

# Understanding the current treatment landscape for paroxysmal nocturnal hemoglobinuria



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# Disclaimer

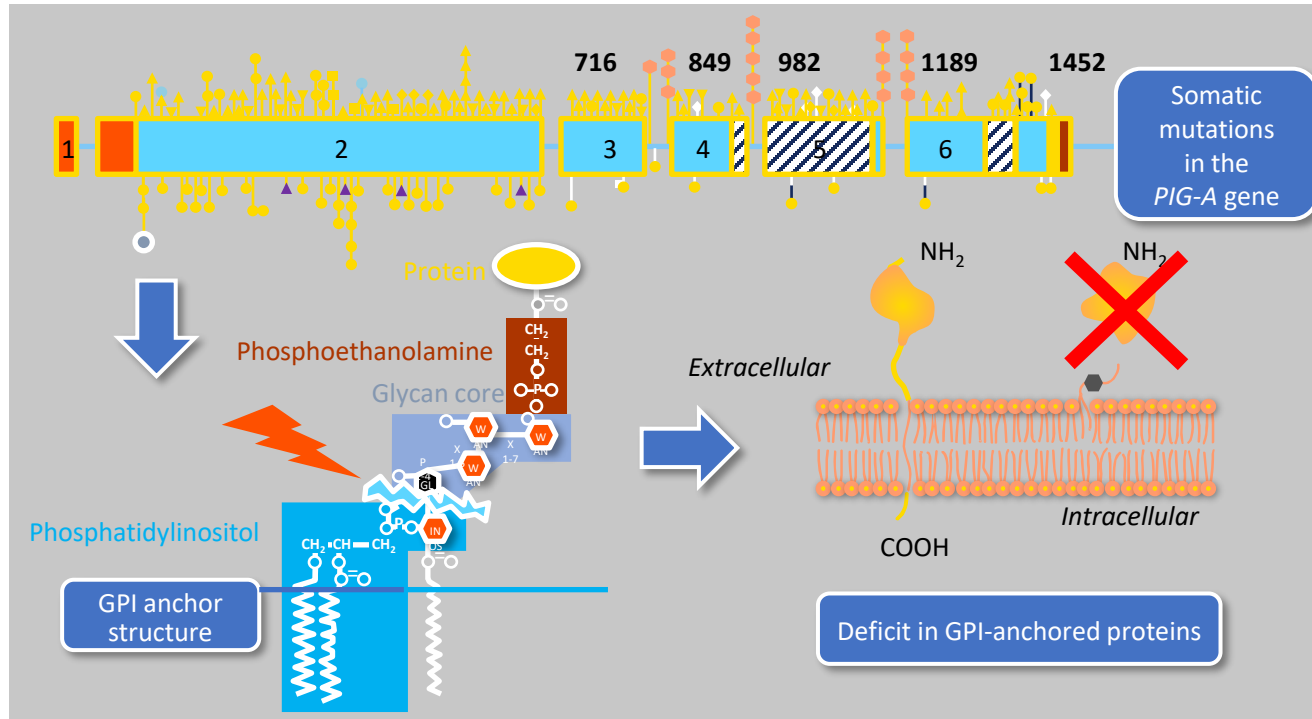
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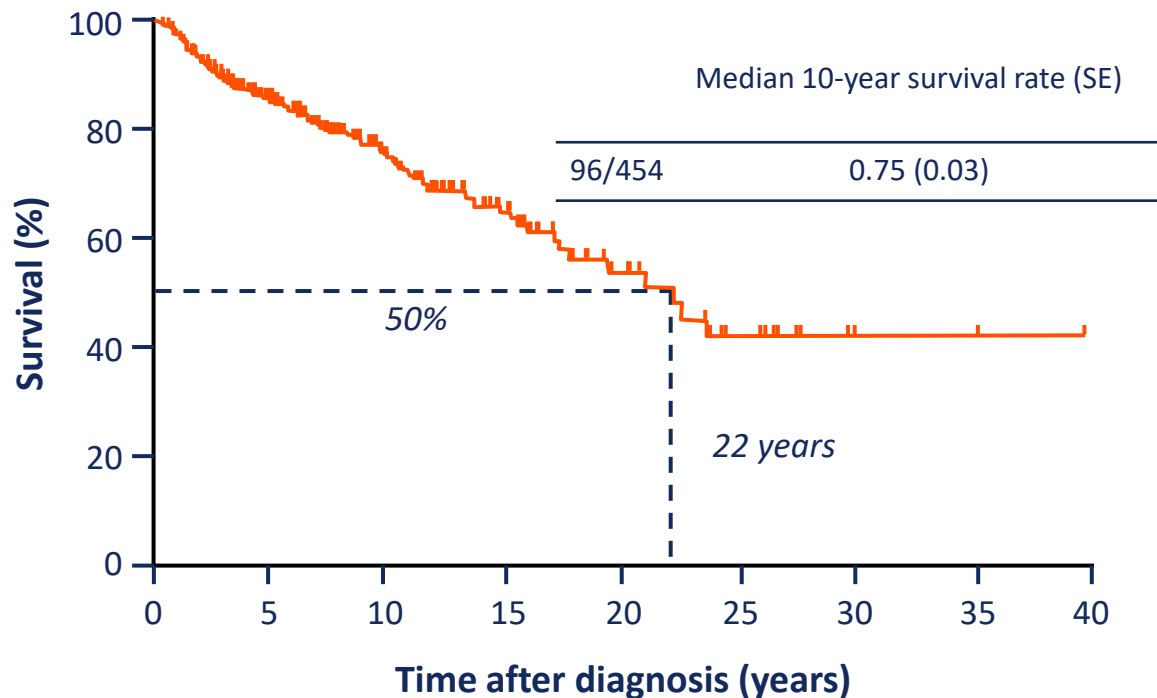
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# Pathophysiology of PNH



Peffault de Latour RP, et al, *Blood*. 2008;112:3099–106.  
 GPI, glycosylphosphatidylinositol; PNH, paroxysmal nocturnal hemoglobinuria.

