

aHUS Facts- a Brief Look

at the Rare Disease Atypical Hemolytic Uremic Syndrome

2017 September



About aHUS

- Atypical Hemolytic Uremic Syndrome (aHUS) is a very rare, chronic and life-threatening genetic condition
- **aHUS can occur at any age, with roughly 60 per cent of children affected and 40 per cent adults**²
- aHUS is caused by chronic, uncontrolled activation of the complement system, a part of the body's natural immune system¹
- As a result, the immune system attacks the body's unhealthy and healthy cells, which can cause abnormal blood clotting and blood vessel damage^{2,3}
- **The presence of blood clots causes damage to organs, leading to heart attack, stroke, kidney failure and death**²
- **Death rates amongst aHUS patients are as high as 25 per cent, and progression to end-stage kidney disease occurs in more than 50 per cent of patients**^{2,5}
- Kidneys are often transplanted in aHUS patients with permanent kidney failure, however, the disease recurs in 60 per cent of patients, and more than 90 per cent of patients experience failure of transplanted kidney²

Diagnosis

- **Atypical HUS encompasses a group of diseases that share in the clinical features of a microangiopathic hemolytic anemia associated with thrombocytopenia and renal failure.**²⁵
- The causes of aHUS are not fully understood, but in 70 per cent of cases it is associated with an underlying genetic or acquired abnormality of the complement system¹⁰
- During initial onset of aHUS, or during recurring episodes, tell-tale signs can be detected from lab findings relating to⁹
 - platelet levels
 - hemoglobin and haptoglobin levels
 - creatinine levels
 - BUN (blood urea nitrogen) levels

2016 aHUS Global Poll <http://ow.ly/1DA7303FoJx>

aHUS Awareness Day is 24 September

Symptoms

- aHUS disease can be characterized by three key features:¹²
 - thrombocytopenia (low platelet count in the blood)
 - anemia (low red blood cell/platelet count in the blood)
 - kidney symptoms (starting as acute kidney failure but can progress to end-stage kidney disease)
- There are a number of symptoms secondary to kidney failure, which include ¹⁰
 - nausea and vomiting
 - confusion
 - shortness of breath (dyspnea)
 - fatigue

Treatment

Plasma Therapy & Dialysis

- The prognosis for patients with aHUS is very poor,¹³ with existing supportive therapies unproven and unreliable
- The management of aHUS has relied on plasma infusion and plasma exchange therapies with variable results¹⁴
- To date, there have been no well-controlled trials that show plasma exchange or plasma infusion to be safe or effective in aHUS¹⁵
- In studies where the majority of patients with aHUS were treated with plasma therapy, patient outcomes were reported as being poor¹⁶
- Dialysis cannot completely compensate for the loss of kidney function, and can lead to deadly infections and shortened life expectancy ¹⁷

Treatment- Therapeutics

- Eculizumab has shown greater efficacy than plasma therapy in the prevention and treatment of aHUS^{16, 19}
- Switching from plasma therapy to eculizumab has been shown to improve renal function even in patients with long-lasting and stable chronic kidney disease ¹⁶
- KDIGO Controversies Conference on aHUS and C3G: See *Treatment Strategies, Section V*. Goodship et al, Dec 2016. Kid. Intl. <http://ow.ly/DCjf30euh7n>
- aHUS Alliance – Therapeutic Drugs, R & D Landscape. Jan 2017. <http://ow.ly/vlDJ30euhZQ>
- Clinical Trials – Currently recruiting as of Aug 2017 on www.ClinicalTrials.gov (atypical HUS): ALXN1210 ID# NCT02949128 and OMS721 ID# NCT03205995

Access to Treatment

- As of August 2017 aHUS patients in many nations still do not have access to eculizumab, and coverage within some of those countries is further restricted: dependent on the aHUS patient's location within their nation or their individual health status ²⁶.
- Inequality in Treatment Options among Nations - Access to eculizumab for treatment of aHUS patients worldwide plummets from 77% to only 37% for poll respondents in nations outside of the US & EU. ²⁸ (White Paper at <http://ow.ly/Dbzb303ZqhU>, with 2016 Poll Results: <http://ow.ly/1DA7303FoJx>)

Note: The aHUS Alliance wishes to extend thanks to aHUS Canada for their efforts in providing core facts here. For Citations & More Info, see the Full Version of this Document: **aHUS Key Facts & Information**

NIH NCBI GeneReview: *Genetic Atypical Hemolytic-Uremic Syndrome* <http://ow.ly/CmhB30euQTI>

