

# Modified Long-term Low-dose Secondary Prophylaxis for Severe and Moderate Hemophilia A Children with Arthropathy : A single-center prospective study in China

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## Objectives:

With the economic and concentrate limitation, full-dose primary prophylaxis is difficult to carry out in China, the majority of children use minimal treatment and their joint status deteriorates rapidly. In 2011 we have reported the benefits of short-term prophylaxis (3 months) using low dose 10 U per kg BIW for Haem A and 20 U per kg weekly for B to reduce joint bleeding by 80% and moderate improve in joint function, daily activities and school attendance. ( Hemophilia 2011,17,70-74)

The objective of this study is to test the benefits of long-term low dose secondary prophylaxis for severe and moderate hemophilia A with arthropathy and comparing the efficacy of a low dose protocol using 10 U/ kg BIW to a modified protocol using 10 U/ kg TIW.

## Method:

### Single Chinese center self-control prospective study:

The former prophylaxis protocol mode: 10U FVIII/kg, 2x/week during February 2009 and October 2010 compared to the modified prophylaxis mode: 10U FVIII/kg, 3x/week using during November 2010 and October 2011.

### Assessments:

1. Joint bleeding frequency;
2. Joint - clinical/radiological;
3. Quality of Life ( BCH QoL score);
4. Psychological;
5. Family Burden scale of Disease (FBSD)

## Results:

Analysis on 6 children who completed 2 period prophylaxes, mean age 9.6 years (6.5 years to 12.5 years), 2 VIII:C <1% and 4 =1%, the reduction rate of frequency of joint (target joint) bleeding: 57%(63%)

	Protocol #1 10 u/kg, Q2W	Protocol #2 10 u/kg, Q3W
<b>Number of Joint Bleeding/Year</b>	68	29
<b>Total Number</b>	12.3	4.83 (0-8)
<b>Mean (range)</b>	(9-15.8)	<b>Reduction 57%</b>
<b>Number of Target Joint Bleeding/Year: Total Number</b>	64.6	24
<b>Mean (Range)</b>	10.8 (9-13.3)	4 (0-6) <b>Reduction 63%</b>
<b>Gilbert Score 17 Target Joints</b>	Improve 2 No-change 10 Deteriorate 5	Improve 1 No-change 13 Deteriorate 3
<b>Petersson Score 17 Target Joints</b>	Improve 0 No-change 12 Deteriorate 7	Improve 0 No-change 17 Deteriorate 0
<b>Psychology Assessment Total (cognition/ Emotion)</b>	Improve 1 (1/1) No-change 5 (5/3) Deteriorate 0 (0/2)	Improve 3 (3/3) No-change 3 (3/3) Deteriorate 0 (0/0)
<b>FBSD</b>	Improve 4 No-change 2 Deteriorate 0	Improve 3 No-change 3 Deteriorate 0

## Conclusions:

The study confirmed the improved benefits of modified long-term low dose secondary prophylaxis Using 10 U/ kg 3x week. With a successful reduction of the mean joint bleeding to 4.83 per year.

This reduction in Joint bleeding to about 5 per year is very significant and comparable to the results of about 5 bleed per year in the group of patients started later at an older age in the Malmo study ( ref)

The results of this small study of 6 patients should be interpreted as a preliminary observation but is a strong support to begin a larger study in our hospital including extending the low dose prophylaxis for a longer period beyond one year.

The result can be better if we have the resources to start prophylaxis earlier between 3 to 6 years before the target joints are well established.

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