

# Five-year treatment report of hospitalized children with Glanzmann's thrombasthenia in a comprehensive hemophilia care centre (2006-2011)

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## OBJECTIVES

In this retrospective study we report on 5 years experience on control bleeding in 15 admitted children with Glanzmann's thrombasthenia (GT) in Mofid comprehensive care centre for Children with Hemophilia in Iran during 2006-2011.

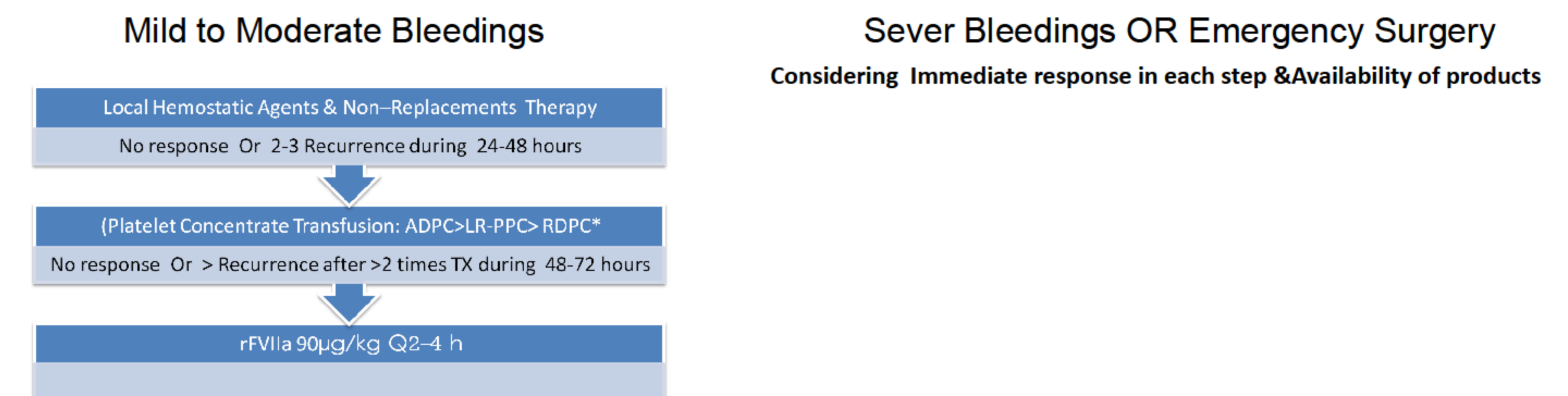
Our main aims were:

- ❖ To find the response AND failure rate of admitted GT patients to each products
- ❖ to define usage indices for products and medicines
- ❖ To find the effect of availability and affordability of needed products for treatment

## METHODS

All of the bleeding events and elective or emergency surgeries in admitted GT Patients were included in this study.

Treatment protocols:



\* Only in case of unavailability of both LR-PCs and rFVIIa we advised RDPCs

Apheresis derived (ADPC), Leukoreduced pooled (L.R.P.P.C), Non-Leukoreduced random donor PCs (RDPC), Recombinant-activated factor VII (rFVIIa)

The main variables we were looking for were age; type of bleedings; number and types of used PCs; usage amount of rFVIIa; response rate of bleedings and patients to PCs, rFVIIa, or both; and the cause of treatment decision (including availability of products, responsiveness, and severity of bleeding). Finally, we defined some indices for treatment requirements

