



PEDIATRIC BLOOD AND MARROW TRANSPLANT PROGRAM

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Pediatric Blood and Marrow Transplant Program Patient Selection

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PEDIATRIC BLOOD AND MARROW TRANSPLANT PROGRAM

PATIENT SELECTION

1 PURPOSE

- 1.1 To provide guidance for the selection of patients across the continuum of care in the Pediatric Transplant and Cellular Therapy (PTCT) Program. The PTCT Program encompasses hematopoietic cell transplantation (HCT) transplant and cellular therapies.

2 INTRODUCTION

- 2.1 The patient selection process is a key component for potential recipients of HCT or cellular therapies.
- 2.2 Patients are evaluated for potential benefits and potential risks 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 HCT Hematopoietic cell transplantation
- 4.8 HLA Human Leukocyte Antigen
- 4.9 PBMT Pediatric Blood and Marrow Transplant
- 4.10 PTCT Pediatric Transplant and Cellular Therapy
- 4.11 LCSW Licensed Clinical Social Worker
- 4.12 LP Lumbar Puncture
- 4.13 MRI Magnetic Resonance Imaging

- 4.14 MRSA Methicillin-Resistant Staphylococcus Aureus
- 4.15 VEP Visual Evoked Potential
- 4.16 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 email to individual program 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 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 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 coordinators, pediatric nurse practitioners, pediatric licensed clinical social worker (LCSW), financial coordinator, and family support program.
 - 8.2.3.1.2 Receive a tour of the inpatient unit and outpatient setting, including the clinic and infusion areas.
 - 8.2.3.1.3 Receive overview information about the transplant or cellular therapy process.

- 8.2.4 The attending physician will have opportunity to review all pertinent and available records. As part of the selection process, the physician, or designee, will obtain a comprehensive history, and will conduct a physical examination. 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 center, and if initial screening does not identify a reason for ineligibility for transplant or cellular therapy 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
 - 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 For HCT Transplant workups, additionally:

- Computed Tomography (CT) of the brain/sinus/chest/ abdomen/pelvis to screen for opportunistic infection
- Neurocognitive testing
- Vision screening; with more specialized testing for patients with metabolic diseases
- Hearing screening; with more specialized testing for patients with metabolic diseases

8.3.1.3.4 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 or cellular therapies.

8.3.2 Results of all studies from the pre-transplant workup will be collated and reviewed by the primary clinical care team.

8.3.2.1 Any unanticipated findings will be addressed.

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

8.4 Eligibility Determination

- 8.4.1 Specifications for eligibility for each diagnosis have been determined and will include the following:
 - 8.4.1.1 Transplant Recipients 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
 - 8.4.1.2 Recipient of cellular therapy must:
 - 8.4.1.2.1 Meet requirements for therapy as outlined by regulatory requirements and/or treatment protocols
 - 8.4.1.2.2 Have an available caretaker
- 8.4.2 If the patient is deemed a candidate for either transplant or cellular therapy:
 - 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 treatment and best approach will be reviewed.
 - 8.4.2.2.1 Pertinent discussion points will be transmitted to the referring physician, if needed, and documented in the patient's electronic medical record.
 - 8.4.2.3 For transplant candidates, 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. Similar outcomes data will be captured and reported for cellular therapy patients as required by CIBMTR, the sponsor, and/or other regulatory requirements.

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 available to the referring physician.
- 8.5.3 The primary care team will present new patients to the full 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

Revision No.	Author	Description of Change(s)
07	Sally McCollum	<ul style="list-style-type: none"> - Section 1.1 –scope broadened to include PTCT - Section 8 – cellular therapy scope added throughout - Section 8 - minor wording revisions throughout to improve document flow - Section 8.3 – information grouped according to HCT or CT - Section 8.4 - information grouped according to HCT or CT - Section 8.4.2.5 – outcomes data requirement added

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