly, younger adults with NPM1-mutated AML who remain MRD-positive after two cycles of chemotherapy have higher relapse rates and seem to benefit from allo-HCT [40, 41]. Although not widely used, auto-HCT may be considered for intermediate-risk AML patients and AML-M3 patients achieving CR2 and MRD negativity [7].

Acute lymphoblastic leukaemia (ALL)

As per EBMT's latest activity survey, ALL is the second most common indication for allo-HCT, accounting for 16% of allo-HCT transplants [5]. While allo-HCT represents the standard of care in Ph+ALL, its role in Ph-ALL has been evaluated in prospective studies considering the availability of a MSD and before the era of systematic MRD monitoring [42]. However, the results of allo-HCT

from unrelated donors are constantly improving including those from non-T-cell-depleted haploidentical donors, as alternatives for transplant-eligible patients lacking a MSD [43, 44]. On the other hand, the evaluation of MRD is today considered as the most important prognostic factor in Ph-B-ALL and most international study groups build the indication for allo-HCT on routine assessment of MRD. This usually refers to adult patients up to 60 years, while there is no consensus regarding older patients [45]. MRD-based indication for allo-HCT requires standardised methodology for MRD detection in an experienced laboratory [45]. Overall, patients with MRD > 0.01% after 3 blocks of standard therapy are considered to have an indication to proceed to allo-HCT [45]. Allo-HCT should also be considered in ALL with high-risk cytogenetics at diagnosis even in MRD-negative patients, at least until additional studies are available [46–48]. Adverse cytogenetics in Ph-ALL include, but, are not limited to, low hypodiploidy, KMT2A (previously MLL) translocations, t(8;14), complex karyotype (≥5 chromosomal abnormalities). With respect to auto-HCT, in the donor versus no donor studies and subsequent meta-analyses, no beneficial effect was observed [49–51]. The question as to whether auto-HCT should be revisited for MRD-negative patients has been discussed but is not routinely considered [52].

In Ph+ ALL treated with imatinib and chemotherapy allo-HCT improves overall survival (OS) and is still indicated in CR1 [53]. However, emerging data show that indications may be restricted in patients treated with third generation TKIs in combination with novel monoclonal antibodies (mAbs) and achieving deep MRD negativity [54, 55]. Allo-HCT is indicated for 'Ph-like' ALL as well, due to the inadequate MRD response but also due to poor outcomes in MRD negative patients as well [56]. Finally, allo-HCT is indicated for relapsed/refractory ALL patients in CR2 or beyond [57] and may rescue a subset of patients with resistant disease responding to novel agents [58].

Regarding novel agents, the treatment paradigm for Ph relapsed/refractory B-ALL has been revolutionised in recent years with the introduction of immunotherapy: a first-in-class bispecific T-cell engager anti-CD19 monoclonal antibody (mAb) blinatumomab and the drug-conjugated anti-CD22-calicheamicin mAb inotuzumab ozogamicin. In addition, anti-CD19 CAR-T cell therapy was approved for patients up to 25 years of age based on the ELIANA study [59], and for adult ALL based on the ZUMA-3 trial [60] and FELIX trial [61]. Most trials of CAR-T cells in relapsed/refractory ALL demonstrate impressive response rates, with >70% of patients achieving CR regardless of cytogenetic background, prior therapies, or age [62]. Prognostic factors associated with higher remission rates and better outcome in adult ALL include lower disease burden (assessed by bone marrow blast count), lower LDH and higher platelet count prior to lymphodepletion [63, 64]. Importantly, conditioning with fludarabine and cyclophosphamide is superior to cyclophosphamide alone [63]. Due to the time delay between detection of relapse and infusion of CAR-T cells, bridging therapy is often necessary, although results may be inferior in patients previously treated with blinatumomab [65] and toxicity may be higher in patients with a previous allo-HCT [64]. Relapse after CAR-T cell therapy occurs in 30–50% of patients (with CD19 negative relapse in up to 40%) [62, 66]. The question of post-CAR-T cell consolidation with allo-HCT is still open and data are controversial. In any case, patients with molecular MRD positivity following CAR-T cell therapy, loss of B-cell aplasia and without a previous HCT are candidates for consolidative allo-HCT post CAR-T cell infusion [66].

It is also recommended to change therapy in patients with persistent or recurrent MRD before proceeding to allo-HCT [45]. Blinatumomab is so far the only agent evaluated in MRD Ph-B-ALL patients which leads to a significant MRD negativity (78%), survival benefit and can effectively serve as bridge to allo-HCT [67, 68]. The choice of conditioning regimen can also affect outcome of allo-HCT, and there is a clear benefit of 12 Gy TBI over chemotherapy-based regimens in terms of reducing relapse in paediatric ALL [69]. Thiotepa and busulfan-based conditioning are an alternative in case of unavailability or intolerance of myeloablative TBI [70, 71].

In contrast to B-ALL, salvage options in T-ALL are limited and consolidation with allo-HCT in CR1 should be considered in high-risk patients. Similarly to B-ALL, MRD is a key predictor of relapse [72]. Other high-risk features include early T-precursor immunophenotype [73] and adverse cytogenetics [74, 75]. Complex cytogenetics are associated with poor outcomes, while NOTCH1 and/or FBXW7 mutations are associated with improved outcomes [72, 75].

Chronic myeloid leukaemia (CML)

Tyrosine kinase inhibitors (TKI) remain the backbone of CML therapy [76, 77], indicated in first line and subsequent lines of therapy. Allo-HCT is not recommended in CML front-line treatment. However, preventing progression to advanced phase CML is the main treatment goal, as outcomes following progression remain poor regardless of therapeutic approach [78]. In this respect, allo-HCT should be indicated in CML patients whilst still in chronic phase 1-CML, according to the following recommendations.

In chronic phase-CML patients failing 1st generation-TKI or 2nd generation-TKI, treatment with ≥3rd generation-TKI is associated with a high progression free survival (PFS) and OS [79, 80]. Allo-HCT is indicated in patients treated with ≥3rd generation-TKI with BCR::ABL1 > 1% (International Scale; IS) or in those with BCR::ABL1 > 0.1% with sustained molecular increase. CML patients carrying high-risk features, such as major route additional chromosomal abnormalities [81] or the BCR::ABL kinase domain mutation T315I, must be closely monitored, and allo-HCT is indicated in case of molecular increase under ≥3rd generation-TKI. Additionally, allo-HCT is recommended in those few chronic phase-CML patients intolerant (i.e. persistent cytopenia) to all available TKI. Donor search should be started in chronic phase-CML patients failing first line TKI with high-risk features, in patients treated with at least two TKIs or after front-line 2nd generation-TKI failure.

De novo advanced phase-CML patients should start treatment with 2nd generation-TKI, or ≥3rd generation-TKI if available; and in this setting, allo-HCT should be considered in de novo advanced phase-CML patients failing front-line TKI. Progression to advanced phase-CML from chronic phase-CML should prompt allo-HCT. Donor search should be commenced as soon as signs of progression are observed, if not started before in chronic phase-CML. HLA-typing should be started in patients diagnosed with de novo or progression to blast phase. These patients can be treated with TKI± intensive chemotherapy adapted to the immunophenotype, and undergo allo-HCT in chronic phase 2, without the need to achieve cytogenetic response [82].

Retrospective data suggest that allo-HCT from MMAD in the PTCy setting is a feasible option [83, 84]; hence, a lack of MSD or MUD is not a contraindication to allo-HCT. BM and PB are both valid options as graft source in chronic phase-CML. In advanced phase CML, PB remains the preferred graft source.

Myeloproliferative neoplasms (MPNs)

Allo-HCT remains the only curative option for patients with MPNs. However, polycythaemia vera and essential thrombocythaemia are not allo-HCT indications unless disease transformation to secondary myelofibrosis (MF) or MDS/leukaemia has occurred [85–87]. Recently, EBMT/ELN guidelines for allo-HCT in MF have been published [88]. Here, eligible patients with primary MF and an intermediate-2 or high-risk Dynamic International Prognostic Scoring System (DIPSS) score, or a high-risk Mutation-Enhanced International Prognostic Score Systems (MIPSS70 or MIPSS70-plus) score, or MYSEC-PM high/intermediate-2 risk (for secondary MF), and a low-risk or intermediate-risk Myelofibrosis Transplant

Scoring System (MTSS) score should be considered candidates for allo-HCT [88, 89]. Ideally, candidates for allo-HCT with splenomegaly >5 cm below the left costal margin should receive spleen-directed therapy prior to allo-HCT, ideally with a JAK inhibitor [88, 90]. As regard younger patients with intermediate risk-1 disease (DIPSS) or intermediate disease (MIPSS70 or MIPSS70-plus) and low MTSS score, allo-HCT could be an option balancing patient preference, donor type, therapeutic options and other risk factors (including multi-hit *TP53* mutations) [88]. Experience of allo-HCT in patients with low-risk index disease is limited and remains controversial. Recent EBMT data suggest no disadvantage with pre-transplant exposure to ruxolitinib, and better outcomes after transplant in ruxolitinib-responsive patients compared with patients with no response or loss of response to ruxolitinib [91]. The effect of other JAK inhibitors on allo-HCT outcome, such as fedratinib and momelotinib remains as yet undetermined. Auto-HCT for MPN is not recommended.