# Mortality in patients with PNH



# CATCH criteria as indications for PNH testing

C

## Cytopenias

Patients for whom a bone marrow examination is considered for otherwise unexplained cytopenia

A

## AA/MDS

All patients with a diagnosis or suspicion of AA  
  
Low or intermediate-1 risk MDS, and especially if hypoplastic

T

## Thrombosis

Unprovoked and/or unusual site thromboembolic event, especially if recurrent and/or despite anticoagulation

C

## Coombs-negative hemolysis

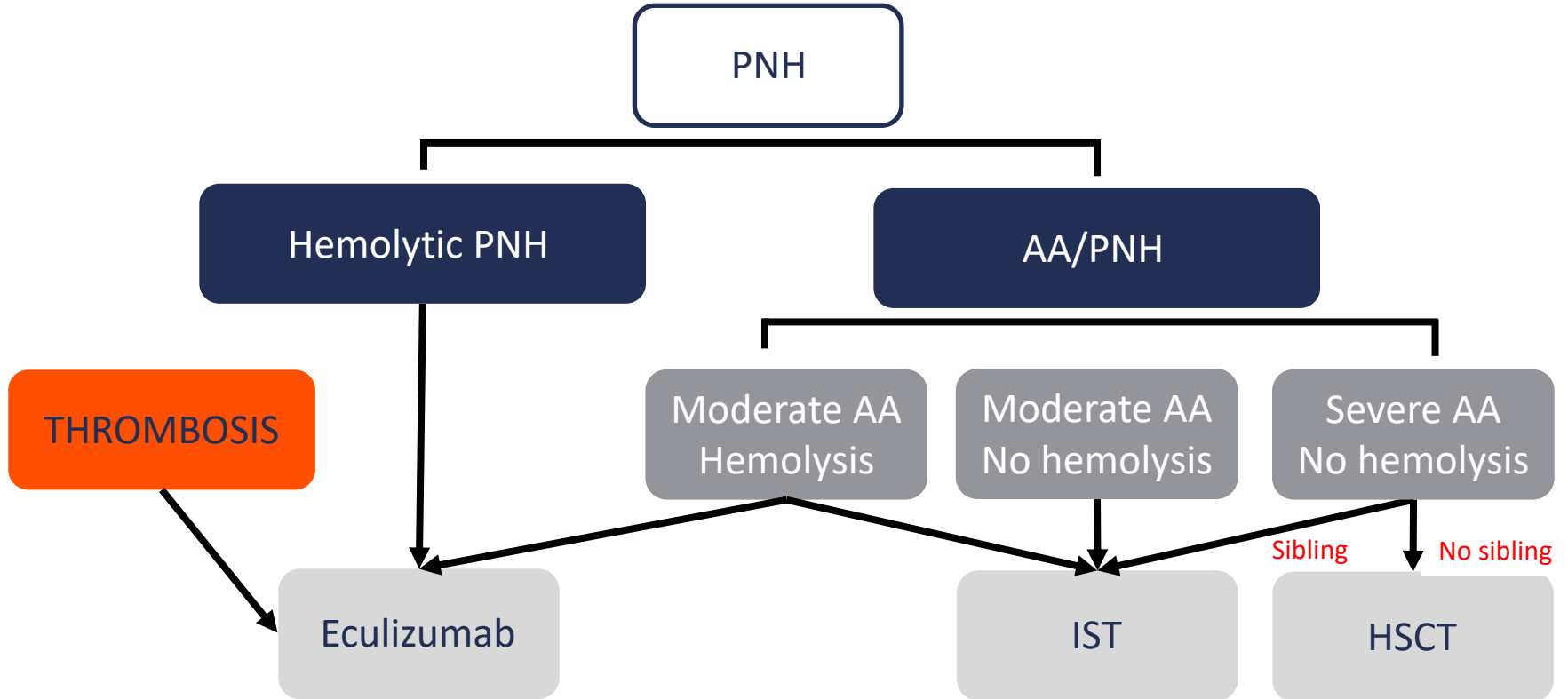
Hemolysis or hemolytic anemia without other clear cause

H

## Hemoglobinuria

Otherwise unexplained hemoglobinuria or cases where “hematuria” has been identified without evidence of erythrocytes on microscopy

# Current management of PNH



# Eculizumab: key trials and studies



## TRIUMPH<sup>1</sup>

- Eculizumab versus placebo
- Transfusion-dependent hemolytic patients with PNH with no overt bone marrow failure
- Stabilization of hemoglobin levels was 0% compared with 49% for placebo and eculizumab respectively (P<0.001)



## SHEPHERD<sup>2</sup>

- Open-label, non-randomized trial
- Only one prior transfusion required in the previous two years
- Patients with lower platelet counts than in the TRIUMPH trial were eligible
- Hemolysis reduced by mean 87%, as measured by lactate dehydrogenase levels (P<0.001)



## Real-world data

- Single-centre, long-term study found 66% patients receiving eculizumab for more than 12 months achieved transfusion independence<sup>3</sup>
- Registry study reported overall survival at six years was 92% in eculizumab cohort compared with 80% in historical controls (P=0.037)<sup>4</sup>