



Canadian Association
for **Porphyrria**

Association Canadienne
de **Porphyrrie**

**Presentation to CBS
Board of Directors
Part II
December 7, 2017**

Presenter: Wendy Sauvé, President, CAP/ACP

- Good Morning. I am here today to follow up on the June 22nd presentation to the Canadian Blood Services Open Board Meeting by the Canadian Association for Porphyrria.



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About Porphyria

Porphyria is a rare, metabolic disorder occurring in 1/20,000. Of those, 1/50 will present with symptoms requiring treatment.

Symptoms

Patients with acute porphyria often present with excruciating abdominal pain. If untreated, it can progress to convulsions and paralysis. Porphyria can be life-threatening.

Treatment

The **only** treatment for acute porphyria is a blood product called hemin. Panhematin was the first orphan drug approved in the US in 1983.

- Porphyria is a rare metabolic disorder that affects approximately one in 20,000 persons. Of those, 1/50 will require treatment. With Canada's population now around 36 million, it is estimated that 86 Canadians would need treatment for this chronic condition.
- Patients often present with excruciating abdominal pain. If untreated, attacks can progress to convulsions and paralysis. Porphyria can be life-threatening.



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Benefits of Treatment

“I am chronically ill and without treatment I am not able to be the father I want to be, the spouse I want to be, or the employee I want to be. My illness affects every aspect of my life and I believe receiving [hemin] could drastically improve my quality of life.”

“Before I began my treatment I was sure I would die, the pain was so bad I wanted to die. I now am receiving preventative treatments as well as emergent, and feel like I've got a life worth living.”

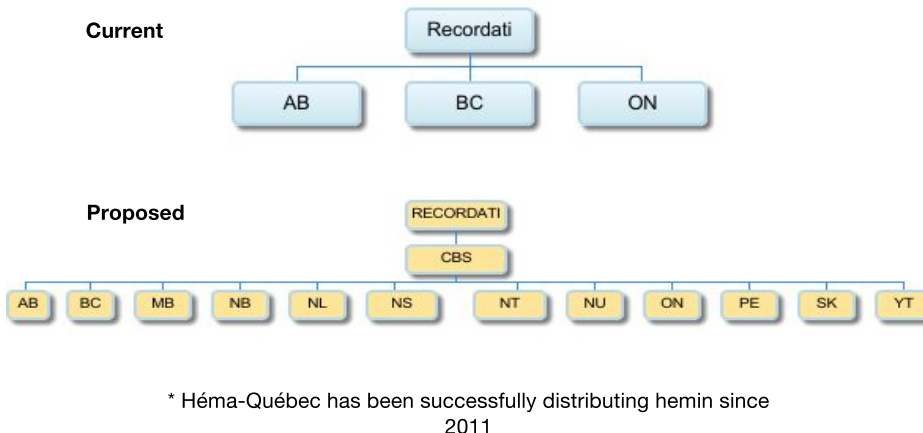
- The only treatment for acute porphyria is a blood product called hemin manufactured by the company, Recordati. Hemin was the first orphan drug approved in the US in 1983 so it is not a new treatment.
- Without treatment, porphyria can become a devastating, debilitating condition. Here's what a patient without treatment said, “1st quote”
- The benefit of treatment is clearly evident in the 2nd patient's comment “2nd quote”



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Hemin Distribution



- In 2016, a CAP survey found that for those diagnosed with an acute porphyria and told by a physician that they should receive hemin only 50% (8 of 16 patients) were able to access treatment.
- The top diagram illustrates the current distribution of hemin. (Québec isn't included here as Héma-Québec has been successfully distributing hemin since 2011.) Although hemin treatment is available in AB, BC and ON not all patients are able to access it. Clearly, the distribution of hemin by the provinces is not meeting the treatment needs of Canadian porphyria patients.
- What's a better solution? In the proposed distribution once CBS becomes the distributor, it is possible for all Canadians to have access to treatment. Once CBS recognized that the blood product, hemin, was unequally available to patients, they invited the manufacturer to submit a proposal to have

- CBS distribute it. CAP provided patient information and support from treating physicians.



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CBS review - Where are we now?

April	The PTBLC discusses the proposal
May	The PTBLC did not provide the required approval to proceed with the review to potentially carry hemin
June	CAP presents to the CBS Board of Directors
October	The PTBLC discusses the proposal
November	The PTBLC discusses the proposal
December	CAP presents to the CBS Board of Directors

- Here's the sequence of the CBS review. In April, the Provincial Territorial Liaison Committee discussed the proposal. In May, the PTBLC did not approve further review on the basis that "this product is already carried with utilization being monitored in many provinces". In June, at the CBS Open Board Meeting, CAP asked that the PTBLC review the proposal again. We were told that the PTBLC discussed this proposal in October and November but we do not know what point the review is at now.



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Why should CBS distribute hemin?

Canadian porphyria patients are waiting...

- Hemin is within the CBS mandate
- Better leverage to negotiate better prices
- Within existing CBS capacity
- Héma-Québec has been distributing hemin since 2011
- All Canadian patients get equal and timely access to treatment

Let me quickly review, Why should CBS distribute hemin?

- Hemin is a blood product.
- Porphyrria patients would benefit from improved distribution.
- CBS has the capacity to negotiate a better price on hemin.
- Héma-Québec has been distributing hemin so there is already a model of effective distribution.

- Earlier, I referred to the estimated number of patients who would require hemin treatment in Canada - 86. We know that 8 patients are currently receiving hemin treatment and that an equal number of patients are **not receiving any treatment!**
- We believe that CBS has a responsibility to agree to be the central distributor of the blood product, hemin, to provide equal access to treatment so that the needless suffering of Canadian porphyria patients can end.
- Thank you!