

# Haemophilia Registry of the Medical Committee of the Swiss Haemophilia Society

## Update and annual survey 2008

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### Keywords

Haemophilia, registry, treatment modality, factor use, quality control

### Summary

The Swiss Haemophilia Registry of the Medical Committee of the Swiss Haemophilia Society was established in 2000. Primarily it bears epidemiological and basic clinical data (incidence, type and severity of the disease, age groups, centres, mortality). Two thirds of the questions of the WFH Global Survey can be answered, especially those concerning use of concentrates (global, per capita) and treatment modalities (on-demand versus prophylactic regimens). Moreover, the registry is an important tool for quality control of the haemophilia treatment centres.

There are no informations about infectious diseases like hepatitis or HIV, due to non-anonymisation of the data. We plan to incorporate the results of the mutation analysis in the future.

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### Schlüsselwörter

Hämophilie, Register, Behandlungstyp, Faktorenverbrauch, Qualitätskontrolle

### Zusammenfassung

Das Schweizer Hämophilieregister, das 2000 von der ärztlichen Kommission der Hämophiliegesellschaft etabliert wurde, erlaubt primär epidemiologische und einfache klinische Angaben zu Hämophilie und einzelnen anderen seltenen Gerinnungsstörungen (Inzidenz, Typ und Schweregrad der Krankheit, Altersverteilung, Zentrenzuordnung, Sterberate). Anhand dieser Daten können etwa 2/3 der Fragen der WFH Global Survey beantwortet werden, insbesondere den Faktorengebrauch (gesamt und pro Kopf) und die Behandlungsart (Bedarf versus Prophylaxe). Das Register ist aber auch ein wichtiges Instrument zur Qualitätskontrolle der Hämophiliezentren.

Da ein Teil der Daten nicht anonymisiert sind, enthält das Register keine Angaben zu Hepatitiden und HIV-Infektionen. Aufgrund der an Bedeutung zunehmenden Mutationsanalyse soll diese Angabe in Zukunft auch erfasst werden.

Hämophilie-Register der Schweizer Hämophiliegesellschaft – Update und Jahresbericht 2008  
Hämostasieologie 2009; 29 (Suppl 1): S16–S18

### Type of haemophilia and other rare coagulation disorders

Generally speaking, the distribution of the registered coagulopathies has not changed over the last years as shown in ►Figure 1: haemophilia A 525 (65%), haemophilia B 106 (13%), von Willebrand disease 118 (15%), other coagulopathies 56 (7%), including deficiencies concerning:

- afibrinogenaemia (n = 9),
- factor VII (n = 23),
- factor X (n = 3),
- factor XI (n = 3),
- factor XIII (n = 14),
- factor V/VIII (n = 4).

Disease severity was classified according to the ISTH standards (1): severe haemophilia A/B < 1%, moderate 1–5%, and mild 6–40%. The corresponding results are shown in ►Figure 2: severe type A patients 188 (39%), moderate type A 100 (21%), mild type A 194 (40%), severe type B patients 26 (28%), moderate type B 36 (39%), and mild type B 31 (33%). Data were missing in 56 patients.

In 2008, there were only two newborns with newly diagnosed haemophilia A. Age distribution of the patients remained stable over the years with a current average age of 37 years.

The current inhibitor status was positive in 19 patients with haemophilia A and one patient with haemophilia B. High responding inhibitors were present in 13 patients (12 with haemophilia A and 1 haemophilia B) and low responding inhibitors in 7 patients, all with haemophilia A. However, the numbers were not fully representative, as data were missing in 95 patients.

Data on treatment types are shown in ►Figure 3: In Switzerland overall, on-demand treatment still exceeds prophylaxis for both haemophilia A and B. In haemophilia A

The base for the IT concept of the Swiss Hemophilia Registry was set in the year 2000. In 2004, the registry was transformed to the actual internet based form with double password security. The latest records from September 30<sup>th</sup> 2008, are presented here.

