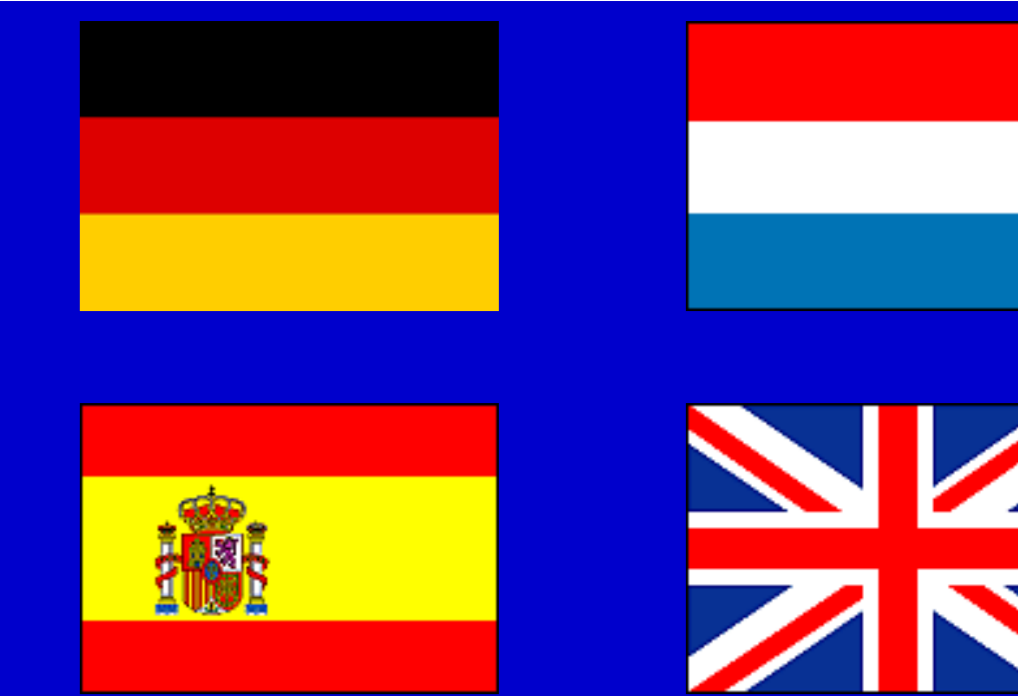


# Impact of Factor Replacement Therapy on Health-Related Quality of Life: an analysis of pooled data from 254 boys with severe hemophilia



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## INTRODUCTION

Prophylaxis reduces the frequency of bleeds in boys with severe hemophilia and is the standard of care for their management in countries with access to safe clotting factor concentrates. However, its effect on Health-Related Quality of Life (HRQoL) remains uncertain as small sample sizes have prevented the exploration of the effect of multiple factors.