Myelodysplastic syndromes (MDS)

Allo-HCT remains the only curative option for patients with higher-risk MDS, but patient-related factors, especially age and co-morbidities, often impact upon feasibility of HCT. The EBMT proposed a transplant-specific risk score for MDS patients [92], which, alongside HCT-CI, may be used to judge the feasibility of allo-HCT and predict outcomes. In addition, disease characteristics that impact the risk of transformation into AML and survival need to be considered.

Both IPSS and the revised IPSS (IPSS-R) have been used to stratify patients and propose transplantation for higher risk eligible patients based on prospective studies [93–97]. Higher risk includes both 'high' and 'very high risk' according to IPSS-R and 'intermediate-2' and 'high risk' according to IPSS. Of note, some lower risk cases have also been included in these prospective studies where they represent a minority of the population. The benefit of transplantation in low risk MDS, including patients who are heavily transfused, has not been proven by prospective trials (in contrast to higher risk patients). More recently, a large international study has utilised IPSS-M to stratify patients and determine potential benefits of transplantation [98]. This study confirmed that eligible patients in the 'moderate-high', 'high' and 'very high-risk' categories benefited from immediate transplantation while other lower risk categories did not (but may benefit for a delayed transplantation approach) which remains in line with previous EBMT and other international recommendations [99]. Of note, some lower risk patients may acquire a transplantation indication when progressing or when presenting with specific high-risk features (transfusion refractoriness, marrow fibrosis, acquisition of poor prognostic somatic mutations). Pre-transplantation therapy has not been proven by prospective trial to be required before transplantation. A recent EBMT study demonstrated that down staging IPSS-R with pre-HCT therapy does not convert into an improved outcome [100]. However, in patients who cannot be transplanted upfront due to logistical issues, hypomethylating agents are usually used (alone or in combination) to prevent disease progression. Marrow blast persistence that is stable over time is not a contraindication for transplantation.

Chronic myelomonocytic leukaemia (CMML)

Allo-HCT represents the only potentially curative treatment option in CMML but is applicable to only a minority of patients. High rates of relapse (30–50%) and NRM (>20%) remain the main reasons for allo-HCT failure historically and careful patient selection is mandated [101]. The EBMT recently generated contemporary guidance focused on the allo-HCT decision in CMML [101]. Recent ICC and WHO classification revisions removed the CMML-0 classification whilst maintaining CMML-1 (<5% blasts in peripheral blood and <10% in bone marrow) and CMML-2 (5–19% blasts in peripheral blood and 10-19% in bone marrow and lowered the threshold to a persistent absolute ($\geq 0.5 \times 10^9$/L) and relative (≥10%) peripheral blood monocytosis. There are two variants, 'dysplastic' and 'proliferative', dependent on the circulating leucocyte count ($\leq 13 \times 10^9$/L and $>13 \times 10^9$/L, respectively). For patient risk stratification, the use of a CMML-specific Prognostic Scoring System (CPSS) is recommended, ideally one including molecular information such as the CPSS-molecular (CPSS-Mol) [102]. Regarding CPSS-Mol high-risk disease, eligible patients with a suitable donor should proceed directly to allo-HCT [102]. For intermediate-2 risk CPSS-Mol, those with ≥1 additional risk factor (e.g. extramedullary disease, splenomegaly, hyperleukocytosis) should also proceed to upfront transplant [102]. Patients with low and intermediate-1 CPSS-Mol disease should be dynamically monitored closely but not routinely undergo allo-HCT [102]. For those with an allo-HCT indication, upfront allo-HCT is preferred rather than undergoing pre-transplant therapy unless delay in donor availability or in the presence of aggressive disease with rapid kinetics [102]. In general, hypomethylating agent-based regimens are preferred if pre-transplant therapy is required. However, available data for this indication are still limited.

Chronic lymphocytic leukaemia (CLL)

The widespread availability of signalling pathway inhibitors, such as the Bruton's TKIs, ibrutinib and acalabrutinib, the BCL2-inhibitor venetoclax, and phosphatidylinositol-3-kinase inhibitors has significantly changed management algorithms and HCT indications across all lines of therapy in CLL. These drugs are frequently used either alone or in combination based on disease risk stratification, licensing and reimbursement status as per each country. Of note, the combination of ibrutinib and venetoclax in the Phase III FLAIR (ISRCTN01844152) trial was associated with improved PFS and OS in untreated CLL patients compared to combination chemoimmunotherapy, with duration guided by MRD response [103]. Eligible patients with relapsed/ refractory CLL who have exhausted their main pharmacological therapeutic options (including sequencing of both covalent and/ or non-covalent Bruton's TKIs and venetoclax-based regimens) may be considered for cellular therapies, including allo-HCT or CAR-T cells, or clinical trials. Such truly 'double-refractory patients' have an estimated survival of 4-6 months without intervention [104]. Cellular and molecular therapies are not mutually exclusive and could be used synergistically to exploit their full potential. Patients with CLL and a concomitant MDS and those with clonally related aggressive transformation of CLL should be considered for allo-HCT regardless of the treatment stage of their CLL [105]. Auto-HCT is generally not recommended in CLL. However, it can still be considered a clinical option in patients with a histological transformation clonally unrelated to CLL [106].

Lymphomas

Since December 2015, lymphoma patients are reported to the EBMT Registry as being in 'true' CR1 (first CR directly by standard first-line treatment), or as being in 'first' CR (achieved by one or more salvage attempts after primary induction failure), clearly segregating patients with different prognoses. Therefore, the recommendations for lymphoma (Table 1) refer to 'true' CR1 if CR1 is mentioned, and CR1 after prior refractoriness is included as CR > 1, and, in some lymphoma types, transplant indications in CR1 are more restrictive than in previous editions of these recommendations. In most types of lymphoma, retrospective analyses now show comparable results for MSD, MUD and haploidentical donor transplantation with PTCy (as cord blood and other alternative donors are used quite rarely in this setting and are not covered by these statements). Therefore, we now have consistency across current lymphoma classifications. While general recommendations and supporting data are discussed in the text below, Table 1 outlines specific transplant indications

along with the corresponding strength of evidence for each disease status.

Large B-cell lymphoma (LBCL)

LBCL is defined as any entity behaving clinically similar to diffuse large B-cell lymphoma (DLBCL) with respect to therapeutic options within the context of this guideline, including high-grade B-cell lymphoma NOS (not otherwise specified), double-hit and triple-hit lymphomas and peripheral mediastinal B-cell lymphoma.

Anti-CD19 CAR-Ts, axicabtagene ciloleucel (axi-cel) and lisocaptagene maraleucel (liso-cel), demonstrated superior PFS compared to standard of care, including salvage immunochemotherapy followed by auto-HCT in responders, as shown in two phase III clinical trials in patients with primary refractory disease or early relapses within 12 months following first-line therapy [107, 108]. For axi-cel a significant OS benefit was shown in the 5-year follow-up [109]. For patients who achieve a CR after salvage treatment prior to cell therapy, a retrospective analysis found better PFS if patients were consolidated with auto-HCT compared to CAR-T cell treatment [110]. Of note, no patients treated with CAR-T cells in second line were included in this analysis and more than half of the patients had received tisagenlecleucel, which is probably less efficient compared to axi-cel [111]. Still, in patients with early chemosensitive relapse, we consider high-dose therapy/auto-HCT to be a valid clinical option. For patients with primary refractory disease or early relapse with untested or chemorefractory disease, treatment with axi-cel or liso-cel represents the current standard of care.

Patients with late relapses face reasonable outcomes with auto-HCT and this standard has not been challenged by a clinical trial as of today. For the treatment of relapses following high-dose therapy/auto-HCT, CAR-T cell therapy can still lead to good responses [112]. In case of failure of second-line cell therapy, allo-HCT remains a clinical option for eligible patients [113–119].

The benefit of auto-HCT as part of the first-line treatment for patients with double-hit lymphomas is questionable and not recommended as routine practice [120]. For patients with high-risk disease defined by inadequate response on the interim PET-CT, auto-HCT as consolidation in CR1 can be considered. Still, superiority of this approach needs to be confirmed by comparative studies [121].