## RESULTS

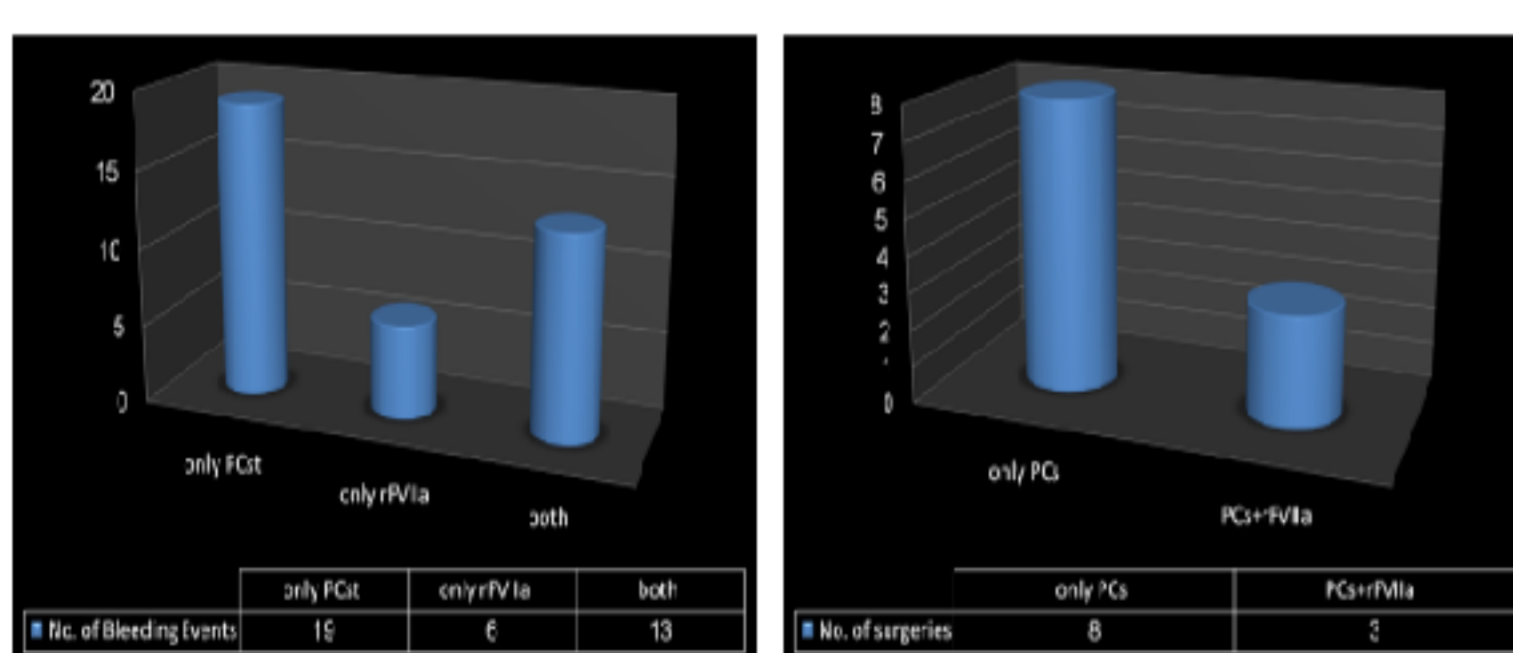
In all, 15 cases of children were admitted with GT (mean age of 3.5 years) for 52 bleeding events (79%) or elective surgeries (21%). Total amount of used rFVIIa was 137 mg and infused PCs were 68 units, among them 35, 29, and 4 units were ADPC, LRPPC, and RDPCs, respectively. Four patients (29%) received only PC and 50% of bleedings were controlled only with PCs. Two out of 11 patients (18%) who received rFVIIa were non-responder to PCs. 9 out of 11 were due to unavailability to LR-PCs. 2 cases (18%) did not respond to rFVIIa.

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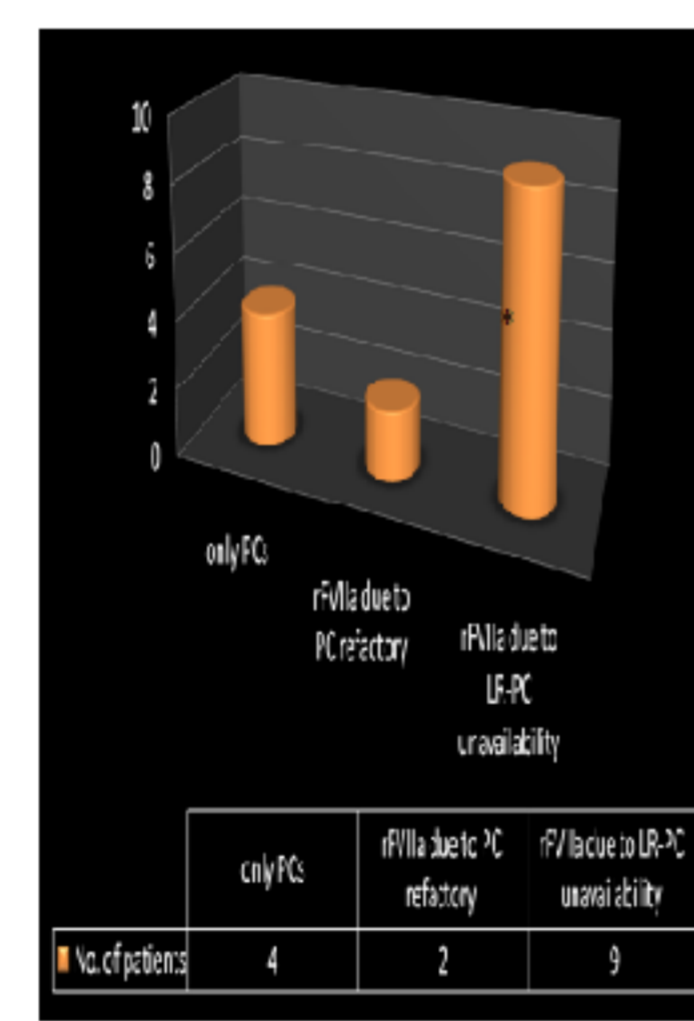
### Bleeding Events

