



Prophylactic vs. on demand treatment in haemophilia patients – real life data according to electronic diary smart-medicationTM

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Background:

Home treatment may be done on regular base (prophylaxis, P), in case of bleeding (on demand, OD) or as mixed regimens (temporary prophylaxis, M). Bleeding rates in real life were analyzed according to the three treatment groups.

Methods:

249 patients completed their electronic smart medication[™] documentation in 2016 and were analyzed according to home treatment regimen and bleeding history.

TAB. 1: DEFINITION OF TREATMENT REGIMEN ACCORDING TO TRANSMITTED PATIENT ENTRIES

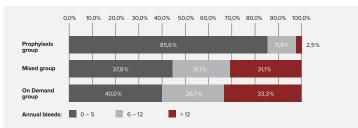
| | N=249 | Entries for prophylaxis | Entries for bleedings |
|------------------|---------|-------------------------|--------------------------|
| Prophylaxe group | 160 64% | >90% | <10% |
| Mixed group | 74 30% | 10% – 90% | 10% – 90% |
| On Demand group | 15 6% | <10% | >90% |

Results:

A majority (64%, n=160) was on prophylactic treatment. A minority (6%, n=15) used > 90% of factor VIIII/X for bleeds and < 10% for temporary prophylaxis (OD). Nearly a third (30%, n=74) of all patients used 10-90% factor VIIII/IX for on demand or temporary prophylaxis (M). Mean age was 25 (P), 42 (OD) and 34 (M). Annual bleeding rates were 2.4 (P), 12,9 (OD) and 11.4 (M). Total 129 joint bleeds in (P) (0,8/patient), 60 in (OD) (4/patient) and 370 in (M) (5/patient).

Annual factor VIII/IX use (IU/kg BW) was 2852 IE/Kg (P), 536 IE(OD) and 2465 IE (M). The average factor VIII/IX dose per bleed (IU/kg BW/bleed) was 26 (P), 25 (OD) and 31 (M).

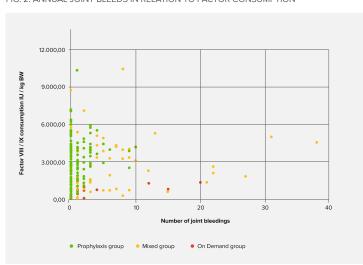
FIG 1: NUMBER OF ANNUAL BLEEDS ACCORDING TO TREATMENT REGIMEN



TAB 3: NUMBER OF ANNUAL JOINT BLEEDS ACCORDING TO TREATMENT REGIMEN

| | N=559 | Mean number of joint bleeds |
|-------------------|-------|--------------------------------|
| Prophylaxis group | 129 | 0,8 |
| Mixed group | 370 | 5,0 |
| On Demand group | 60 | 4,0 |

FIG. 2: ANNUAL JOINT BLEEDS IN RELATION TO FACTOR CONSUMPTION



TAB 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 | 25 | 2852 | 26 | 2,4 |
| Mixed group | 34 | 2465 | 31 | 11,4 |
| On Demand group | 42 | 536 | 25 | 12,9 |

Summary:

Real life data are in accordance to published data showing substantial less bleeding during prophylactic treatment in contrast to on demand treatment. Nearly one third of patients treat on mixed regimens with subsequently high bleeding rates, in spite of the highest dosing per bleed and may profit from more regular treatment.