Hematology/Oncology/BMT

Short Name	Comments	Type	Investigator	Contact
	Hematology			
Kid-DOTT	**Prospective Evaluation of the Duration of Therapy for Thrombosis in Children (the "Kids-DOTT" Trial)** *This study plans to learn more about the best type of treatment for children with blood clots in the veins. This study is being done to find out how long to treat blood clots in a vein by best balancing the risks of bleeding versus clotting again. The study involves using the follow-up pictures (scans) of the clot, performed as part of standard care after the initial treatment of a clot, to help determine the total length of treatment needed. Another part of this study involves learning whether different blood thinning medicines affect long-term outcomes. A third part of this study involves collecting blood samples for testing that is designed to look for new risk factors for blood clots and their long-term effects.* For More Information	Treatment	Mitchell, Deanna	Kathy Nystrom Kathy.Nystrom@spectrumhealth.org (616) 486-2062
	BMT			
BMT Cord Blood Access Protocol	**A Multicenter Access and Distribution Protocol for Unlicensed Cryopreserved Cord Blood Units (CBUs) in Pediatric and Adult Patients with Hematologic Malignancies and other Indications** *This is an access study in which umbilical cord blood used for bone marrow transplant is only available through this access and distribution study. In October 2011, the Food and Drug Administration (FDA) began considering cord blood as a biological drug. In the United States, drugs must meet standards set by the FDA to make sure they are safe. Cord blood units that were not collected, tested, or stored exactly according to FDA standards may be used for transplant if the transplant is done as part of a study.* For More Information	Symptom Management	Abdel-Mageed, Aly	Emily Gleason Emily.Gleason@helendevoschildrens.org (616) 486-6332

NYBB UCB	**A multicenter safety study of unlicensed, investigational cryopreserved cord blood units (CBUs) manufactured by the National Cord Blood Program (NCBP) and provided for unrelated hematopoietic stem cell transplantation of pediatric and adult patients** *Umbilical cord blood used for bone marrow transplant which is only available through this access and distribution study. In October 2011, the Food and Drug Administration (FDA) began considering cord blood as a biological drug. In the United States, drugs must meet standards set by the FDA to make sure they are safe. Cord blood units that were not collected, tested, or stored exactly according to FDA standards may be used for transplant if the transplant is done as part of a study.*	Treatment	Abdel-Mageed, Aly	Kathy Nystrom Kathy.Nystrom@spectrumhealth.org (616) 486-2062
Multiple Cord Blood	**Allogeneic Stem Cell Transplantation Using Multiple Unrelated Donor Umbilical Cord Grafts** *This research study will evaluate the ability of umbilical cord blood cells, which have been obtained from multiple unrelated donors, to serve as a source of stem cells for patients undergoing stem cell transplant. The use of a single unrelated umbilical cord blood unit is common for small pediatric patients requiring stem cell transplantation when a related or unrelated donor is not available. For larger patients, the use of more than one umbilical cord blood unit provides an adequate number of stem cells and increases the chances of successful engraftment and survival.*	Treatment	Abdel-Mageed, Aly	Kathy Nystrom Kathy.Nystrom@spectrumhealth.org (616) 486-2062
CliniMACS T-Cell Depletion	**An Expanded Access Study of the Feasibility of Using the CliniMACS® Device for CD34+ Cell Selection and T-Cell Depletion for Graft-versus-Host Disease Prophylaxis in Alternative Donor Stem Cell Transplant Recipients** *This research study will help doctors learn about how to prevent serious side effects that can happen when a patient receives a stem cell transplant that uses cells from an alternative donor. This will allow your doctor to use a potentially lifesaving investigational treatment that is not FDA-approved for use in the treatment of the recipient's disease at this time. This protocol will also allow researchers to gather additional information about the safety and effectiveness of using the CliniMACS® device to help prevent GVHD after an alternative-donor transplant.*	Symptom Management	Abdel-Mageed, Aly	Emily Gleason Emily.Gleason@helendevoschildrens.org (616) 486-6332

