

CADTH RAPID RESPONSE REPORT: REFERENCE LIST

Injectable Hemin for Patients with Acute Porphyria: Clinical-Effectiveness, Cost- Effectiveness, and Guidelines

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Research Questions

1. What is the clinical effectiveness of injectable hemin for the treatment of acute attacks of porphyria?
2. What is the cost-effectiveness of injectable hemin for the treatment of acute attacks of porphyria?
3. What are the evidence-based guidelines regarding the use of injectable hemin for the treatment of acute attacks of porphyria?

Key Findings

No relevant clinical effectiveness evidence or evidence-based guidelines were identified regarding injectable hemin for patients with acute porphyria.

Methods

A limited literature search was conducted on key resources including PubMed, The Cochrane Library, University of York Centre for Reviews and Dissemination (CRD), Canadian and major international health technology agencies, as well as a focused Internet search. No filters were applied to limit the retrieval by study type. Where possible, retrieval was limited to the human population. The search was also limited to English language documents published between January 1, 2009 and January 17, 2019. Internet links are provided where available.

Selection Criteria

One reviewer screened citations and selected studies based on the inclusion criteria presented in Table 1.

Table 1: Selection Criteria

Population	Patients with acute attacks of porphyria (of any etiology)
Intervention	Injectable hemin (e.g., Panhematin)
Comparator	Q1-2: Haem arginate; Supportive treatment for symptoms (e.g., fluids, anti-emetic medications, anti-seizure medications); Carbohydrates administered by mouth or intravenous, placebo, no treatment Q3: No comparator
Outcomes	Q1: Clinical effectiveness (e.g., reduction in duration of attack, resolution of attack and symptoms, prevention of neurological symptoms or other severe or life threatening symptoms), safety (e.g., rate of adverse events) Q2: Cost-effectiveness Q3: Evidence-based guidelines
Study Designs	Health technology assessments, systematic reviews, meta-analyses, randomized controlled trials, non-randomized studies, economic evaluations, evidence-based guidelines

Results

Rapid Response reports are organized so that the higher quality evidence is presented first. Therefore, health technology assessment reports, systematic reviews, and meta-analyses are presented first. These are followed by randomized controlled trials, non-randomized studies, economic evaluations, and evidence-based guidelines.

No relevant health technology assessments, systematic review, meta-analyses, randomized controlled trials, non-randomized studies, economic evaluations, or evidence-based guidelines were identified regarding injectable hemin for patients with acute porphyria.

References of potential interest are provided in the appendix.

Health Technology Assessments

No literature identified.

Systematic Reviews and Meta-analyses

No literature identified.

Randomized Controlled Trials

No literature identified.

Non-Randomized Studies

No literature identified.

Economic Evaluations

No literature identified.

Guidelines and Recommendations

No literature identified.

Appendix — Further Information

Randomized Controlled Trials – Trials in the Recruiting Stage

1. University of Texas Medical Branch - Galveston. NCT02180412: Controlled trial of Panhematin in treatment of acute attacks of porphyria. *ClinicalTrials.gov*. Bethesda (MD): U.S. National Library of Medicine; 2018: <https://clinicaltrials.gov/ct2/show/results/NCT02180412>
Accessed 2019 Jan 22
2. University of Texas Medical Branch - Galveston. NCT02922413: Panhematin for prevention of acute attacks of porphyria. *ClinicalTrials.gov*. Bethesda (MD): U.S. National Library of Medicine; 2018: <https://clinicaltrials.gov/ct2/show/NCT02922413>
Accessed 2019 Jan 21

Non-Randomized Studies - Case Studies

3. Attarian S, Yu C, Anderson KE, Friedman EW. Effects of hemin and hemodialysis in a patient with acute intermittent porphyria and renal failure. *Blood Adv*. 2017 Jun 13;1(14):915-917.
[PubMed: PM29296735](#)
4. Bonnefoy Mirralles AM, Torres-Castro R, Ovalle Guzman C. A comprehensive rehabilitation program and follow-up assessment for acute intermittent porphyria . *Am J Phys Med Rehabil*. 2017 May;96(5):e85-e88.
[PubMed: PM27584135](#)
5. Shen J, O'Keefe K, Webb LB, DeGirolamo A. Acute porphyria in a patient with Arnold Chiari malformation. *Am J Case Rep*. 2015 Feb 20;16:99-103.
[PubMed: PM25697467](#)
6. Anyaegbu E, Goodman M, Ahn SY, Thangarajh M, Wong M, Shinawi M. Acute intermittent porphyria : a diagnostic challenge. *J Child Neurol*. 2012 Jul;27(7):917-921.
[PubMed: PM22190498](#)
7. Asselbergs FW, Kremer Hovinga TK, Bouwsma C, van Ingen J. Acute intermittent porphyria as a cause of respiratory failure: case report. *Am J Crit Care*. 2009 Mar;18(2):180, 178-189.
[PubMed: PM19255109](#)

Qualitative Studies

8. Naik H, Stoecker M, Sanderson SC, Balwani M, Desnick RJ. Experiences and concerns of patients with recurrent attacks of acute hepatic porphyria : a qualitative study. *Mol Genet Metab*. 2016 Nov;119(3):278-283.
[PubMed: PM27595545](#)

Review Articles

9. Arora S, Young S, Kodali S, Singal AK. Hepatic porphyria : a narrative review. *Indian J Gastroenterol*. 2016 Nov;35(6):405-418.
[PubMed: PM27796941](#)

10. Bissell DM, Lai JC, Meister RK, Blanc PD. Role of delta-aminolevulinic acid in the symptoms of acute porphyria . *Am J Med.* 2015 Mar;128(3):313-317.
[PubMed: PM25446301](#)

Additional References

11. Gouya L, Bloomer JR, Balwani M, Bissell DM, et al. An analysis of healthcare utilization and costs associated with patients with acute hepatic porphyrias (AHP) with recurrent attacks in EXPLORE: a prospective, multinational natural history study of patients with AHP. Cambridge (MA): Alnylam Pharmaceuticals; 2017: <https://www.alnylam.com/wp-content/uploads/2017/06/ICPP-2017-EXPLORE-Healthcare-Utilization-Poster-Capella.pdf>. Accessed 2019 Jan 21