

# Dr. Paula James

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HDH Department – Hematology Clinic

**Fields of Specialization:** Hematology (Adult) - bleeding disorders

**Keywords:** Bleeding, von Willebrand disease, hemophilia

## Current Studies Recruiting Patients at HDH

Title of Study: **Determinants of health-related quality of life among patients with von Willebrand Disease**

This research study is looking at the health burden among people living with von Willebrand disease (VWD). Currently, the quality of life among VWD patients has not been clearly established, especially in Canada. We would like to learn how living with VWD has affected the daily lives of these patients, as a first step towards potentially coming up with strategies to improve their quality of life. This study is recruiting patients previously diagnosed with VWD. A summer student award was granted to Yan Xu (3<sup>rd</sup> year medical student) by the Canadian Hemophilia Society to carry out this project.

Title of Study: **Generation and Validation of the Self-Administered Bleeding Assessment Tool (Phase 3)**

In Phases 1 & 2 we generated and validated a self-administered bleeding questionnaire that patients could complete themselves as a tool to identify abnormal bleeding. The final questionnaire showed it could discriminate between people diagnosed with bleeding disorders and those without as well as the previously validated expert administered questionnaire. This phase of the research study is to test how well this self-administered bleeding questionnaire works as a screening tool for inherited bleeding disorders in patients referred to hematology because of bleeding symptoms. This study is funded by an Investigator Initiated Grant from CSL Behring.

Deforest M, Grabell J, Tuttle A, Thibeault L, Hopman W, James P. Generation and Optimization of the Self-Administered Bleeding Assessment Tool (Self-BAT). *Haemophilia* 2012; 18: Suppl 3 FP-TU-04.3-4.

Title of Study: **Surveillance of Safety and Efficacy of wilate in patients with von Willebrand Disease (Wil-20)**

This is a non-interventional, surveillance study designed to collect data about the regular treatment of patients diagnosed with von Willebrand disease (VWD) with a drug called wilate during surgeries or procedures. The study is funded by Octapharma, the manufacturers of wilate. Any patient previously diagnosed with VWD undergoing a surgery or procedure requiring replacement therapy is eligible.

Title of Study: **A Genome Wide Association Study (GWAS) to Determine Factors that Contribute to Mucocutaneous Bleeding**

This study aims to determine how well a previously validated bleeding questionnaire works as a screening tool for inherited bleeding disorders and to identify genetic changes that cause mucocutaneous (mucous membrane and skin) bleeding. The study is funded by the Baxter

Canadian Hemophilia Epidemiological Research Program (B-CHERP), and is open to newly referred patients having investigations for problems with bleeding or bruising.

Jackson S, Poon M-C, Grabell J, Lillicrap D and James P. The Condensed MCMDM-1 VWD Bleeding Questionnaire: Utility as a Diagnostic Tool in the Hematology Clinic. ISTH Kyoto 2011 P-TU-459.

**Title of Study: Validation of the ISTH-BAT (International Society on Thrombosis and Haemostasis Bleeding Assessment Tool) in Hemophilia Carriers**

This is a research study to validate the ISTH-BAT (International Society on Thrombosis and Haemostasis Bleeding Assessment Tool) in hemophilia carriers. The ISTH-BAT is a standardized questionnaire that is currently used in other inherited bleeding disorders, but has not yet been assessed in the hemophilia carrier population. This study is open to anyone who has been diagnosed as a Hemophilia carrier. It is an international, multi-center study being carried out within the context of GEHEP (Global Emerging HEMophilia Panel). Members of GEHEP are from North and South America, Europe and South Africa. The study is funded by an Investigator Initiated Grant from Bayer.

**Title of Study: Validation of the Self BAT (Self-Administered Bleeding Assessment Tool) in Hemophilia Carriers.**

This is a research study to validate the Self-Administered Bleeding Assessment Tool in hemophilia carriers. The Self-BAT has been shown to be effective in differentiating between people with Type 1 VWD and people without bleeding disorders. This study is to investigate the use of the Self-BAT in hemophilia carriers and to quantify their bleeding symptoms to compare with groups with other inherited bleeding disorders.

This study is open to anyone who has been diagnosed as a Hemophilia carrier. An application for funding has been made to the World Federation of Hemophilia Clinical Research Grant Program.

**Title of Study: Assessment of Bleeding Symptoms in Individuals with Bleeding Disorders using a Comprehensive Bleeding History Phenotyping System**

A research study for adults who have been identified as having Type 1 Von Willebrand Disease (VWD). This research study is designed to validate two Web-based Bleeding Questionnaires developed by researchers at Rockefeller University in New York. Bleeding Questionnaires are tools which help identify people that have a problem with bruising and/or bleeding symptoms. The Bleeding Questionnaire we use routinely is paper-based, and the data must be manually entered into our database. In this study we are collecting information on adults who have been diagnosed as having Type 1 VWD using two Web-based Bleeding Questionnaires. These systems enable all data to be captured electronically.

In addition, by employing new methods of analysis we are hoping to find new relationships between bleeding symptoms and other variables

**Title of Study: Generation and Validation of the Self-Administered Pediatric Bleeding Questionnaire (Self-PBQ)**

The doctors conducting this study have previously developed a tool called The Pediatric Bleeding Questionnaire which helps to identify children that have a problem with bruising and/or bleeding. This tool must be administered by an expert. We believe that the modification of this screening tool into a format in which the child and/or their parent/ward could fill out themselves would be of even greater benefit in helping doctors understand the needs of these children and young adults. We are currently recruiting children known to have Type 1 VWD and healthy

controls, and will soon be moving on to recruit children referred to a specialist for a problem with bleeding and/or bruising. Funding has been awarded by C17 Pediatric Research Network.  
Rand M, Grabell J, Moorehead P, Deforest M, Wu J, Steele M, Klaassen R, Silva M, James PD. Development of the Self-PBQ (Self-administered Pediatric Bleeding Questionnaire): Pre-testing and optimization. *J Thromb Haemost*, 2013; 11(Suppl 2), PA 1.09-6