



Canadian Association
for **Porphyrria**

Association Canadienne
de **Porphyrie**

Presentation to CBS Board of Directors

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Porphyria

- **Rare: 1 in 20,000**
- **Symptoms:**
 - severe abdominal pain
 - nausea, vomiting, constipation
 - hypertension, increased heart rate
 - agitation, insomnia, confusion, and psychosis
 - convulsions
 - decreased sodium in the blood
 - urinary retention, incontinence ... and more



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Heme

- The **only** treatment
- A blood product
- Marketed as:
 - Panhematin
 - Normosang



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Unequal access





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Does treatment make a difference?

“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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CBS

- Excellent distribution model
- Works well for patients
- Mandate: the management of the national supply of blood and blood products



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Submission to PTBLC/CBS

- Recordati Rare Diseases
- CAP 2016 Survey Data
- Physician Support



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Outcome

- PTBLC refuses CBS review
- Believe there is easy access to treatment
- Use pan-Canadian pharma route



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Access to Heme

Very limited
access to heme,
the only porphyria
treatment



CBS deliver on its
mandate:
distribute blood
product



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Our concern

- **No** further disclosure on how decision was made
- **No** opportunity for a future meeting
- **No** avenue for appeal



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CBS

“...we build into the process input from stakeholders at all steps of the procurement process. Our program... gives them a voice in the decision-making on product selection and procurement...”



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Process

No avenue for
input



Accountability &
Openness:

Engagement of all
stakeholders



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Our request

It is our wish that the submission be reviewed again and that all stakeholders — patients, patient support group representatives, physicians, and pharmaceutical representatives — be invited into an open, transparent discussion