

Plain language summary – treatment preference and quality of life impact: ravulizumab vs eculizumab for atypical hemolytic uremic syndrome

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*At the time of the study.

INTRODUCTION TO aHUS

- Atypical hemolytic uremic syndrome, also called aHUS, is a rare disease that causes blood clots to form that block small blood vessels throughout the body. These blood clots can damage vital organs, such as the kidneys.
 - aHUS happens when a part of the immune system, called the **complement system**, becomes overactive.
- Ravulizumab and eculizumab lower the risk of kidney damage in patients with aHUS. They achieve this by stopping the complement system from becoming overactive.
 - Ravulizumab, also called ULTOMIRIS[®], was approved in the USA in 2019 and in the European Union in 2020 for the treatment of aHUS.
 - Eculizumab, also called SOLIRIS[®], was approved in the USA and European Union in 2011 for the treatment of aHUS.
- These treatments are given by intravenous (IV) infusion, where the medication is infused into the patient's bloodstream.
- Although the treatments have been shown to work as well as each other, ravulizumab doesn't need to be infused as often as eculizumab.
 - Ravulizumab is infused every 4 to 8 weeks, while eculizumab is infused every 2 weeks.
 - Less frequent infusions may be preferred by patients and caregivers, as it is more convenient.



WHY WAS THIS STUDY NEEDED?

- Understanding treatment preferences and how those treatments affect people's lives can help doctors to support people in making decisions about the management of their, or their child's, aHUS.

HOW WAS THIS STUDY DONE?

- Researchers created two separate surveys for people with aHUS who received eculizumab and then switched to the 10 mg/mL or 100 mg/mL formulation of ravulizumab.
 - One survey was given to adults with aHUS.
 - The other survey was given to caregivers, such as the parents of children with aHUS.
- The surveys were used to find out whether these people preferred eculizumab or ravulizumab, and how the treatments affected their lives.
- Surveys were written in English.
- In total, 50 adults with aHUS and 16 caregivers (each with one child with aHUS) were recruited and took part in the study.
- All participants were from the USA.

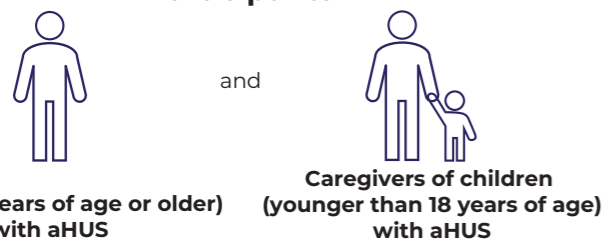


The average (mean) age of the adults was **46.5 years**

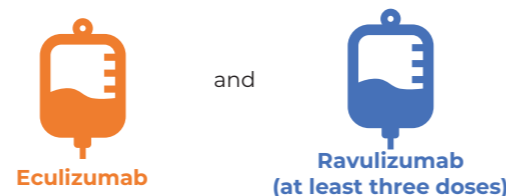


The average (mean) age of the children was **10.1 years**

Participants

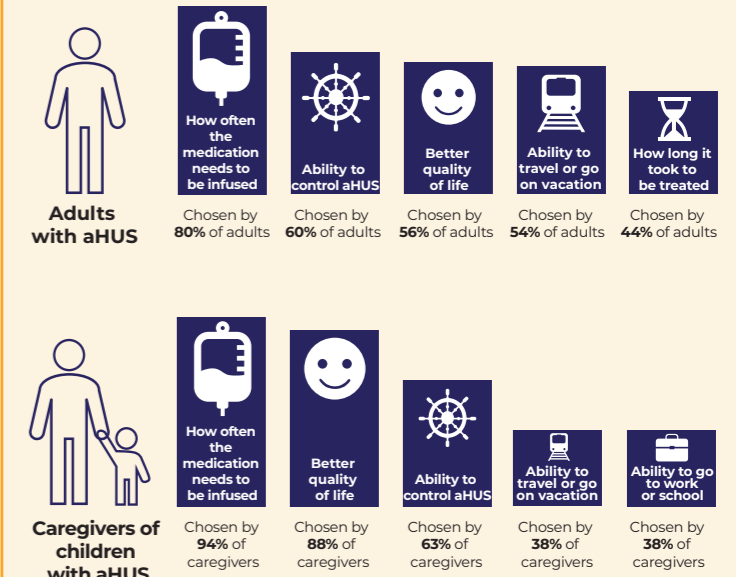
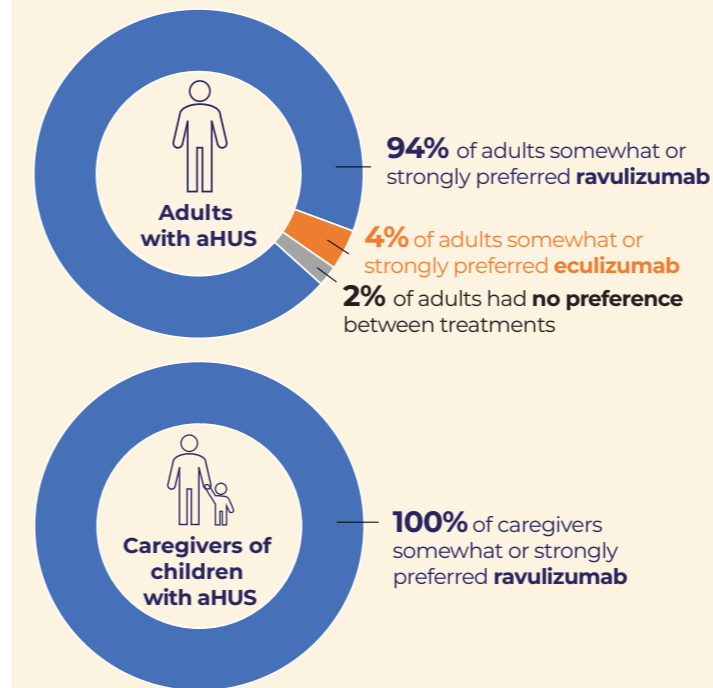


