Title: Literature Review of Fatigue Scales and Association with Clinically Meaningful Improvements in Outcomes among Patients with and without Paroxysmal Nocturnal Hemoglobinuria

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**Supplemental Table 1.** Participant-Intervention-Comparison-Outcome-Study criteria

| Element            | Criteria                                                                                                                                 |
|--------------------|------------------------------------------------------------------------------------------------------------------------------------------|
| **Population**     | • Patients ≥18 years of age with PNH  
                      • Patients ≥18 years of age with cancer (including cancer patients with anemia receiving or not receiving chemotherapy) |
| **Interventions**  | N/A                                                                                                                                     |
| **Outcomes**       | • Average FACIT-Fatigue scores at baseline  
                      • Change in FACIT-Fatigue score over follow-up  
                      • Proportion of patients who achieved a clinically meaningful improvement in FACIT-Fatigue score  
                      • Association between FACIT-Fatigue score and hemoglobin value  
                      • Association between incremental improvement in FACIT-Fatigue score and incremental improvement in hemoglobin value |
| **Study Design**   | Clinical trials and observational studies                                                                                              |
| **Language**       | English                                                                                                                                |
| **Geographic Setting** | U.S.; Multi-country studies with U.S. sites; Non-U.S. studies with North American or Western European sites                             |
| **Publication Type** | Full-text articles; conference abstracts                                                                                               |
| **Date of Publication** | Conference abstracts must not be published >2 years ago (i.e., 2017)                                                                    |

**Abbreviations:** FACIT, Functional Assessment of Chronic Illness Therapy; N/A, not applicable; PNH, paroxysmal nocturnal hemoglobinuria; U.S., United States.
### Supplemental Table 2. Description of electronic databases

| Database                                      | Description                                                                                                                                                                                                                                                                                                                                 |
|-----------------------------------------------|---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| MEDLINE                                      | Produced by the National Library of Medicine, MEDLINE covers the international literature on biomedicine, including the allied health fields and the biological and physical sciences, humanities, and information science as they relate to medicine and health care. The MEDLINE database contains more than 27 million references, indexed from approximately 5,600 journals world-wide and published in more than 30 languages. Coverage ranges from 1946 to the present with daily updates. |
| Embase                                       | Produced by Elsevier B.V., Embase (Excerpta Medica Database) is a biomedical and pharmacological database, and is especially strong in its coverage of drug and pharmaceutical research, pharmacology and toxicology. The Embase database contains more than 30 million records and includes articles from more than 8,500 journals (including all titles of the MEDLINE database) published world-wide. Embase adds over 1 million records annually. Coverage ranges from 1947 to the present. |
| Evidence-based Medicine Reviews - Cochrane Central Register of Controlled Trials | The Cochrane Central Register of Controlled Trials (CCTR) is a bibliographic database of definitive controlled trials, and part of Ovid’s Evidence-based Medicine Reviews collection. The CCTR contains over 300,000 bibliographic references to controlled trials in health care together with references to clinical trials identified by contributors to the Cochrane Collaboration in MEDLINE and Embase. Contributors follow quality control standards to ensure that only reports of definite randomized controlled trials or controlled clinical trials are included. Coverage ranges from 1991 to the present. |
| PsycINFO                                     | PsycINFO is an electronic bibliographic database providing abstracts and citations to the scholarly literature in the psychological, social, behavioral, and health sciences. The PsycINFO database contains more than 3.5 million records from approximately 2,290 journals (~99% are peer-reviewed). 30% of the database contains material of European origin, while an additional 12% of the database is from U.S. dissertations. Coverage begins as early as the 1800s and is updated weekly. |

**Abbreviations:** CCTR, Cochrane Central Register of Controlled Trials; U.S., United States.
### Supplemental Table 3. Description of search terms and search strategy

| No. | Search String                                                                 | Hits   |
|-----|-------------------------------------------------------------------------------|--------|
| 1   | (PNH or paroxysmal nocturnal hemoglobinuria).ti,ab.\(^a\)                     | 7,360  |
| 2   | (FACIT-Fatigue or fatigue).ti,ab.\(^a\)                                      | 274,557|
| 3   | 1 and 2                                                                      | 243    |
| 4   | Remove duplicates from 3                                                      | 185    |
| 5   | Limit 4 to English language                                                   | 173    |
| 6   | Limit 5 to human subjects                                                    | 142    |

*Accessed: November 6, 2019*

### Google Scholar

Results of the Google Scholar search were sorted by “relevance” and the first 100 search results were assessed for inclusion in this targeted literature review. Patents were not included in the search results.

*Accessed: November 6, 2019*

**Abbreviations:** FACIT, Functional Assessment of Chronic Illness Therapy; PNH, paroxysmal nocturnal hemoglobinuria.

**Note:**

\(^a\) “ti,ab” refers to terms in either the title or abstract fields of a search result.