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CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

Hematopoietic Cell Transplantation (HCT) Predictions for the Year 2023

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Page exit logic: Skip / Disqualify Logic
IF: (#1 Question "Is your primary specialty hematopoietic cell transplantation?
" is one of the following answers ("No") OR #2 Question "Do you work at a transplant center in the United States?" is one of the following answers ("No"))
THEN: Jump to page 7 - We're sorry. You do not meet the qualifications for this survey. Flag response as complete

Show/hide trigger exists.

1. Is your primary specialty hematopoietic cell transplantation? *

- Yes
- No

Hidden unless: #1 Question "Is your primary specialty hematopoietic cell transplantation?
" is one of the following answers ("Yes")

2. Do you work at a transplant center in the United States? *

- Yes
- No

Consent to participate in research

Page exit logic: Skip / Disqualify Logic
IF: #3 Question "Please indicate whether or not you would like to participate in this study by selecting one of the following options:" is one of the following answers ("I do not agree to participate in this survey for research.") **THEN:** Jump to page 7 - We're sorry. You do not meet the qualifications for this survey. Flag response as complete

Hidden unless: (#1 Question "Is your primary specialty hematopoietic cell transplantation?
" is one of the following answers ("Yes") AND #2 Question "Do you work at a transplant center in the United States?" is one of the following answers ("Yes"))

3. Please indicate whether or not you would like to participate in this study by selecting one of the following options: *

- I agree to participate in this survey for research.
- I do not agree to participate in this survey for research.

The following section asks about you, your practice and center's characteristics

Show/hide trigger exists.

4. For what patient population do you provide care? *

- Adult only
- Pediatric only
- Both adult and pediatric

5. How old are you in years? *

- <30
- 30-40
- 41-50
- 51-60
- 61-70
- >70

6. Please select your sex *

- Male
- Female
- Prefer not to respond

7. Since completing your fellowship, for how many years have you been in active clinical practice? *

- < 5
- 5-10
- 11-14
- 15-20
- >20

8. How many years of experience do you have in hematopoietic cell transplantation and cellular therapy? *

- <5
- 5-10
- 11-14
- 15-20
- >20

9. During the past 12 months, approximately what percentage of your professional time do you spend in the following activities? (Numbers must add up to 100%)
*

Patient care

Clinical research

Basic research

Administration/Teaching

0 out of 100 Total

10. How would you best characterize your practice setting? *

- Office/clinic not affiliated with a hospital
- Non-teaching hospital
- Teaching hospital- affiliated with university/academic center
- Teaching hospital- not affiliated with university/academic center
- Other, Specify

Show/hide trigger exists.

11. What type(s) of HCT does your center perform? *

- Autologous HCT only
- Allogeneic HCT only
- Both Autologous and Allogeneic HCT

Hidden unless: #11 Question "What type(s) of HCT does your center perform?" is one of the following answers ("Autologous HCT only","Both Autologous and Allogeneic HCT")

12. How many autologous transplants does your center perform annually? *

- <25
- 25-49
- 50-100
- >100
- I don't know

Hidden unless: #11 Question "What type(s) of HCT does your center perform?" is one of the following answers ("Allogeneic HCT only","Both Autologous and Allogeneic HCT")

13. How many allogeneic transplants does your center perform annually? *

- <5
- 5-10
- 11-49
- 50-100
- >100
- I don't know

14. Please describe your center's participation in the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) *

- BMT CTN Core/Consortium Center (Not PBMTC)
- BMT CTN/Consortium Center (PBMTC)
- BMT CTN Affiliate Center
- Does not participate in BMT CTN trials

The following questions ask about your prediction of where the HCT and cellular therapy field will be in the year 2023 (4 years from now).

