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## Patient Survey - Access to Heme Treatment in Canada, 2016

### Executive Summary

The mission of the Canadian Association for Porphyrria/Association Canadienne de Porphyrrie (CAP/ACP) is to deliver evidence-based information and support to patients with porphyria, their families, health care providers and the general public across Canada and to achieve standards and evidence-based comprehensive care for all people with porphyria throughout their lifespans.

The CAP/ACP asserts that patients with acute porphyria should have access to Normosang and/or Panhematin treatment, as needed, regardless of where they live in Canada. We are advocating for a future where these blood products are distributed efficiently and equitably, and by so doing, greatly improve the lives of those suffering from acute hepatic porphyria.

### Acute Porphyrria Symptoms and Treatment

Porphyrria is a rare, serious and life-threatening condition. The following are a list of the clinical features during an attack<sup>1</sup> for those with an acute porphyria:

- o Abdominal pain – severe, poorly localized. Pain can also affect back, legs and other sites
- o Nausea, vomiting, constipation
- o Hypertension, tachycardia, and rarely, arrhythmias
- o Agitation, insomnia, confusion, psychosis with hallucinations and unusual behaviour
- o Convulsions – frequently associated with hyponatraemia
- o Peripheral motor neuropathy – may progress to flaccid paralysis, respiratory insufficiency, difficulty swallowing, urinary retention or incontinence
- o Dark urine – colour darkens to orange or red on exposure to light
- o Hyponatraemia

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<sup>1</sup> P Stein; Department of Medicine, Addenbrooke's Hospital, Cambridge CB2 0QQ, *Best practice guidelines on clinical management of acute attacks of porphyria and their complications*

<sup>2</sup> Normosang Product Information: SUMMARY OF PRODUCT CHARACTERISTICS, Orphan Europe

<sup>3</sup> Panhematin Patient Information, Orphan Europe

In addition to the excruciating pain experienced during an attack, many patients also experience chronic pain as a result of neuropathic damage sustained during untreated attacks.

Normosang and/or Panhematin (a concentrate for solution for infusion and consisting primarily of processed human red blood cells) is the **only** treatment available to patients with acute porphyria. Both Normosang and/or Panhematin products are distributed by Recordati/Orphan Europe and are indicated for treatment of acute attacks of hepatic porphyria (acute intermittent porphyria, porphyria variegata, hereditary coproporphyrin).<sup>2,3</sup>

### **Current Treatment Availability in Canada**

An Online Survey sent October 28, 2016 was used to gather information about patients' experiences with Normosang and/or Panhematin treatment in Canada. This survey was distributed to members and contacts of the CAP/ACP via Facebook, Website and Emails. Results were anonymously collected and compiled to collect the experiences of Canadians with acute porphyria (see Appendix). 39 responses from Canadians diagnosed with acute porphyria were received. Quotes from survey respondents are included in italics throughout this submission.

Patients in the survey spoke of the excruciating pain of attacks and the long term effects of not receiving adequate treatment for themselves or their family members.

*"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 PanHematin/Normosang could drastically improve my quality of life."*

*"Suffering intense pain for several days knowing nothing could be done to help me, husband and family worried and feeling available treatment should never be denied to patient if it exists somewhere on this planet. Simply unacceptable"*

Porphyria is treatable. Patients can be treated with Normosang and/or Panhematin and in the survey spoke of the benefits of receiving adequate treatment.

*"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."*

*"When I did get normosang, it lessened nerve pain, lessened anxiety, and as a result, lessened my need for other drugs."*

### **Restricted Access to Normosang and/or Panhematin Treatment**

The *Access to Heme Treatment, 2016 Survey* results clearly demonstrates that there is unequal access to Normosang and/or Panhematin treatment across Canada. For those

diagnosed with an acute porphyria and told by a physician that they should receive Normosang and/or Panhematin only 50% (8 of 16 patients) were able to access treatment. Although some patients receive timely treatment, half of them suffer through attacks, receiving only supportive care (medication for nausea & pain) in Emergency or as an inpatient as they are not able to receive Normosang and/or Panhematin treatment.

### Access to Normosang and/or Panhematin Treatment in Canada, 2016

Province	Access	No Access
AB	3	4
BC	2	1
ON	2	1
QC	1	1
SK	-	1
Total	8	8

Of particular concern is access to Normosang and/or Panhematin treatment in the provinces of Saskatchewan and Alberta. The CAP/ACP previously received information that treatment was not available in Saskatchewan and the one respondent from this province confirmed this. Alberta, however, was the only province where two patients were denied ongoing access to Normosang and/or Panhematin treatment despite being successfully treated with Normosang and/or Panhematin previously.

