

Disease Burden and Healthcare Utilization Among Patients with Acute Intermittent Porphyria Experiencing Chronic Neuropathy: Analyses from a National Healthcare Database

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Background, Objective, and Methods

Acute Hepatic Porphyria (AHP) Background

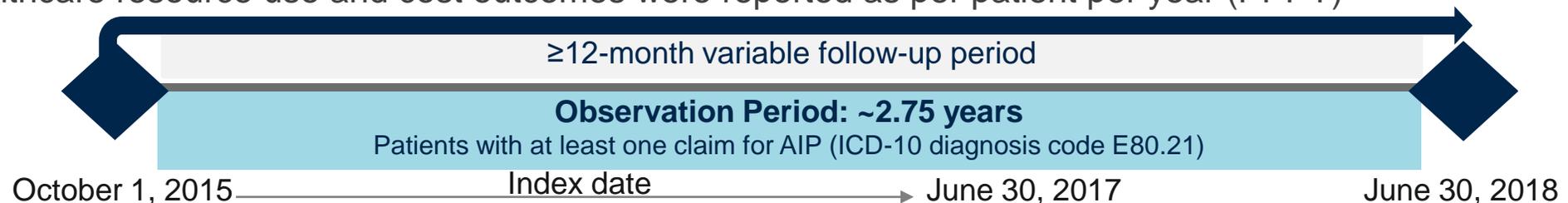
- AHP is a family of rare genetic diseases resulting from a deficiency in one of the enzymes responsible for heme biosynthesis in liver^{1–4}; acute intermittent porphyria (AIP) is most common^{1–4}
 - Patients with AHP can develop chronic pain associated with axonal motor polyneuropathy.^{5–7} Chronic neuropathy can result from a single attack or as ongoing damage from repeated attacks^{5–7}

Objective

- To estimate healthcare resource utilization among various segments of patients with AIP defined by porphyria attack rates, chronic symptoms, and comorbidities
 - This analysis focused on the patient segment specific to chronic neuropathy

Methods

- Retrospective analysis of IBM[®] MarketScan[®] Commercial Claims and Medicare Supplemental Databases*
 - Healthcare resource use and cost outcomes were reported as per patient per year (PPPY)



*Database does not capture the majority of Medicare and Medicaid patients. 1. Bonkovsky et al. *Am J Med* 2014;127:1233–41; 2. Elder et al. *J Inherit Metab Dis* 2013;36:849–57; 3. Balwani et al. *Hepatology* 2017;66:1314–22; 4. Bonkovsky et al. *Mol Genet Metab* 2019;128:213–18; 5. Puy et al. *Lancet* 2010;375:924–37; 6. Jaramillo-Calle & Aguirre Acevedo. *JIMD Reports* 2018;125; 7. Wang. *Hepatology Communications* 2019;3:193-206