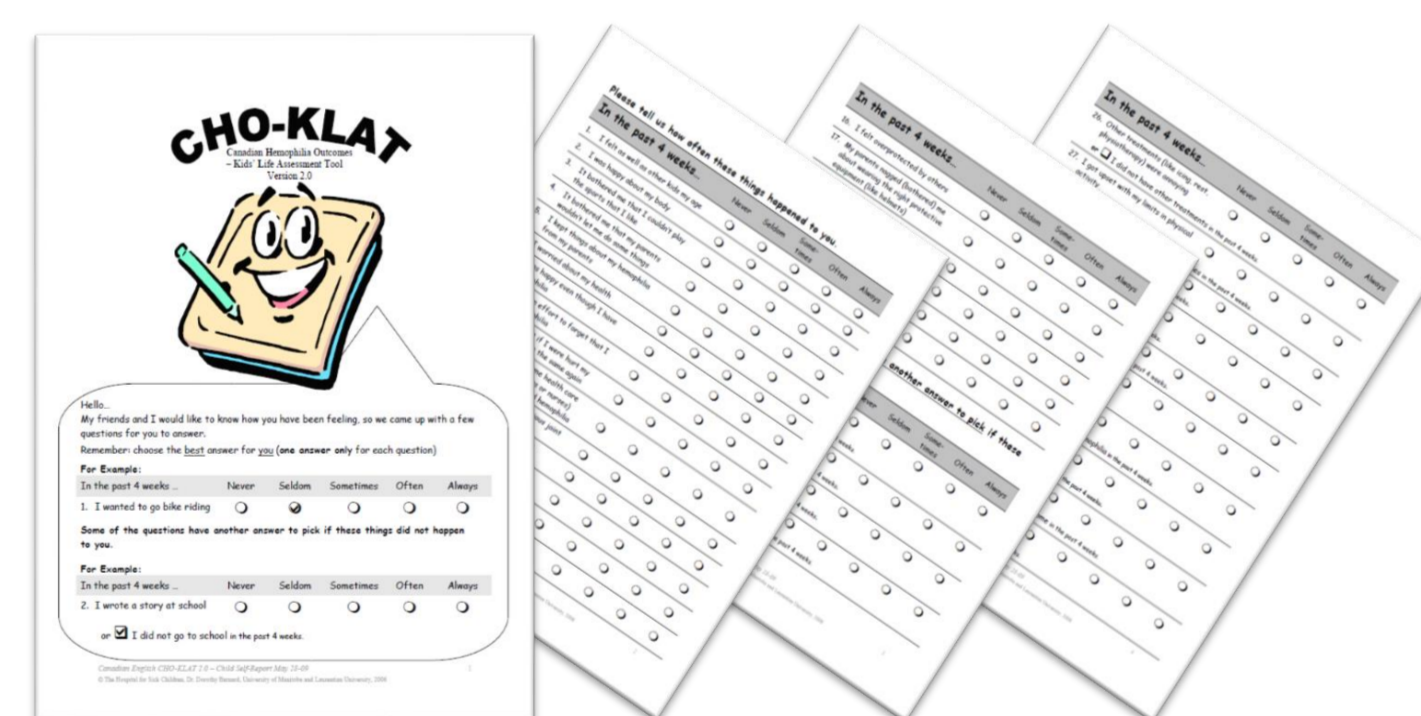
### Aim of this study:

To assess the impact of treatment regimen on HRQoL in boys, < 18 years of age, with severe hemophilia.

## METHODS

▪ **Data:** Data were pooled from 6 studies across 8 countries that measured HRQoL using the Canadian Hemophilia Outcomes-Kids' Life Assessment (CHO-KLAT) <sup>1-6</sup>.

▪ **Outcome:** Child reported CHO-KLAT scores were used: (0-100; where 100 is the best score).



▪ **Groups:** Subjects were classified into one of 5 treatment groups, defined by expert consensus, based on their treatment at the time of participation in the studies. :

- A. Early initiation with intensive prophylaxis (the Netherlands, Germany, UK, Spain)
- B. Gradual initiation with intensive prophylaxis (Canada, France)
- C. Late initiation with limited prophylaxis (China, Brazil)
- D. On-demand with good access to factor (Canada and Europe)
- E. On-demand with variable/limited access to factor (China and Brazil)

▪ **Analysis:** Linear Regression analysis was employed to assess the effect of treatment group on HRQoL. CHO-KLAT scores in boys with mild hemophilia were estimated from the pooled dataset, and used as a comparison.

## RESULTS

### ❖ Demographics:

Data from 254 boys with severe hemophilia were analyzed. Mean age was 11.4 (SD=3.4) years with range between 4.4\* and 17.9 years. Of those 220 (86.6%) had hemophilia A and 34 (13.4%) had hemophilia B.

\* A total of 21 children were < 7 years of age

### ❖ Regression model (Table 1):

The treatment groups had an effect on HRQoL, with group A (early initiation with intensive prophylaxis) having the highest scores, followed by:

- ↓ B (gradual initiation with intensive prophylaxis)
- ↓ C (late initiation with limited prophylaxis)
- ↓ D (on-demand therapy with good access to factor)
- ↓ E (on-demand with limited access to factor)

- Boys with severe hemophilia who receive limited on-demand factor replacement have significantly lower HRQoL scores.
- Although age influenced CHO-KLAT scores, it did not alter the effect between the groups, therefore, simple regression analysis was used.