For patients with primary CNS lymphoma, auto-HCT in first remission after first-line therapy remains a standard approach in eligible patients, supported by several randomised trials and shown to be feasible even in fit older patients [122–126]. For patients with primary CNS lymphoma experiencing relapse or refractory disease, auto-HCT can also represent a promising option [127, 128]. In addition, there is growing evidence that CAR-T cell treatment has the potential to offer similar outcomes to those observed in other relapsed/refractory LBCL with an acceptable response duration in this setting [129–131] and is therefore considered to be the clinical option in second line.

Follicular lymphoma (FL)

Most advanced-stage FL patients benefit from a long PFS after induction and maintenance and should not be offered any cellular therapy in first remission [132]. Previously untreated FL patients with high-grade transformation should be treated as DLBCL. Auto-HCT is a clinical option in patients with transformed FL, if they had received prior chemoimmunotherapy. Patients with untransformed FL in 2nd remission or beyond and patients with POD24 should be considered for auto-HCT [133]. The FL treatment algorithm is changing. Bispecific antibodies are conditionally approved by EMA from 3rd line. [134–136] Two CAR-T cell products are EMA-approved for FL based on Phase 2 non-comparative trials: axi-cel [137] from 4th line and tisa-cel [138] from 3rd line. Liso-cel also shows promising results in FL [139]. CAR-T cells can be used in accordance with the EMA-label in countries where they are available. Allo-HCT is recommended for relapses after auto-HCT, if CAR-T is not available [140–142].

Waldenström's macroglobulinemia (lymphoplasmacytic lymphoma with IgM gammopathy; WM)

The treatment advances for patients with WM using immunotherapy with anti-CD20 monoclonal antibody, chemoimmunotherapy [143, 144], purine analogues [145], proteosome inhibitors [146–148], Bruton's TKIs [149, 150] and most recently bcl2 inhibitors have achieved deep and durable responses. Therefore, auto-HCT should not be considered as first-line clinical option. Auto-HCT should be considered as clinical option at subsequent disease relapses depending on the type of prior treatment, duration of response, age, performance status and comorbidities. Allo-HCT might be considered for younger patients with multiple relapses, those not achieving durable responses, or patients with refractory disease to immunochemotherapy, proteosome inhibitors-based treatment and/or Bruton's TKIs and bcl-2 inhibitors.

Mantle cell lymphoma (MCL)

Studies testing Bruton's TKIs as part of first-line therapy are questioning the benefit of 1st-line auto-HCT consolidation in MCL [151]. In 2nd line, auto-HCT is an option in HCT-naïve patients responding to salvage chemoimmunotherapy.

Available evidence does not suggest benefit of allo-HCT in MCL in CR1. In the salvage setting, allo-HCT is an option in patients without access to CAR-T or who have failed CAR-T [152, 153].

CAR-T should not be used as 1st-line treatment of MCL outside of clinical trials. The labelled indication of CAR-T in MCL is patients having failed at least two prior therapies including Bruton's TKIs [153]. With Bruton's TKIs moving to 1st-line treatment, CAR-T has become a clinical option as 2nd-line therapy in patients with primary Bruton's TKIs failure.

T-cell lymphomas (TCL)

Peripheral TCLs (PTCL) usually carry a poor prognosis. Allo-HCT is the only curative option for transplant-eligible patients with primary refractory PTCL or those who are in relapse. While allo-HCT is not an indication for major PTCL entities as first-line consolidation (T Follicular Helper Lymphoma (TFH-L), Peripheral TCL, Not Otherwise Specified (PTCL-nos) or Anaplastic Large Cell Lymphoma (ALCL)), auto-HCT is a recommended strategy in CR1 [154, 155]. In Enteropathy-Associated T-Cell Lymphoma (EATL) auto-HCT is also recommended in CR1. In Hepatosplenic TCL (HSTL), Monomorphic Epitheliotropic Intestinal T-Cell Lymphoma (MEITL), Extranodal NK/TCL (ENKTL), and Adult T-Cell Leukaemia/Lymphoma (ATLL), allo-HCT is strongly recommended in first-line therapy. While patients undergoing transplant in CR and PR show superior survival, patients with stable disease or progression should be considered for allo-HCT because other treatments do not typically result in long-term remission.

Primary cutaneous TCL (CTCL) in early stage have an excellent outcome, and HCT is generally not recommended. However, patients with EORTC/ISCL advanced stages (IIB to IV) have a dismal prognosis with conventional therapy [156]. Allo-HCT offers these patients a clinically relevant and persistent graft-versus-lymphoma effect [157–159].

A prospective matched controlled study in patients with advanced CTCL [160] demonstrated superior PFS of patients treated with allo-HCT as compared to conventional therapy. Therefore, allo-HCT is recommended for all patients with poor-risk CTCL stage IIB-IV, refractory or relapse after first-line therapy, large-cell transformation, nodal or visceral involvement, as soon as they reach CR, very good partial response (VGPR) or stable disease with minimal residual disease. Because of high relapse rates, auto-HCT is generally not recommended.

Hodgkin lymphoma (HL)

Targeted agents such as brentuximab vedotin (BV) and checkpoint inhibitors (CPIs) have already modified results of both auto-HCT and allo-HCT in patients with relapsed/refractory cHL. For now, as in previous recommendations, auto-HCT remains standard of care for patients with relapsed HL chemosensitive to standard therapy, and allo-HCT in those after a failed prior autograft [161–165].

The combination of BV and CPIs with salvage therapeutic strategies increases the percentage of metabolic complete remissions (mCR) before auto-HCT, thus potentially improving results of the procedure and the number of potential candidates for high-dose therapy [166–171]. Consolidation strategies after auto-HCT in high-risk of relapse patients have also improved long-term outcomes of auto-HCT. In the absence of prospective randomised clinical trials, allo-HCT is generally considered in patients who fail both BV and CPIs. CAR-Ts have been tested in the setting of prospective clinical trials; the few available results do not show convincingly better results than other treatment modalities to date [172]. Nodular lymphocyte predominant HL comprises only 5% of all patients being diagnosed with HL; HCT is restricted to those high-risk patients with relapsed disease [173].

Multiple myeloma (MM)

The development over the last decade of new agents such as anti-CD38 monoclonal antibodies and bispecific antibodies targeting BCMA and GPRC5D among other antigens, represent major advances. The incorporation of these agents into induction regimens may ultimately displace high-dose melphalan and upfront auto-HCT from its current central role in the treatment of younger, fitter patients. At present, however, first-line auto-HCT remains the standard of care for younger MM patients [174–176]. Age should be considered in conjunction with general health and fitness [177]. TBI should not be used in the conditioning regimen due to increased toxicity without appreciable benefit, and the addition of bortezomib or lenalidomide to conditioning regimens was not found to improve patient outcomes [178]. Double or 'tandem' autografting was found to be superior to a single auto-HCT in historical studies, although the benefit of the second transplant procedure in patients receiving contemporary quadruplet induction is not clear. Immunomodulatory drugs and bortezomib patients with high risk myeloma [179–182].

Two trials randomised patients who relapsed late after an autologous transplant to either a second salvage auto-HCT or a chemotherapy-only approach [183, 184]. Cook et al. reported a superior median time to progression (19 vs 11 months) in the salvage auto-HCT group without post-transplant maintenance [184]. In the randomised GMMG phase III ReLApsE trial, a post-hoc landmark analysis beginning at the time of HCT suggested an OS benefit for HCT (hazard ratio [HR] 0.56; 95% CI 0.32-0.99). In general, more durable remissions have been reported in patients whose time to progression was >24 months after the first auto-HCT [185].

Allo-HCT is a treatment with curative potential but is associated with considerable NRM and is generally reserved for selected high-risk patients in experienced centres [186]. The combination of auto-HCT followed by RIC allograft ('auto-allo') has shown a survival benefit in selected high-risk patients, albeit inconsistently in various clinical trials [187–190]. Recently, allo-HCT with PTCy has been shown to be feasible in MM, but relapse remains a major challenge [191]. Overall, there has been a gradual decline in the use of allo-HCT in patients with myeloma in EBMT centres over the last decade. There were approximately 200 allo-HCT reported to the EBMT in 2023.