The current system of delivering Normosang and/or Panhematin treatment to acute porphyria patients in Canada is failing and in some cases increases suffering unnecessarily. Where you live in Canada determines whether or not you will get treatment for acute porphyria. The province you live in will also determine how consistent treatment will be and how difficult it is to get approval for treatment.

The outcome of not adequately treating acute porphyria patients concerns us all. In addition to dealing with the medical symptoms, acute porphyria patients spoke of decreased social interactions, loss of their capacity for work and an inability to parent as a direct result of their condition. Sadly, one respondent spoke about suicide as a way to end the suffering.<sup>2</sup>

The CAP/ACP asserts that patients with acute porphyria should have access to Normosang and/or Panhematin treatment, as needed, regardless of where they live in Canada. We are advocating for a future where these blood products are distributed efficiently and equitably, and by so doing, greatly improve the lives of those suffering from acute hepatic porphyria.

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<sup>2</sup> W Sauv , J Reimer, 2016 Survey: Access to Heme Treatment in Canada

## APPENDIX

### SUMMARY - 2016 Survey: Access to Heme Treatment in Canada

#### **Question 1: Do you live in Canada?**

All 39 respondents live in Canada.

#### **Question 2: In what province or territory do you live?**

Provinces represented; AB (14), BC (4), MB (3), NS (3), ON (11), QC (3), SK (1)

#### **Question 3: Have you been diagnosed with acute porphyria?**

39 respondents indicated that they were diagnosed with an acute porphyria.

#### **Question 4: If you have received a diagnosis of porphyria, which type have you been diagnosed with?**

- Acute Intermittent Porphyria (AIP) **26**
- Variegate Porphyria (VP) **13**
- Hereditary Coproporphyria (HCP) **3**
- Acute Intermittent Porphyria (AIP) and Hereditary Coproporphyria (HCP) **2**

The survey results are representative of the acute porphyria community where AIP is most frequently diagnosed, followed by VP and, less frequently, HCP. Two respondents indicated that they had been diagnosed with two types of porphyria.

#### **Question 5: Have you ever been told by a physician that you should receive Heme treatment (Normosang and/or Panhematin)?**

16 respondents were told by their physician that they require heme treatment.

#### **Question 6: Which of the following treatments have you received (if you have been told by a physician that you should receive heme treatment – Normosang and/or Panhematin)?**

Panhematin	3
Normosang	3
Panhematin & Normosang	4
No Treatment Available	6

**Question 7: If you have not been able to receive Normosang or Panhematin, why can't you get treatment?**

# of Responses	<u>Reason for <b>NOT</b> being able to access Normosang and/or Panhematin Treatment</u>
3	Patient would have to travel to get the treatment
2	Treatment was not paid for where the patient lived
2	Physician did not order the treatment
2	Patient did not know of the treatment option
2	Treatment was denied after physician's application
1	Physician would not order the treatment
1	Physician did not accept the diagnosis
1	Patient told that treatment was not available in Canada

**Question 8: If you have not been able to receive Normosang or Panhematin, please describe the impact to you, your family, and your quality of life.**

# of Responses	<u>Impact of <b>NOT</b> receiving Normosang and/or Panhematin treatment</u>
4	Negatively impacts family life
3	Suffering intense pain
3	Negatively impacts employment
2	Irreversible nerve damage
1	Negatively impacts mental health
1	Increased hospitalizations
1	Patient is chronically ill
1	Uncertainty of intermittent symptoms

**Question 9: If you have been able to receive Normosang or Panhematin, please describe the impact to you, your family, and your quality of life.**

# of Responses	<u>Impact of RECEIVING Normosang and/or Panhematin treatment</u>
2	Less time in hospital
2	Quality of life greatly improved
2	Less pain
2	No more porphyria crises
1	Less nausea
1	Improved social life
1	Fewer sick days
1	Fewer sleepless nights
1	Improved health
1	Lessened anxiety
1	Lessened need for additional drugs

**Question 10: Please use this area to share information that was not included above.**

# of Responses	<u>Additional Comments</u>
6	Frustration with lack of access to treatment
5	Lack of support from the medical community
3	Support for effective treatment
2	Satisfaction with access to treatment
1	Support for Advocacy for treatment

<b>Name of the disorder:</b>	Acute hepatic porphyria (Acute Intermittent Porphyria, Variegate Porphyria, and Hereditary Coproporphyrinuria)
<b>Name of the blood product(s):</b>	Normosang and/or Panhematin
<b>Name of the patient group:</b>	Canadian Association for Porphyria/Association Canadienne de Porphyrie
Email	<a href="mailto:CanadianAssocForPorphyria.acp@gmail.com">CanadianAssocForPorphyria.acp@gmail.com</a>
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<b>Conflict of Interest Declaration:</b>	None to declare