15. Will the numbers of HCTs increase or decrease in 2023 compared to 2018? *

	Increase	Decrease	Stay about the same
Umbilical cord blood transplants (UCBT):	○	○	○
HCT for hematologic malignancy:	○	○	○
HCT for immunodeficiency:	○	○	○
HCT for sickle cell disease:	○	○	○
HCT for genetic disorders:	○	○	○
HCT for "regenerative medicine":	○	○	○
HCT for autoimmune diseases:	○	○	○

Hidden unless: #4 Question "For what patient population do you provide care?" is one of the following answers ("Adult only","Both adult and pediatric")

16. Autologous HCT in adults (age ≥18 years) will be considered as a "standard of care" indication for which diseases in 2023, i.e., widely covered by private or/and public insurance? (Check all that apply)

- ☐ Systemic sclerosis
- ☐ Multiple sclerosis
- ☐ Crohn's disease
- ☐ Rheumatoid arthritis
- ☐ Systemic lupus erythematosus

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17. Will the numbers of allogeneic HCT for hematologic malignancies in adults (age ≥18 years) increase or decrease in 2023 compared to 2018 *

	Increase	Decrease	Stay about the same
IPSS-R Intermediate-2/high risk myelodysplastic syndromes (MDS):	○	○	○
AML in CR2:	○	○	○
Acute lymphoblastic leukemia with high risk features:	○	○	○
Chronic phase chronic myelogenous leukemia (CML) (tyrosine kinase inhibitor intolerant/ refractory):	○	○	○
Intermediate/high risk myelofibrosis:	○	○	○

Hidden unless: #4 Question "For what patient population do you provide care?" is one of the following answers ("Adult only","Both adult and pediatric")

18. Please predict the most common donor source for adults (age ≥18 years) with AML in CR2 undergoing allogeneic HCT in 2023 by ranking the list of preferred donors by dropping the preferred donor into the right-hand list. If you anticipate a donor type will not be used, leave in the left-hand list. (1 = highest preference; 2 = second highest preference, etc; options left in left-hand list will not be used) *

Drag items from the left-hand list into the right-hand list to order them.

- Matched related donor
- Haploidentical related donor
- Matched unrelated donor
- Mismatched unrelated donor
- Umbilical cord blood

Hidden unless: #4 Question "For what patient population do you provide care?" is one of the following answers ("Pediatric only","Both adult and pediatric")

19. What will be the most common graft source for children (age <18 years) with hematologic malignancies in 2023? *

- ○ Bone marrow
- ○ Peripheral blood
- ○ Umbilical cord

20. Will autologous transplantation with gene therapy be the first line therapy for patients for adolescents and young adults patients (12 - 35 years of age) with transfusion dependent beta-thalassemia in 2023? *

- ○ Yes
- ○ No

21. Will frontline alternative donor HCT (i.e., before treatment with anti-thymocyte globulin [ATG] be the preferred treatment for children and adolescents with severe aplastic anemia who lack an HLA-matched related donor in 2023? *

- Yes
- No

Hidden unless: #4 Question "For what patient population do you provide care?" is one of the following answers ("Pediatric only","Both adult and pediatric")

22. Please predict the most common donor source in 2023 for children (age ≤ 18 years) with primary immune deficiency who lack a matched related donor by ranking the list of most commonly used donors by dropping the preferred donor into the right-hand list. If you anticipate a donor type will not be used, leave in the left-hand list. (1 = highest preference; 2 = second highest preference, etc; options left in left-hand list will not be used) *

Drag items from the left-hand list into the right-hand list to order them.

