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Long-term ravulizumab treatment in complement inhibitor-experienced patients with PNH provides durable control of intravascular hemolysis with low incidence of major adverse vascular events and death

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PLAIN LANGUAGE SUMMARY

Why did we perform this research?

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare long-term blood disease that can lead to serious, potentially life-threatening complications (such as organ damage and blood clots) or death if left untreated. Ravulizumab is a type of treatment known as a 'complement inhibitor.' It works by binding to a protein (also known as 'complement component 5') in the immune system, which helps to reduce the symptoms of PNH, including potentially life-threatening blood clots, and improves patient quality of life. As PNH is a life-long disease, it is important to understand the long-term impact of treatment. So far, studies have shown that ravulizumab continues to manage most patients' PNH symptoms for up to 2 years. This poster describes ravulizumab treatment results for up to 4 years.

How did we perform this research?

Study 302 (an international phase 3 clinical study) is currently investigating the long-term impact of ravulizumab treatment in patients with PNH who were previously treated with eculizumab (another complement inhibitor for treating PNH). In this study, patients with PNH were randomly chosen to receive treatment with either ravulizumab or eculizumab for up to 26 weeks. After 26 weeks, depending on the treatment they received, patients either continued ravulizumab or switched from eculizumab treatment to ravulizumab for the long-term study period.

What were the findings of this research and what are the implications?

The results show that patients with PNH treated with ravulizumab for up to 4 years continued to have improved symptoms of PNH and their disease was well controlled. This study also showed that there were few side effects related to ravulizumab treatment and a very low number of patients had to stop treatment because of ravulizumab. Finally, none of the 3 deaths that occurred during this long-term study were linked to ravulizumab treatment.

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