Atypical HUS: In Brief
Atypical Hemolytic Uremic Syndrome - a Rare Disease

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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 percent of children affected and 40 percent adults²
• 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 damage²,³
• The presence of blood clots causes damage to organs, leading to heart attack, stroke, kidney failure and death²
• Death rates amongst aHUS patients are as high as 25 percent, and progression to end-stage kidney disease occurs in more than 50 percent of patients²,⁵
• 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.²⁰
• 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 system²⁰
• 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


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 include10
  - 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 expectancy17

Treatment- Therapeutics

- Eculizumab has shown greater efficacy than plasma therapy in the prevention and treatment of aHUS16,19
- 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
- aHUS Alliance – aHUS Therapeutic Drugs, R & D, with tables. May 2018 https://bit.ly/2xpfg0T

Access to Treatment

- As of Sept 2019, 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. Proposal: Global Panel for aHUS Drug Access https://bit.ly/2MKPIQO

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