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Pegcetacoplan, a Novel C3 Complement Inhibitor – Beginning of a new era in the Treatment of Paroxysmal Nocturnal Hemoglobinuria (PNH)?

PNH is a rare but severe and usually life-long blood disease in which the blood cells are fragile and easily destroyed by a part of the patient's own immune system, called complement. In PNH, this results in fatigue and severe anaemia, often requiring regular blood transfusions, passing red or black urine due to the breakdown of red cells, and severe life-threatening blood clots in critical organs such as the liver. An earlier approved complement therapy, eculizumab, a C5 inhibitor, is the current standard of treatment for PNH. Although it is helpful, many patients still remain anaemic and fatigued on eculizumab and continue to need transfusions. A new investigational drug called pegcetacoplan targets C3, a central part of the complement system and in early studies, promised to be more effective than eculizumab resulting in the resolution of anaemia and greatly improved symptoms.

We present the PEGASUS study results that show pegcetacoplan is significantly more effective than eculizumab in improving haemoglobin as well as other key disease markers in patients with PNH. PEGASUS is the first randomised Phase III trial of a proximal C3 inhibitor and shows a rapid and sustained improvement in haemoglobin, a reduction in ongoing break down of blood cells, a marked reduction in blood transfusions, and marked improvement in fatigue. Pegcetacoplan promises to be a novel, more effective therapy for PNH.

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Abstract: **#S192** RESULTS OF THE PEGASUS PHASE III RANDOMIZED TRIAL DEMONSTRATING SUPERIORITY OF THE C3 INHIBITOR, PEGCETACOPLAN, COMPARED TO ECULIZUMAB IN PATIENTS WITH PAROXYSMAL NOCTURNAL HEMOGLOBINURIA

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