

Prophylaxis with increasing doses, our experience about 8 cases

F.MEZHOUD, A.KRIM, N.SALHI, N.SIDI MANSOUR
Department of hematology, CHU Benbadis CONSTANTINE, ALGERIA

Introduction

➤ Prophylactic treatment of severe haemophilia A is likely to be more effective than on-demand treatment, but is more expensive considering the highest amount of units used.

Objective

- Reduction of the bleeding frequency.
- Prevention of hemophilic arthropathy.

Méthodes

- It's a prospective study.
- We recruited 8 patients with severe hemophilia A, with an age range of 21 months to 9 years over a 7-22 months period depending on the availability of recombinant factor VIII, to ensure full Protocol compliance.
- 5 patients with severe hemophilia A live in Constantine, 3 others in Mila (54 Km from Constantine)
- 8 boys and their parents have benefited from 8 therapeutic education sessions.
- Prophylactic treatment is done at home.
- They were first treated with once a week infusions (dose = 50 IU/kg), and the frequency was increased step by step if occurrence of unacceptable bleeding according to the previously described escalating (Canadian Protocol)
- All hemorrhagic events and the dose of recombinant factor VIII received during the prophylaxis period or for breakthrough bleed have been reported on the hemophilia logbook.
- Patients were followed every 3 months during outcome visits with the hemophilia team, in order to assess the frequency of bleeding and to determine the development of the target joint.

Our patients with severe hemophilia A at start of prophylaxis:

patient	Age at start of prophylaxis	type of prophylaxis	Target joint	radiological score of Peterson	inhibitors
M A	6 years	Long term Secondary P *	Right ankle	0	No
B M	4 years	Long term Secondary P	No	0	No
M T	4,5 years	Long term Secondary P	Right knee	0	No
K A	4,5 years	Long term Secondary P	No	0	No
G A	9 years	Long term Secondary P	Left knee	0	No
M Z	21 months	Primary prophylaxis	No	0	No
G A S	7,5 years	Long term Secondary P	No	0	No
K H	4 years	Long term Secondary P	No	0	No

Secondary P *: secondary prophylaxis

Results

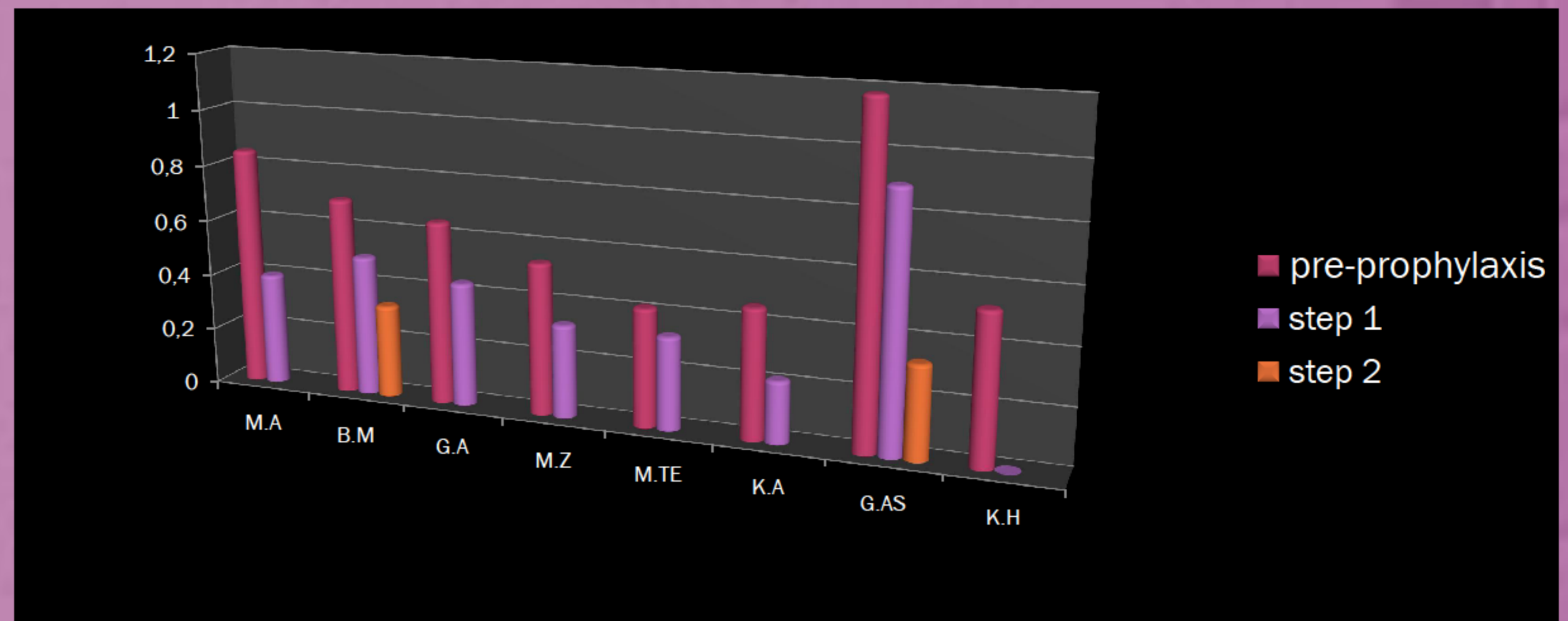
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- All the 8 boys with severe hemophilia A exhibited a decrease in the monthly frequency of bleeding following the initiation of prophylaxis.
- 1 patient of 8 didn't report a single event since the start of prophylaxis (during 7 months)
- 2 others met the criteria for escalation to step 2:
- ✓ 1 patient has developed 4 hemarthrosis on the same joint (left knee) during the period of 3 months.
- ✓ 1 patient presented multiple bruises on both arms every month.
- A marked improvement after escalation to step 2.

