Epidemiology, Healthcare Resource Utilization, and Costs of Hemophilia A and B by Treatment Regimen: A Retrospective Claims Data Analysis in Germany From 2016 to 2021

HSD54

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Background & Objectives

- Hemophilia is a rare, X-recessive, congenital bleeding disorder characterized by a deficient endogenous production of blood clotting factors VIII (FVIII) in Hemophilia A (HA) and IX (FIX) in Hemophilia B (HB).
- The standard of care in Germany for patients with moderate -severe hemophilia is a lifelong prophylactic treatment with intravenous concentrated FVIII or FIX to prevent bleeding events [1]. To assess the economic value of emerging treatment options studies describing the economic burden of hemophilia under current standard of care in Germany are needed.
- Here we present a structured approach classifying hemophilia patients by treatment regimen in administrative claims data to examine real-world economic burden of Hemophilia in Germany from 2016 to 2021.

Methods

- This retrospective study used anonymized SHI claims data from 01 January 2015 to 31 December 2021 from the InGef research database which includes longitudinal claims data of approx. 4.8 million subjects from approx. 50 SHI (mainly company or guild health insurances) in Germany. The database is representative to the total German population with regards to age and sex [2]. All analyses were conducted in a cross-sectional design where patients were examined per calendar year. Hemophilia patients aged 12 years and older were identified in InGef statutory health insurance claims data via ICD-10 codes D66 (Hemophilia A, HA) and D67 (Hemophilia B, HB) in combination with ≥1 claim for hemophilia-related medication in the study year.
- Each patient's factor regimen type was classified as either indicative of a severe phenotype predominantly requiring prophylactic treatment or a non-severe phenotype predominantly treated on-demand using a prespecified classification threshold of 100.000 IU for HA and 80.000 IU for HB (40.000 IU if patient had received extended-half-life factor products). Patients with current evidence of inhibitors (≥ 1 prescription of a bypassing agent or, (HA only:) ≥ 1 prescription of Emicizumab in combination with a diagnosis of inhibitor status) were excluded from classification by factor consumption and were classified separately (Data not shown due to low patient numbers).
- The three treatment groups 'severe'; 'non severe' and 'inhibitors' combined formed the overall study population, consisting of all treated HA, resp. HB patients with ≥ 1 prescription of a medication for HA resp. HB treatment.
- HCRU and cost outcomes were captured for each study year and included all-cause hospitalizations, physician visits, and all cause and hemophilia-related medication.
- Patient numbers were projected to the total German population using official population statistics from the German Federal Bureau of Statistics [3].

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Disclosures

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Results

Hemophilia A						
	2016	2017	2018	2019	2020	2021
Overall	2,748 (100)	2,878 (100)	2,834 (100)	2,781 (100)	3,337 (100)	3,272 (100)
	3.73 [3.59-3.87]	3.90 [3.76-4.04]	3.83 [3.69-3.98]	3.76 [3.62-3.90]	4.51 [4.36-4.67]	4.43 [4.28-4.58]
Severe	1,176 (42.8)	1,244 (43.2)	1,368 (48.3)	1,482 (53.3)	1,586 (47.5)	1,425 (43.8)
	1.60 [1.51-1.69]	1.68 [1.59-1.78]	1.85 [1.75-1.95]	2.00 [1.90-2.11]	2.15 [2.04-2.25]	1.96 [1.86-2.06]
Non-Severe	1,529 (55.6)	1,569 (54.5)	1,442 (50.9)	1,251 (45.0)	1,615 (48.4)	1,740 (53.5)
	2.07 [1.97-2.18]	2.12 [2.02-2.23]	1.95 [1.85-2.05]	1.69 [1.60-1.79]	2.18 [2.08-2.29]	2.35 [2.25-2.47]
Hemophilia B						
	2016	2017	2018	2019	2020	2021
Overall	640 (100)	570 (100)	566 (100)	641 (100)	684 (100)	802 (100)
	0.87 [0.80-0.94]	0.77 [0.71-0.84]	0.77 [0.70-0.83]	0.87 [0.80-0.94]	0.93 [0.86-1.00]	1.09 [1.01-1.16]
Severe	255 (39.8)	255 (44.7)	283 (50.0)	267 (41.7)	381 (55.7)	436 (54.4)
	0.35 [0.31-0.39]	0.35 [0.31-0.39]	0.38 [0.34-0.43]	0.36 [0.32-0.41]	0.52 [0.47-0.57]	0.59 [0.54-0.65]
Non-Severe	385 (60.2)	315 (55.3)	283 (50.0)	374 (58.4)	279 (40.8)	342 (42.6)
	0.52 [0.47-0.58]	0.43 [0.38-0.48]	0.38 [0.34-0.43]	0.51 [0.46-0.56]	0.38 [0.34-0.42]	0.46 [0.42-0.51]

