

Prevalence and treatment of cardiovascular disease in congenital bleeding disorders

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This project will involve:

1. Mentored training of a young hematologist in the diagnosis, evaluation and planning of management strategies in patients with bleeding disorders; 2. Investigation of the prevalence of cardiovascular disease (CVD) in the congenital bleeding disorder population through an epidemiological single center analysis (i.e. a population-based natural history cohort study).

The evaluation for CVD in at-risk groups will include the indicated screening tests and imaging. The project will record the following information in all patients found to have cardiovascular complications:

1. Treatment of the bleeding disorder, including:

a. Therapy and regimen utilized for correction of the bleeding disorder in patients undergoing invasive procedures

b. Adverse events (AEs) related to either inadequate replacement, or procoagulant overload (e.g. thrombotic events)

2. CVD treatment including:

a. Therapy and regimen utilized

for treatment of CVD

b. AEs due to these treatment or interventions (e.g. bleeding) or related to inadequate treatment (e.g. restenosis, recurrent stroke, etc)

The last decade has seen significant advances in safety and availability of replacement products for bleeding disorders. Furthermore, treatment of bloodborne infections (HIV, hepatitis C & B) has improved. Thus, an increased longevity is observed in patients with bleeding disorders.

CVD (i.e. CAD, MI, stroke) is the leading cause of morbidity and mortality in the general population, and is now emerging in the aging bleeding disorder population. Despite the high prevalence of CVD, optimal interventions and outcome are not well established. There are no published guidelines specific to this population with regard to:

1. Prediction of the patients at the highest risk

2. Evaluation of the impact of replacement regimens such as prophylaxis with intermittent normalization of coagulation

3. Need, optimal use and outcome of anti-platelet agents and/or other anticoagulants, and the supportive infusion regimen required.

Additionally, there are insufficient new hematologists dedicating their careers to the field of hemostasis and thrombosis.

The project objectives are:

1. To provide mentored training in diagnosis, evaluation and planning of management strategies in patients with bleeding disorders to a young physician early in the course of

their hematology career. This mentored training will occur via:

a. Integral participation in the evaluation and care of all patients at the Indiana Hemophilia & Thrombosis Center (IHTC), the largest hemophilia treatment center in the United States

b. Design, conduct and analysis of a key clinical research project by the trainee

2. To determine the prevalence of CVD in a bleeding disorder population

3. To determine optimal treatment strategies for CVD and treatment sequelae.

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