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Haemophilia

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MONITORING OF EMICIZUMAB USING A PATIENT REGISTRY

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Introduction: The longitudinal monitoring of new expensive medicines by patient registries providing real world data is encouraged by the European Medicines Agency. Since August 2020, the non-replacement therapy emicizumab also became available for patients with severe hemophilia A without inhibitors in the Netherlands. The aim of this study is to evaluate the use and outcomes of emicizumab, using the Dutch Hemophilia Registry 'HemoNED'.

Methods: Almost all patients with hemophilia in the Netherlands are registered in the HemoNED registry after informed consent. Six Dutch Hemophilia Treatment Centers (HTCs) register patient characteristics, the treatment plan and outcomes (e.g. adverse events). Patients use a digital infusion log (app/web page 'VastePrik') to record all emicizumab and clotting factor infusions and/or bleeds.

Results: By July 2021, 1333 patients with hemophilia A (572 (43%) with severe hemophilia A, 575 (43%) on prophylaxis), were registered in HemoNED. 162 patients were treated with emicizumab. Forty three percent of emicizumab users were younger than 18 years and 92% had severe hemophilia A. For more than 80% of the patients emicizumab was prescribed once every one or two weeks. The mean prescribed dose was 1,60 mg/kg/week. The reported reasons to start emicizumab were e.g.: venous access problems (16%), inhibitor with bleeding tendency (12%) or recurring bleeds despite regular prophylaxis (9%). However, more than half (52%) of the patients started for non-specific reasons, most likely patient preference.

A subset of 119 (73%) of patients on emicizumab reported at least one infusion with emicizumab in the infusion log; median follow-up time was 3.2 months (IQR 1.2- 5.5 months). 77% reported no bleeds during the treatment with emicizumab.

So far, two times a reason to stop emicizumab treatment was registered and three patients reported side effects in the digital infusion log (rash, joint pain).

Discussion/Conclusion: A patient registry like HemoNED, combined with an infusion log for patients (VastePrik), provides useful information on the clinical effectiveness and safety of new medicines in a real world setting. However, the quality of the data depends on the registration efforts made by HTC staff and patients. Pilots on automatic data extractions from electronic hospital records are set up to substantially decrease the burden of registration and improve data quality.

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