50% of bleeding accidental events were controlled only with PLCs

73% of surgeries were controlled only with PLCs

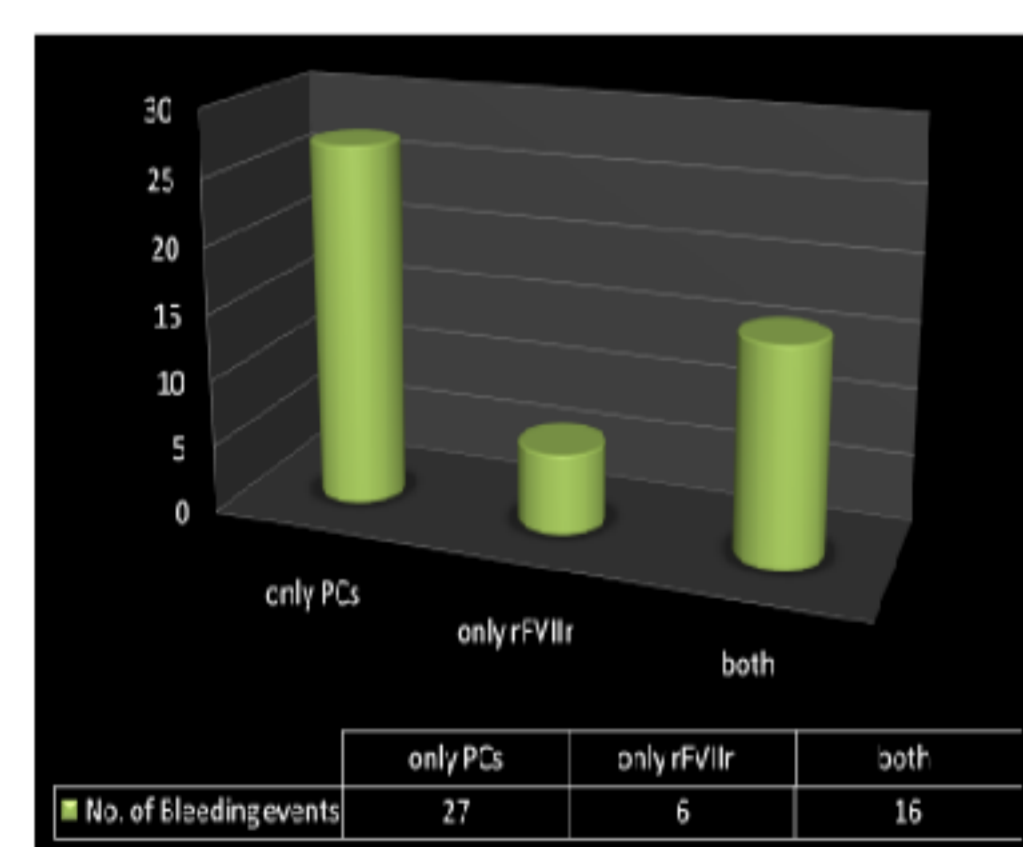


### Product usage in patients



- 4 patients (29%) received only PC
- Just 2 out of 11 patients (18%) who received rFVIIa were non-responder to PCs. 9 out of 11 were due to unavailability to LR-PCs
- 2 cases (18%) did not respond to rFVIIa.

### Product usage in Bleeding Events



Leukoreduced PCs (LRPC) were not available in at least in 22/49 (45%) of bleeding events as needed

### Treatment Indices

- Mean admission per Patients per Year : 0.69
- Mean LRPC units per patients per Year : 0.85 U
- Mean LRPC units per Bleeding Event: 3.1 U
- Mean rFVII<sub>a</sub>(mg) per patients per Year :1.83 mg
- Mean rFVII<sub>a</sub>(mg) per Bleeding Event:2.6 mg

## CONCLUSIONS

- The rate of bleeding events lead to admission was obviously less than our expectation: efficacy of LHAs and Education
- With our standard protocol, product and medicine demands for children with GT could be very low (at most 150 U/Y of LR-PCs and 290 mg/Y rFVIIa in Iran)
- Full availability of LR-PCs would reduce the needs to rFVIIa and the cost of treatment about 50%
- We should remind, not all of the GT patients are responding to rFVII

