



## Access to Heme Treatment in Canada - Survey 2016

The Canadian Association for Porphyria/Association Canadienne de Porphyrie (CAP/ACP) asserts that patients with acute porphyria should have access to Hemin treatment, as needed, regardless of where they live in Canada. We support a system of medical care where this blood product is distributed efficiently and equitably, and by so doing, greatly improve the lives of those suffering from acute hepatic porphyria.

In order to have a clearer understanding of Canadians with porphyria, an Online Survey was used to gather information about patients' experiences with Hemin treatment in Canada. This survey was distributed to members and contacts of CAP/ACP via Facebook, Website and Emails. Results were anonymously collected and compiled to collect the experiences of Canadians with acute porphyria (see Appendix).

### Overview: Acute Hepatic Porphyria Symptoms and Treatment

Porphyria 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 hepatic 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*

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.

Hemin (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 hemin products (Normosang and Panhematin) are distributed by Orphan Europe and are indicated for treatment of acute attacks of hepatic porphyria (acute intermittent porphyria, porphyria variegata, and hereditary coproporphyria).<sup>2,3</sup>

## Access to Heme Treatment: 2016 Survey Results

### Current Treatment Availability in Canada

Thirty-nine 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 for themselves and/or their family members of **not** receiving adequate 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 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”*

*“I have started having irreversible nervous system damage because of my frequent attacks and only receiving the carb loading treatment. I may have to change careers... It's a drain on my mental status and my family feels they should bear this load.”*

Porphyria is treatable. Patients can be treated with Hemin 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.”*

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

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

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

*“I had numerous recurring attacks before doctors were able to identify AIP. During my last attack (Nov 2014) I was lucky to receive Panhematin and since that time I have not had any attacks. Thanks to the treatment my life has tremendously improved.*

## **Restricted Access to Hemin Treatment**

The *Access to Heme Treatment, 2016 Survey* results clearly demonstrates that there is unequal access to Hemin treatment across Canada. For those diagnosed with an acute porphyria and told by a physician that they should receive Hemin treatment 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 Hemin treatment.

### **Access to Appropriate Hemin Treatment in Canada, 2016**

<b>Province</b>	<b>Access</b>	<b>No Access</b>
AB	3	4
BC	2	1
ON	2	1
QC	1	1
SK	-	1
Total	8	8

Of particular concern is access to Hemin treatment in the provinces of Saskatchewan and Alberta. 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 Hemin treatment despite being successfully treated with Hemin previously.

The current system of delivering hemin 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>

### **Information to Patients about Hemin Treatment**

Several respondents mentioned that they were unaware of hemin treatment or even more alarmingly, that their physician had told them that hemin treatment was unavailable in Canada. In addition to the problems of accessing appropriate hemin treatment, not being made aware of the **only** treatment for their condition is unacceptable.

### **Advocacy by CAP/ACP**

The mission of CAP 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.

Based on the findings of the 2016 Survey: Access to Heme Treatment, our goals for advocacy will include:

1. Providing accurate information regarding access to treatment for porphyria patients
2. Advocating that Canadian acute porphyria patients, in general, receive hemin treatment, as needed.
3. Advocating with individual Canadian acute porphyria patients that they receive hemin treatment, as needed.
4. Proposing that Hemin treatment be distributed as other blood products are in Canada - through Canadian Blood Services.

The CAP/ACP view the 2016 survey as the beginning of a discussion around how best to ensure that those with porphyria access treatment, as needed. We invite you to contact us at [canadianassocforporphyria.acp@gmail.com](mailto:canadianassocforporphyria.acp@gmail.com) .

### **The 2016 Survey: Access to Heme Treatment was prepared by:**

Wendy Sauv  (President CAP/ACP) and Jeannie Reimer (Treasurer CAP/ACP)

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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 Hemin 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 Hemin treatment.

#### **Question 6: Which of the following treatments have you received (if you have been told by a physician that you should receive Hemin 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 Hemin 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 Hemin 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 Hemin 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
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	Coproporphyrin (hereafter referred to as acute porphyria)
<b>Name of the blood product</b>	Normosang and/or Panhematin (hereafter referred to as Hemin)
<b>Name of the patient group</b>	Canadian Association for Porphyria/Association Canadienne de Porphyrie (hereafter referred to as CAP/ACP)
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<b>Conflict of Interest Declaration</b>	None to declare