aHUS One Year On

By Monique Burgin

Nephrotic Renal Nurse, E17 QMC

Ext 62243

Patient History

- 2 year old boy
- Diagnosed with Atypical Haemolytic Uraemic Syndrome (aHUS) one year ago.
- Presented with diarrhoea, vomiting and acute kidney injury.
- Transferred to PICU with severe shock, metabolic acidosis and anaemia.
- Discharged after 1 month, now seen in clinic.

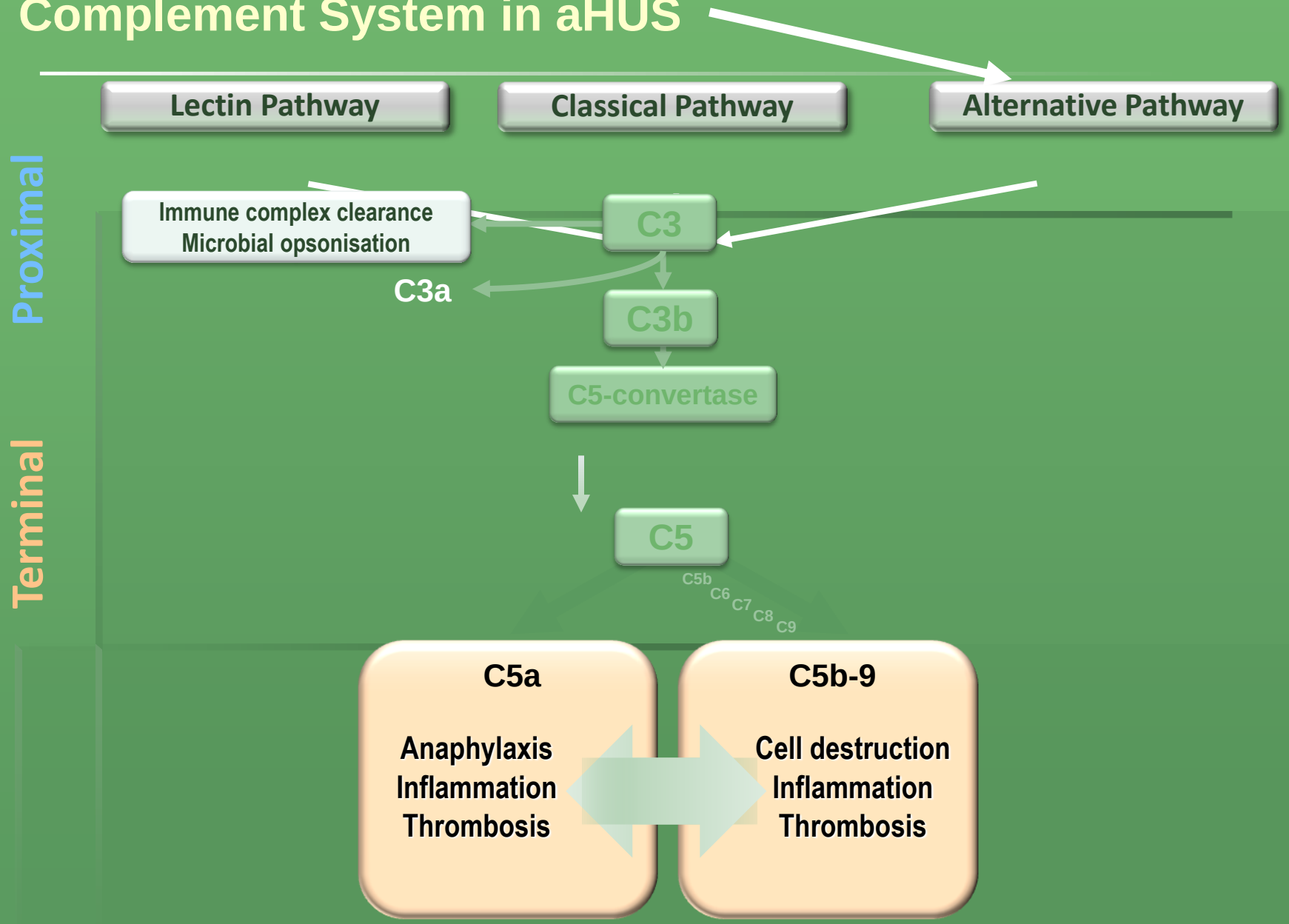
Initial Presentation

- Consistent with haemolytic uraemic syndrome (HUS)
- Kidney failure, haemolysis and thrombocytopenia.
- 16weeks old & breast-fed - E.coli O157 associated HUS extremely unlikely.
- Stool sample negative.
- Atypical Haemolytic Uraemic Syndrome (aHUS) suspected.

What Is aHUS?

- Atypical Haemolytic Uraemic Syndrome (aHUS)
- Approx 3 per million in children
- 65% die, require dialysis, or have permanent renal damage within 1 year.
- Triggered by genetic predisposition and environmental factors.
- Complement- mediated condition
- Over activation of the alternative complement pathway

Complement System in aHUS



What Is Eculizumab?

- Eculizumab blocks the complement pathway.
- Humanised version of anti-C5 antibody, binding specifically to the complement protein C5 (NHS 2012).
- Stabilises terminal complement protein C5 and prevents out of control turnover of the alternative complement pathway.

Treatment

- Current treatment for patients with aHUS was inadequate.
- Standard treatment for AHUS is plasma therapy
- Children often become resistant to the plasma infusions.
- Eculizumab as the first line therapy for aHUS avoiding plasma exchange and central venous catheters owing to the high risk of complications (Alexion 2013).
- Urgent approval was put in to trial a dose of the drug.

Eculizumab Trial

- Individual funding request due to cost of the drug.
- Consent given for 3 doses before agreeing to future funding.
- Day 3 after Eculizumab dialysis independent
- Lactate dehydrogenase (LDH) is used as a marker for tissue damage.
- LDH peaked at 2388 units/L prior to infusion.
- Last clinic result 530units/L.

Impact Upon Family

- Life long treatment of Eculizumab,
- 8 months after relapsed, following a chest infection.
- LDH 922 units/L, presented with macroscopic haematuria,
- Complement blocked at relapse. Possibly mediated by pathway independent of complement.
- suggested stopping the infusions. Samples have been sent to the USA for further testing.

- NHS England funding Eculizumab for all new cases.
- 3 hour round trip every 3 weeks
- Infusions increased to every 2weeks due to recent relapse.
- Difficulties in venous access and increasingly stressful for the family.
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- PortaCath was inserted to minimise the trauma of these regular venepunctures.
- Vast improvement made to all the lives of our aHUS patients & families


My First Christmas

THANK YOU SO MUCH FOR LISTENING

Any Questions?