Efficacy of emicizumab prophylaxis in patients with severe hemophilia A in Germany: Follow-up evaluation of real-life-data documented by smart medication eDiary

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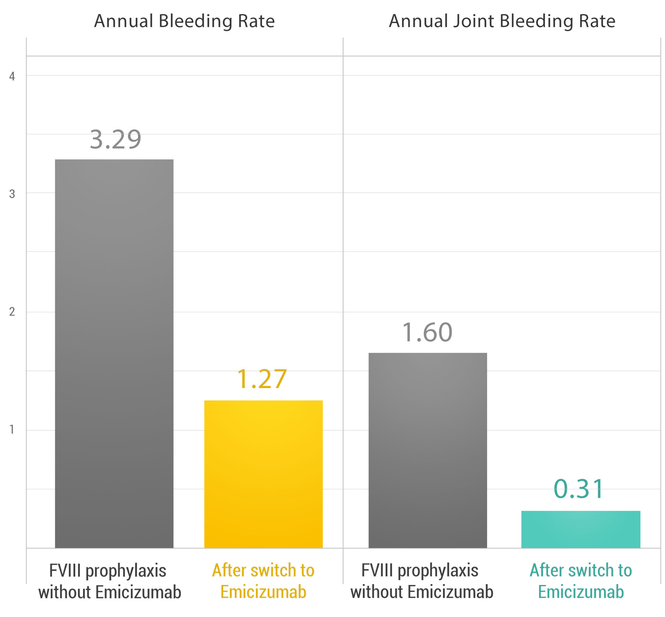
Introduction

Systematically documented data on real-world use of emicizumab prophylaxis in patients with severe haemophilia A (PwSHA) in Germany are still lacking. We present real-life data on efficacy of emicizumab in PwSHA across German haemophilia Treatment Centres (HTCs) as of September 2022.

Method

Annual bleeding rate (ABR), annual joint bleeding rate (AJBR) and proportion of bleed-free patients were documented using the electronic diary platform smart medication. Data of PwSHA before and after switch of treatment with FVIII concentrates to emicizumab were evaluated. Patients with >24 weeks of electronic documentation after switch were evaluated.

Figure 1: Change of ABR and AJBR before and after switch

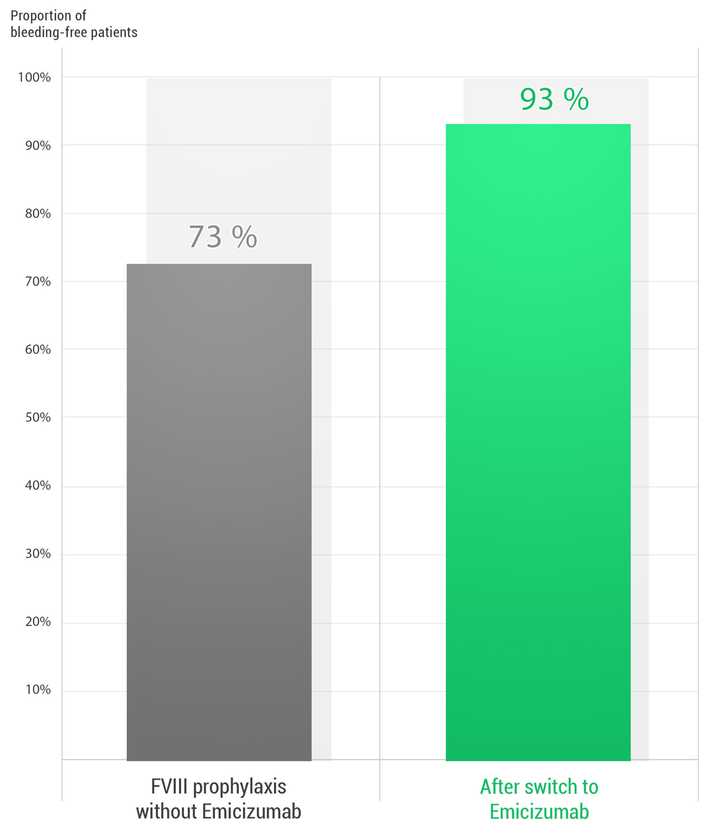


Results

79 PwSHA from HTCs in Germany in which PwSHA are using smart medication could be included. The median age was 33 years (IQR 36); 66% were 18 years and older. 39 PwSHA started with electronic documentation when switched to emizicumab. In 40 patients complete electronic documentation before and after switch could be evaluated. All PwSHA were on prophylactic treatment at 1 - 5 days intervals.

After switch to emicizumab, the mean AJBR was 0.31 and the mean ABR 0.84 in all patients. In the subgroup of 40 PwSHA with documentation before and after switch, the mean AJBR dropped significantly from 1.60 before and 0.39 after switch ($p<0.01$) and the mean ABR from 3.29 to 1.27. The proportion of bleeding-free patients increased from 73% before to 93% after switching to emicizumab. Despite of additional FVIII treatment in 41% of patients after the switch to emicizumab, only 4 (5%) needed additional FVIII due to joint bleeds.

Figure 2: Bleeding-free patients before and after switch



Conclusion

The real life-data collected with the electronic patient diary smart medication show a statistically significant decrease of bleeding episodes in this increasing cohort of PwSHA after switching from prophylaxis with FVIII concentrates to emicizumab. The preliminary results of this ongoing study confirm the favourable data with emicizumab prophylaxis from clinical trials.