### Patients

A total number of 856 patients (compared to 839 in year 2007) were included in the registry, corresponding to an increase of 2%. 51 patients were not assigned to a specific center and, because of lack of updating, excluded for the evaluation.

346 (66%) patients performed on-demand treatment vs. 102 (19%) with a prophylactic treatment. The figure in haemophilia B was even more obvious: 78 (74%) on-demand vs. 13 (12%) prophylactic treatment. Over time, however, a progressive shift was observed towards prophylactic treatment, also in adults (data not shown).

Recently, a new treatment group (sporadic therapy) (2), representing 8% of haemophilia A and 9% of haemophilia B patients, was introduced to describe mainly mild haemophiliacs requiring such a minimal treatment that it cannot be called on-demand. It is highly probable that not all concerned patients have already been correctly transferred from the on-demand to the sporadic group. Treatment status of about 5 to 6% of all patients remained unknown.

## Mortality

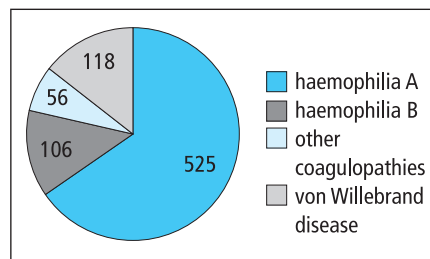
Death registration represents a small file in the registry. In the period 1996–2008, a total number of 58 patients died. During the year 2008 six patients died: three from cancer, one each from bleeding and liver disease and one from an unknown cause. The last HIV-related death was back in 2003.

## Factor concentrate

The module includes following components:

- name and type of the concentrate,
- delivery of the concentrate: amount in U or mg, date of delivery and treatment indication
  - home treatment,
  - acute bleeding,
  - surgery,
  - stockpile for holidays,
- total amount of concentrate use per year,
- history of the actual concentrate and former ones.

The main data input comes from an automatic file transfer from the pharmaceutical companies which deliver the concentrates for home treatment to the registry. The total amounts of delivered concentrates are reported quarterly to the administrator responsible for the data input.



**Fig. 1** Haemophilia type of registered patients (n = 805)

Data can also be put in manually by any user. Despite the convenient and easy handling of the module, data are probably not completely collected yet.

In 2004 the software was enhanced by a statistics program allowing evaluation of the concentrates in various combinations of selection, quantity and period of time. The different factor concentrates used are presented in ► Figure 4.

In Switzerland as in other countries (3), recombinant products clearly lead the market in haemophilia A with about 75% vs. 25% plasma-derived factor VIII. In haemophilia B,

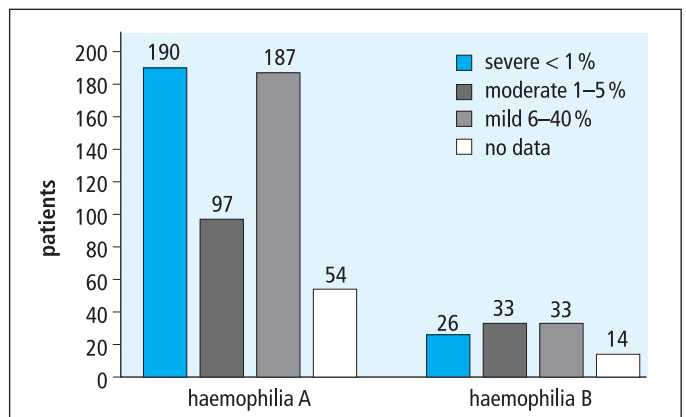
plasma-derived factor IX concentrates remain clearly on top: 92% vs. 8% rFIX. It is somewhat disappointing to note that the type of concentrate remains unknown in many patients (30–40%). The total amounts of concentrates used between October 2007 and September 2008 are shown in ► Figure 5.