CAR-T has shown promising results in patients with refractory/relapsed MM [192–194]. Idecabtagene vicleucel is the first cell-based gene therapy approved by the FDA for adults with relapsed/refractory MM after four or more lines of therapy, including an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 mAb. The second anti-BCMA CAR-T therapy, ciltacabtagene autoleucel, is indicated for the treatment of adult patients with relapsed and refractory MM, who have received at least one prior therapy, including an immunomodulatory agent and a proteasome inhibitor who have demonstrated disease progression on the last therapy, and are refractory to lenalidomide. The 27-month PFS and OS rates in patients with advanced disease were 54.9% (95% CI, 44.0 to 64.6) and 70.4% (95% CI, 60.1 to 78.6), respectively [195]. There are several trials evaluating the efficacy and tolerability of anti-BCMA CAR-T therapy at earlier disease stages though logistic and cost constraints have limited access to these agents so far.

AL amyloidosis

Patients with systemic immunoglobulin-light-chain (AL) amyloidosis without severe heart failure may benefit from auto-HCT [196]. The Mayo Clinic Prognostic Staging System for Light Chain Amyloidosis Incorporating cardiac biomarkers may aid in patient selection [197]. The benefit of auto-HCT was not confirmed in a prospective randomised trial that included patients with advanced cardiac amyloidosis [198]. Many recently published studies have reported improved early mortality after an appropriate risk assessment and consistently good haematologic responses and impressive long-term survival [199, 200]. Cytogenetic aberrations, as t(11;14), can also guide therapy [201]. The recently reported efficacy of bispecific antibodies in this disease is likely to reduce the use of auto-HCT.

Acquired severe aplastic anaemia (SAA)

HLA-identical sibling allo-HCT is considered the standard of care for adult patients with SAA; outcomes decline depending on the age, with increased mortality which is meaningful above 40 years, and unacceptable above 50 years [202–205]. Thus, in addition to age, careful assessment of co-morbidities prior to HCT is essential to assess the risk of mortality and morbidity for upfront HCT, especially in border-line age groups (i.e. 35–50 years). To reduce the risk of chronic GVHD, all patients should receive in vivo lymphodepletion with ATG or alemtuzumab, and bone marrow is the recommended source of stem cells [206–208]. The choice of conditioning regimen also depends on age; patients <30 years old should receive high-dose cyclophosphamide (200 mg/kg), and those aged 30–40 years old, a fludarabine-based (120 mg/m^2) regimen with lower dose cyclophosphamide (120 mg/kg). There is no indication for using radiation in the conditioning for MSD HCT. MSD HCT is also the preferred treatment option for all patients reasonably young (up to 70 years) and fit who have failed (refractory or relapsed) an initial non-transplant treatment, which is immunosuppressive therapy (IST), now based on the triple therapy ATG (preferably horse ATG), ciclosporin and eltrombopag [209, 210]. Failure of IST also includes progression/transformation into a myeloid malignancy (MDS or AML), somehow defined as 'clonal evolution; however, while allo-HCT is recommended in case of proven MDS or AML, the presence/emergence of somatic mutations alone does not qualify as an indication to allo-HCT [211].

MUD HCT can be considered as a first-line choice in young patients aged <18 years based on the excellent outcome compared to historical matched controls [212], provided transplant is feasible within the first two months after diagnosis. Alemtuzumab-based conditioning is also recommended in this situation [213]. If the interval to find a suitable MUD and proceed to HCT is predicted to be longer, IST with triple therapy is recommended as first-line treatment as in adults.

Otherwise, MUD HCT is indicated in young and adult patients after failure to respond to one course of IST (again, triple therapy), normally assessed at 3–6 months. Age of recipient is also an issue for MUD HCT, and along with assessment of co-morbidities and other patient and transplant characteristics (e.g. CMV status,

source of cells, use of ATG, interval from diagnosis to transplant, HLA matching degree), should help in evaluating patients who would benefit most from the procedure. On the other hand, since IST with triple therapy has maximised the likelihood of response to IST, allo-HCT is now considered the preferred treatment option for all patients who have failed initial IST, as defined above. In this setting, recipient age, HLA disparity and interval from diagnosis to transplant (within 1 year) impact on transplant outcome. A non-transplant approach is still recommended in elderly (>60–70 years), and it can be considered also in patients who had an initial response to triple therapy (e.g. second course of IST, or reintroduction of eltrombopag), as well as in other clinical settings (e.g. eltrombopag if not given first line, androgens, second course of immunosuppressive therapy) [206–208, 212–216]. Here, the conditioning regimen is usually based on the standard association of fludarabine and cyclophosphamide (120 mg/m^2 and 120 mg/kg, respectively), associated with lymphodepletion with ATG or alemtuzumab. As in MSD HCT, bone marrow is the recommended stem cell source for MUD HCT in SAA, especially for ATG-based conditioning regimens, while alemtuzumab-based conditioning may result in excellent outcome even using peripheral blood as stem cell source. Ongoing studies are exploring the impact of PTCY as an alternative GVHD prophylaxis in the context of MUD allo-HCT in SAA.

Alternative donors for allo-HCT (e.g. MMUD, haploidentical or cord blood) may be considered after failure to respond to IST. Excellent results have been reported especially in young patients up to 20 years of age in the absence of MSD or MUD [217–223]. Registry studies have documented that the outcome of MMUD allo-HCT for SAA is worse than that of MUD, but still acceptable; recent data suggest that the survival difference may be somehow mitigated or even overcome with the introduction of PTCY as GVHD prophylaxis [224]. This approach of GVHD prophylaxis has drastically changed the field, with very promising data even in older patients [217, 225]. Haplo-HCT in SAA should follow the so-called 'DeZern' protocol (fludarabine, cyclophosphamide, ATG and low-dose TBI, followed by PTCY, CNI and MMF). This regimen, albeit in small studies, resulted in excellent outcomes, making this strategy a clinical option for refractory/relapsed patients [226, 227]. Haplo-HCT is now under investigation also as possible initial therapy of SAA [228]. Although the treatment algorithm of SAA is under debate [229, 230], it is too early to recommend haplo-HCT as initial treatment for SAA patients [231]. Similarly, the question whether this promising conditioning regimen (which requires TBI, even at the dose of 400 cGy as upfront therapy) might be extended to other donor types [232] requires further investigation.

Cord blood HCT is also an option for refractory/relapsed SAA patients. Even if results from small studies are promising, this strategy remains experimental and should be delivered through specific trials or protocols (a SAAWP-recommended protocol is available), always seeking advice from a SAA specialist centre.

Constitutional SAA and bone marrow failure (BMF) syndromes, including Fanconi anaemia (FA), dyskeratosis congenita (DKC) and other telomere diseases, may not only be present in childhood but also in adults, often with more subtle clinical features. All cases of SAA being considered for HCT should have an appropriate diagnostic workup, including molecular genetics, to establish or exclude constitutional BMF syndromes, as it has important consequences for donor choice and conditioning regimen (see relevant sections below). On the other hand, additional testing may be useful to investigate clonal evolution even in possible (or confirmed) inherited BMF, since several factors (e.g. disease penetrance, concurrent mutations and further somatic mutations—including possible somatic rescue) may impact on disease phenotype, possibly impacting both the indication for allo-HCT, as well as the specific HCT procedure, including donor selection.

Paroxysmal nocturnal hemoglobinuria (PNH)

The introduction of anti-complement therapy with eculizumab changed the natural history of the disease, and allo-HCT became generally not recommended for patients with classical PNH (haemolysis ± thrombosis) for whom eculizumab is available. In recent years, additional complement inhibitors have been developed aiming to improve the haematological benefit and/or patients' convenience (e.g. long-lasting agents and oral agents) [233–236]. They have further limited the room for allo-HCT in patients with suboptimal response to complement inhibitors. Potential indications remain dependent on the individual clinical manifestations: (i) severe bi- or tri-lineage cytopenia due to severe BM failure (the so-called AA/PNH syndrome), using the same criteria for SAA above (i.e. age, disease severity, and failure to respond to one course of immunosuppressive therapy in case of donor other than MSD) and (ii) evolution/transformation of PNH to a myeloid malignancy (MDS or AML) [237, 238]. Given the improved outcome of allo-HCT for PNH [239], allo-HCT remains a potential treatment option when anti-complement treatment is not available [240]. Given the pleiomorphic clinical presentation of PNH, expert advice should be sought from a PNH specialist centre.

Details about these indications as well as the specific treatment regimens and challenges in AA patients are summarised in the most recent guidelines on the management of AA from the British Society of Haematology [241].

Solid tumours

Currently, the EBMT Registry includes >70,000 HCT procedures in >50,000 patients, of whom >40,000 are adults with solid tumours. Over 7500 procedures have been performed in adults in the last 5 years. However, with the possible exception of patients with germ cell tumours, and highly selected patients with breast cancer, sarcoma and medulloblastoma, HCT is generally not recommended or remains developmental for most indications in solid tumours [7]. With limited evidence from prospective studies published recently, the new recommendations in 2025 have changed little compared to prior indications.

