

Annual Report, Novel Therapeutics Committee May 2022

The Committee welcomed Dr. Victor Blanchette as a member. The other members are Jerry Teitel (Chair), Molly Warner, Adrienne Lee, Jayson Stoffman, and Mark Belletrutti.

In light of the recent introduction of emicizumab to the formulary of both the CBS and HémaQuébec, the Committee considered options for potential research projects, and for assistance to AHCDC members in laboratory monitoring. Dr. Davide Martino joined us as a guest at a recent (virtual) meeting to describe several projects which might be of interest to our members.

- The HEMNOR project is an investigator-initiated 4-year project to study the efficacy and safety of Hemlibra in Canada. The principal investigators are Dr. Poon and Dr. Lee. Data will be captured from CBDR so it will impose minimal workload on participating HTC's.
- Calibra is an app that works through WAPPS-Hemo. It is designed to recommend regimens that allow for adjustment of dosing intervals of Hemlibra that maintain target plasma levels with minimal injection volume and without having to discard any volume from a vial. Calibra is up and running.
- Hemalytic is a Hamilton company whose owner is a patient at the McMaster HTC. Dr. Iorio is a consultant to the company. Hemalytic offers services that can expedite and simplify clinical trial initiation, assisting with REB and contract submissions and other administrative activities. Using Hemalytic's services could reduce institutional overhead costs by taking on tasks that would otherwise be performed by hospital research offices.

The Committee drafted a letter of support for the AHCDC Executive to forward to the Québec Ministry of Health as it was considering the introduction of emicizumab. In it we recommended that it be made available for all Hemophilia A patients with a severe clinical phenotype, rather than for "severe Hemophilia A patients", the criterion being followed by the CBS. This expanded access criterion was in fact ultimately approved by HémaQuébec.

Anticipated Committee activities in the coming year:

- The Committee will assist AHCDC in lobbying CBS for expanded access to Hemlibra for prophylaxis in selected patients with non-severe hemophilia.
- We will undertake a survey of our members to determine whether enrolment in clinical trials of new products can be offered to patients from HTC's which are not study sites, and to establish which sites are able to accept samples for laboratory testing.
- Dr. Warner informed the Committee that she has access to research funding for which we may be able to provide assistance in project design or recruitment.

- The Committee will revisit its mandate, its membership, and its leadership.

Jerry Teitel
Committee Chair