

# The Health and Socioeconomic Burden of Paroxysmal Nocturnal Hemoglobinuria (PNH) in Germany

Authored by WifOR

Ioannis Athanasiou, MSc

Malina Müller, PhD

June 2024

# Table of Content

1	Background .....	3
2	Objectives .....	4
3	Methodology .....	4
4	Results .....	4
5	Conclusion .....	5
	References .....	6

# 1 Background

Paroxysmal Nocturnal Hemoglobinuria (PNH) is a rare and life-threatening hematologic disorder that poses significant challenges not only for patients but also for the healthcare system and the broader society. PNH is characterized by the destruction of red blood cells (RBC), thrombosis, and bone marrow failure, which impose a substantial burden on patients, healthcare systems, and society (Bektas et al., 2020; Panse et al., 2022).

## **Patient Burden**

For individuals diagnosed with the condition, the implications are profound and complex. The debilitating symptoms of PNH, such as severe fatigue and the destruction of large numbers of RBC over a short period of time (hemolytic crisis), impair the ability to maintain regular employment, exacerbating financial instability and reducing overall quality of life. The chronic nature of the disease necessitates lifelong management, involving frequent medical consultations, routine RBC transfusions, and advanced treatments such as eculizumab and ravulizumab, which are administered to the patients intravenously. These therapies are effective in preventing symptoms and prolonging the life expectancy of patients but are also costly, representing a significant financial strain on patients and their families. Furthermore, receiving such treatments can be a time-consuming process; the PNH patient would lose significant productive time to travel to the clinic or hospital and receive intravenous therapy with eculizumab and ravulizumab.

## **Societal Burden**

Apart from the overall impact that PNH has on patients, it also imposes a substantial indirect economic burden to society. The frequent medical complications that patients exhibit, such as hemolytic crisis and sustained fatigue, and the time needed to receive treatment with complement inhibitors, impair their ability to work consistently. This loss of productivity translates into economic losses for both the individuals affected and the broader economy.

## 2 Objectives

In Germany, the health and socioeconomic impact of PNH is insufficiently studied due to the rarity of the disease and scarcity of information available. WifOR's study, commissioned and supported by Novartis, utilized data from the international PNH registry and the "Adelphi PNH Disease Specific Program" (DSP)<sup>1</sup> of PNH patients receiving eculizumab and ravulizumab, to calculate the health and socioeconomic burden of disease for patients receiving complement inhibitors, in a lifetime horizon (Anderson et al., 2008; Schrezenmeier et al., 2020).

## 3 Methodology

The health burden in Germany due to PNH was measured as 1) the number of RBC transfusions and blood units transfused, 2) the number of hospitalizations and hospital days, 3) the number of patients reporting moderate and severe fatigue and 4) the total time spent for IV infusions. These outcomes were linked to the potential productivity losses to estimate the socioeconomic burden of PNH, i.e. the productive hours lost due to the disease. Finally, the socioeconomic burden was monetized using the average wages of all types of employees in the German economy to produce the total and per patient monetized socioeconomic burden.

## 4 Results

The epidemiological estimates showed that in 2023 1,137 adult patients were diagnosed with PNH in Germany, of whom 825 were under treatment with eculizumab and ravulizumab (C5 treatment). The study results highlighted that over a lifetime, patients under C5 treatment would still be in need of RBC transfusions (mean value: 144,226 RBC transfusions in total, Standard Deviation (SD)<sup>2</sup>: 13,898) and lose significant time to receive their IV infusions with C5 inhibitors (mean value: 15,896,874 total hours lost to receive IV infusions, SD:1,815,945 hours).