Despite the encouraging role of immune surveillance and immune responses against several solid tumours [242–244] recommendations for allo-HCT would require further prospective trials, which are not a priority for medical oncology.

CAR-T, often associated with adjunctive agents, and other forms of adaptive immune effector therapies [245–250] are currently an area of active clinical research with data from Phase I/II studies suggesting a potential role of these approaches in several solid tumours [251, 252].

The role of auto-HCT in breast cancer at high risk of recurrence and metastatic disease has been assessed by several randomised trials and meta-analyses of individual patient data [253, 254]. As discussed in more detail in previous reports [255], the overall conclusion is that auto-HCT in breast cancer improves PFS but not OS in most studies. However, auto-HCT may still represent a clinical option for selected patients with specific biological characteristics and/or having gross involvement of axillary nodes (adjuvant setting) or highly chemosensitive disease (advanced setting) [255–258].

In germ cell tumours, auto-HCT is a standard of care for patients with disease refractory to platinum-based chemotherapy or with a second or further relapse, a clinical option as a second line in high-risk patients, and generally not recommended as first-line therapy [259–261], with the possible exception of primary mediastinal non-seminoma germ cell tumours [262]. Finally, auto-HCT can be considered a potential clinical option in selected patients with Ewing's and soft tissue sarcomas [263, 264], and medulloblastoma [265].

Auto-HCT, being per se capable of inducing marked and rapid tumour regression, may still represent a treatment modality for

selected chemosensitive solid tumours and is worthy of further study in combination with effective target agents, including immunotherapy. While awaiting results of further prospective trials, the EBMT registry remains an important source to survey indications, outcome and clinical risk factors in patients with solid tumours treated with auto-, allo-HCT and cellular therapies.

Autoimmune diseases (AD)

Autologous and, less frequently, allo-HCT represent promising therapeutic approaches for many severe AD cases that are resistant to state-of-the-art therapies, following careful consideration of the associated risks and benefits [266]. Worldwide, most transplant procedures for AD have been performed for multiple sclerosis (MS), followed by systemic sclerosis (SSc), currently representing the two standard indications for auto-HCT in this population [7].

Evidence is continually evolving for auto-HCT, predominantly in MS [267–271], and SSc [272–276], for which auto-HCT is regarded as a standard of care [277]. In addition, there is evidence to support treatment of carefully selected patients with Crohn's disease [278, 279], systemic lupus erythematosus (SLE) [280–282], neuromyelitis optica [283, 284], chronic inflammatory demyelinating polyradiculoneuropathy [285], myasthenia gravis [286], stiff person syndrome [287, 288], systemic vasculitis (ANCA positive [289], Takayasu [290], Behçet's disease [291] and refractory coeliac disease [292]. Collectively, the available data support the consideration of auto-HCT as an efficacious, on-off intensive therapeutic procedure for severe and refractory AD. Indeed, in the last decade, better outcomes have been obtained with auto-HCT, owing to a growing centre experience in selecting the most appropriate patients to transplant paralleled by advances in conditioning and supportive care regimens, certification and national socioeconomic factors [293]. Health economic reports have supported cost-effectiveness in some AD [269, 294, 295].

Despite improved survival over time, allo-HCT has remained predominantly used in younger patients [293]. According to recent EBMT registry data, this strategy can potentially provide long-term disease control in refractory AD, warranting further investigations mainly in younger patients [296]. The EBMT and other professional societies have published multidisciplinary guidelines and recommendations for both autologous and allo-HCT across various ADs, providing support to clinicians, scientists, patients, and caregivers [277, 293, 297–303]. Recently, the EBMT has also provided updated recommendations for the best practice of HCT in AD during the COVID-19 pandemic [304].

Recently, CAR-T cell therapy has been successfully used to treat patients with refractory autoantibody-driven ADs [305, 306]. Evidence for CAR-T therapies initially derived from small pilot studies [11] or single cases using autologous CD19 CAR-T in SLE [307–309], SSc [310] and idiopathic inflammatory myopathies (IIM) [311–313], followed by positive results from the first phase I/II trial of the BCMA-CD19 compound CAR-T in SLE [314]. Positive results were also obtained for refractory cases of rheumatoid arthritis [315, 316]. Meanwhile, first experiences have been obtained for CD19 CAR-T cell therapy for neurologic indications, including refractory cases of myasthenia gravis [315, 317], MS [318] and SPS [319]. More recently, positive results have been reported from phase 1 trials using BCMA CAR-T therapy in NMOSD [320] and autologous RNA CAR-T therapy in MG [321] with an acceptable safety profile.

Although representing a unique and promising therapeutic approach, further studies are required to position CAR-T cell therapies in the current treatment landscape of AD. EBMT recommendations for the use of CAR-T cells in ADs have been published [12]. A multidisciplinary team is strongly recommended to ensure appropriate patient selection and to carefully monitor the efficacy and toxicity of CAR-T cells in ADs.

Inherited diseases in adults

While allo-HCT in inherited diseases is predominantly performed in childhood, adult patients with inherited diseases, including haemoglobinopathies, constitutional BMF syndromes and inborn errors of metabolism (IEM) and immunity (IEI) are increasingly considered for HCT [322]. Given the complexity of these cases, it is advisable to seek expert guidance from specialised centres. The indications are the same as for inherited paediatric diseases (covered below), although presentation in adults may differ, including age of onset, course and prognosis. In some cases, access to HCT during childhood has been limited by health service resource and other non-clinical factors, necessitating consideration of HCT in adulthood.

As for the paediatric patients, there are differences between *Transfusion dependent Thalassaemia (TDT)* and *Sickle Cell Disease (SCD)*.

In TDT, limited experience has been registered in patients older than 14 years over the last years, with success rates, although improved, still not exceeding 80% [323]. However, because it has been clearly demonstrated that clinical condition and iron toxicity long-life-control is a major determinant of transplant outcome [324], improved clinical care and management of TDT makes transplant a today rationale option in patients beyond the above defined threshold [325].

In SCD, the EBMT hemoglobinopathies registry data have recently demonstrated that age is no longer a factor determining adverse outcomes [326] with success rates over 90% in adults as in children.

The recommendation to perform transplant for hemoglobinopathies in disease highly experienced centres already expressed for children is even more stringent for adult patient [325]

Transplant indications in children and adolescents

Allo-HCT in children and adolescents accounts for over 20% of overall allo-HCT activity, with particular use in congenital and non-malignant diseases, many of which are rare. Transplant complications in paediatric patients are compounded by the vulnerabilities of the developing child, including organ dysfunction related to development, such as infertility, delayed hormonal development, growth retardation, and a high risk of malignancies in congenital disorders with chromosomal breakage syndromes.

Advances in high-resolution HLA matching for UDs, conditioning regimens, supportive care for both infectious and non-infectious complications and the improvements in haplo transplants especially in non-malignant diseases (e.g. PIDs), have progressively reduced mortality. These developments have encouraged the use of allo-HCT, particularly for non-malignant indications, at earlier stages of disease progression, with patients in better performance status, rather than as a 'last chance for cure'.

GVHD remains a major limitation for patients without optimally matched donors. Emerging allo-HCT strategies aim to improve outcomes for patients with MMAD. The updated 2025 classification of HCT procedures in children and adolescents is presented in Table 2.

Acute myeloid leukaemia (AML)

Childhood AML is a rare and heterogeneous disease, with increasing cure and survival rates achieved through intensive chemotherapy, particularly for patients with favourable prognostic markers. As a result, allo-HCT is not recommended as frontline therapy for low-risk patients but remains the standard of care for those in CR1 with high- or very-high-risk disease who have a well-matched donor [327–330].

Alternative donors, particularly haploidentical family members, are playing an increasingly significant role in treating high- and very-high-risk childhood AML and patients beyond CR1 [331, 332]. Children who relapse and achieve a second CR are also candidates for allo-HCT from the best available donor.

Table 2. Proposed classification of transplant indications for children and adolescents—2025.