## Quality control

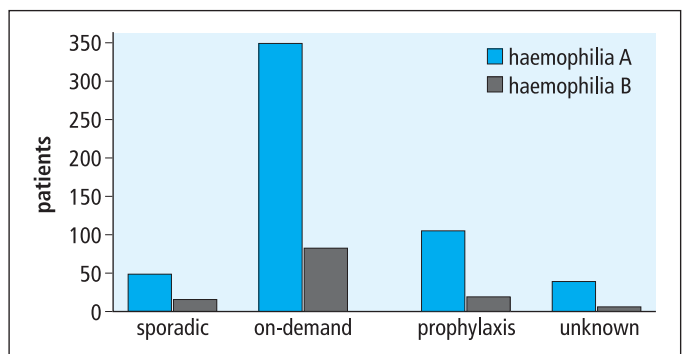
Currently there are 47 authorized users in the registry, all accredited members of the Medical Committee of the Swiss Hemophilia Society. The history of their logins as well as the quality and the regularity of their inputs in the registry are controlled once a year. Compliance to the rules set by the Medical Committee is one of the key criteria for the accreditation and re-accreditation/validation of the corresponding haemophilia reference or treatment centers.

Currently, two reference and eight treatment centers for children and five reference and six treatment centers for adults are established in Switzerland. Apart from three small centers, all actually meet the compliance

**Fig. 2** Severity grading of haemophilia A and B (classification by ISTH standards)



**Fig. 3** Treatment type



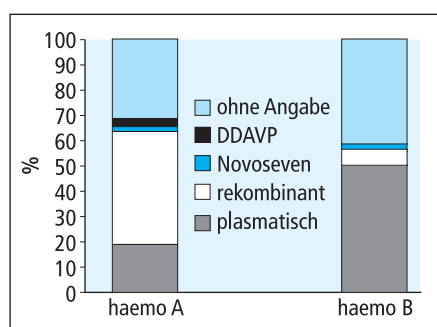


Fig. 4 Type of factor concentrates

criteria, yearly editing the medical data of at least 80% of their patients and reporting on the follow-up of at least 3 (for treatment centers) or 20 (for reference centers) severe haemophiliacs during the last 12 months.

The registry will start to exert a continuous control of the last check-up dates; this will be a very valuable tool to prospectively assess quality of medical care in haemophilia and help to ensure at least one annual check-

up in an accredited center for patients with severe disease. Currently, compliance rate to this rule is only about 50%.

## Conclusions

The current status and the outlook of the registry are very encouraging. After 14 years it really starts to be self-acting. For haemophilia specialists it has become a motivating tool to put in data, as it provides useful epidemiological, clinical but also economical data.

The registry soon will be used for prospective quality control of haemophilia centers and care in Switzerland.

The data of the Swiss Registry will enable international comparisons in the fields of clinical and epidemiological research in haemophilia (4). It is planned to expand the spectrum of registered diagnoses, newly including severe inherited platelet disorders

(e.g. Bernard-Soulier syndrome and Glanzmann's thrombasthenia) and other coagulopathies.

Finally and after obtaining every individual informed consent to that, the registry will integrate the results from the national mutation analysis of haemophilia families in Switzerland.

Patients indirectly take advantage from the registry, as they get accurate and up-to-date information on accredited haemophilia centers in the country. Swiss medical authorities as well as suppliers show increasing interest in the data of the registry for their planning of emergency supplies of factor concentrates and prediction of overall usage in Switzerland. The registry will require continuous efforts of the Medical Committee and support from the Swiss Hemophilia Society Board to meet highest quality requirements.

## Interessenkonflikt

Alle Autoren erklären, dass kein Interessenkonflikt besteht.

	haemo A	von Willebrand	
FVIII r	19 080 850		
FVIII pd	6 770 250	571 500	
<b>Total FVIII total</b>		<b>26 422 600</b>	
			haemo B
FIX r			354 750
FIX pd			3 258 600
<b>Total FIX total</b>			<b>3 613 350</b>
Novoseven	1 592	0	828
akt. Prothrombin	249 000	0	

Fig. 5 Annual consumption of factor concentrates

## References

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