Table 1. Extrapolated Numbers of Hemophilia A/Hemophilia B patients in the German population stratified by treatment group. n (% of Overall); Prevalence per 100.000 Persons [SD]

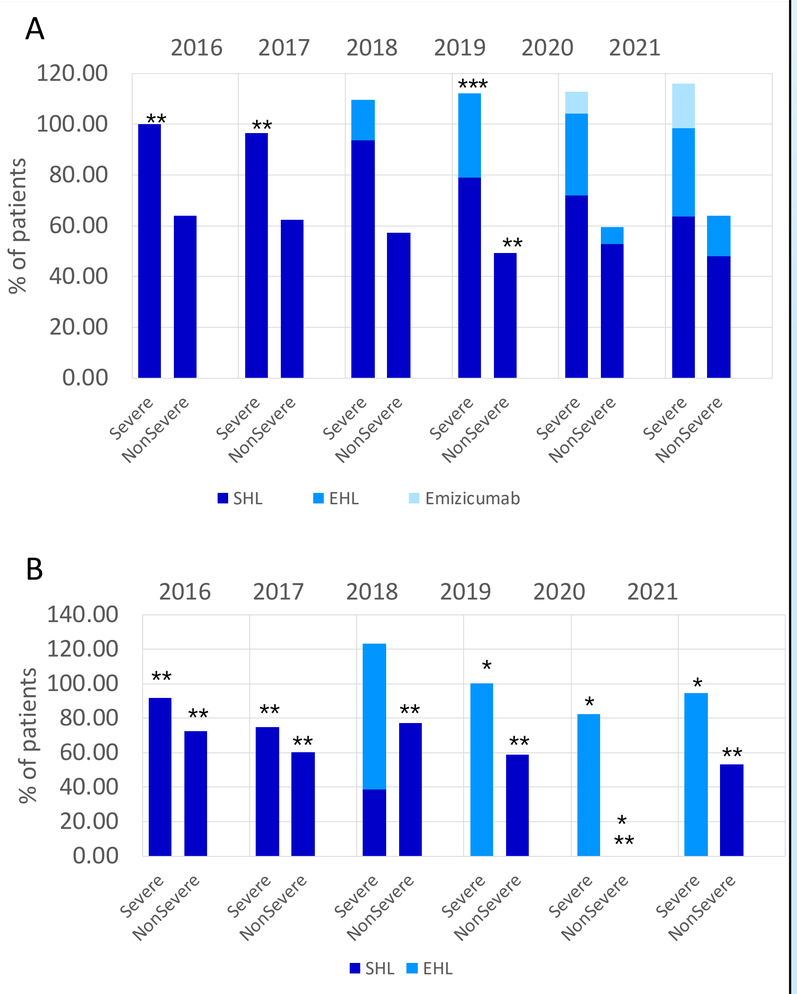

*Figure 1 . Distribution of factor medication in prevalent hemophilia patients (age 12+ years) by product category and treatment group. A) Hemophilia A – Factor VIII and Emizicumab prescriptions. B) Hemophilia B – Factor IX prescriptions . *) <5 patients with SHL prescriptions; **) <5 patients with EHL prescriptions; ***) <5 patients with Emicizumab prescriptions*