- Matched unrelated donor
- Haploidentical transplant TCRαβ+/CD19+-depleted grafts
- Haploidentical transplant with post-transplant cyclophosphamide
- Umbilical cord blood
- Mismatched unrelated donor

23. What do you predict to be the most common graft-versus-host disease (GVHD) prophylaxis for matched unrelated allogeneic stem cell transplant in the future? *

- Calcineurin inhibitor-based prophylaxis
- Post-transplant cyclophosphamide (Cytoxan)
- Graft manipulation or engineering strategies
- Novel agents, specify
- Other, specify

24. What will be the most common treatment for newly diagnosed acute GVHD in 2023? *

- Steroids
- Steroid in combination with another agent
- Infusions of regulatory cells, immunomodulatory molecules or antibodies
- Other, specify

25. Will treatment of acute GVHD primarily be based on clinical and/or biomarker risk stratification in 2023? *

- Clinical features
- Biomarker results
- Both clinical and biomarker features

26. What will be the most common treatment for newly diagnosed chronic GVHD in 2023? *

- Steroids
- Steroid in combination with other agents
- Infusions of regulatory cells, immunomodulatory molecules, or antibodies
- Other, specify:

27. Will high dose total body irradiation (TBI) be used as the most common conditioning regimens for adolescents and young adults (15-39 years old) with acute lymphoblastic leukemia in 2023? *

- Yes
- No

28. Will allogeneic HCT be routinely done entirely in the outpatient setting in 2023? *

- Yes
- No

29. In the setting of all hematologic malignancies, CAR-T or other immune cell therapies in 2023 will: *

- Completely replace HCT
- Reduce HCT numbers, but not eliminate them
- Serve as a bridge to HCT
- Will not affect HCT practice

30. How will CAR-T cell therapy or other immune cell therapies be used for treatment of multiple myeloma in 2023? (check all that apply) *

- Remain a modality for late lines of therapy for patient who have exhausted all other treatment options (relapse/refractory disease)
- Used in front line of therapy
- Used to eliminate residual disease prior to autologous HCT
- Used to eliminate residual disease after autologous HCT
- Replace autologous HCT

31. What will be the future application of CAR-T in non-Hodgkin lymphoma in 2023? (Check all that apply). *

- [ ] Remain a modality for late lines of therapy for patient who have exhausted all other treatment options (relapse/refractory disease)
- [ ] Used in front line of therapy
- [ ] Used to eliminate residual disease prior to autologous HCT
- [ ] Used to eliminate residual disease after autologous HCT
- [ ] Replace autologous HCT

32. Which of the following will be the first solid tumor to receive an FDA-approved indication for CAR-T therapy? *

- ○ Breast
- ○ Colorectal
- ○ Glioblastoma multiforme
- ○ Lung
- ○ CAR-T cell therapy will not be approved for treatment of solid tumors
- ○ Other (please specify):

33. Will patient reported outcomes (PROs) be incorporated into formal tools for risk stratification and treatment planning for allogeneic HCT recipients? (PRO is any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else) *

- ○ Yes
- ○ No

The following questions ask about your prediction of the results of current Blood and Marrow Transplant Clinical Trials Network (BMT CTN) studies

Hidden unless: #4 Question "For what patient population do you provide care?" is one of the following answers ("Adult only","Both adult and pediatric")

34. **BMT CTN 1101:** The randomized clinical trial of reduced intensity conditioning and transplantation of double unrelated umbilical cord blood (dUCB) versus haploidentical related bone marrow (Haplo) for patients with hematologic malignancies will show: *

- ○ No significant difference will be observed in the 2-year progression-free-survival (PFS) and overall survival (OS)
- ○ Higher 2-years PFS and OS with Haplo
- ○ Higher 2-years PFS and OS with dUCB
- ○ Higher 2-years PFS after Haplo with comparable OS rates between the two arms
- ○ Higher 2-years PFS after dUCB with comparable OS rates between the two arms
- ○ I cannot predict the results as I am not aware of this study

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35. **BMT CTN 1102:** Reduced intensity conditioning allogeneic HCT in patients 50 to 75 years of age with a history of de novo intermediate-2 or high-risk myelodysplastic syndrome by International Prognostic Scoring System (IPSS) compared to non-transplant/best supportive care will: *

