

Prophylactic vs. On Demand treatment in haemophilia patients – real life data according to electronic diary smart-medication™

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Background:

Haemophilia home treatment may be done on regular base (prophylactic), in case of bleeding (on demand) or as mixed regimens such as temporary prophylaxis. Bleeding rates as well as the number of follow-up treatments in case of bleeding according to the three treatment groups were analyzed from real life data acquired from the electronic patient diary smart medication™.

Methods:

195 patients with haemophilia A or B completed their electronic smart medication™ documentation in 2014 and were analyzed according to home treatment regimen and bleeding history. Three groups were compared according to documented patient entries (Tab. 1). Patients with more than 90% prophylactic entries, accordingly less than 10% for bleeding were defined as prophylaxis group. Vice versa, patients with less than 10% entries as prophylactic treatment and more than 90% for bleeds were defined as on demand group. All others with varying treatment causes were defined as mixed group.

TABLE 1: DEFINITION OF TREATMENT REGIMEN ACCORDING TO TRANSMITTED PATIENT ENTRIES

	N=192	Entries for prophylaxis	Entries for bleedings
Prophylaxis group	127	>90%	<10%
Mixed group	49	10 – 90%	10 – 90%
On Demand group	16	<10%	>90%

Results:

A majority (64%, n = 125) was on prophylactic treatment with <10% treatment episodes for bleeding (Fig. 1 and 2). A minority (9%, n = 18) treated on demand with less than 10% for occasional prophylactic purpose. Nearly one third (27%, n = 52) of all patients were on mixed regimens using 10% to 90% of factor for either prophylactic or bleeding reason. Patients in the prophylaxis group were younger, than in the mixed and the on demand group (Tab. 2). Annual factor VIII/IX consumption/kg BW was highest in the prophylaxis group, similar in the mixed group and lowest in the on demand group. Factor consumption per bleed was highest in the mixed group, followed by the prophylaxis and the on demand group. Accordingly, follow-up treatment was most frequent in the mixed group.

FIG 1: NUMBER OF ANNUAL BLEEDS ACCORDING TO TREATMENT REGIMEN

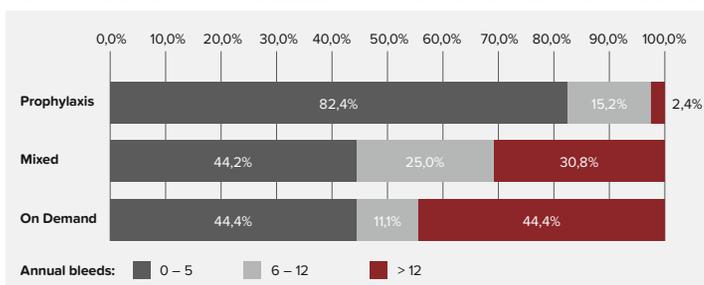


TABLE 3: NUMBER OF BLEEDS, NUMBER OF FOLLOW-UP TREATMENTS PER PATIENT AND NUMBER OF FOLLOW-UP TREATMENTS PER BLEED ACCORDING TO TREATMENT REGIMEN

	N=142	Bleeds/Patient	Follow-up treatment/Patient	Follow-up treatment/Bleed
Prophylaxis	77	4,2	1,9	0,6 ± 1,2
Mixed	49	11,2	12,1	2,1 ± 4,2
On Demand	16	18,0	30,9	1,2 ± 3,3

FIG 2: ANNUAL JOINT BLEEDS IN RELATION TO FACTOR CONSUMPTION

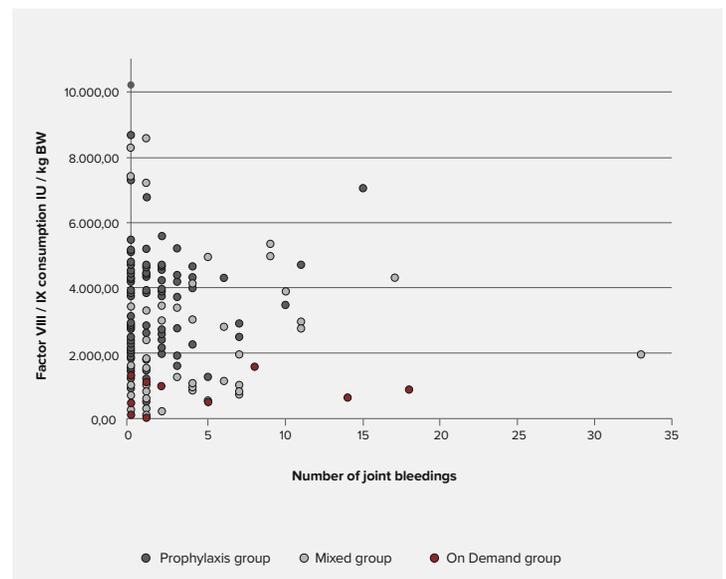


TABLE 2: MEAN AGE, MEAN ANNUAL FACTOR VIII/IX CONSUMPTION AND MEAN FACTOR TRANSFUSION PER BLEED

	Mean age	Mean annual factor VIII/IX consumption IU/kg BW	Mean factor VIII/IX requirement per bleed IU/kg BW	Mean number of bleedings
Prophylaxis group	24	2760	39	4,2
Mixed group	29	2222	62	11,2
On Demand group	34	542	34	18

Summary:

- Real life data are in accordance to published data showing less bleeding during prophylactic treatment in contrast to on demand treatment. Mixed regimens showed similar high bleeding frequency as in the on demand group in spite of similar high factor requirement as in the prophylaxis group.
- Follow-up was longer with higher factor requirement in the mixed in contrast to the on demand group. The prophylactic group may not be compared in this context, as it was not clear to distinguish between follow-up and regular prophylaxis in this group.
- In general, mixed regimens are not advisable because of frequent bleeds and high factor consumption. Prophylaxis should be increased in order to prevent spontaneous bleeds in the first place.
- Younger patients rather use prophylaxis, older are rather on demand regimens.
- All data were acquired automatically from patient smart medication™ entries and did not require cumbersome and erroneous transferal from paper diaries.