

PB2354 THE IMPACT OF ACUTE HEPATIC PORPHYRIA ON MENTAL HEALTH: RESULTS FROM THE PORPHYRIA WORLDWIDE PATIENT EXPERIENCE RESEARCH (POWER) STUDY

Topic: 35. Quality of life, palliative care, ethics and health economics

Danielle Nance¹, Desiree Lyon², Sean Hegarty³, Rocco Falchetto⁴, Jasmin Barman-Aksözen^{4, 5}, Joana E. Matos⁶, Stephen Meninger⁷, Stephen Lombardelli⁸, Amy Dickey⁹

¹ Banner Health, Gilbert, United States; ² American Porphyria Foundation, Bethesda, United States; ³ Global Porphyria Advocacy Coalition, Durham City, United Kingdom; ⁴ Swiss Society for Porphyria, Zürich, Switzerland; ⁵ Stadtspital Zürich, Triemli, Institute of Laboratory Medicine, Zürich, Switzerland; ⁶ Cerner Enviza, North Kansas City, United States; ⁷ Alnylam Pharmaceuticals, Cambridge, United States; ⁸ Alnylam Pharmaceuticals, Maidenhead, United Kingdom; ⁹ Massachusetts General Hospital, Boston, United States

Background: Acute hepatic porphyria (AHP) is a group of rare genetic diseases of heme biosynthesis characterised by potentially life-threatening attacks, most frequently occurring in the abdomen. AHP is also associated with psychiatric symptoms, including depression and anxiety. Prior studies on AHP have also documented its psychosocial burden, which can limit patients' day-to-day activities, including social interactions and ability to work.

Aims: To investigate the burden of AHP on mental health, the study evaluated the effects of the disease on social life, personal life/goals, depression, and anxiety.

Methods: Patients age ≥ 18 years who experienced >1 AHP attack within the past 2 years or who were receiving intravenous hemin and/or glucose for attack prevention were recruited from the United States, Italy, Spain, Australia, Mexico, and Brazil from January 19 to April 26, 2021. Patients taking givosiran were excluded. Participants were administered an online survey that used standardised questionnaires and validated screening instruments to evaluate the impact of AHP symptoms on social life, personal life/goals, and mental health. The 8-item Patient Health Questionnaire depression scale (PHQ-8; range, 0–24) and the 7-item Generalized Anxiety Disorder scale (GAD-7; range, 0–21) were used to screen patients for depression and anxiety. Current moderate to severe depression was identified with a PHQ-8 cutoff score of ≥ 10 , and threshold scores of 5, 10, and 15 on the GAD-7 identified mild, moderate, and severe anxiety, respectively. Depression and anxiety were evaluated among the overall patient population as well as several subgroups, including those with sporadic attacks (0–5 attacks over 2 years) vs recurrent attacks (≥ 6 attacks over 2 years), those receiving vs not receiving prophylactic treatment for AHP, and those with an active disease duration of 0–5 years vs those with a disease duration of ≥ 6 years.

Results: Ninety-two patients with AHP completed the survey. Mean age was 41.1 years; 90% were female. Patients reported substantial impact on social life—76.1% of patients reported that most of their symptoms were hidden and that people in their social circle did not know they had AHP. An equal percentage of patients reported feeling frustrated that people do not understand the challenges they face, because their symptoms are not visible. Similarly, 72.8% of patients reported feeling guilty/upset that their symptoms and disabilities affect others (**Figure 1A**). Patients also reported AHP has impacted their personal life/goals. For example, $>80\%$ of patients reported having had to modify or give up goals important to them, more than half reported that the decline in their mental and physical health feels never-ending, and over a third reported they had lost their sense of purpose (**Figure 1B**). PHQ-8 scores indicating moderate to severe depression were reported in more than half of patients with AHP (58.7%) regardless of attack rate or prophylactic treatment status. GAD-7 scores indicating moderate to severe anxiety were reported in 48.9% of patients, and were highest in patients experiencing recurrent attacks (56.8%) (**Figure 1C**).

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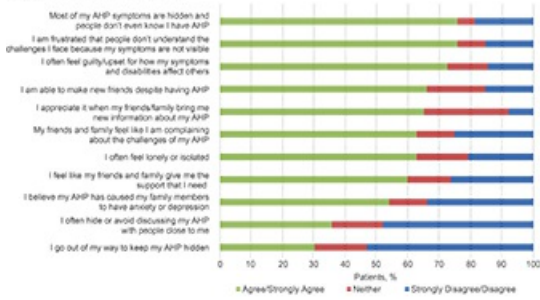
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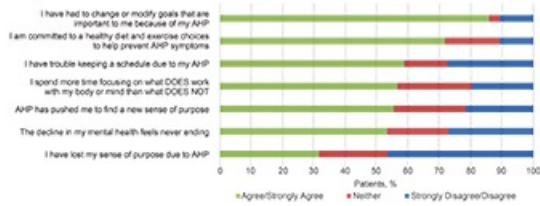
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Figure

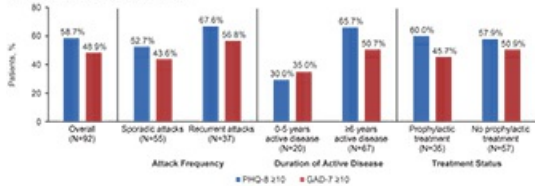
A. Impact of AHP on Social Life



B. Impact of AHP on Personal Life/Goals



C. PHQ-8 Scale and GAD-7 Scale



AHP, acute hepatic porphyria; GAD-7, Generalized Anxiety Disorder 7-item scale; PHQ-8, Patient Health Questionnaire 8-item depression scale.

Summary/Conclusion: Patients with AHP, regardless of attack rate, treatment received, or duration of active disease, experience a high mental health burden on their personal and social life. This study suggests that approximately half of patients with AHP experience moderate to severe anxiety or depression. These results highlight the importance of mental health monitoring as part of disease management for AHP.

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