BMT CTN 1507 Haplo BMT for SCD	**Reduced Intensity Conditioning for Haploidentical Bone Marrow Transplantation in Patients with Symptomatic Sickle Cell Disease (Posted on clinicaltrials.gov as NCT03263559)** *This study is designed as a Phase II multi-center trial evaluating the feasibility of a high rate of event-free survival (EFS) at 2 years post transplant using pre-conditioning hydroxyurea (HU) with a conditioning regimen of Thymoglobulin/Cyclophosphamide/Fludarabine/Thiotepa/Total Body Irradiation with post-grafting high-dose cyclophosphamide in patients with severe SCD who have HLA-haploidentical donors. This is a single arm study in which participants will be enrolled into one of two strata. The first stratum will be restricted to children age 5 - 14.99 years who have stroke. The second stratum will consist of adult patients age 15 - 44.99 years with severe sickle cell disease. HDVCH is only enrolling patients age 5 - 21.99 years at the time of Segment A Enrollment.*	***Treatment***	*Duffner, Ulrich*	Emily Gleason Emily.Gleason@helendevoschildrens.org (616) 391-5075
PBMTC SUP1701 ACES	**Antiviral Cellular Therapy for Enhancing T-cell Reconstitution Before or After Hematopoietic Stem Cell Transplantation (ACES) PBMTC SUP1701 (Posted on clinicaltrials.gov as NCT03475212)** *The purpose of this study is to evaluate whether virus-specific T cell lines (VSTs) are safe and can effectively control three viruses (EBV, CMV, and adenovirus) in patients who have had a stem cell transplant and also in patients that have a primary immunodeficiency disorder with no prior stem cell transplant. Patients after any type of allogeneic hematopoietic stem cell transplant and patients with a primary immunodeficiency disorder diagnosis with persistent CMV, adenovirus and/or EBV infection that persists despite standard therapy are eligible for enrollment. Eligible patients must be ≤25 years old at the time of enrollment.*	***Treatment***	*Duffner, Ulrich*	Emily Gleason Emily.Gleason@helendevoschildrens.org (616) 391-5075
	Other			
Rare Disease Registry (Registry)	**Genzyme Rare Disease Registry (Gaucher, Pompe, Fabry, and MPS I)** *The Rare Disease Registry sponsored by Genzyme collects information on patients with Gaucher, Pompe, Fabry, and mucopolysaccharidosis type I (MPS I) diseases to better understand how to prevent, diagnose and treatment them.* For More Information	Registry	Adams, Stacie	Rosa Sifontes-Escamilla Rosa.Sifontes-Escamilla@helendevoschildrens.org (616) 486-6342

	Protocols that are NOT disease site specific			
Brincidofovir	**Expanded Access Protocol to Provide Brincidofovir for the Treatment of Serious Adenovirus Infection or Disease** *This study is an access protocol to provide treatment of the investigational drug called brincidofovir (or BCV for short) to treat the virrus called adenovirus (or AdV).* For More Information	Symptom Management	Duffner, Ulrich	Laura Paulsen cancer.research@spectrumhealth.org (616) 486-8500
Edoxaban	**A Phase 3, Open-Label Randomized, Multi-Center, Controlled Trial to Evaluate the Pharmacokinetics and Pharmacodyamics of Edoxaban and to Compare the Efficacy and Safety of Edoxaban with Standard of Care Anticoagulant Therapy in Pediatric Subjects from Birth to Less Than 18 Years of Age with Confirmed Venous Thromboembolism (VTE)** The primary objective is to demonstrate the non-inferiority of edoxaban to standard of care (SOC; including low molecular weight heparin (LMWH), vitamin K antagonist (VKA), or synthetic pentasaccharide (SP) Xa inhibitors) in the treatment and secondary prevention of VTE in pediatric subjects with regard to the composite efficacy endpoint (ie, symptomatic recurrent VTE, death as result of VTE, and no change or extension of thrombotic burden) during the first 3-month treatment period.		Braunreiter, Chi	Kathy Nystrom Kathy.Nystrom@spectrumhealth.org (616) 486-2062