- Improve three-year overall survival (OS)
- Have comparable 3-year OS but worsen quality of life
- Have comparable 3-year OS but improve quality of life
- Reduce OS compare to non-transplant/best supportive care
- I cannot predict the results as I am not aware of this study

36. **BMT CTN 1301:** Which one of the following GVHD prophylaxis regimens will provide the lowest rate of chronic GVHD/relapse-free survival (CRFS) in patients with AML and MDS undergoing myeloablative conditioning allogeneic HCT? *

- CD34 selected T-cell depleted peripheral blood stem cell graft
- Unmanipulated bone marrow graft followed by post-transplant cyclophosphamide
- Unmanipulated bone marrow graft with Tacrolimus/Methotrexate
- All three methods lead to comparable rate of CRFS
- I cannot predict the results as I am not aware of this study

37. **BMT CTN 1503:** In comparison to standard of care in adolescents and young adults with severe sickle cell disease, allogeneic HCT will: *

- Improve 2-year overall survival (OS)
- Reduce 2-year OS
- Have comparable 2-year OS
- I cannot predict the results as I am not aware of this study

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38. **BMT CTN 1506:** Two years of maintenance FLT-3 inhibitor, Gilteritinib, after allogenic HCT in patient with FLT-3 ITD positive AML in first morphologic remission (CR1) will: *

- Improve relapse-free survival and overall survival
- Improve relapse-free survival but have no significant impact on overall survival
- Not improve relapse-free survival
- I cannot predict the results as I am not aware of this study

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39. **ECOG-ACRIN EA4151/BMT CTN 1601:** Autologous HCT followed by maintenance rituximab compared to rituximab alone (without autologous HCT) for patients with mantle cell lymphoma in first complete remission with no evidence of minimal residual disease will: *

- Improve progression free survival (PFS) and overall survival (OS)
- Improve PFS but have no impact on OS
- Have comparable PFS and OS
- Reduce OS
- I cannot predict the results as I am not aware of this study

40. **BMT CTN 1702:** For patients with (AML, ALL, MDS, NHL, HL) without an HLA-identical sibling, is it better to proceed quickly to an alternative donor graft or to take longer to find a matched unrelated donor. *

- Survival is better with a matched unrelated donor (delay transplant to identify a matched unrelated donor)
- Survival is better with an alternative donor (proceed quickly to transplant)
- I cannot predict the results as I am not aware of this study

41. **BMT CTN 1703:** Which one of the following GVHD prophylaxis regimens will provide the best rate of GVHD-free, relapse-free survival (GRFS) in patients with malignant diseases receiving matched related or matched unrelated allogeneic PBSC transplant after a reduced intensity conditioning regimen? *

- Post-transplant cyclophosphamide / tacrolimus / mycophenolate mofetil
- Tacrolimus / methotrexate
- Both a and b lead to comparable rate of GRFS
- I cannot predict the results as I am not aware of this study

Hidden unless: #4 Question "For what patient population do you provide care?" is one of the following answers ("Adult only","Both adult and pediatric")

42. **BMT CTN 1704:** Will geriatric assessment measures have independent prognostic utility in older (age 50 years and older) allogeneic HCT recipients compared with standard clinical variables? *

- Yes
- No

The following questions ask about your prediction of the impact of patient-reported outcomes on therapy outcomes

Show/hide trigger exists.

43. Will patient reported outcomes (e.g., quality of life, symptoms) be used as primary endpoints in BMT CTN therapeutic studies? *

- Yes
- No

Hidden unless: #4 Question "For what patient population do you provide care?" is one of the following answers ("Adult only","Both adult and pediatric")

44. Many HCT survivors may feel stressed or be unsure of what health care they need. A self-management program plus a personalized survivorship care plan, as compared to a personalized survivorship care plan alone, will improve what? (check all that apply). *

- [ ] Health care adherence for cardio-metabolic surveillance
- [ ] Subsequent malignancy surveillance
- [ ] Distress level
- [ ] None of the above