All patients had received eculizumab then at least three doses of ravulizumab

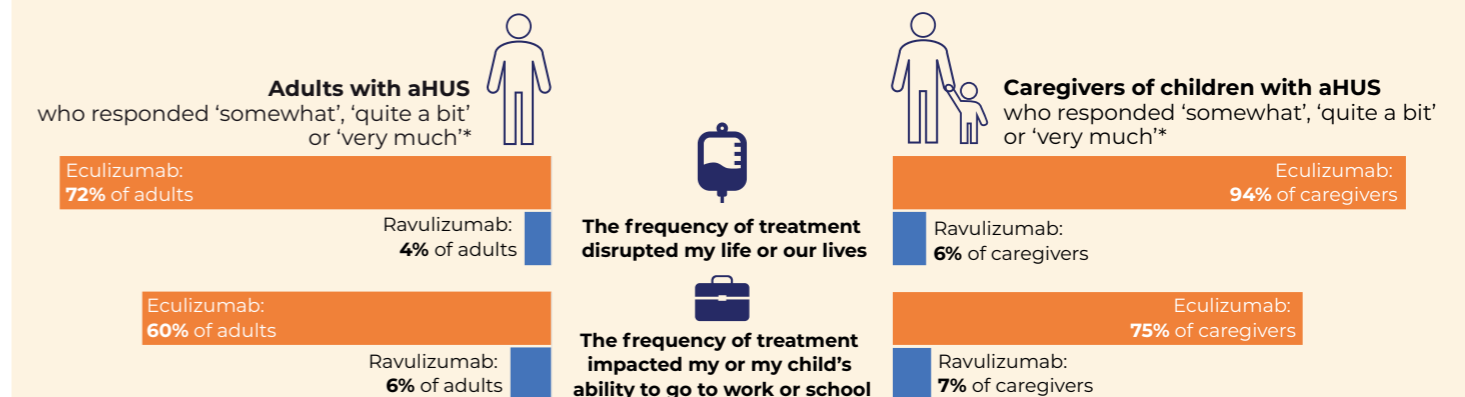


WHAT DID THIS STUDY FIND?

- Most participants preferred ravulizumab over eculizumab and the statistics show that the difference between the number of participants who preferred ravulizumab and those who preferred eculizumab is unlikely to be due to chance.
- The most important thing for participants when selecting their preferred treatment was how often it needed to be infused.



- More participants reported that eculizumab disrupted their or their child's life and ability to go to work/school compared with ravulizumab.



*The alternative response options were 'not at all' or 'a little bit'

WHAT DOES THIS MEAN FOR PATIENTS?

- Most participants preferred ravulizumab over eculizumab for the treatment of aHUS.
 - This was mostly because ravulizumab needs to be infused less often than eculizumab, meaning that ravulizumab had less of an impact on participants' daily lives.
 - These findings can help patients, caregivers and doctors to decide whether to choose ravulizumab or eculizumab to treat aHUS.

WHERE CAN I FIND MORE INFORMATION?

This plain language summary is based on the article called 'Treatment preference and quality of life impact: ravulizumab vs eculizumab for atypical hemolytic uremic syndrome' which has been published in the *Journal of Comparative Effectiveness Research*.

ACKNOWLEDGMENTS & SPONSOR INFORMATION

Alexion, AstraZeneca Rare Disease, Boston, MA, USA (hereafter 'Alexion') sponsored this study. Alexion and the authors would like to thank all patients and their families, physicians and patient organizations for their assistance. Alexion also thanks independent patients and patient organizations for their reviews of this summary. The authors of the original research article reviewed and approved the summary. Summary prepared by Rebecca Prince, BMBS, MSc, of Oxford PharmaGenesis Ltd. Funding for preparation of the summary and editorial review was provided by Alexion.