Disease	Disease status and subtypes	MSD allo	MUD allo	MMAD allo	Auto	CAR-T
Haematological malignancies						
AML	CR1 (low risk)[a]	GNR/II	GNR/II	GNR/III	GNR/II	
	CR1 (high and very high risk)[a]	S/II	S/II	CO/II	GNR/II	
	CR2	S/II	S/II	S/II	GNR/II	
	>CR2	S/II	CO/II	CO/II	GNR/II	
ALL	CR1 (low risk)[a]	GNR/II	GNR/II	GNR/III	GNR/II	GNR
	CR1 (high risk)[a]	S/II	S/II	CO/II	GNR/II	CO/II (refractory pB-ALL)
	CR2	S/II	S/II	CO/II	GNR/II	CO/II (refractory pB-ALL or relapse post-HCT)
	>CR2	S/II	S/II	CO/II	GNR/II	S/II (pB-ALL)
CML	1st CP, failing 2nd or 3rd line TKI	S/II	S/II	CO/II	GNR/III	
	Accelerated phase, blast crisis or >1st CP	S/II	S/II	CO/II	GNR/III	
MDS and JMML		S/II	S/II	CO/III	GNR/III	
NHL	CR1 (low risk)	GNR/II	GNR/II	GNR/II	GNR/II	
	CR1 (high risk)	CO/II	CO/II	CO/II	CO/II	
	CR2	S/II	S/II	CO/II	CO/II	
HL	CR1	GNR/II	GNR/II	GNR/II	GNR/II	
	1st relapse, CR2	CO/II	CO/III	CO/III	S/II	
Non-malignant disorders and solid tumours						
IBMFS		S/II	S/II	CO/II	NA	
Acquired SAA	Newly diagnosed	S/II	S/II	D/II	NA	
	Relapse/Refractory	S/I	S/II	CO/II	NA	
Germ cell tumours (refractory)		GNR	GNR	GNR	D	
Sarcoma	Ewing's sarcoma (high risk or >CR1)	D/II	D/III	D/III	CO	
	Soft tissue sarcoma (high risk or >CR1)	GNR	GNR	GNR	GNR	
	Osteogenic sarcoma	GNR/III	GNR/III	GNR/III	GNR	
Neuroblastoma	High risk or >CR1	D	D	D	S/II	D/II (refractory)
Brain tumours		GNR/III	GNR/III	GNR/III	D	
Wilms' tumour	>CR1	GNR/III	GNR/III	GNR/III	D	
Primary ID	SCID	S/II	S/II	S/II	NA	
	Non-SCID CID	S/II	S/II	S or CO/II	NA	
	Primary HLH S/II	S/II	S/II	S/II	NA	
	Other primary ID	S/II	S/II	CO/II	NA	
IEM	MPS-IH	S/II	S/II	S/II	NA	
	Wolman disease[b]	CO/III	CO/III	CO/III	NA	
	MPSII–VII[b]	CO/II	CO/II	CO/II	NA	
	MLD	S/II	S/II	CO/II		
PSD	X-ALD	S/II	S/II	CO/II	NA	
Osteopetrosis		S/II	S/II	S/II	NA	
AD	Including monogenic AD	CO/II	CO/OO	CO/II	CO/II	
TDT	Absence of iron related severe/ irreversible tissue/organ damage	S/II	S/II	CO/II[#]	NA	
SCD	Severe clinical disease and absence of severe organ/tissue damage	S/II	D/II	CO/II[#]	NA	

This classification does not cover patients for whom a syngeneic donor is available.

AD autoimmune disorders, *ALL* acute lymphoblastic leukaemia, *Allo* allogeneic transplantation, *AML* acute myeloid leukaemia, *Auto* autologous transplantation, *CML* chronic myelogenous leukaemia, *CO* clinical option (can be carried after careful assessment of risks and benefits), *CP* chronic phase, *CR1, 2* first, second complete remission, *D* developmental (further trials are needed), *GNR* generally not recommended, *HL* Hodgkin lymphoma, *HCT* haematopoietic cell transplantation, *IBMFS* inborn marrow failure syndromes (Fanconi anaemia, dyskeratosis congenita, Blackfan–Diamond anaemia and others), *ID* immunodeficiency, *IEM* inborn error of metabolism, *JMML* juvenile myelomonocytic leukaemia, *MDS* myelodysplastic syndromes, *MLD* metachromatic leukodystrophy, *MMAD* mismatched alternative donors (cord blood, haploidentical and mismatched unrelated donors), *MPS* mucopolysaccharidosis, *MSD* matched sibling donor, *MUD* well-matched unrelated donor (8/8, 10/10, or 9/10 if mismatched is in DQB1), *PSD* peroxisomal storage diseases, S standard of care (generally indicated in suitable patients), *SAA* severe aplastic anaemia, *SCD* sickle cell disease (high risk), *SCID* severe combined immunodeficiencies, *TDT* Transfusion dependent Thalassaemia, *TKI* tyrosine kinase inhibitors, *X-ALD* X-linked adrenoleukodystrophy.

[a]Categories are based on number of white blood cells, cytogenetics and molecular markers at diagnosis and time to achieve remission (see text).

[b]For Wolman disease, MPSII and VII, decision is individualised after expert evaluation.

[#]For thalassaemia and SCD, MMAD indication applies ONLY to haploidentical allo-HCT.

Two randomised studies are currently underway to evaluate conditioning regimens in children with AML: one comparing BuCyMel versus TreoFluTT (AML-AIEOP BFM 2020) and another comparing BuCyMel versus BuFluClo (SCRIPT). Auto-HCT in this context is generally not recommended [333].

Acute lymphoblastic leukaemia (ALL)

Allo-HCT from MSD and MUD is the standard of care for high-risk ALL patients in CR1, as well as for those in CR2 or later [334–338]. Classical risk factors include molecular markers, chromosomal abnormalities, biological factors, and resistance to initial chemotherapy [339]. However, minimal residual disease (MRD) has now emerged as the most critical prognostic factor to distinguish high- from very-high-risk ALL groups [340–342].

When an MSD or MUD is unavailable, mismatched alternative donors (MMAD), such as cord blood (CB), mismatched unrelated donors (MMUD), or haploidentical family donors, are viable clinical options [343]. Unlike in adults, PBSCs offer no significant advantage over BM in terms of engraftment or relapse incidence. Therefore, BM is the preferred stem cell source for children undergoing MSD-HCT [344].

The FORUM trial (EudraCT: 2012-003032-22; ClinicalTrials.gov: NCT01949129) recently evaluated the role of total body irradiation (TBI) in a non-inferiority study involving 417 patients. In the intention-to-treat population, the 2-year OS was significantly higher following TBI compared to chemotherapy-only conditioning. The 2-year cumulative incidence of relapse and treatment-related mortality (TRM) was 0.12 and 0.02 for TBI, versus 0.33 and 0.09 for chemotherapy-only conditioning, respectively.

Regrettably, the trial demonstrated that improved OS and lower relapse rates were achieved with TBI plus etoposide compared to chemotherapy-only conditioning. At present, TBI plus etoposide is recommended for patients >4 years old with high-risk ALL undergoing allo-HCT [345].

In regions without access to TBI or for children with contraindications, chemotherapy regimens such as BuFluTT or TreoFluTT remain valuable alternatives [346].

Tisagenlecleucel is a CD19-specific chimeric antigen receptor T cell therapy, EMA- and US FDA-approved for children, adolescents, and young adults with relapsed and/or refractory B-ALL. The registration for tisagenlecleucel was based on a CR rate of 81%, 12-month OS of 76%, and EFS of 50% [59].

Chronic myeloid leukaemia (CML)

As mentioned earlier, for adult patients, with the advent of TKIs, allo-HCT is no longer recommended as a first-line treatment for CML in children and adolescents. However, it remains a standard option for patients experiencing treatment failure, recurrence after receiving salvage second-generation TKI therapy, and advanced-phase CML [347–349].

For paediatric patients, the decision to proceed with allo-HCT requires careful individual consideration to balance the well-established long-term complications of HCT with the potential adverse effects of prolonged TKI treatment, which may include growth failure, hepatic, and cardiac complications [350–352]. Stronger evidence from prospective cooperative studies is needed to better understand disease progression after TKI discontinuation and to address issues specific to paediatric patients with CML [348, 353].

A recent paper by Pichler et al. recommends FluMelTT as the optimal conditioning protocol for children with CML [354].

Myelodysplastic Syndromes (MDS) and Juvenile Myelomonocytic Leukaemia (JMML)

Allo-HCT from an MSD or MUD is the treatment of choice for children with primary MDS, including JMML, as well as secondary AML [355–357]. Auto-HCT is not recommended outside clinical trials.

Lymphoma

Nearly all children and adolescents with HL and NHL are cured with multidrug chemotherapy. Only a small subset of paediatric patients are eligible for HCT (Table 2) [358–362]. These include patients with residual disease after re-induction therapy with contemporary chemotherapy protocols, patients with early relapses of NHL, and those with an inadequate response or relapse of ALK-positive ALCL. All other approaches should be discussed with experts in frontline chemotherapy trials.

