



Canadian Hemophilia Society
Help Stop the Bleeding
Société canadienne de l'hémophilie
Arrêtons l'hémorragie

PROVINCES STALL ON ACCESS TO LIFE-CHANGING HEMOPHILIA THERAPY

Montreal, May 6, 2019 – The Canadian Hemophilia Society (CHS) calls on the Provinces and Territories to stop stalling and immediately make Hemlibra® (emicizumab) available to all Canadians with hemophilia A and an inhibitor to factor VIII. The case of young [Callum Guthrie](#) from Nova Scotia illustrates the urgency.

Patients like Callum affected by this rare and serious complication—an inhibitor to factor VIII—are at constant risk of life- and limb-threatening bleeding. Current therapies are only marginally effective and extremely burdensome.

“Hemlibra represents a rare win/win/win opportunity,” said CHS President, Paul Wilton. “Dramatic **benefits in health outcomes** over existing therapies, **life-changing improvements** in quality of life for patients and their families, and significant **cost savings** for the health system. The Provinces and Territories need to stop stalling and say yes today.”

Hemlibra was approved for patients with hemophilia A and inhibitors by Health Canada on August 2, 2018, and has become the standard of care in the U.S. and many European countries. It has received all necessary approvals in Quebec and is expected to be available this month. But the rest of Canada lags woefully behind.

Canadian Blood Services (CBS) is responsible for providing blood, blood products and their alternatives to the Provinces and Territories (except Quebec). It has responsibility for the medical review of Hemlibra. It concluded last August, “Based on the unanimously favourable medical and scientific review, the Internal Review Team recommends that HEMLIBRA® (emicizumab) be added to the plasma protein products considered for distribution by Canadian Blood Services and has recommended it being added to its list of products to be provided to Canadians.”

The Canadian Agency for Drugs and Technologies in Health (CADTH), responsible for the economic review of Hemlibra, concluded in December, “CADTH estimated the following budget impact associated with treating hemophilia A with inhibitors with emicizumab: cost savings of \$32,920,731 in year 1; \$34,750,021 in year 2; and \$36,545,226 in year 3.” This means that the Provinces and Territories have foregone \$20,000,000 in savings since August 2018.

A very small number of Canadian patients have gained access to Hemlibra through registration in clinical trials or via compassionate use programs. The results have been impressive. Patients have seen dramatic reductions in the number of bleeding episodes. Children and young people have been able to return to school and resume their educations.

The failure in making Hemlibra available to all Canadians in a timely manner is the latest, and perhaps the most egregious, example of a review process for innovative products for rare blood disorders that appears designed to deny Canadians access to needed therapies.

About hemophilia A, inhibitors and Hemlibra

Hemophilia A is a congenital bleeding disorder affecting 1 in 10,000 Canadians. It is characterized by frequent and painful bleeding into joints and muscles. Untreated, it

can be fatal. Current therapies, consisting of intravenous infusions of the missing protein factor VIII, are burdensome but effective. In the presence of an **inhibitor**, however, the infused factor VIII is quickly eliminated by the body's immune system and it becomes ineffective in controlling bleeding. Alternative therapies are extremely expensive and less effective. **Hemlibra** is a novel monoclonal antibody that acts like factor VIII. It is injected subcutaneously once a week. Clinical trials with hundreds of patients have shown it to be a safe, effective therapy to prevent bleeding in people with hemophilia A and an inhibitor.

About the Canadian Hemophilia Society (CHS)

Founded in 1953, the CHS is a national voluntary health charity. Its mission is to improve the health and quality of life of all people in Canada with inherited bleeding disorders and ultimately to find cures. Its vision is a world free from the pain and suffering of inherited bleeding disorders.

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