Conclusion

- Young patients with severe hemophilia A on prophylaxis exhibit a decreased bleeding tendency that is associated with the preservation of joint structure and function.
- Prophylaxis improves quality of life.
- Prophylactic treatment is an expensive treatment but is profitable in the long term because it eliminates the high cost of subsequent management of patients whose joints are damaged.

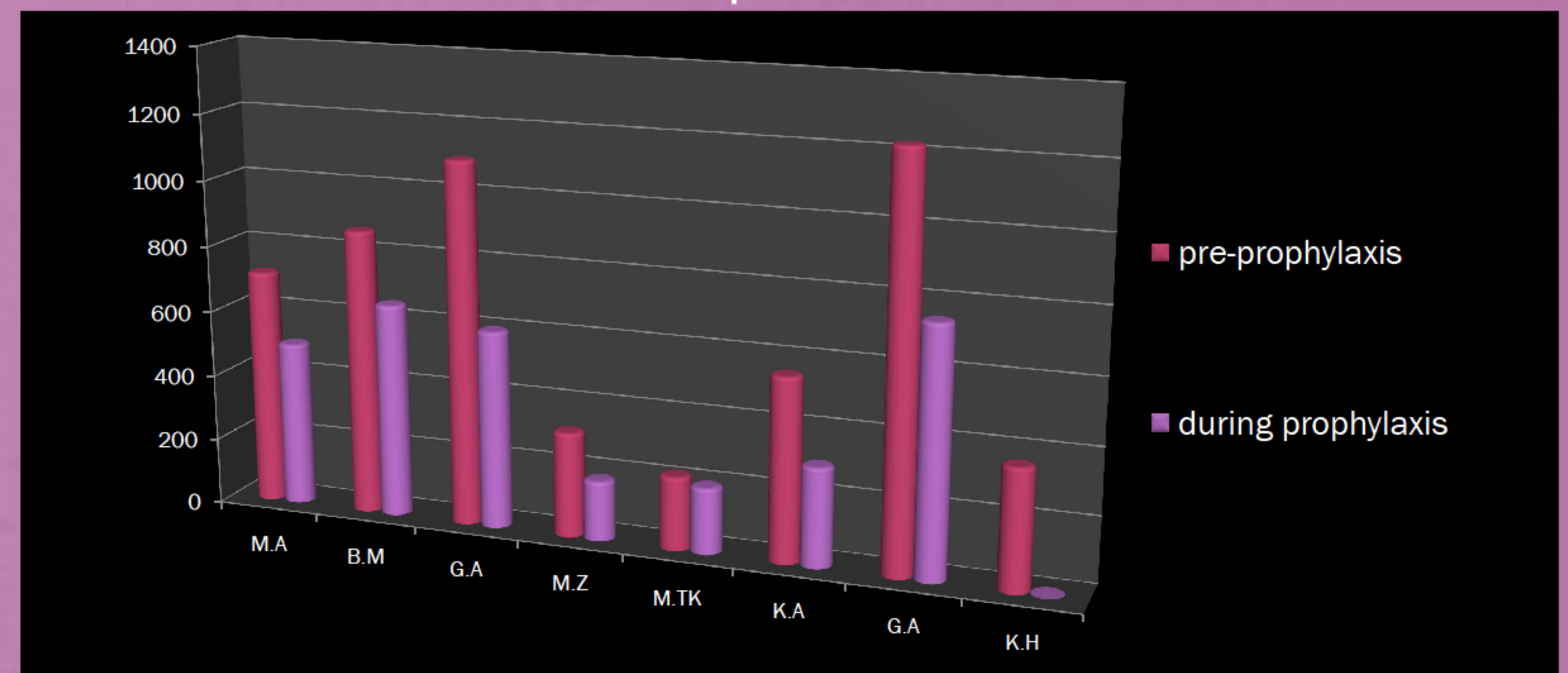
Monthly frequency of hemorrhagic event:



