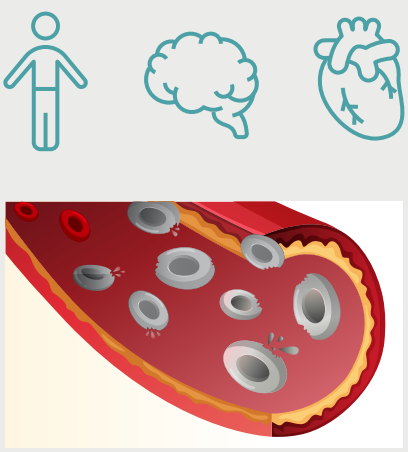
The Science behind Paroxysmal Nocturnal Haemoglobinuria

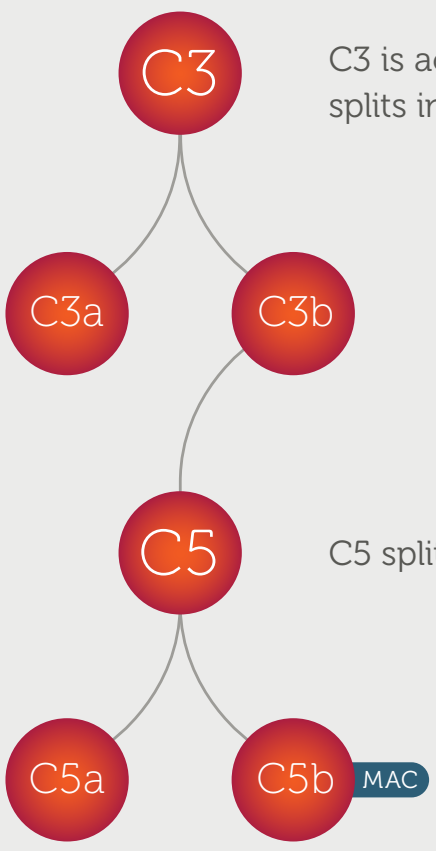
Paroxysmal nocturnal haemoglobinuria (PNH) is a potentially life-threatening bone marrow disease which has significant effects on patients physically, mentally and emotionally.[1]

PNH is **chronic** and **acquired**, caused by mutations in the **bone marrow stem cells**. In people with PNH, the body 'breaks apart' its own red blood cells because they are missing important protective proteins.[1]



The 'breaking apart' of these red blood cells is known as **haemolysis**. It occurs after a cascade of activity is started within the **complement system**, which is a **complex immune surveillance** system made up of multiple protective proteins that helps destroy dangerous toxins, **viruses or bacteria**, and gets rid of damaged or dying cells. Due to the missing protection, haemolysis can occur uncontrolled in people with PNH.[2,3]

Complement Cascade[4]



C3 is activated and splits into C3a and C3b

C3b, part of the C5 enzyme, activates C5

C5 splits into C5a and C5b

The **membrane attack complex (MAC)** is formed to help destroy dangerous foreign bodies, like viruses and bacteria, in the body[4]

Striving to help

PNH is a relatively unknown condition with symptoms that can make daily life challenging, and is associated with many serious and potentially fatal complications.[3]

Sobi is striving to help patients manage their disease and overall, improve their quality of life. For more information, visit my-PNH.com.

References:
1. Mitchell R, *et al*. Path to diagnosis of paroxysmal nocturnal hemoglobinuria: the results of an exploratory study conducted by the aplastic anemia and MDS international foundation and the national organization for rare disorders utilizing an internet-based survey. *SM Clin Med Oncol.* 2017;1(1):1001. **2.** Devos T, *et al*. Diagnosis and management of PNH: Review and recommendations from a Belgian expert panel. *Eur J Haematol.* 2018;101:737–749. **3.** Brodsky RA. Paroxysmal nocturnal hemoglobinuria. *Blood.* 2014;124(18):2804–2811. **4.** Merle NS, *et al*. Complement System Part I – Molecular Mechanisms of Activation and Regulation. *Front Immunol.* 2015;6:262.

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