

Abstract: PB3262

Title: THE PATIENT EXPERIENCE WITH WARM AUTOIMMUNE HEMOLYTIC ANEMIA: A TARGETED LITERATURE AND PATIENT-REPORTED OUTCOME MEASURE REVIEW

Abstract Type: Publication Only

Topic: Enzymopathies, membranopathies and other anemias

Background:

Warm autoimmune hemolytic anemia (wAIHA), an autoimmune disease in which autoantibodies target antigens on the red blood cell membrane, shortening their lifespan, can result in a constellation of signs and symptoms impacting one's health-related quality of life (HRQoL).

Aims:

To 1) review published literature to identify wAIHA signs/symptoms (S/S), 2) identify and evaluate patient-reported outcome measures (PROMs) that have been used in wAIHA to support their use in the target patient population, and 3) evaluate the concept coverage of each potential PROM.

Methods:

The literature review was conducted via OvidSP (limited to articles published between 2013 and 2023), Google/Google Scholar, and PubMed. Due to the limited literature on wAIHA, articles on autoimmune hemolytic anemia (AIHA) more broadly were included. S/S concepts identified in the literature were summarized. PROMs used in wAIHA were then identified via ClinicalTrials.gov. Concepts assessed by the identified PROMs were "mapped" against the summarized concepts from the literature review. A gap analysis was conducted for relevant PROMs to assess evidence of content validity, psychometric performance, and score interpretation. The gap analysis utilized data from research in analogue conditions (e.g., other AIHAs, chronic anemias or iron deficiencies) as no wAIHA-specific information was identified. The PROMs that related specifically to the key concept of interest (based on the literature results) were evaluated for appropriateness of use in a clinical trial including conceptual coverage, performance indicators, and completion burden.

Results:

From 15 articles, 38 S/Ss were identified, 15 of which may be suggested for measurement via patient-report. Among these 15 S/Ss, the most common reported S/S was fatigue (5/15 articles, 62% of patients in one article reviewed). Fatigue was considered the key S/S identified in the literature for assessment via PROM. Of eight PROMs included in the concept mapping exercise, the FACT-An (47 items) provided the most S/S coverage, although, for the key concept of fatigue, the FACT-An and FACIT-Fatigue/PROMIS Fatigue 13a (13 items) have the same level of coverage. The gap analysis, conducted for the five most relevant PROMs, demonstrated that evidence was most available for the FACIT-Fatigue (12/14 performance indicators) followed by the FACT-An (9/14 performance indicators). Considering all evidence, the FACT-An and FACIT-Fatigue/PROMIS Fatigue 13a have supportive data in populations similar to wAIHA. The remaining two PROMs, the SF-36v2 and EQ-5D, did not demonstrate strong evidence for the assessment of S/Ss, although both had more coverage when considering HRQoL more broadly.

Summary/Conclusion:

As a rare condition, there is limited information describing the wAIHA patient experience and few publications to guide development of a patient-reported outcome strategy for clinical trials. Fatigue was frequently described within the published literature and with high prevalence among patients, demonstrating it is a key concept of interest. While FACT-An and FACIT-Fatigue/PROMIS Fatigue 13a provide the same coverage assessing fatigue, patient burden should be considered when using multiple assessments in trials (i.e.,

minimizing length and focusing on assessment of key concepts). Thus, the FACIT-Fatigue/PROMIS Fatigue 13a may be most appropriate for assessing fatigue as a clinical trial endpoint, providing comprehensive coverage of the key concept, while minimizing burden on patients.

Keywords: Autoimmune disease, AIHA (see autoimmune hemolytic anemia), Autoimmune hemolytic anemia (AIHA)