SWISS ONCOLOGY & HEMATOLOGY CONGRESS

Use of Pegcetacoplan in Swiss Patients with Paroxysmal Nocturnal Hemoglobinuria – Retrospective Case Series Analysis

E. Häfliger[1], B. Drexler[2], A. Holbro[2], M. Bissig[3], G. Stüssi[4], N. Fiorelli[4], P. Lovey[5], A. Rovó[1]

Affiliations:

[1]Department of Hematology, Inselspital, Bern University Hospital, University of Bern, Bern, Switzerland ; [2] Department of Hematology, Basel University Hospital, Basel, Switzerland

[3] Department of Hematology, Zürich University Hospital, Zürich, Switzerland ; [4] Department of Hematology, Oncology Institute of Southern Switzerland, Bellinzona, Switzerland;

[5] Service of Hematology, Hôpital du Valais – Institut central, Sion, Switzerland

Introduction

Pegcetacoplan (PEG), a C3 inhibitor, offers a novel treatment for Paroxysmal Nocturnal Hemoglobinuria (PNH) by preventing both intravascular (IVH) and extravascular hemolysis (EVH), improving outcomes beyond C5 inhibitors. We aimed to report real-world experience with PEG in PNH patients from Switzerland.

Methods

We retrospectively reviewed clinical and lab data from all identified Swiss PNH patients treated with PEG via an expert physician network

Results

- Seven patients treated at five Swiss centers were analyzed: five female; two with aplastic anemia; one with myelodysplastic syndrome.
- At diagnosis, all patients presented with symptomatic hemolysis and 3 had thrombosis.
- Previously all patients received C5 inhibitors (either eculizumab and/or ravulizumab).
- The main indications for initiating PEG were ongoing IVH and EVH (in 6/7 and 5/7 patients, respectively) and persistent transfusion dependency (3/7 patients).
- At PEG initiation, the median hemoglobin (Hb) level was 84.5 g/L (range 68 - 100) and LDH was 308 U/L (201-600). PEG was administered at a baseline dose of 1080 mg twice weekly. After 12 months of therapy, the median Hb level had increased to 116 g/L (89 -128) and LDH had normalized to 196 U/L (158 - 324).
- One episode of breakthrough hemolysis occurred, associated with infection and was managed by intensifying PEG therapy.
- Two patients died during PEG treatment, one due to a new neoplasm, another due to acute sepsis; this last patient was admitted in a coma due to an acute infection and PEG was discontinued due to lack of information about the underlying disease. The patient died of PNH-related complications.

Figure 1: Summary of results

Patient	Age	Gender	Related disease	Hb before pegcetacoplan (g/l)	Hb after pegcetacoplan (g/l)	LDH before pegcetacoplan (U/l)	LDH after pegcetacoplan (U/l)
1	78	Male	MDS	68	95	600	324
2	46	Female	No	81	115	317	202
3	77	Male	AA	97	116	165	216
4	42	Female	No	100	119	274	176
5	36	Female	No	67	89	396	136
6	56	Female	No	NR	119	NR	196
7	34	Female	AA	88	128	292	128

Abbreviations: Hb: hemoglobin; LDH: lactate dehydrogenase; MDS: myelodysplastic neoplasm; AA: aplastic anemia; NR: not reported

Conclusions

- In line with data reported in clinical trials, pegcetacoplan significantly improved anemia and hemolysis in Swiss PNH patients with a favorable safety profile and minimal breakthrough hemolysis.
- Awareness of therapy discontinuation in critical acute clinical situations should be raised.

Presented in the abstract category of hemostasis, transfusion medicine, vascular, laboratory medicine and benign hematology at SOHC 2025 from 19 – 21 November 2025