# Pediatric Blood and Marrow Transplant Program

## Document Information

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PBMT-GEN-050
PEDIATRIC BLOOD AND MARROW TRANSPLANT PROGRAM
PATIENT SELECTION

1 PURPOSE
1.1 To provide guidance for the selection of patients for the Pediatric Blood and Marrow Transplant (PBMT) Program.

2 INTRODUCTION
2.1 The patient selection process is a key component to the transplantation process.
2.2 Patients are evaluated for risk and benefit of the transplantation process to determine candidacy and optimize patient outcomes.
2.3 Selection processes rely on excellent and coordinated communication across the health care team, including referral teams.

3 SCOPE AND RESPONSIBILITIES
3.1 The attending physician is primarily responsible for the patient selection process and the contents of this procedure.
3.2 The nurse coordinator serves as a key member of the patient selection team.
3.3 The licensed clinical social worker (LCSW) is responsible for assessment of the patient for psychosocial barriers to transplantation.

4 DEFINITIONS/ACRONYMS
4.1 BAER Brainstem Auditory Evoked Response
4.2 CIBMTR Center for International Blood and Marrow Transplant Research
4.3 CSF Cerebrospinal Fluid
4.4 CT Computed Tomography
4.5 DTI Diffusion Tensor Imaging
4.6 EEG Electroencephalogram
4.7 HLA Human Leukocyte Antigen
4.8 PBMT Pediatric Blood and Marrow Transplant
4.9 LCSW Licensed Clinical Social Worker
4.10 LP Lumbar Puncture
4.11 MRI Magnetic Resonance Imaging
4.12 MRSA Methicillin-Resistant Staphylococcus Aureus
4.13 VEP Visual Evoked Potential
4.14 VRE Vancomycin-Resistant Enterocci
5 MATERIALS
5.1 N/A

6 EQUIPMENT
6.1 N/A

7 SAFETY
7.1 N/A

8 PROCEDURE
8.1 Referral to the Program
   8.1.1 Patients are referred by their local physicians or through direct parental
         contact by phone or through email, most commonly directed to
         individual PBMT physicians or nurse coordinators.
   8.1.2 Occasionally, third party payer case managers or the Duke Referral
         Center generates referrals.

8.2 Initial consultation
   8.2.1 Prior to initial consult, insurance clearance will be obtained.
   8.2.2 The PBMT physician and/or nurse coordinator is responsible for
         returning patient calls and/or sending a secure email. The team will
         conduct initial interviews with the parent(s) and /or legally authorized
         representative(s) (LARs) and perform an informal screening to
         determine if the patient should come for a consultation.
   8.2.3 Parent(s) and/or the legally authorized representative(s) may also
         request a telephone or on-site consultation to learn more about the Duke
         PBMT program without a formal evaluation of their child.

   8.2.3.1 During on-site visits, the patient, parent(s) and/or legally
         authorized representative(s) will:

   8.2.3.1.1 Receive information about clinical team and
         structure including but not limited to the
         transplant physician, nurse coordinator, pediatric
         licensed clinical social worker (LCSW), financial
         coordinator, and family support program.

   8.2.3.1.2 Receive a tour of the PBMT inpatient unit and
         outpatient setting, including the clinic and
         infusion areas. If time does not allow for a tour,
         the tour will occur during subsequent visits.

   8.2.3.1.3 Receive overview information about the
         transplant process.

8.2.4 The attending physician will have opportunity to review all pertinent
         and available records. Depending on patient status and time allotment,
         the physician, or designee, will obtain a comprehensive history, and will
conduct a physical examination, if required. If time or situation does not allow for this in the consult visit, this will be performed at subsequent workup visits.

8.3 Longitudinal Evaluation/Pre-transplant “workup”

8.3.1 If the parents identify Duke as their choice for a transplant center, and if initial screening does not identify a reason for ineligibility for transplant, a primary clinical team will be assigned to the patient.

8.3.1.1 The primary clinical team will include an attending physician, an advanced practice provider/and or fellow, and a nurse coordinator.

8.3.1.2 The primary clinical team will coordinate the patient workup. The primary nurse coordinator will schedule each aspect of the initial workup.

8.3.1.3 The primary nurse coordinator team will work directly with the primary attending physician to obtain relevant records and to determine the required studies for evaluation of the applicable patient.

