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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BACKGROUND

Systematically documented data on real-world use of emicizumab prophylaxis in patients with severe hemophilia A (PwSHA) in Germany are still lacking. We present real-life data on efficacy of emicizumab in PwSHA across German Hemophilia Treatment Centres (HTCs) as of Dec 31, 2022, documented with the electronic diary smart medication for monitoring home treatment of hemophilia patients – developed and provided by VFTH, a non-profit association focused on the advancement of telemedicine.

AIMS

Evaluation of self-reported bleed rate (total treated bleeds per year/ABR, total joint bleeds per year/AJBR and life-threatening bleeds) in PwSHA undergoing emicizumab prophylaxis and compared with previous FVIII prophylaxis captured in the real-world data set of smart medication eDiary.

PATIENTS AND METHODS

Included were patients from an unselected cohort when switching to emicizumab prophylaxis fulfilling the following inclusion criteria:

- Severe hemophilia A (FVIII<1%)
- Negative FVIII inhibitor (<0.6 BU/mL)
- 24 weeks of documented prophylaxis with emicizumab using smart medication eDiary app
- Subgroup evaluation – 24 weeks of documented FVIII prophylaxis before switch to emicizumab using smart medication eDiary app

smart medication is a mobile website platform to interconnect patients, physicians, pharmacies and public registry (e.g. DHR – German hemophilia registry) and a EU registered medical device (MDD* class I). Data on treatment modalities (medication, dose, batch number, date, reason of treatment as well as bleeding events (location, bleeding type, bleeding cause, pain, photo upload, etc.) are captured in real-time by the patient and transmitted to the HTC. Outcome of emicizumab treatment and self-reported bleeds (total treated bleeds per year/ABR, total joint bleeds per year/AJBR and life-threatening bleeds) over a period of 24 weeks were evaluated. Whenever available, 24-weeks FVIII prophylaxis before switching to emicizumab and outcome was compared to outcome of consecutive emicizumab prophylaxis (Fig 1).

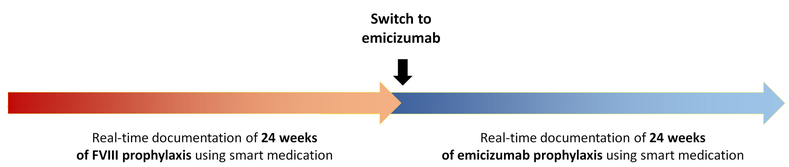

Figure 1: Subanalysis: real-world data on emicizumab prophylaxis

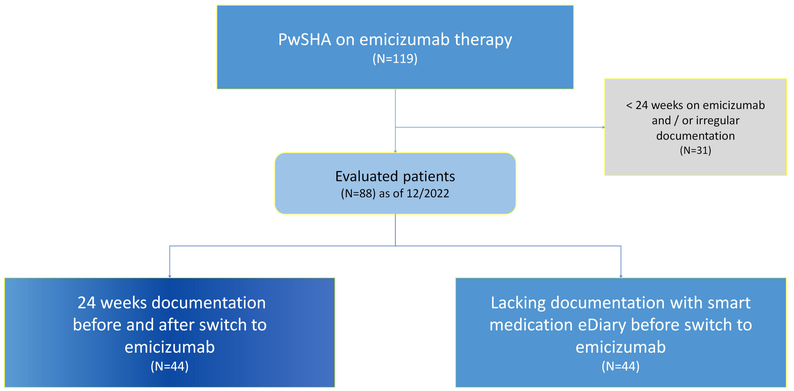

Figure 2: Study patient cohort as of 12/2022

RESULTS

As of December 2022, 88 pediatric and adult PwSHA from HTCs in Germany meeting all inclusion criteria were included (Fig 2). The median age was 35 years (IQR 35); 71% were 18 years and older. 44 PwSHA started with electronic documentation when switched to emicizumab. 44 patients with electronic documentation before and after switch could be evaluated and 24 weeks of FVIII prophylaxis could be compared with 24 weeks of emicizumab prophylaxis. FVIII prophylaxis included prophylaxis with SHL and EHL-FVIII at 1 - 5 daily intervals with weekly doses from 21.2 – 179.8 IU/kg

After switching, patients showed a mean AJBR of 0.30 and the mean ABR of 0.78 under emicizumab prophylaxis (N=88).