Conclusions

- Based on projected patient numbers, 2,748 to 3,337 treated HA patients and 566 to 802 treated HB patients were estimated from 2016 to 2021, with 43 to 53% and 40% to 56% categorized in the severe treatment group.
- Our data show that EHL are now the mainstay treatment in severe HB patients, with over 95% treated with EHL; whereas SHL remain in use for prophylactic treatment in a high percentage of HA patients.
- Differences in treatment costs are driven by the medication costs constituting 87%-99% of total costs and consistently and statistically significantly higher in the severe than in the non-severe group.
- In 2021, mean (SD) per-patient medication costs were €321,987 (€157,915) in the severe treatment group vs. €43,487 (€92,821) in non-severe group for HA and € 289,411 (€132,400) vs. €19,253 (€23,655) for HB.
- The results demonstrate the high economic burden in severe HA and HB patients in Germany, driven by the need for continuous factor replacement therapy and give an estimate of treatment costs based on a real-world therapy mix.
- This claims-data based approach remains methodological challenging. Robust registry data are an important source to address and confirm our results.

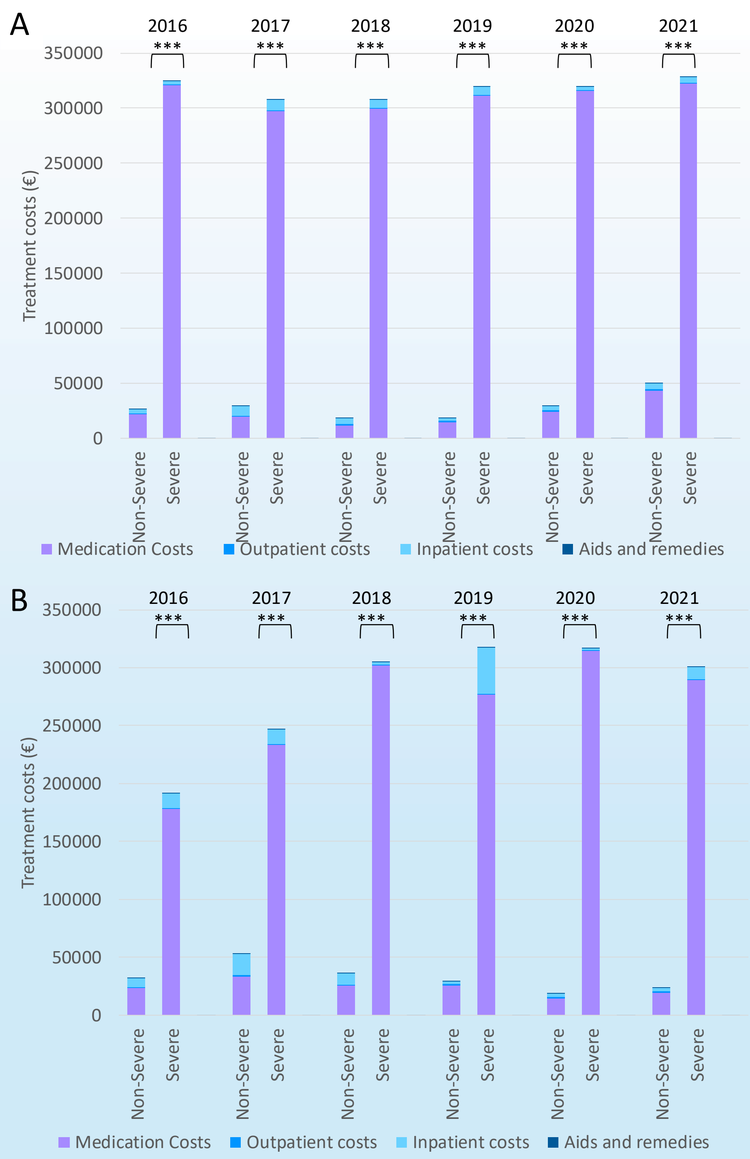

*Figure 2. Mean Per-patient Healthcare costs per treatment group and cost category (in €) A) Patients with Hemophilia A; B) patients with Hemophilia B. *** for p < 0.001*

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