Emicizumab is cost-saving for Finnish hemophilia A patients with factor VIII inhibitors

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Background

- Emicizumab, a bispecific antibody for hemophilia A, has been reimbursed in Finland since November 2018 with certain restrictions (Figure 1). Until October 2023, reimbursement was restricted for patients with hemophilia A and factor VIII (FVIII) inhibitors (HAwI) when immunotolerance induction therapy (ITI) is unsuccessful or unsuitable, and for patients with severe hemophilia A without FVIII inhibitors when prophylactic treatment with FVIII is unsuccessful or unsuitable. Restrictions were removed in October 2023, when reimbursement was extended also for patients with moderate hemophilia A (FVIII 1-2%) and severe bleeding phenotype.
- FVIII replacement therapy has been the standard of care in hemophilia A for decades. FVIII treatment is individually tailored based on pharmacokinetic profiling and lifestyle factors, and thereby subject to large inter-individual variation.
- On HAwI patients, the need for immunotolerance induction (ITI) with very high and frequent FVIII dosing and bypassing agents (BPAs) multiplies treatment costs compared to regular FVIII prophylaxis.
- In contrast, emicizumab dosing (mg/kg) and associated treatment costs are fixed, which improves the predictability of the hemophilia treatment budget. In addition, as the only product in the Finnish hemophilia market, emicizumab has conditional reimbursement associated with a confidential net price agreement.

Objectives

- Due to significant uncertainty related to the cost of previous hemophilia A treatment, the actual budget impact of switching to emicizumab is not known in Finland. We aimed to investigate the real-life treatment costs of Finnish HAwI patients before and after emicizumab initiation, as required by the Pharmaceutical Pricing Board in its reimbursement decision.

Methods

- According to the Finnish law on secondary use of health and social care data, approval of an ethical committee or informed consent was not required, as the study was based on administrative register data and patients were not directly contacted.
- All patients who had purchased emicizumab under the inhibitor reimbursement scheme (reimbursement entitlement number 1503) were identified in the Dispensations reimbursable under the National Health Insurance Scheme register of the Finnish national payer, the Social Insurance Institution. A patient was considered an emicizumab user if he had more than one emicizumab purchase during the follow-up.
- Reimbursed expenses and purchase dates of emicizumab, FVIII, and BPAs were recorded for the length of emicizumab treatment to the end of June 2022, and three years before the emicizumab switch, as indicated by each patient's initial emicizumab purchase.
- Treatment costs for other hemophilia treatments than emicizumab were summed up and reported in aggregate as an annual average cost during emicizumab treatment.
- Average daily treatment cost per patient was calculated and annualized to account for individual differences in treatment duration.
- Reimbursed expenses are reported using public wholesale list prices.

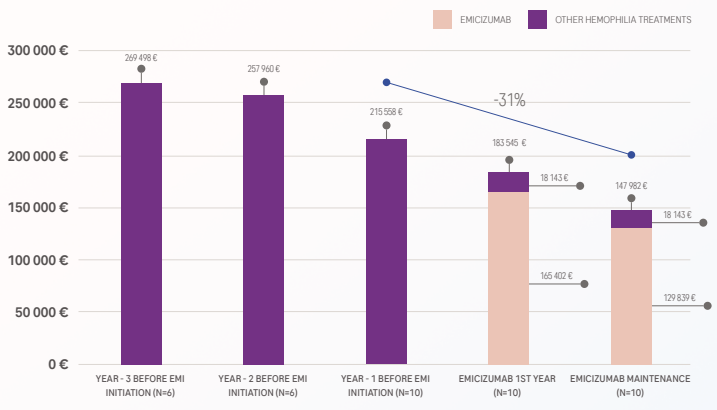

Figure 2. Average annual treatment costs of the Finnish hemophilia A patients with FVIII inhibitors who have switched to emicizumab.

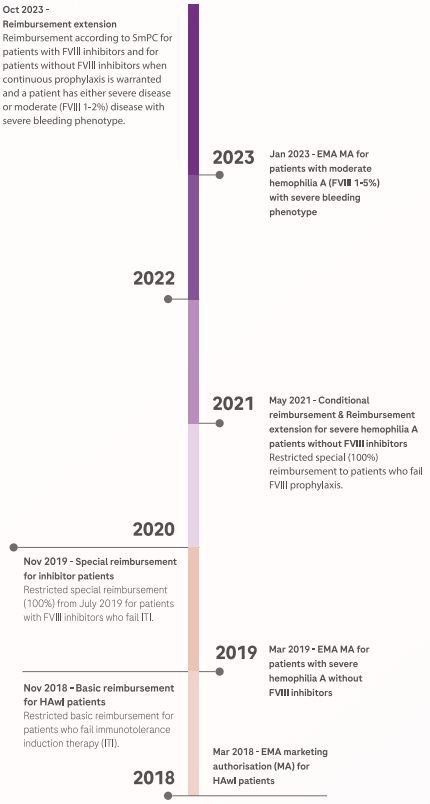

Figure 1. Evolving patient access to emicizumab in Finland.

Results

- Ten HAwI patients had recurring emicizumab purchases until the end of follow-up (30 June 2022).
- All ten patients had purchased other reimbursable hemophilia treatments during the year preceding the emicizumab treatment initiation. Six patients had reimbursed expenses also in years -2 and -3 prior to the emicizumab switch.
- The average annual treatment cost per patient declined -31% from 215 558 € in year -1 prior to emicizumab initiation to 147 982 € during emicizumab maintenance (Figure 2).
- Because emicizumab is started with a loading phase, expenses in the first treatment year are higher than in subsequent years.
- Seven out of ten patients had concomitant use of other hemophilia drugs during their emicizumab treatment.
- Concomitant use of other hemophilia drugs amounted to an additional average annual cost of 18 143 € across all ten patients.

Conclusions

- Switching to emicizumab prophylaxis reduces drug costs of Finnish HAwI patients compared to their previous treatment.
- Expenses of concomitant reimbursable hemophilia treatments were modest in comparison to emicizumab, suggesting no significant need for additional bleed control during emicizumab treatment.
- Emicizumab is also an interesting case from the price regulation perspective in Finland. The Pharmaceutical Pricing Board has been able to apply decision making criteria and gradually extend reimbursement, even though emicizumab is the only hemophilia medication with a confidential net price.

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