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 (FEVI) and diffusing capacity for carbon monoxide (DLCO) are greater than forty-five percent predicted. If the FEVI 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.