

Taking a Deep Data Dive into PNH: Focus on New and Emerging Agents

References (Order of Appearance)

- Cançado RD, Araújo A da S, Sandes AF, et al. Consensus statement for diagnosis and treatment of paroxysmal nocturnal haemoglobinuria. *Hematol Transfus Cell Ther.* 2021;43:341-348. doi: [10.1016/j.htct.2020.06.006](https://doi.org/10.1016/j.htct.2020.06.006)
- Fattizzo B, Serpenti F, Giannotta JA, Barcellini W. Difficult cases of paroxysmal nocturnal hemoglobinuria: diagnosis and therapeutic novelties. *J Clin Med.* 2021;10:948. doi: [10.3390/jcm10050948](https://doi.org/10.3390/jcm10050948)
- Schrezenmeier H, Röth A, Araten DJ, et al. Baseline clinical characteristics and disease burden in patients with paroxysmal nocturnal hemoglobinuria (PNH): updated analysis from the International PNH Registry. *Ann Hematol.* 2020;99:1505-1514. doi:[10.1007/s00277-020-04052-z](https://doi.org/10.1007/s00277-020-04052-z)
- Brodsky RA. How I treat paroxysmal nocturnal hemoglobinuria. *Blood.* 2021;137:1304-1309. doi: <https://doi.org/10.1182/blood.2019003812>
- Lee JW, Brodsky RA, Nishimura J-I, Kulasekararaj AG. The role of the alternative pathway in paroxysmal nocturnal hemoglobinuria and emerging treatments. *Expert Rev Clin Pharmacol.* 2022;15:851-861. doi: [10.1080/17512433.2022.2109462](https://doi.org/10.1080/17512433.2022.2109462)
- Wong RSM. Safety and efficacy of pegcetacoplan in paroxysmal nocturnal hemoglobinuria. *Ther Adv Hematol.* 2022;13:20406207221114673. doi: [10.1177/20406207221114673](https://doi.org/10.1177/20406207221114673)
- Notaro R, Luzzatto L. Breakthrough hemolysis in PNH with proximal or terminal complement inhibition. *N Engl J Med.* 2022;387:160-166. doi: [10.1056/nejmra2201664](https://doi.org/10.1056/nejmra2201664)
- Hillmen P, Szer J, Weitz I, et al. Pegcetacoplan versus eculizumab in paroxysmal nocturnal hemoglobinuria. *N Engl J Med.* 2021;18:384:1028-1037. doi: [10.1056/nejmoa2029073](https://doi.org/10.1056/nejmoa2029073)
- Peffault de Latour R, Szer J, Weitz IC, et al. Pegcetacoplan versus eculizumab in patients with paroxysmal nocturnal haemoglobinuria (PEGASUS): 48-week follow-up of a randomised, open-label, phase 3, active-comparator, controlled trial. *Lancet Haematol.* 2022;9:e648-e659. doi: [10.1016/s2352-3026\(22\)00210-1](https://doi.org/10.1016/s2352-3026(22)00210-1)
- Wong R, Fishman J, Wilson K, et al. Comparative effectiveness of pegcetacoplan versus ravulizumab and eculizumab in complement inhibitor-naïve patients with paroxysmal nocturnal hemoglobinuria: a matching-adjusted indirect comparison. *Adv Ther.* 2023;40:1571-1589. doi: [10.1007/s12325-023-02438-9](https://doi.org/10.1007/s12325-023-02438-9)

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References (Order of Appearance)

Wong RSM, Navarro-Cabrera JR, Comia NS, et al. Pegcetacoplan controls hemolysis in complement inhibitor-naïve patients with paroxysmal nocturnal hemoglobinuria. *Blood Adv.* 2023 Feb 27. [Online ahead of print] doi: [10.1182/bloodadvances.2022009129](https://doi.org/10.1182/bloodadvances.2022009129)

Liu H, Xia L, Weng J, et al. Results from the first phase 3 crovalimab (C5 inhibitor) study (COMMODORE 3): efficacy and safety in complement inhibitor-naïve patients with paroxysmal nocturnal hemoglobinuria (PNH). *Blood.* 2022;140(Suppl 1):714-716. doi: [10.1182/blood-2022-162452](https://doi.org/10.1182/blood-2022-162452)

Kelly R, Houghton N, Munir T, et al. Phase 2, open-label study evaluating the safety and efficacy of combination pozelimab and cemdisiran therapy in patients with paroxysmal nocturnal hemoglobinuria who switch from eculizumab. *Blood.* 2022;140(Suppl 1):8174-8175. doi: [10.1182/blood-2022-166547](https://doi.org/10.1182/blood-2022-166547)

Peffault de Latour R, Roeth A, Kulasekararaj A, et al. Oral monotherapy with iptacopan, a proximal complement inhibitor of factor B, has superior efficacy to intravenous terminal complement inhibition with standard of care eculizumab or ravulizumab and favorable safety in patients with paroxysmal nocturnal hemoglobinuria and residual anemia: results from the randomized, active-comparator-controlled, open-label, multicenter, phase III APPLY-PNH study. *Blood.* 2022;140(Suppl 2):LBA-2. doi: [10.1182/blood-2022-171469](https://doi.org/10.1182/blood-2022-171469)

Kulasekararaj AG, Lazana I. Paroxysmal nocturnal hemoglobinuria: where are we going? *Am J Hematol.* 2023;98(Suppl4):S33-S43. doi: [10.1002/ajh.26882](https://doi.org/10.1002/ajh.26882)

Dingli D, Matos JE, Lehrhaupt K, et al. The burden of illness in patients with paroxysmal nocturnal hemoglobinuria receiving treatment with the C5-inhibitors eculizumab or ravulizumab: results from a US patient survey. *Ann Hematol.* 2022;101:251-263. doi: [10.1007/s00277-021-04715-5](https://doi.org/10.1007/s00277-021-04715-5)

Daly RP, Jalbert JJ, Keith S, Symonds T, Shamma J. A novel patient-reported outcome instrument assessing the symptoms of paroxysmal nocturnal hemoglobinuria, the PNH-SQ. *J Patient Rep Outcomes.* 2021;5:102. doi: [10.1186/s41687-021-00376-0](https://doi.org/10.1186/s41687-021-00376-0)

Risitano AM, Peffault de Latour R. How we('ll) treat paroxysmal nocturnal hemoglobinuria: diving into the future. *Br J Haematol.* 2022;196:288-303. doi: [10.1111/bjh.17753](https://doi.org/10.1111/bjh.17753)

Szlendak U, Budziszewska B, Spychalska J, Drozd-Sokołowska J, Patkowska E, Nowak J. Paroxysmal nocturnal hemoglobinuria: advances in the understanding of pathophysiology, diagnosis, and treatment. *Pol Arch Intern Med.* 2022;132:16271. doi: [10.20452/pamw.16271](https://doi.org/10.20452/pamw.16271)