In the subgroup of 44 PwSHA with documentation before and after switch, the mean AJBR dropped significantly from 2.08 under FVIII prophylaxis to 0.40 under emicizumab prophylaxis ($p < 0.01$). The mean ABR for all bleeds decreased from 3.19 to 1.20 (Fig 3), corresponding to a bleed reduction of 81% and 62% respectively. The proportion of patients with zero bleeds increased from 64% with FVIII prophylaxis to 91% with emicizumab prophylaxis (Fig 4). Despite of additional FVIII treatment in 39% of patients after the switch to emicizumab (e.g. during loading phase), only 6 (7%) needed additional FVIII due to joint bleeds. No life-threatening bleeds have been reported.

CONCLUSION

The real life-data show a significant decrease of bleeding episodes after switching PwSHA from regular FVIII prophylaxis to emicizumab prophylaxis and confirm the favorable data from previous clinical trials. E-diaries like smart medication support real-life documentation and evaluation of current and new treatment strategies.

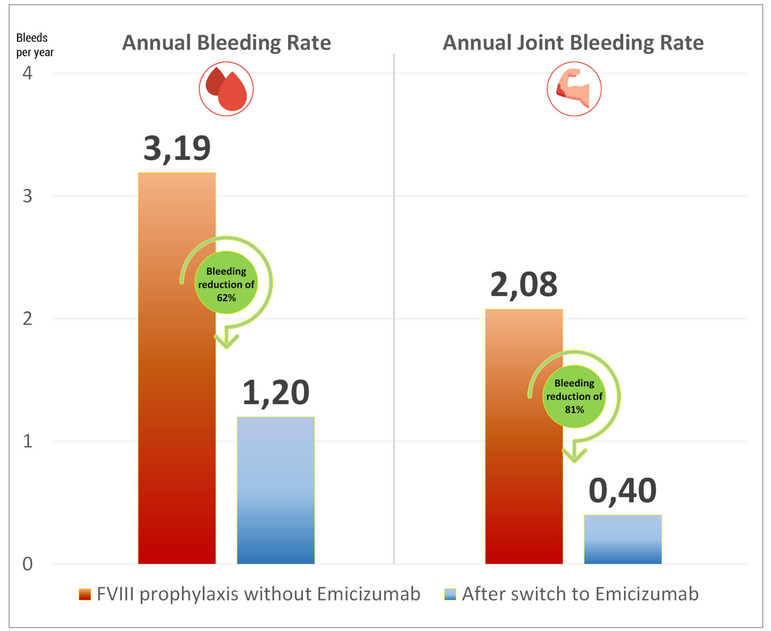

Figure 3: Change of ABR und AJBR before and after switch

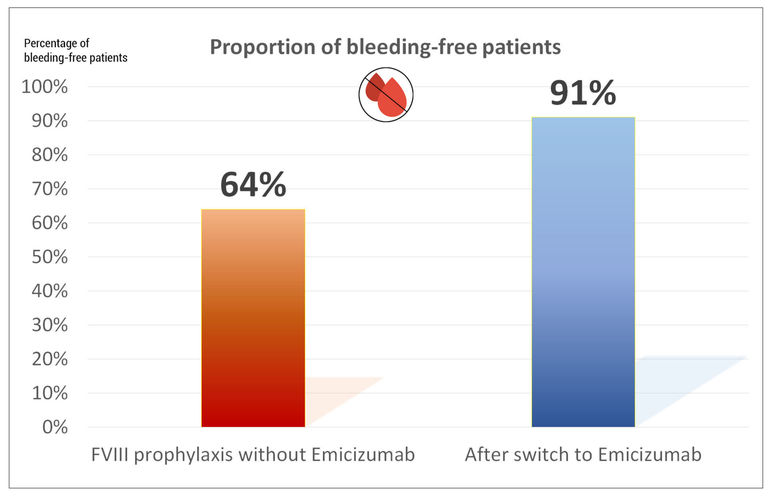

Figure 4: Bleeding free patients before and after switch

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