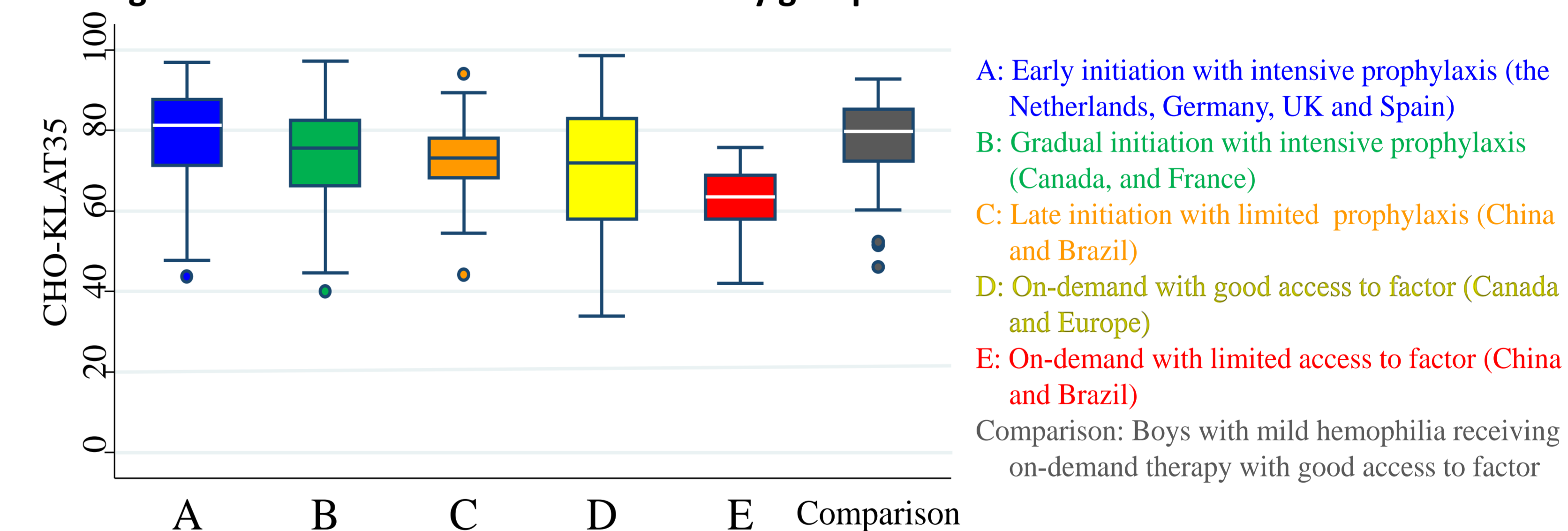
### ❖ Score Distribution (Figure 1):

- Boys with severe hemophilia who receive prophylaxis have similar HRQoL to the boys with mild hemophilia with good access to factor.

Table 1: Linear Regression Model

Treatment Group	n	Mean	Standard Deviation	Regression Co-Efficient	P value
A: early initiation with intensive prophylaxis	60	79.5	12.1	Reference Group	-
B: gradual initiation with intensive prophylaxis	120	73.7	12.5	-5.79	0.003
C: late initiation with limited prophylaxis	40	72.4	10.2	-7.03	0.005
D: on-demand with good access to factor	17	70.5	17.1	-8.96	0.008
E: on-demand with limited access to factor	17	62.6	9.4	-16.81	<0.001
Constant				79.5	<0.001

Figure 1: CHO-KLAT score Distributions by group



## DISCUSSION & CONCLUSION

- This poster presents differences by treatment group, that were defined by expert consensus. It is possible that these groups may not reflect changes in treatment protocols that have occurred over the past decade. Despite this limitation, important differences were observed between groups.
- Early initiation of intensive prophylaxis has the greatest impact on HRQoL in boys with severe hemophilia.
- HRQoL in boys with severe hemophilia exposed to early initiation of intensive prophylaxis is comparable to boys with mild hemophilia receiving on-demand therapy.
- These data strongly support the use of prophylaxis over on-demand therapy in boys with severe hemophilia.

## REFERENCES

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