

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

*Angelika L. Erwin<sup>1</sup>, Samuel Silver<sup>2</sup>, **Stephen Meninger<sup>3</sup>**, Joseph Tkacz<sup>4</sup>, Virginia Noxon<sup>4</sup>, John J. Ko<sup>3</sup>*

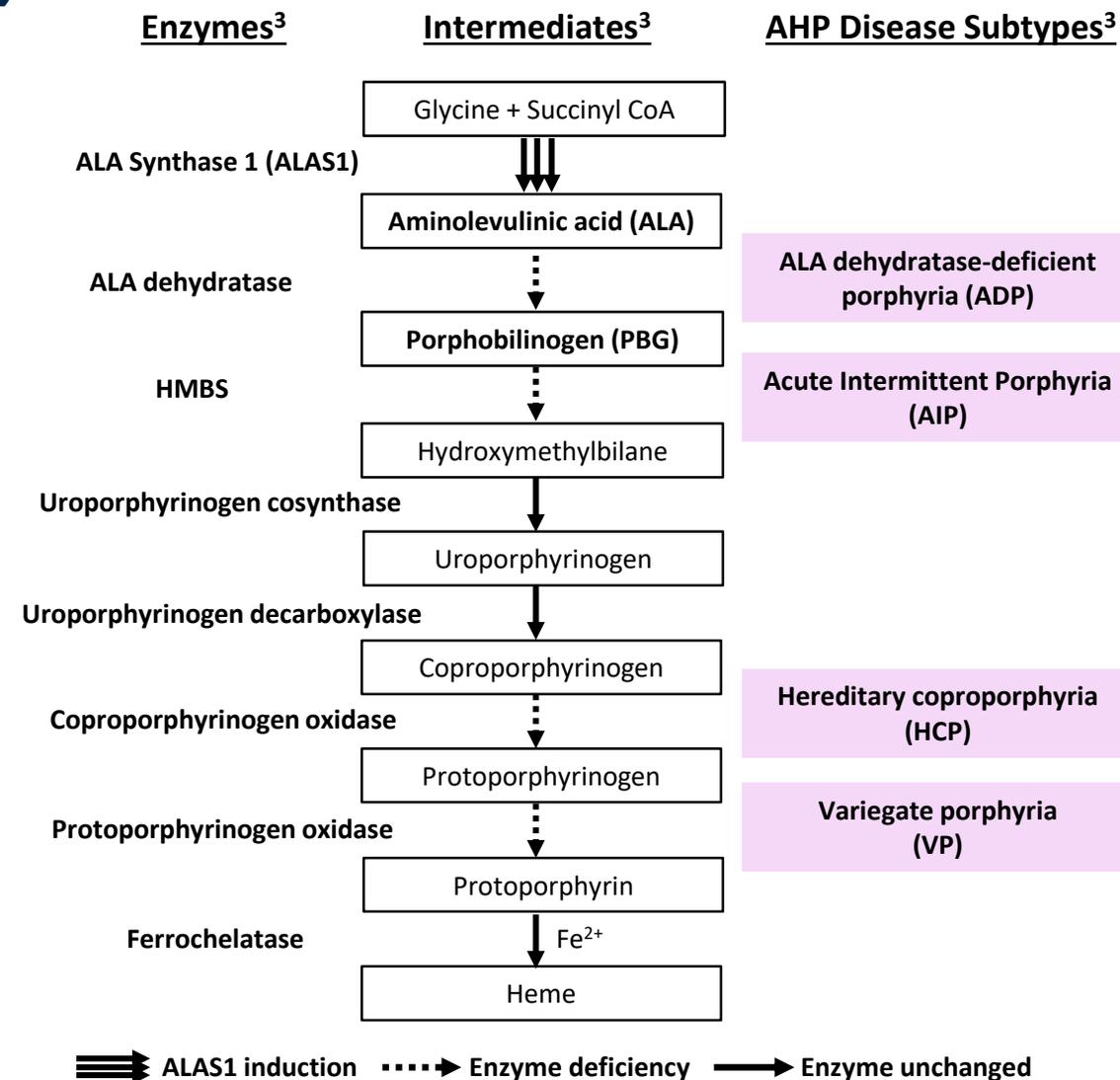
<sup>1</sup>Cleveland Clinic Cleveland, Cleveland, OH, United States; <sup>2</sup>University of Michigan Medical Center, Ann Arbor, MI, United States; <sup>3</sup>Anylam Pharmaceuticals, Cambridge, MA, United States; <sup>4</sup>IBM Watson Health, Bethesda, MD, United States



# Acute Hepatic Porphyria (AHP)

## Disease Overview and Pathophysiology

- Family of rare, genetic diseases resulting from a deficiency in one of the enzymes in heme biosynthesis in liver<sup>1-4</sup>
  - Acute intermittent porphyria (AIP) is most common<sup>1-4</sup>
- AHP is characterized by acute neurovisceral attacks and, for some patients, chronic debilitating symptoms and long term complications<sup>1-4</sup>
- Patients can develop chronic pain associated with axonal motor polyneuropathy<sup>5-7</sup>
  - Chronic neuropathy can result from a single attack or as on-going damage from repeated attacks<sup>5-7</sup>
- Attacks typically require hospitalization with supportive care, opioid analgesics and hemin<sup>4</sup>



