



Access to Heme Treatment in Canada - Survey 2018

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, Online Surveys in 2016 and 2018 were used to gather information about patients' experiences with Hemin treatment in Canada. These surveys were 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 attack¹ 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

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.

¹ 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*

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. Two hemin products (Normosang and Panhematin) are distributed by [Recordati](#) and are indicated for treatment of acute attacks of hepatic porphyria (acute intermittent porphyria, porphyria variegata, and hereditary coproporphyria).^{2,3} As of November, 2018, [Panhematin](#) has been approved by Health Canada and is the hemin treatment available for treatment of acute porphyria symptoms (unless there are medical circumstances that warrant Normosang).

Access to Heme Treatment: 2018 Survey Results

Current Treatment Availability in Canada

Twenty-five 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, once again, of the excruciating pain of attacks and the long term effects for themselves and/or their family members of **not** receiving adequate treatment.

“Having to be in full attack before I can access drugs that could have prevented the attack in the first place is a terrible drain on myself and my family in every aspect imaginable from physical, to mental, to monetary.... The inability to lead a normal life free of attacks due to not having access to preventative therapy is cruel.”

“I have no quality in my life. It is pain and illness constantly.”

Porphyria is treatable. Patients can be treated with Hemin and in the survey spoke, once again, of the benefits of receiving adequate treatment.

“With Normosang in preventive treatment one dose every 10 days...no more serious crisis needing to go to emergency and intensive care, Before it was a crisis a month minimum”

“Improves my quality of life. Less neurological symptoms after the attack. Less sick days.”

“I had two treatments of panhematin in 2012-13. It was amazing. It’s the only time I’ve been symptom free in 40 yrs.”

² Normosang Product Information: SUMMARY OF PRODUCT CHARACTERISTICS, Orphan Europe

³ Panhematin Patient Information, Orphan Europe

Restricted Access to Hemin Treatment

The *Access to Heme Treatment, 2018 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 40% (4 of 10 patients) were able to access treatment as needed, 40% (4 of 10 patients) had limited access to Hemin treatment and 20% (2 of 10 patients) had no access. The majority of Canadian porphyria patients (60%) are not receiving timely Hemin treatment.

Access to Appropriate Hemin Treatment in Canada, 2016

Province	Access	Limited Access	No Access
AB	2	2	
BC	2	2	
MB			1
NS			1
QC	1		
Total	4	4	2

Of particular concern is access to Hemin treatment in the provinces of Manitoba and Nova Scotia where the respondent in each province stated that they had no access to hemin treatment.

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 the stress of advocating for appropriate care,² loss of their capacity for work and an inability to parent as a direct result of their condition.

Information to Patients about Hemin Treatment

One respondent stated that they were unaware of Hemin treatment or even more alarmingly, several respondents mentioned that the hospitals or physicians were refusing to provide Hemin treatment because of the cost. These porphyria patients were not able to access appropriate Hemin treatment - the **only** treatment for their condition.

Advocacy by CAP/ACP

² W Sauv , J Reimer, 2018 Survey: Access to Heme Treatment in Canada

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 and 2018 Surveys: Access to Heme Treatment, our goals for advocacy will include:

1. Proposing that Hemin treatment be distributed as other blood products are in Canada - through Canadian Blood Services (CBS). Please find the CAP/ACP presentations to CBS [here](#)
2. Providing accurate information regarding access to treatment for porphyria patients
3. Advocating that Canadian acute porphyria patients, in general, receive hemin treatment, as needed.
4. Advocating with individual Canadian acute porphyria patients that they receive hemin treatment, as needed.

The CAP/ACP view the 2016 and 2018 surveys as basis for discussions around how best to ensure that those with porphyria access treatment, as needed. We invite you to contact us at canadianassocforporphyria.acp@gmail.com .

The 2018 Survey: Access to Heme Treatment was prepared by:

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APPENDIX

SUMMARY - 2016 Survey: Access to Hemin Treatment in Canada

Question 1: Do you live in Canada?

All 25 respondents live in Canada.

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

Provinces represented; AB (8), BC (6), MB (1), NS (2), ON (5), QC (3)

Question 3: Have you been diagnosed with acute porphyria?

25 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) 16
- Variegate Porphyria (VP) 4
- Hereditary Coproporphyrinuria (HCP) 2
- Acute Intermittent Porphyria (AIP) and Hereditary Coproporphyrinuria (HCP) 2
- Not Indicated 1

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)?

12 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	2
Normosang	3
Panhematin & Normosang	5
No Treatment Available	4

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

“I was given a treatment of panhematin because I was in crisis (the treating doctors didn't believe I was having a severe reaction to the medications). I asked the doctor if I could receive a treatment if it was needed and the doctor answered “no” because it was too expensive for the hospital.”

# of Responses	Reason for NOT being able to access Hemin Treatment
2	Physician would not order the treatment
2	Hospital stated that the treatment was too expensive
1	Patient did not know of the treatment option
1	Physician did not order the treatment
1	Government would not approve

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.

“Have been put on disability and no longer working or able to take proper care of my family. I do what I can when I can and limit things as needed.”

# of Responses	Impact of NOT receiving Hemin treatment
3	Negatively impacts family life
2	Negatively impacts quality of life
2	Negatively impacts mental health
2	Patient is chronically ill
1	Poorer health
1	Lack of preventative treatment
1	Negatively impacts finances
1	Suffering intense pain
1	Negatively impacts employment

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.

“It saved my life! If I didn’t receive it in August or 2002 I wouldn’t be alive today. Thankfully I got it and since then I am able to live normal life.”

# of Responses	Impact of RECEIVING Hemin treatment
1	Less time in emergency
1	Less time in intensive care
1	Quality of life greatly improved
1	No more porphyria crises
1	Fewer sick days
1	Free of porphyria symptoms
1	Saved patient’s life
1	Allowed patient to live a normal life
1	Less neurological symptoms

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

“With Normosang the life is not normal but far better than before, I wasted 400 days in hospital mainly emergency and intensive care before the Normosang”

# of Responses	Additional Comments
3	Lack of knowledge by physicians
2	Lack of support from physicians
2	Lack of hospital staff knowledge
2	Frustration with lack of access to treatment
1	Benefit from effective treatment
1	Lack of care for elderly porphyria patients
1	Patient reassured if treatment was made available
1	Patient told they were mental by nurses
1	Patient told there was no such disease
1	Patient made to feel guilty for the cost of treatment
1	Diagnosis (AIP) questioned by physicians
1	Appropriate Hemin treatment improved condition

2018 Access to Heme Treatment in Canada Survey

Name of the disorder	Acute hepatic porphyria (Acute Intermittent Porphyria, Variegate Porphyria, and Hereditary Coproporphyria) (<i>hereafter referred to as acute porphyria</i>)
Name of the blood product	Normosang and/or Panhematin (referred to as Hemin)
Name of the patient group	Canadian Association for Porphyria/Association Canadienne de Porphyrie (<i>hereafter referred to as CAP/ACP</i>)
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Conflict of Interest Declaration	None to declare