



Canadian Association
for **Porphyrria**

Association Canadienne
de **Porphyrrie**

Presentation to CBS Board of Directors Part IV

December 6, 2018

Presenter: Wendy Sauvé, Co-President, CAP/ACP

- Good Morning. I am here today on behalf of the Canadian Association for Porphyrria to present Part IV of our presentations to the Canadian Blood Services Open Board Meetings. We have presented at the last three Open Board meetings beginning in June 2017.



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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

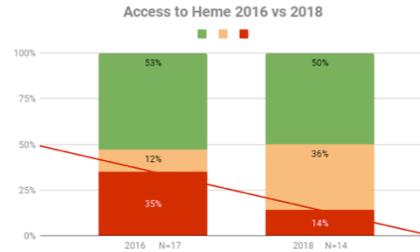
The **only** treatment for acute porphyria is a blood product called hemin.

- I will provide a brief overview of porphyria for those who are new. I apologize for repeating this information - especially for those of you who are hearing this for the fourth time.
- 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. 24 patients are currently receiving treatment.
- Patients often present with excruciating abdominal pain. If untreated, attacks can progress to convulsions and paralysis. Porphyria can be life-threatening.
- 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.
- **New since our last presentation**, Recordati announced in November that one of their hemin products, Panhematin, became the first prescription medication approved by Health Canada for the treatment of recurrent attacks of acute intermittent porphyria.

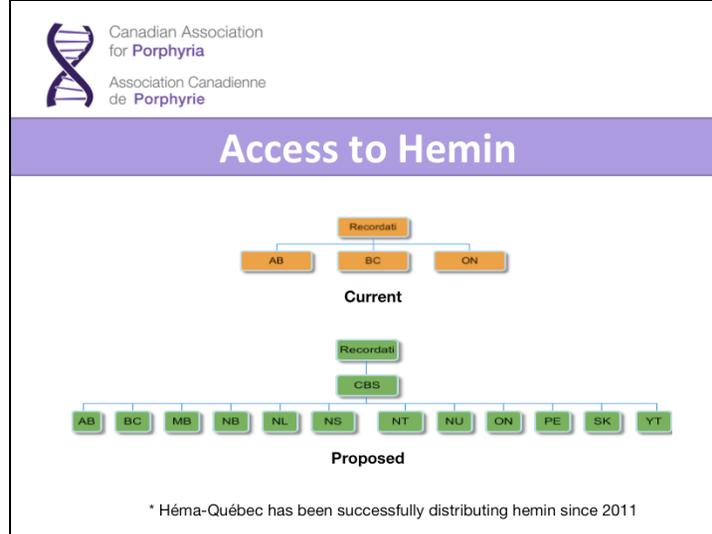


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Hemin Access 2016 vs 2018



- In 2016 and 2018, the Canadian Association for Porphyria surveyed Canadian porphyria patients to evaluate their access to hemin treatment. This graph shows the results for those diagnosed with an acute porphyria and told by a physician that they should receive hemin.
- What we want is 100% access - the green area of the graph. What we found in both surveys is that only 50% of porphyria patients are accessing treatment
- What changed in 2018 was that more patients could sometimes access treatment rather than not receiving it at all. Certainly a move in the right direction but still not appropriate access.

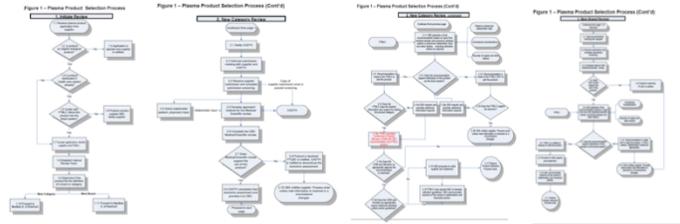


- So let's talk about what it is currently like - even if you can access treatment. Here's a comment from a patient on the 2018 survey:
- "I have received both Panhemitan and Normosang but my husband and I had to advocate strongly for it each time due to unwilling hospital physicians. I have been made to feel guilty because of the cost of the heme treatment and because they don't believe I have Acute Intermittent Porphyrria."
- The other thing that did not change from 2016 to 2018 is the number of provinces providing access to hemin. There were only 3 - AB, BC and ON and none of them provided access to all patients in their jurisdiction. (Québec isn't included here as Héma-Québec has been successfully distributing hemin since 2011.)
- Appropriate access to treatment for porphyria patients is important for all of us. Here's a comment from a patient on the 2018 survey. "With [hemin] life is not normal but far better than before. I wasted **400** days in hospital - mainly emergency and intensive care before [receiving] Normosang"
- Clearly, the current distribution of hemin by the provinces/territories is not meeting treatment needs of Canadian porphyria patients which is why we eagerly anticipate the proposed distribution by



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CBS Process of Review



CBS PLASMA PRODUCT SELECTION PROCESS - Process Summary

- The review of hemin by CBS began in March 2017. It was delayed a year by a decision in April 2017 by the Provincial/Territorial Blood Liaison Committee to stop the review. The review restarted in March 2018 and we are now told that it is “ahead of schedule” and that it is “proceeding apace”.



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CBS Commitment

"We believe Panhematin is an appropriate drug for us to carry. The Provincial and Territorial Blood Liaison Committee has agreed to move forward with a review of this drug using the Canadian Blood Services selection process for plasma protein products as a guide.

The vendor's submission was recently received, and it has been vetted and deemed appropriate from a medical and scientific standpoint. Knowing the review can take 12 to 18 months to complete, we are committed to moving forward on this and will streamline and accelerate the process wherever possible and appropriate."

Mel Cappe, Chair, CBS Board of Directors - July, 2018

- The Canadian Association for Porphyrria recognizes that there is support to have CBS carry Panhematin. We have had support from Graham Sher, CBS CEO; Mel Cappe, Chair, Board of Directors, CBS and, most recently, from Dr. Isra Levy and Ron Vezina. We also recognize that there is commitment to expediting the review process.
- However, I will end by asking four questions on behalf of Canadian porphyria patients....



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What do we tell patients?



1. Which of the 40 steps in the CBS review process have been completed?
2. When do you expect the CBS review will be completed?
3. Are there additional steps after the review before CBS can make Panhematin available?
4. When do you expect that CBS will make Panhematin available to patients?

Thank you!