2

- All patients consumed less factor VIII during bleeding event since the start of prophylaxis but with varying degrees.
- Before the start of prophylaxis: the monthly consumption of recombinant factor VIII during the bleeding episodes ranged of 222 IU/month to 1227 IU/month.
- After the start of prophylaxis: the monthly consumption of recombinant factor VIII during the bleeding episodes range of 181 IU/month to 750 IU/month.

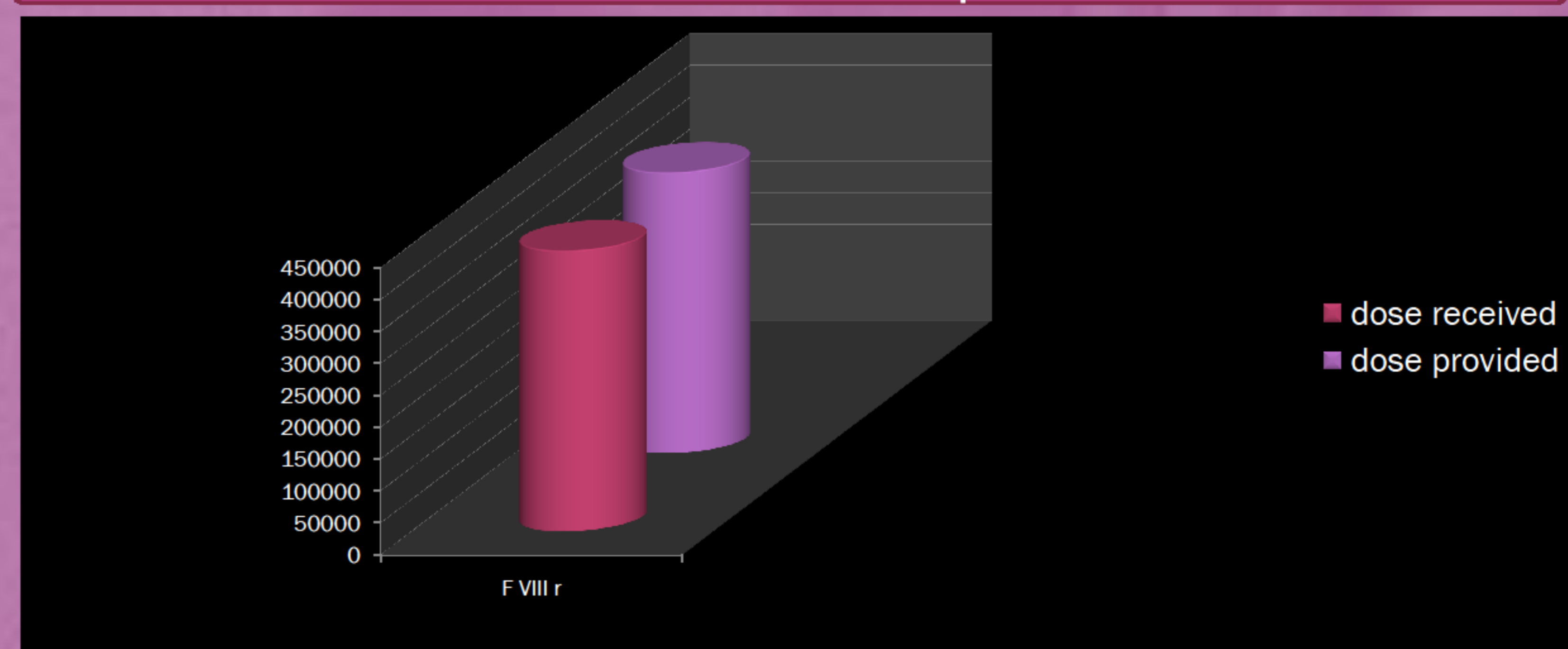
the monthly consumption of recombinant factor VIII during the bleeding episodes



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- Treatment adherence was excellent.
- dose received = infusions provided = 440 000 IU of recombinant FVIII.
- The satisfaction level of parents is good.
- Parents of hemophiliacs are more optimistic about the health of their children.
- Peripheral venous capital is preserved.
- Decrease of truancy
- Good quality of life.
- Cost: 33 000 000 AD = 264 000 €
- Prophylactic treatment is expensive.

dose received = infusions provided



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	New target joint	Radiological score of Peterson	inhibitors
all our patients During prophylaxis	No	0	No