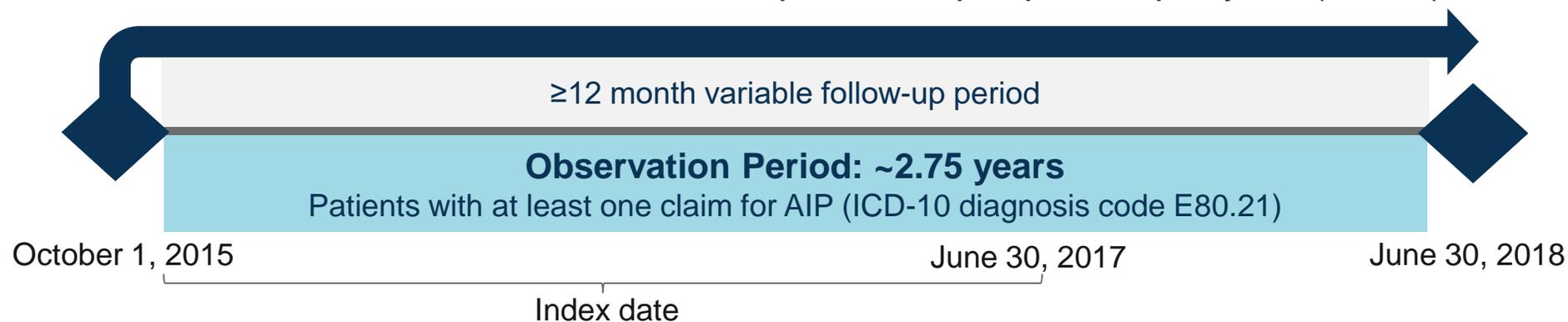
# Objective and Methods

## Objective

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

## Methods

- This retrospective analysis utilized the IBM® MarketScan® Commercial Claims and Medicare Supplemental Databases. Patients with at least one claim for AIP (ICD-10 diagnosis code E80.21) between October 1, 2015–June 30, 2018 were selected for analyses
- Medication Possession Ratio (MPR) was used to calculate medication usage and adherence based on pharmacy claims data. MPR usually ranges from 0 to 1. A value of 1 corresponds to 100% adherence
- Healthcare resource use and cost outcomes were reported as per patient per year (PPPY)



# Results

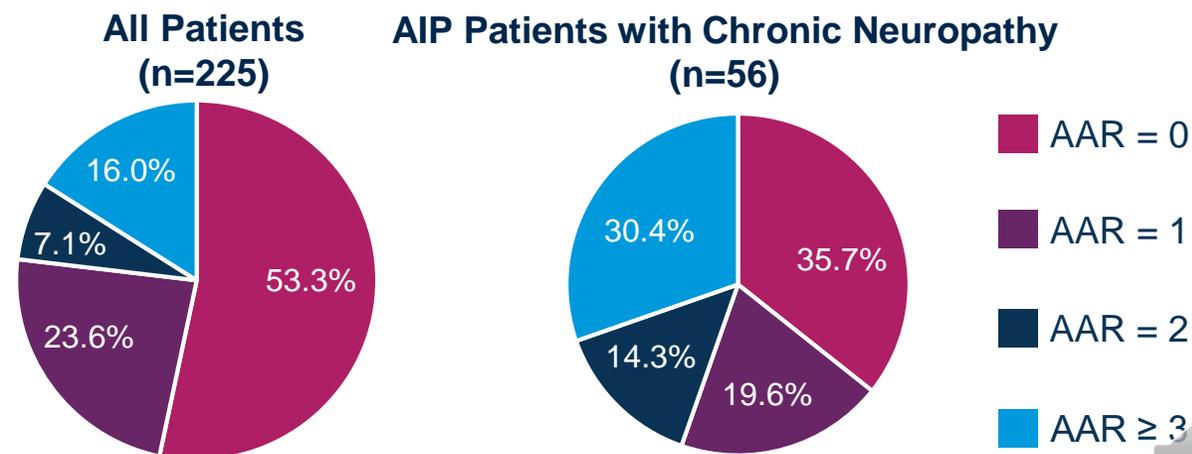
## Patient Demographics

- 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)
	Mean/n (SD/%)	Mean/n (SD/%)
Age	45.6 (16.4)	49.9 (14.8)
Gender, Female	157 (69.8%)	45 (80.4%)
Payer, Commercial	207 (92.0%)	51 (91.1%)
Length of Follow-Up, Years	1.8 (0.6)	2.0 (0.7)
Number of Attacks*	2.2 (2.8)	2.7 (3.4)
Liver Disease	28 (12.4%)	10 (17.9%)
Chronic Kidney Disease	21 (9.3%)	8 (14.3%)
Hypertension	106 (47.1%)	35 (62.5%)



# 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 medications (15.4)
  - Patients had frequent utilization, with Medication Possession Ratios (MPR) of 0.43 and 0.6, respectively

HCRU / Comorbidities	AIP Patients with Chronic Neuropathy (n=56)
	Mean/n (SD/%)
<b>Hospitalization</b>	1.0 (1.4)
<b>≥ 1 Hospitalization</b>	32 (57%)
<b>ER visit</b>	7.5 (23.2)
<b>≥ 1 ER visit</b>	42 (75%)
<b>Outpatient Visit</b>	19.9 (13.8)
<b>Pharmacy Claims</b>	57.4 (32.4)
<b>Opioids</b>	24.2 (28.7)
<b>MPR</b>	0.43 (0.39)
<b>Neuropathic Pain Meds</b>	15.4 (11.7)
<b>MPR</b>	0.60 (0.36)
<b>Any Pain Meds</b>	33.2 (34.2)
<b>MPR</b>	0.62 (0.40)



# 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

