

# The German Pediatric Hemophilia Research Database

## Newly diagnosed children and adolescents with haemophilia A and B in Germany – UPDATE 2020

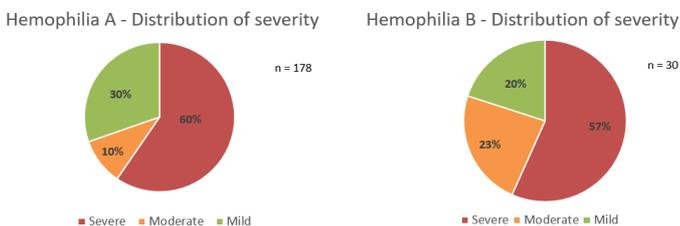
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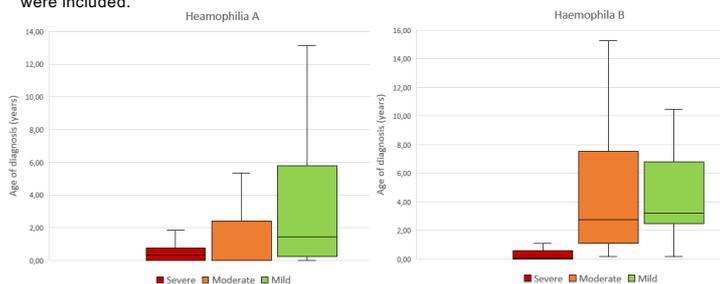
**Background:** In Germany, 40-60 newborns are expected to be diagnosed with haemophilia per year. Haemophilia leads to recurrent bleeds and increased morbidity and mortality. Prophylaxis is the standard of care to prevent bleeds and sequela. The **optimal timing** or regimen to start prophylaxis to avoid the development of **joint disease** or **neutralizing antibodies** to clotting factors are still being discussed. New therapeutic options need independent collection of data on safety and efficacy.

**Formalia:** GEPHARD stands under the official auspices of the GTH. The study has IRB approval (Munich Az 572-16) and is registered at Clinicaltrials.gov (NCT02912143). Financial support has been agreed with most FVIII and FIX producing companies in Germany and contracts with PedNet have been signed.

### Results:



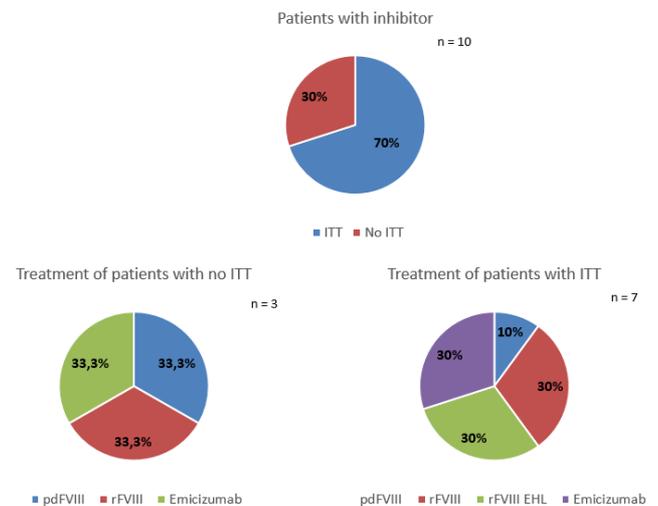
**Fig. 1:** Overview of the distribution of the severity of haemophilia A (left) and haemophilia B (right). Total n = 153 patients with haemophilia A and n = 29 patients with haemophilia B were included.



**Fig. 2:** Overview of the diagnosis age of patients with haemophilia A (n = 178) and B (n = 30). Median age of diagnosis of patients with haemophilia A is 0.42 (severe, n = 106), 0.04 (moderate, n = 18) and 1.75 (mild, n = 54). Median age of diagnosis of patients with haemophilia B is 0.17 (severe, n = 17), 2.75 (moderate, n = 7) and 3.21 (mild, n = 6).

**Method:** The Standing Commission Pediatrics of the GTH established a multicenter, prospective, observational registry for newly diagnosed patients. This pediatric registry cooperates with existing registries and supports other pediatric hemophilia studies. It focusses on the first 100 ED in children with hemophilia but has the potential for long term follow up and the implementation of sub studies.

**Status:** 36 centres have anonymously reported 216 (cut-off date 31.06.2020) patients with haemophilia A or B since 2017. For those children and adolescents with information available, 178 children were diagnosed with haemophilia A including 106, 18 and 54 with a severe, moderate or mild phenotype, respectively. Thirty children were diagnosed with haemophilia B including 17, 7 and 6 with a severe, moderate or mild phenotype, respectively. The IT database has now been established and longitudinal documentation has been started.



**Fig. 3:** Overview of patients with inhibitor development. A total of 10 patients were found to have an inhibitor. 7 patients receive an ITT and 3 patients do not (above). The patients are treated with pdFVIII, rFVIII, rFVIII, Emicizumab (bottom right/left). (Cut-off date 31.12.2019)

**Conclusion:** The GEPHARD community has included 216 children from January 2017 to June 2020. Following administrative issues which have been solved, the current pandemic poses a further burden on a central and longitudinal documentation. Nevertheless, longitudinal documentation has been started and will provide additional data in the near future.

In cooperation with



Support YOUR registry !



Diagnosed 01.01.2017 onwards  
 Age birth - 18 years  
 FVIII / FIX 0 - 25 %

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