

## AHCDC Inhibitor Committee

Annual Report 2018 – 19

### 1. Emicizumab Review Process

The Inhibitor Committee has been involved in reviews of Emicizumab through CBS (P Moorehead along with B Ritchie provided medical review) and CADTH (data related to anticipated usage provided by Inhibitor Committee and CBDR). The Inhibitor Committee was offered the opportunity to review the CADTH report prior to its release, but declined to do this because there would not have been sufficient time to provide meaningful feedback.

### 2. Emicizumab Guide

A guide to the use of Emicizumab for FVIII inhibitor patients is currently being drafted by members of the Inhibitor Committee and the Novel Therapies Committee (A Lee, N Rydz, P Moorehead). It is hoped that this document will be drafted, edited, reviewed by the two contributing committees, and ready for distribution to the membership by August.

### 3. Inhibitor Management Survey

A brief survey of the current management of FVIII inhibitors at Canadian centres is being prepared by N Rydz. The purpose of this survey is to provide baseline pre-Emicizumab data, so that a followup survey can assess the impact of Emicizumab on the FVIII inhibitor management landscape.

### 4. Inhibitor Data in CBDR

The Inhibitor Committee has been assisting CBDR with the implementation of a new logic system for classifying the inhibitor status of patients in CBDR.

### 5. CANHC Representation on Inhibitor Committee

The Inhibitor Committee has agreed in principle to CANHC representation on the Committee. We are waiting to hear back from CANHC as to whether representation on this committee would be of interest.

6. Study: The Genetic Basis of FVIII Inhibitor Development

The Inhibitor Committee strongly encourages Canadian centres to consider participation in a new multicenter international study. GENESIS is just getting underway to provide a comprehensive genome-wide evaluation of loci associated with inhibitor generation. The study will conduct whole genome sequencing and genome-wide association analysis on 500 severe hemophilia A patients with past or current high titer (>5 BU) FVIII inhibitors and 3,000 severe HA patients with no inhibitor history. Two factors will facilitate enrollment - a) Central IRB guidance concerning the genetic information that will be generated and b) Individual study site funding will be available with additional per patient funding. The study leader is Dr. Dan Hart and the study sponsor is Sanofi.

Paul Moorehead MD FRCPC  
for the Inhibitor Committee