

3701-84-27**APPENDIX A****Patient Selection Criteria**

- I. Patients transplanted on protocols: Any patient that is registered on and treated on a protocol that has been reviewed and approved by the National Institute of Health is an appropriate bone marrow transplantation (BMT) candidate.
- II. Criteria for eligibility for patients who do not meet the conditions of paragraph I, above:
 - A. General Considerations
 1. Pulmonary function: forced expiratory volume in one second (FEV1) and diffusing capacity for carbon monoxide (DLCO) are greater than forty-five percent predicted. If the FEV1 or DLCO is less than forty-five percent of predicted value, the patient should be evaluated by a pulmonologist. For pediatric patients 5 and under and those who failed an attempt to complete PFTs: Include venous blood gases (VBG). Exception to VBGs: If the patient is on room air and does not have a history of a lung comorbidity.
 2. Cardiology: multigated blood-pool imaging (MUGA) or echocardiogram showing the left ventricular ejection fraction of greater than or equal to forty-five percent. If a left ventricular ejection fraction is less than fifty percent, the patient must be consulted to and approved by a cardiologist to proceed to transplant. For pediatric patients, if a left ventricular shortening fraction by echocardiogram is less than twenty-nine percent, the patient must have an evaluation and clearance by a pediatric cardiologist prior to transplant.
 3. Renal: the patient must have a serum creatinine of less than two milligrams/milliliter or a glomerular filtration rate (GFR) of greater than or equal to sixty milliliters/minute/one point seven three meters squared. If the patient does not meet this criteria, a nephrology consult and clearance must be obtained prior to transplant, unless multiple myeloma is the known cause of the renal dysfunction.
 4. Hepatic: patients with abnormal hepatic enzymes (direct bilirubin, aspartate aminotransferase (AST) or alanine aminotransferase (ALT) greater than two times normal) need to be calculated and cleared by a gastroenterologist in order to proceed with transplant.
 5. Performance status: the patient must have an Eastern Cooperative Oncology Group (ECOG) performance status of zero, one, or two or a Lansky performance of seventy percent or greater.
 - B. Criteria for Exclusion

1. The presence of an uncontrolled infection
2. A female patient who is pregnant or lactating
3. A patient with known human immunodeficiency virus (HIV) disease unless receiving HAART therapy and having a negative viral load.
4. Patients with known metastatic intra-cranial disease (with the exception of metastatic germ cell tumors with treated intra-cranial disease or patients having neuroblastoma with active leptomeningeal disease)

C. Disease Indications for Allogeneic Bone Marrow Transplantation

1. Background

- a. Ablative bone marrow transplantation, including reduced intensity procedures.
- b. Non-myeloablative BMT: defined as infusion of hematopoietic cells after a nonmyeloablative preparative regimen for the purpose of attempting to treat a patients underlying disease with donor marrow and possibly the immunologic by-product of donor marrow.
- c. Source of stem cells
 - i. Five out of six-phenotypic antigen related or matched unrelated donor
 - ii. Haploidentical donor
 - iii. For cord blood, single or double units with a three-antigen mismatch or less by high resolution typing

2. Indications

- a. Acute leukemias
- b. Chronic myelogenous leukemia
- c. Chronic lymphocytic leukemia
- d. Myelodysplastic syndrome
- e. Severe combined immune deficiency (SCID)
- f. Wiscott-Aldrich syndrome
- g. Paroxysmal nocturnal hemoglobinuria

- h. Adrenoleukodystrophy, Hurlers syndrome, and other storage diseases
- i. Hodgkin's disease, excluding first complete remission
- j. Non-Hodgkin's lymphoma, excluding first complete remission
- k. Multiple myeloma
- l. Hemoglobinopathy
- m. Myeloproliferative disorder
- n. Juvenile myelomonocytic leukemia (JMML)
- o. Hemophagocytic lymphohistiocytosis (HLH)
- p. Bone marrow failure syndromes
- q. Osteopetrosis
- r. Aplastic anemia
- s. Neuroblastoma
- t. Renal cell carcinoma
- u. Sickle Cell Disease from matched related donor (donor without sickle disease) or haploidentical donor
- v. Other severe life-threatening immune deficiencies for which transplant has been shown to be effective

3. Indications for Repeat Blood or Marrow Infusion

- a. Graft rejection
- b. Disease relapse
- c. Engraftment failure

D. Indications for Donor Leukocyte Infusions for Relapsed or Persistent Hematologic Malignancy or Mixed Chimerism after Allogeneic BMT from Original Donor

- 1. General Considerations
 - a. Expected survival of greater than or equal to four weeks
 - b. Absence of active acute or chronic graft versus host disease (GVHD)

2. Indications: any patient treated with an allogeneic BMT suffering from disease relapse and/or persistent mixed chimerism

E. Disease Indications for Autologous Hematopoietic Cell Transplantation

1. Indications
 - a. Hodgkin's disease, excluding first complete remission
 - b. Non-Hodgkin's lymphoma
 - c. Acute myelogenous leukemia
 - d. Acute lymphoblastic leukemia
 - e. Chronic myelogenous leukemia
 - f. Neuroblastoma (including active leptomeningeal disease)
 - g. Germ cell tumors (including treated intracranial disease)
 - h. Plasma Cell Neoplasms (including multiple myeloma; smoldering myeloma; light chain deposition disease; polyradiculoneuropathy, organomegaly, endocrinopathy, monoclonal plasma cell disorder, and skin changes (POEMS); amyloidosis; and monoclonal gammopathy of renal significance)
 - i. Ovarian cancer
 - j. Pediatric tumors (up to age twenty-one)
 - i. Brain tumors
 - ii. Wilms tumor
 - iii. Soft tissue sarcomas
 - iv. Ewing's family of tumors
 - v. Desmoplastic small round cell tumors
 - vi. Neuroblastoma
 - k. For pediatric patients with primary CNS tumors: No progression of metastatic intracranial and or leptomeningeal disease since original diagnosis
 - l. Refractory relapsing multiple sclerosis

- m. Systemic sclerosis
 - 2. Repeat transplants
 - a. Second transplants are acceptable if they are part of the “initial therapy.” Thus, a protocol that demands multiple sequential myeloablative therapies as a planned treatment course is appropriate if the patient is treated on such a protocol.
 - b. Treatment of relapse: if a patient achieved a complete remission after the last autologous transplant and that complete remission lasted a minimum of one year, then a second transplant is potentially reasonable. A second transplant at less than one year requires a case review as established under paragraph (B) of rule 3701-84-27 of the Administrative Code.
- F. Indications for Hematopoietic Stem Cell Harvesting Without a Planned Transplant
 - 1. Acute myelogenous leukemia: patients in complete remission
 - 2. Acute lymphoblastic leukemia: patients in complete remission
 - 3. Matched unrelated donor: patients undergoing a matched, unrelated donor allogeneic transplant with marrow used as “back up”
 - 4. Haploidentical donor patients undergoing a haploidentical transplant which have HLA antigens with marrow harvest or peripheral cell harvest, whichever is determined the most suitable procedure for the recipient
 - 5. Pediatric tumors: (Wilms’ tumor, soft tissue sarcoma, neuroblastoma, brain tumors, Ewing’s family of tumors) and lymphomas in complete remission to receive future pelvic radiation therapy.
 - 6. Multiple myeloma
- G. Any other disease treated with bone marrow, stem cell or cord blood transplant must be an accepted indication for transplantation or done with a formal treatment/clinical research protocol that is approved by the transplant institution’s Internal Review Board (IRB) and renewed on an annual basis.