Solid tumours

Although published results have not yet demonstrated an unequivocal benefit for most indications, children and adolescents with solid tumours may undergo auto-HCT following high-dose chemotherapy as a clinical option or as part of research protocols, preferably within first-line treatment strategies (Table 2). Neuroblastoma (stage 4 in children older than 1 year, or with high-risk factors in lower stages) remains the only indication where the benefit of auto-HCT has been proven by randomised trials [363, 364]. The use of GD2-CART01 cells was feasible and safe in treating high-risk neuroblastoma in a single-centre study. Treatment-related toxic effects developed, and the activation of the suicide gene controlled side effects. GD2-CART01 cells may have a sustained antitumor effect [365].

In general, allo-HCT for children with solid tumours should only be explored within prospective clinical trials conducted at highly experienced centres.

Acquired severe aplastic anaemia

Allo-HCT from MSD is the standard front-line therapy for children and adolescents (up to 18–20 years) with acquired SAA. In patients without MSD, a well-matched (10/10) unrelated HCT is now also considered a standard front-line therapy in many patients if the donor is readily available and the transplant feasible within the first two months after diagnosis. The search should in any case be initiated before starting any immunosuppressive therapy [366–370]. For those who lack a well-matched donor (i.e. MMUD and Haplo) allo-HCT is currently not recommended as front-line treatment, even if recent data using an haplo donor may be promising [220, 221, 223–228]. MMUD and haplo-HCT is considered standard in patients failing their first course of immunosuppression, being preferred over a second course of immunosuppression.

Constitutional bone marrow failure syndromes

Allo-HCT is the only treatment able to restore normal haematopoiesis in these patients. Transfusion-dependent FA patients with a suitably well-matched family or UD should be transplanted while in the phase of moderate cytopenia with no poor-risk clonal abnormalities and no MDS/AML [371–374]. Haplo donor HCT offers very good results when either in vivo or ex vivo TCD is included [371–373] but for patients who lack a well-matched donor, HCT from MMAD should be better considered as a clinical option in the context of a clinical protocol. Although outcomes are reported to be better at age <10 years, this is not the only criterion for decision-making. Details on transplant conditioning for specific indications are beyond the scope of these recommendations, but it is important that standard doses of chemotherapy and/or irradiation are absolutely avoided in HCT for FA due to the underlying defect in DNA repair. Although radiation-free regimens including busulfan, cyclophosphamide, fludarabine, ATG with the infusion of a T-cell-depleted graft provide excellent outcomes in HCT from allogeneic donors other than MSD [373], the addition of low-dose TBI may be indicated for those patients with clonal evolution or receiving transplantation from a UD due to a higher risk of graft rejection. In addition, MSD must be tested for chromosomal fragility, given that some FA subjects can have nearly normal somatic and haematological phenotype. BM is

recommended above PB as HSC source, as PB is an independent risk factor for second malignancies.

Patients with DKC and other inherited BMF syndromes should be transplanted if they have an MSD or a MUD [375–377]. A recent large retrospective SAAWP study on allo-HCT for DKC and other telomeropathies showed that pre-transplant organ damage (lung and liver) was associated with poorer outcome [375], supporting thorough organ assessment before HCT. RIC regimens incorporating fludarabine are currently recommended [376, 377]. Potential sibling donors should be tested for telomere length and for mutations of gene of the telomerase-shelterin complex to rule out alterations despite normal somatic and haematologic phenotype. Patients with Blackfan–Diamond anaemia with a MSD should be transplanted if they do not respond to steroids. If an MSD is not available, allo-HCT may be performed with a MUD in experienced centres [378]. Discussion with a specialist centre is advised regarding possible HCT in patients with constitutional BMF.

Autoimmune diseases (AD)

Autologous and allo-HCT may be considered clinical options for children and adolescents with AD [266, 293, 296, 379]. Overall, given the overlap between autoimmune, autoinflammatory conditions, and inborn errors of immunity (IEI) in the paediatric age group, appropriate specialist expertise in diagnostics is essential (such as NGS, WGS/exome sequencing) and in evaluating alternative treatment options when selecting patients for HCT. Special consideration should be given to AD that remains refractory to multiple lines of conventional and disease-modifying treatments, for which allo-HCT might represent the final opportunity for disease control and cure [296].

Auto-HCT may be considered for carefully selected subpopulations of patients with juvenile inflammatory arthritis (e.g. polyarticular or systemic course or onset, inadequate response and/or intolerance to prednisone or disease-modifying antirheumatic drugs) and other ADs including SSc, SLE, vasculitis and polymyositis-dermatomyositis. Paediatric MS is a rare indication for auto-HCT, and long- term responses have been reported [368].

Crohn's disease is a potential indication for auto-HCT. However, there should be careful consideration of monogenic forms of inflammatory bowel disease (e.g. IL-10 signalling defects, immunodysregulation polyendocrinopathy enteropathy X-linked—IPEX [369]—syndrome, chronic granulomatous disease or increasingly X-linked inhibitor of apoptosis—XIAP–deficiency [300], which are IEI for which allo-HCT is appropriate. Auto-HCT and allo-HCT have both been performed in severe autoimmune cytopenias, with similar outcomes [380]. Allo-HCT may also result in long-term responses in severe juvenile inflammatory arthritis [381]. A recent retrospective EBMT study reported the long-term outcome of allo-HCT in various haematological and non-haematological severe AD, including also paediatric patients [368]. Better transplant outcomes have been reported for patients <18 years and in more recent years of transplant.

Inherited diseases

Allo-HCT for inherited conditions is most commonly used in the paediatric and teenage and young adult populations, though an increased number of older patients require assessment and treatment. Genetically modified auto-HCT has become available for few diseases and is being explored in others with promising results.

Inborn errors of immunity (IEI)

IEIs are an expanding group of over 500 genetic disorders often involving susceptibility to infections, immune dysregulation related manifestations such as autoimmunity and autoinflammation [382]. Patients may experience chronic lymphoproliferation or cancers. Allo-HCT offers curative options for adaptive immune defects and in specific innate immune deficiencies. The decision to proceed to HCT depends on factors like immunological parameters, disease severity, organ damage, HLA matching, and family willingness [383]. Early intervention is critical to prevent damage from the disease or treatments [384–386]. A thorough, multidisciplinary evaluation is essential for optimal treatment planning.

T-cell immunity defects are among the most serious conditions, often making allo-HCT necessary. Severe combined immunodeficiency (SCID) represents the most critical forms, typically leading to an early mortality unless HCT, or in some instances, gene therapy is initiated during infancy. Non-SCID-T cell deficiencies are varied, resulting in a broad spectrum of symptoms and severity. For the most severe conditions, such as MHC class II deficiency [387], Wiskott-Aldrich syndrome, DOCK8 deficiencies [388], CD40 ligand deficiency [389] among others, allo-HCT has to be strongly considered early in life, particularly when a suitable HLA-identical donor is available.

Advances in haploidentical transplants, including selective in vitro graft depletion (e.g. TCRab/CD19) [390–392] or T-replete graft with in vivo depletion with PTCy have further broaden the indications for transplantation [393, 394]. Feasibility has been shown with both approaches in the field of IEIs [386].

Primary immune regulation disorders have been increasingly recognised and are characterised by diverse auto-immune and auto-inflammatory features. While allo-HCT can be an option, decision to go to transplant, and determining the appropriate timing remain complex. Control of the inflammation as much as possible prior to the procedure is crucial. The place of emerging biotherapies and targeted immunosuppressive drugs will need to be clarified, either as an alternative to transplantation or as a bridge to the procedure [386, 395–397].

Primary HLH, regardless of the underlying genetic cause is a clear indication for allo-HCT, including alternative donors. Achieving disease remission prior to allo-HCT plays a critical role in improving overall survival. In certain cases, pre-emptive HCT, before the onset of any HLH-related symptoms, can be considered [398, 399].

Among innate immune deficiencies, complete leucocyte adhesion deficiency is a straightforward indication of HCT [400]. In chronic granulomatous disease, the improved outcomes following HCT have expanded the HCT indication, particularly when an HLA-matched donor is available [401].

Stem cell gene therapy has been pioneered in X-linked and ADA-SCID, leading to EMA licensing of the gammaretroviral product Strimvelis® [402], while excellent results have recently been reported with a lentivirus-based approach [402, 403]. The most recent lentiviral gene therapy studies in other IEI have provided encouraging results and positioning of this alternative curative treatment option is expected in the next years [404–406].

However, accessibility to these innovative therapies for rare or ultra-rare conditions remains challenging [407, 408].

