



Canadian Association
for **Porphyrria**

Association Canadienne
de **Porphyrie**

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

Presenter: Wendy Sauvé, President, CAP/ACP



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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.



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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.”

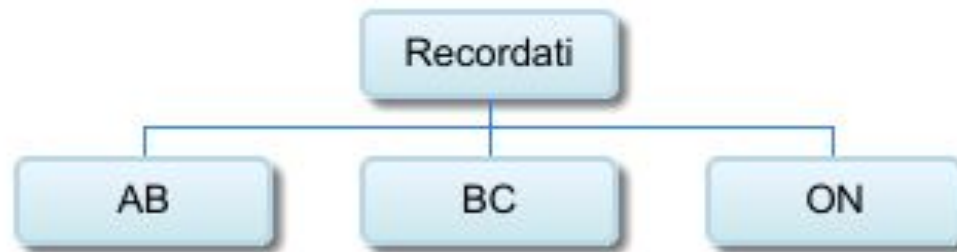


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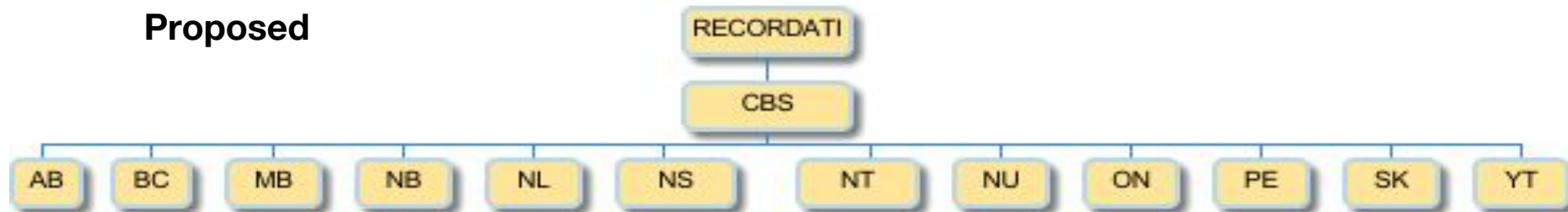
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Hemin Distribution

Current



Proposed



* Héma-Québec has been successfully distributing hemin since 2011



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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



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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