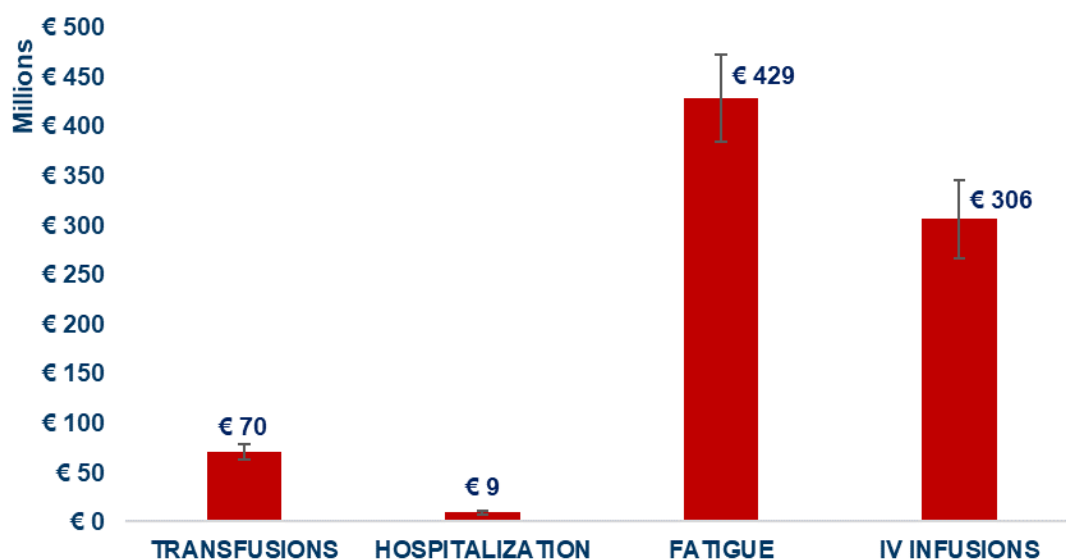
---

<sup>1</sup> The "Adelphi PNH Disease Specific Program" (DSP) is a real-world, cross-sectional survey of physicians and their patients with PNH, conducted in Europe, China and Japan. The DSP methodology incorporate both physician perceptions and prescribing behaviour, as well as patient perspectives.

<sup>2</sup> The Standard Deviation is a statistical measure which shows the amount of variation of a variable around the expected value (mean).

Furthermore, disease complications would lead many patients to an overnight hospital stay (mean value: 68,142 total days spent in hospital, SD:12,416 days) PNH incurred total monetized productivity losses that reached a mean of 813,939,969 euros (SD: 70,069,631 euros) in German society. The translated figures of the per patient, per year monetized burden corresponded to a mean of 3,890 euros (SD: 238 euros). Notably, fatigue levels (moderate and severe) impacted patients the most, accounting for 52% of the total monetized burden (Figure 1) and caused patients in the most productive working age groups (30-64 years old) to lose one month of paid work per year, for their remaining lifetime.

**Figure 1. Monetized Burden per Health Outcome, (€)**



## 5 Conclusion

Addressing the socioeconomic impact of PNH in Germany requires a multifaceted approach. WifOR's study revealed that investment in research and development of novel therapies is an essential step towards improving the lives of those affected by PNH. By recognizing and addressing these challenges, Germany can better support PNH patients and alleviate the broader socioeconomic consequences of this debilitating condition.

# References

- Anderson, P., Benford, M., Harris, N., Karavali, M., & Piercy, J. (2008). Real-world physician and patient behaviour across countries: Disease-Specific Programmes - a means to understand. *Current Medical Research and Opinion*, *24*(11), 3063–3072.  
<https://doi.org/10.1185/03007990802457040>
- Bektas, M., Copley-Merriman, C., Khan, S., Sarda, S. P., & Shammo, J. M. (2020). Paroxysmal nocturnal hemoglobinuria: Patient journey and burden of disease. *Journal of Managed Care & Specialty Pharmacy*, *26*(12-b Suppl), S8–S14.  
<https://doi.org/10.18553/jmcp.2020.26.12-b.s8>
- Panse, J., Sicre De Fontbrune, F., Burmester, P., Piggin, M., Matos, J. E., Costantino, H., Wilson, K., Hakimi, Z., Nazir, J., Desgraz, R., Fishman, J., Persson, E., & Kulasekararaj, A. (2022). The burden of illness of patients with paroxysmal nocturnal haemoglobinuria receiving C5 inhibitors in France, Germany and the United Kingdom: Patient-reported insights on symptoms and quality of life. *European Journal of Haematology*, *109*(4), 351–363. <https://doi.org/10.1111/ejh.13816>
- Schrezenmeier, H., Röth, A., Araten, D. J., Kanakura, Y., Larratt, L., Shammo, J. M., Wilson, A., Shayan, G., & Maciejewski, J. P. (2020). Baseline clinical characteristics and disease burden in patients with paroxysmal nocturnal hemoglobinuria (PNH): Updated analysis from the International PNH Registry. *Annals of Hematology*, *99*(7), 1505–1514.  
<https://doi.org/10.1007/s00277-020-04052-z>



## WifOR Institute – If you measure it, you can shape it.

WifOR is an independent economic research institute, founded in 2009 as a spin-off from the Technical University of Darmstadt. As experts in macroeconomic analysis, our research focuses on the areas of labor markets, health, and sustainability. Through our studies, WifOR's mission is to enable data-based solutions to challenges on the labor market, set global standards in sustainability measurement, and illuminate the value of health investments worldwide. WifOR has over 75 employees located in Germany, Greece, Latin America, and the USA.



-  In 4 countries and 6 locations: Germany (Berlin, Darmstadt, Leipzig), Greece, Latin America and the USA
-  Active projects in 55 countries - global, regional, and national analyses