Results

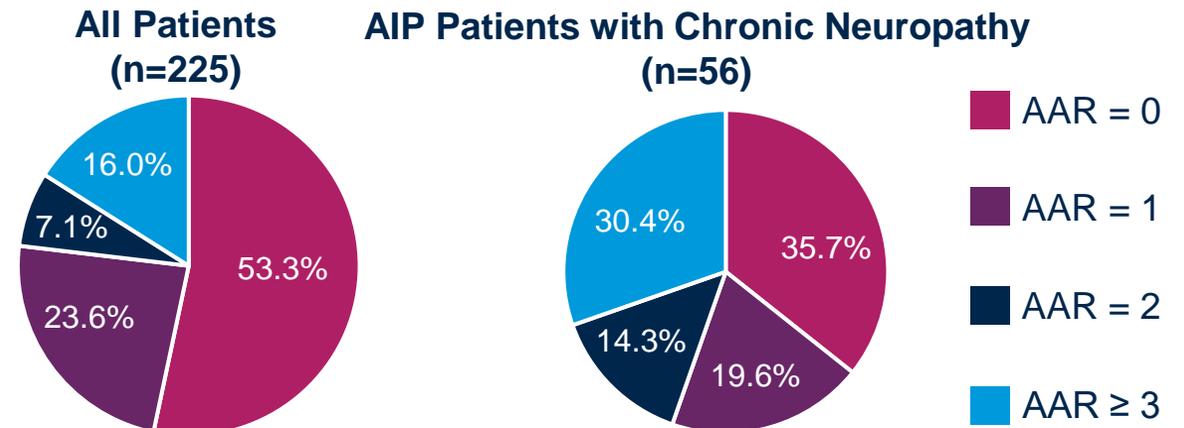
Patient Demographics

- Chronic neuropathy occurred in 56 (24.9%) of AIP patients
- The majority of total AIP patients, along with the subset patients with chronic neuropathy, were female with a mean age of 45.6 and 49.9, respectively

Disease Characteristics

- AIP patients with chronic neuropathy had a mean annualized attack rate (AAR) of 2.7
 - 35.7% had an AAR of 0 during the observation period
- AIP patients with chronic neuropathy experienced other comorbidities often associated with AHP including liver disease (17.9%), chronic kidney disease (14.3%), and hypertension (62.5%)

Characteristic	Total AIP Patients (n=225)	AIP Patients with Chronic Neuropathy (n=56)
Age, mean (SD)	45.6 (16.4)	49.9 (14.8)
Gender, % female	69.8%	80.4%
Payer, % Commercial	92.0%	91.1%
Length of Follow-Up, Years, mean (SD)	1.8 (0.6)	2.0 (0.7)
Number of Attacks*, mean (SD)	2.2 (2.8)	2.7 (3.4)
Liver Disease, %	12.4%	17.9%
Chronic Kidney Disease, %	9.3%	14.3%
Hypertension, %	47.1%	62.5%



*Calculated based on annualized attack rate (AAR); AAR was rounded to the nearest integer;

Attacks are defined as an outpatient ED visit or inpatient admission with a diagnosis of porphyria, abdominal pain, back pain, chest pain, or nausea/vomiting in any position on the claim. For patients who do not have any indication of prophylactic hemin use, use of hemin will also be counted as an attack. Attacks identified within a 7-day period were counted as a single attack. Prophylactic hemin is defined as patients who have more than 5 instances where two hemin claims are between 7 and 38 days apart based on a previously developed algorithm representing weekly, bi-weekly, and monthly prophylactic hemin regimens

Results (continued)

Healthcare Resource Utilization (HCRU) in AIP Patients with Chronic Neuropathy

- Mean annualized hospitalization and ER visit rates among AIP patients with chronic neuropathy were 1.0 and 7.5, respectively
- AIP patients with chronic neuropathy presented high utilization of pain medication, including opioids (24.2 annualized prescriptions) and neuropathic pain (NP) medications (15.4)
 - Patients had frequent utilization, with 82% and 59% of patients having ≥ 1 claim for opioids and neuropathic pain medication, respectively

HCRU / Comorbidities (annualized data)	AIP Patients with Chronic Neuropathy (n=56)
% with ≥ 1 Hospitalization	57%
Hospitalizations, mean (SD)	1.0 (1.4)
% with ≥ 1 ER visit	75%
ER visits, mean (SD)	7.5 (23.2)
% with ≥ 1 Outpatient Visit	100%
Outpatient visits, mean (SD)	19.9 (13.8)
% with ≥ 1 Opioid claim	82%
Opioids, mean (SD)	24.2 (28.7)
% with ≥ 1 NP Med Claims	59%
NP med claims, mean (SD)	15.4 (11.7)
% with ≥ 1 Any Pain Med Claims	91%
Any pain med claims, mean (SD)	33.2 (34.2)

Conclusion

- Results from this national representative healthcare claims database demonstrated chronic pain and long-term complications of AIP including chronic neuropathy in 24.9% of patients
- AIP patients experiencing chronic neuropathy had high utilization of opioid, non-opioid, and neuropathic pain medications
- When diagnosing and managing patients with AIP, chronic neuropathy, both during and outside of the setting of an attack, should be considered
- Additional analyses are planned to estimate healthcare resource utilization among various segments of the AIP patients defined by porphyria attack rates, chronic symptoms, and disease related comorbidities