Functional Assessment of Fatigue and Other Patient-Reported Outcomes in Patients Enrolled in the Global aHUS Registry

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This is a summary of outcomes reported by patients with atypical hemolytic uremic syndrome (aHUS for short) enrolled in the Global aHUS Registry

- Patient-reported outcomes are very important because they give a patient's perspective on a disease or therapy

Background

- aHUS is a rare disease
- In aHUS, a part of the body's immune system, called the complement system, becomes overactive and causes damage to the body
- Eculizumab (Soliris®), developed by Alexion Pharmaceuticals, Inc., was approved to treat aHUS in 2011

What did this analysis look at?

- This analysis looked at patient-reported outcomes in patients enrolled in the Global aHUS Registry

The observational **Global aHUS Registry** was established for long-term follow-up of patients with aHUS. It is the largest collection of information about patients with aHUS. It provides important insights into the course of the disease and real-world outcomes

- Patients can be enrolled in the Global aHUS Registry if they have a diagnosis of aHUS, but some patients are not able to be enrolled (like patients with evidence of an infection from a bacterium called *Escherichia coli* that makes a poison known as Shiga toxin)

- The FACIT-Fatigue scale is a validated questionnaire in multiple languages and was used to measure patients' fatigue
- Another questionnaire asked about general health and symptoms
- There were also questions about work and school, how often patients with aHUS visited their health care providers and the emergency room, and how often they were hospitalized

Change (→) in fatigue and other patient-reported outcomes were measured by comparing the results of the first and last surveys that patients completed

Which patients from the Global aHUS Registry were included in this analysis?

551 patients split into 3 groups

1. 233 **never** treated with eculizumab
2. 295 **already on** eculizumab at enrollment
3. 23 **started** eculizumab **after** enrollment

77% adults

23% children ages 5–17 years

- In patients who were already on eculizumab at the time they were enrolled, the median duration of eculizumab treatment before enrollment was 6 months (a median is the middle number in a list of numbers)

What are the results of this analysis?

- Patients who started eculizumab after enrollment had decreased fatigue, decreases in symptoms such as weakness, irritability, and nausea/vomiting, and improved general health status
- Patients on eculizumab at enrollment in the registry had FACIT-Fatigue scores close to normal and had minimal changes in their scores from first to last survey
- Patients never treated with eculizumab had near-normal FACIT-Fatigue scores
- The number of emergency room visits and hospitalizations were similar across all patients. Patients who started eculizumab after enrollment reported more health care provider visits and missed days of work, likely because they started a new therapy

Patient group	Change in FACIT-Fatigue score	Clinically meaningful decrease in fatigue
1 **Never** treated with eculizumab	0-Point change	33%
2 **Already on** eculizumab at enrollment	1-Point change	27%
3 **Started** eculizumab **after** enrollment	9-Point change	80%

Patient symptoms

- Fatigue, headache, and weakness were the symptoms reported by the most patients at the beginning and end of the analysis (measured by percent)

61.4% → 64.4%
Fatigue

54.4% → 55.0%
Headache

46.4% → 42.3%
Weakness

- The group with the biggest decrease in fatigue as a symptom was the group that started eculizumab after enrollment

Effects of dialysis and hospitalization

- Patients on dialysis reported lower FACIT-Fatigue scores than patients not on dialysis on both their first and last surveys
- The same was true for patients who were recently hospitalized versus those who were not recently hospitalized

General health

- Overall, patients' general health status did not change very much over time (measured by percent)

69.1% → 76.5%
Reported good/very good/excellent

30.9% → 23.5%
Reported fair/poor

- The group with the most patients whose health status improved was the group that started eculizumab after enrollment

Resource utilization

- Overall, patients reported:

0.38
Health care provider visits per year

0.13
Emergency room visits per year

0.17
Hospitalizations per year

- Patients who started eculizumab after enrollment reported more health care provider visits

Work status

- 32.1% of patients reported working either full-time or part-time
- Patients who started eculizumab after enrollment reported missing more days of work, likely because they started a new treatment

Overall, patients missed 1.58 work days per year

What were the main conclusions from this analysis?

Changes in FACIT-Fatigue scores appeared to be tied to changes in the treatment of aHUS

- Patients who started eculizumab after being included in the registry saw the greatest improvement
- Patients always on eculizumab and never on eculizumab reported similar scores on their first and last surveys

The symptoms most commonly reported by patients were fatigue, headache, and weakness

Overall, 69%–77% of patients reported good/very good/excellent general health, compared with 24%–31% reporting poor or fair general health

Who sponsored this analysis?

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Are there any plans for further analysis?

The Global aHUS Registry is continuously updated, and new findings will be reported, including data on ravulizumab, a treatment for aHUS approved by the FDA in 2019

This summary is based on the following research article:

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