

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

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Introduction

Real-life data showing the efficacy of emicizumab treatment in patients with severe hemophilia A (PwSHA) across multiple Hemophilia Treatment Centres (HTCs) in Germany are limited.

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 retrospectively. Included were patients with ≥ 24 weeks of electronic documentation after switch.

Results

36 PwSHA from 8 HTCs in Germany in which PwSHA are using smart medication could be included. The median age was 42 years (IQR 31); 79% were 18 years and older. 12 PwSHA started with electronic documentation together with the switch to emicizumab. Data from paper documentation prior to switch in these patients were not available.



Fig. 1: Change of ABR and AJBR before and after switch

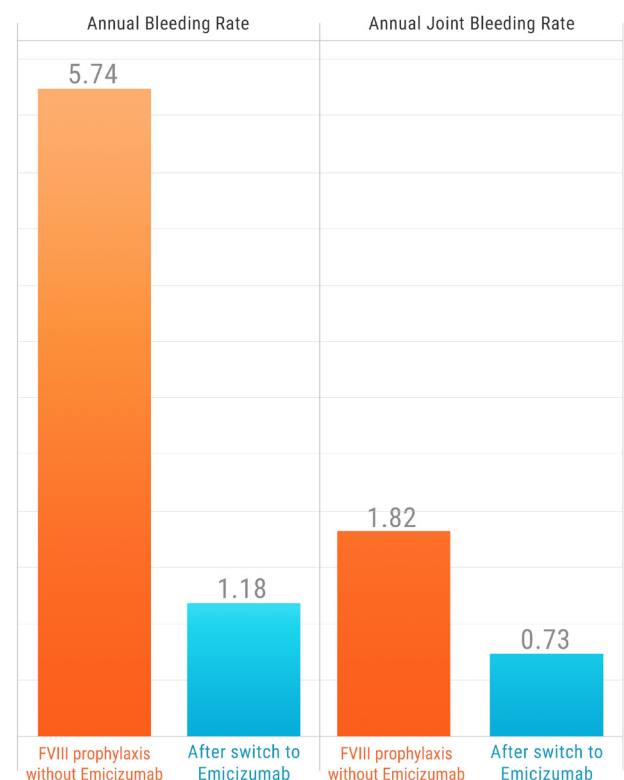


Fig. 2: Bleeding-free patients before and after switch



In 24 patients complete electronic documentation before and after switch could be evaluated. All PwSHA were on prophylactic treatment in 2 - 4 days interval. After switch to emicizumab, the mean AJBR was 0.49, the ABR 0.79 in all patients. In the subgroup of 24 PwSHA with documentation before and after switch, the mean AJBR was 1.82 before and 0.73 after switch, the mean ABR dropped from 5.74 to 1.18 after switch. The proportion of bleeding-free patients increased from 45% before to 62% after switching to emicizumab. Despite of additional FVIII treatment in 29% of patients after the switch to emicizumab, only 2 (7%) needed additional FVIII due to joint bleeds.

Conclusion

Real-life data documentation by using electronic diary smart medication and analysis across various centres may allow early clinical consequences. As these data show a significant decrease of bleeding episodes by switching PwSHA on prophylactic treatment from FVIII concentrates to emicizumab, a more effective prophylaxis, consistent with data from clinical trials, may be discussed.