Inborn errors of metabolism (IEM)

Allo-HCT is effective in selected patients with peroxisomal diseases (PSD), lysosomal storage diseases (LSD), and some other IEMs [409]. Among LSD, the most successful application of this approach is seen in in type I mucopolysaccharidosis (or Hurler's syndrome), a severe, multi-system disorder that progressively affects neurocognitive function. Engraftment of donor-derived myeloid cells, including microglia, provides the missing enzyme to the recipient. Early intervention (before the age of 2) with a wild type donor, and a tailored busulfan-based conditioning regimen to reach full donor chimerism achieve the best results. Cord blood and bone marrow are both eligible as HSC source. HCT can mitigate disease progression but does not completely eliminate long-term disease manifestations.

In metachromatic leukodystrophy, a disorder characterised by central and peripheral demyelination, allo-HCT is ineffective in

early infantile disease but can have a beneficial impact when applied in juvenile or adult forms, provided the intervention occurs early in the disease's progression. Allo-HCT also has a role in other LSD, such as MPSII, MPSVII and Wolman disease, but given the rarity of these later conditions, a thorough assessment by an expert multidisciplinary team is crucial.

Among PSD, allo-HCT is effective in preventing disease progression in childhood, (or in rare instances adult) forms of X-linked adrenoleukodystrophy (X-ALD), a cerebral inflammatory disease. Allo-HCT is not recommended for individuals with advanced disease but transplant is offered at the earliest sign of disease.

Gene therapy using gene-corrected autologous HSC holds considerable promise in treating IEMs by facilitating supra-physiological enzyme production [410]. This approach is now an established treatment for late-infantile metachromatic leukodystrophy [411]. A phase I-II trial has been completed in MPS IH, with very promising results in the short and medium term [412, 413] while this approach is currently being investigated in MPS II and MPS IIIA. Lentiviral GT product has also been approved by the EMA for X-ALD [414] but the risk of this product needs to be carefully weighted since a significant risk of MDS has been reported, related to clonal vector insertions within oncogenes [415].

Osteopetrosis (OP). OP is a heterogeneous genetic disease related to several gene defects that impair bone resorption. Urgent HCT is indicated after exclusion of neurodegenerative and osteoclast-extrinsic defects. Atypical, delayed disease may occur, for whom HCT may be considered in case of haematological insufficiency or imminent visual impairment [416].

Haemoglobinopathies

Haemoglobinopathies are non-malignant diseases and have prolonged survival with modern medical therapy, albeit life-long, complex and expensive and is 'standard-of-care' only in limited parts of the world, different to where hemoglobinopathies are most prevalent. Importantly, allo-HCT for haemoglobinopathies should be performed early in life to reduce disease related complications like irreversible damage due to iron overload in patients with transfusion dependent thalassaemia (TDT) and systemic vasculopathy in patients with Sickle cell Disease (SCD).

Moreover, since TDT and SCD present specific clinical characteristics potentially leading to unexpected and disease-specific complications, transplantation (particularly experimental approaches) should only be performed in highly experienced centres [325, 417].

Transfusion-dependent thalassaemia (TDT)

TDT can today benefit from optimal transfusion and iron chelation therapy permitting, is consistent long-life, a prolonged survival until adulthood. However, transfusion consequences and iron toxicity are not completely avoidable [418].

For patients without an MSD, a transplant from a MUD can obtain similar results and is therefore a practicable clinical option [323, 325, 419, 420].

HCT in TDT from haploidentical related donors is now increasingly performed as a clinical option in experienced centres with outstanding results [421–423].

Sickle cell disease (SCD)

Although SCD standard clinical care improved over recent years, current management relies on lifelong use of palliative short-term and long-term therapies and survival and quality of life remain significantly reduced. For this reason, allo-HCT from a MSD is standard of care and should be offered prior to the emergence of serious complications [325, 424].

Recently, hemoglobinopathies registry data demonstrated improved results in the last years with a probability of success clearly exceeding 90% of cases [326]. The general theme that MSD and MUD produce overlapping OS and event-free survival has not been reproduced in SCD, where HCT from MUD is inferior to MSD with a significant decline in outcome [425]. Recent reports demonstrated successful outcomes with haploidentical transplantation [417, 426] and alternative family donors are considered a clinical option using either PTCy or T-cell-depleted strategies.

Gene therapy in haemaoglobinopathies

CRISPR-Cas9 gene-editing approaches have been recently approved by FDA and EMA and are currently available in few EU countries for TDT [427] and SCD [428]. A lentiviral-based gene therapy approach for TDT [429] and SCD [430] has received market authorisation but is not available in Europe [431].

CONCLUSIONS

For over two decades, the EBMT indications reports have incorporated developments in HCT practice based on scientific and technical developments in HCT. We encourage harmonisation of practice, where possible, to ensure meaningfully aggregated experience across indications via registry outputs. We also recommend working according to JACIE certification standards and participation in benchmarking of survival outcomes to maintain quality in HCT and other cellular therapy practice [1–3].

Reporting in the EBMT registry allows the collection of crucial real-world data also in long-term follow up, alongside basic reporting of activity data that is published in EBMT Survey reports [4, 5]. The EBMT is increasingly engaging with the patient community and considering Patient Reported Outcomes as part of data collection [432].

Moving forward, all treatment decisions, whether HCT or non-HCT, need to be considered in conjunction with the risk of the disease, risk of HCT procedure and non-HCT strategies, and impact on both quantity and quality of life. HCTs are evolving in complexity alongside health economic costs associated with their delivery. Local delivery of HCT should be balanced against working in close partnership ('shared care') with more distant HCT centres with more developed facilities and expertise is also an important consideration. The increasing application of CAR-T and other genetically modified cell therapies will require ongoing consideration in clinical decision-making in HCT across the indications.

DATA AVAILABILITY

Data sharing is not applicable to this article as no datasets were generated or analysed during the current study.

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AUTHOR CONTRIBUTIONS

RG, AR, IS-O and JAS coordinated the writing of the manuscript following approval of the structure and authorship at the EBMT Board and Scientific Council on 19 October 2024. Relevant sections were drafted by authors designated according to roles within EBMT Scientific Council, Working Parties (WPs) and other roles: namely, AR (Cellular Therapy and Immunobiology WP), TA with RG (Autoimmune Diseases WP); DPM (Chronic Malignancies WP), FC (Acute Leukaemia WP), ZP (Transplant Complications WP), AB (Lymphoma WP); PP (for Solid Tumours, within Cellular Therapy and Immunobiology WP chaired by AR); KK (Paediatric Diseases WP), AMR (Severe Aplastic Anaemia WP); EA (Haemoglobinopathies WP), BN (Inborn Errors WP), with input from DA (Infectious Diseases WP), JAS (for Quality, JACIE and EBMT Benchmarking), MK and HM (Nurses Group). RG, AR, IS-O, JAS, MDH and AS provided cross-cutting advisory expertise throughout. RG, AR and IS-O edited the manuscript and all authors provided input across all sections in the final drafts of the manuscript according to expertise. Other specialists from EBMT are acknowledged. The manuscript was agreed and finalised at the EBMT Board and Scientific Council Meeting on 29 March 2025. All authors approved the final version.

COMPETING INTERESTS

RG discloses speaking honoraria from Biotest, Pfizer, Medac, BMS, MSD, Neovii, Kyverna and Magenta; advisory board from Pfizer. AR: declares honoraria from Gilead and Kyte. JAS declares advisory board membership for the last 36 months with Medac, Jazz, Vertex and BMS. TA received study support from Johnson & Johnson. DA delcares MSD – local PI in a clinical trial, advisory board. AB: declares Speaker bureau or advisory board: Novartis, Roche, Sanofi, Jazz, Takeda, Hikma, Biologix, Jansen, MSD, Abbvie, Pfizer and Amgen and Research support: Novartis, Roche, Takeda, Biologix, Hikma, Pfizer and Jansen. MDH: none MK advisory board Jazz, Alexion. KK: declares speaker's bureau for Novartis, medac, Pierre Fabre. ZP declares advisory board for Amgen, Novartis and Pfizer. AMR: declares payment or honoraria for lectures, presentations, speakers' bureaus, manuscript writing, or educational events from Alexion (AstraZeneca Rare Disease), Apellis, Kira, Novartis, Omeros, Pfizer, Roche, and Sobi; and support for attending meetings and/or travel from Novartis and Sobi. AS declares TakedaBMS/Celgene, MSD. DPM, EA, MK, HM, BN, PP, ISO and FC declare no conflict of interests.

ETHICS APPROVAL AND CONSENT TO PARTICIPATE

This manuscript did not require ethical approval as it did not involve human participants, human tissue, animals, or identifiable personal data. It is based on EBMT recommendations, providing guidance on the indications for HCT and CAR-T in haematological diseases, solid tumours, and immune disorders, with reference to previously published literature, guidelines, and supporting evidence.

ADDITIONAL INFORMATION

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Correspondence and requests for materials should be addressed to R. Greco or A. Ruggeri.