8.3.1.3.1 Medical records from the patient’s treating physicians will be obtained.

8.3.1.3.2 All patient workups will include:

- Organ function screening with blood work
- Pulmonary function tests
- Echocardiogram
- Chest X-ray
- Infectious disease screening
- Human leukocyte antigen (HLA) typing (if indicated)
- Endocrine and growth function screening
- Pregnancy testing, if the patient is a female of childbearing age with signs of puberty
- Dental health; age appropriate
- Cultures of stool (if symptomatic), urine, skin, for colonization with methicillin-resistant staphylococcus aureus (MRSA), vancomycin-resistant enterococci (VRE), or viral pathogens
- Computed Tomography (CT) of the brain/sinus/chest/abdomen/pelvis to screen for opportunistic infection
8.3.2.1 Any unanticipated findings will be addressed.

8.3.2.2 Results will be reviewed and discussed with the patient’s parent(s) and/or legally authorized representative(s) and final candidacy for transplantation will be determined.

8.4 Transplant Eligibility Determination

8.4.1 Specifications for eligibility for each diagnosis have been determined and will include the following:

- Neurocognitive testing
- Vision screening; with more specialized testing for patients with metabolic diseases
- Hearing screening; with more specialized testing for patients with metabolic diseases
- Disease restaging in patients with malignancies, individualized for the patient. The patient will receive applicable scans if bulky disease was present in past manifestations of disease.

8.3.1.3.3 Additionally:

- Patients with hematological malignancies will be assessed with bone marrow aspirates and biopsies, lumbar puncture (LP) for cerebrospinal fluid (CSF) cytopathology.
- Patients with a history of chronic transfusion will be assessed with studies to determine iron overload, liver and cardiac iron burden.
- Patients with inborn errors of metabolism will be assessed for neurologic, skeletal, cardiac, developmental manifestations by the clinical manifestations of the disease.
- Patients with leukodystrophies will be assessed with brain MRI (magnetic resonance imaging), electroencephalogram (EEG), visual evoked potential (VEP), brainstem auditory evoked response (BAER), nerve conduction studies and neurodevelopmental-neurocognitive testing.

8.3.1.4 The LCSW will assess all new patients for psychosocial barriers to transplantation.
8.4.1.1 Patients must:

8.4.1.1.1 Be expected to complete the preparative regimen
8.4.1.1.2 Have adequate organ function
8.4.1.1.3 Be free of active uncontrolled infections
8.4.1.1.4 Have an available caretaker including a back-up care taker

8.4.2 If the patient is deemed a transplant candidate:

8.4.2.1 The parent(s) and/or legally authorized representative(s) will be extensively educated about the plan for treatment, possible risks and benefits, late effects and logistics of treatment at Duke.

8.4.2.2 Discussions regarding appropriateness for transplant and best approach will be reviewed.

8.4.2.2.1 Pertinent discussion points will be transmitted to the referring physician by phone or fax and documented in the patient’s electronic medical record.

8.4.2.3 Final donor selection and eligibility will be determined. (See related SOPs: APBMT-COMM-001 Donor Selection, Evaluation and Management.)

8.4.2.4 Third party coverage and prior approval will be documented.

8.4.2.5 Eligibility for active protocols will be considered and identified, if appropriate. The patient (if age appropriate), parent(s) and/or legally authorized representative(s) will sign the informed consent for transplant and for reporting of outcomes data to the CIBMTR.

8.5 Documentation and Multidisciplinary Communication

8.5.1 A summary of all patient consultation visits, workup visits, and pertinent discussions will be documented in the patient’s electronic medical record.

8.5.2 Visit summaries, including pertinent discussion points, will be provided to the referring physician via fax, phone or secure email.

8.5.3 The primary care team will be able to present new patients to the PBMT clinical team in the weekly multidisciplinary new patient meetings.

9 RELATED DOCUMENTS/FORMS

9.1 APBMT-COMM-001 Donor Selection, Evaluation and Management

10 REFERENCES

10.1 N/A
## 11 REVISION HISTORY

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<td>Longitudinal Evaluation/Pre-transplant “workup” Section updated to include the following additions:</td>
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<td>- Dental health; age appropriate</td>
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