Disease burden analysis of prophylactic treatment versus on-demand treatment for adult patients with moderate-to-severe haemophilia A in China



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Introduction

- Hemophilia A is a genetic disorder classified by deficient or defective coagulation factor VIII (FVIII) that puts those affected at risk for spontaneous bleeding episodes.
- Currently, a limited number of hemophilia A adult patients are treated with prophylactic FVIII, which requires costly and frequent infusions in China.

Objectives

To compare clinical outcomes and economic burden of prophylactic treatment with on-demand treatment in adult patients with moderate-to-severe haemophilia A in China.

Methods

- The analysis was conducted from a societal perspective over a one-year period.
- A disease burden model was developed to evaluate clinical outcomes and costs
 of prophylactic treatment versus on-demand treatment with recombinant factor
 VIII in 5 selected centres in China.
- Total bleeding events, hospitalizations for major surgeries, outpatient visits and productivity loss due to haemophilia were modelled and compared.
- Treatment patterns, treatment effectiveness, healthcare resource utilization, and direct medical costs were derived from a survey conducted in 5 centres across the country, including Beijing, Guangzhou, Hangzhou, Chengdu and Yangzhou.

 Opinions from patient organization experts were used for inputs regarding indirect and societal costs.
- Scenario analyses were undertaken to assess the impact of an increased proportion of adults receiving prophylactic treatment.

Results

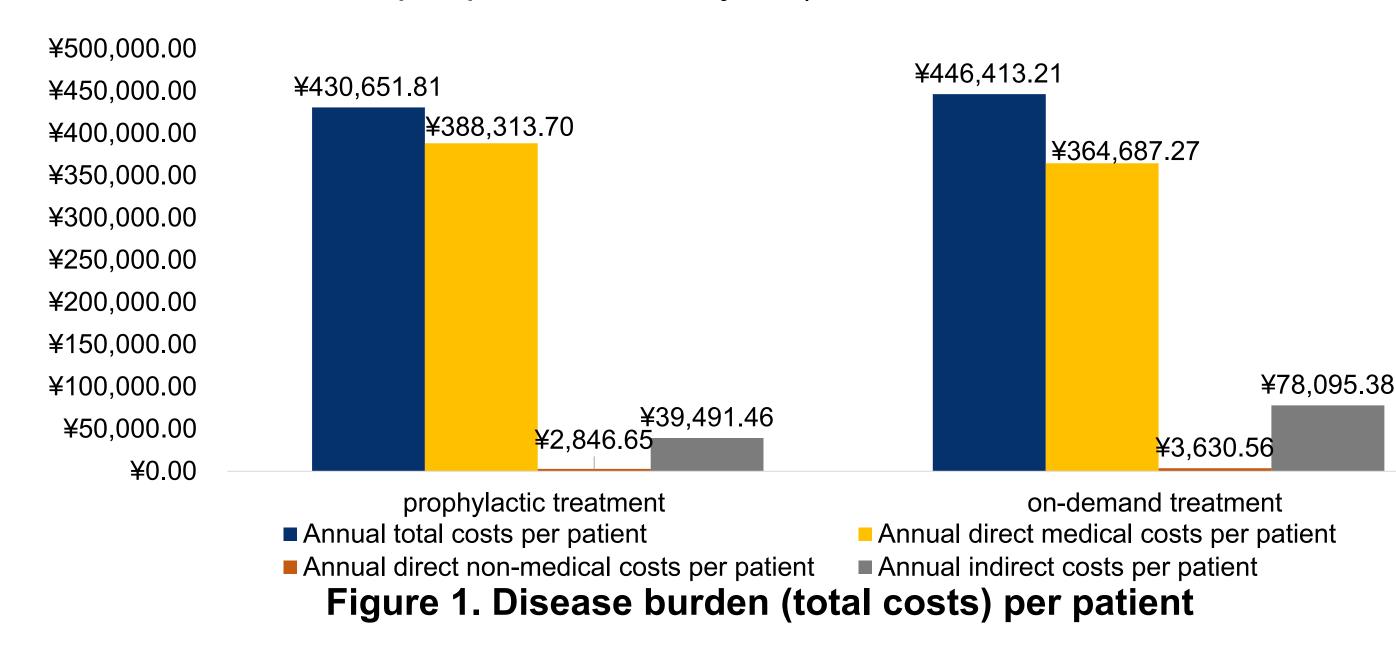
• The mean annual number of total bleeds was lower in patients receiving prophylaxis than those receiving on-demand treatment (4.40 vs. 27.00).

Table 1. Model inputs

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	Prophylactic treatment	On-demand treatment	Source
Base case: proportion of patients (%)	49.00%	51.00%	Survey
Scenario analysis 1: Assuming a 10% increase in prophylactic treatment	59.00%	41.00%	Assumption
Scenario analysis 2: Assuming a 20% increase in prophylactic treatment	69.00%	31.00%	Assumption
Scenario analysis 3: Assuming a 30% increase in prophylactic treatment	79.00%	21.00%	Assumption
Annual joint bleeding rate	2.80	23.40	Survey
Annual life-threatening bleeding rate	0.00	0.93	Survey
Annual bleeding rate	4.40	27.00	Survey
The incidence of hemophilic arthropathy (%)	60.00%	79.00%	Survey
Proportion of patients with hemophilic osteoarthritis requiring osteoarticular surgery (%)	4.46%	4.46%	Survey

Results

• The base-case analysis favoured prophylaxis over on-demand as it is associated with lower total costs per patient in one-year(¥ 430,652 CNY vs. ¥ 446,413 CNY).



- Prophylactic treatment saved ¥ 42,952 CNY in non-drug costs compared to ondemand treatment due to decreased bleeding and productivity loss, which offset more expensive prophylactic drug costs. When extrapolated to national level, the societal savings would be 531 million CNY per year.
- Compared with the base-case, assuming that the proportion of adult prophylaxis
 is increased by 10%, 20% and 30%, the projected incremental cost saving
 would be ¥12, ¥ 24 and ¥ 36 million CNY each year, respectively.

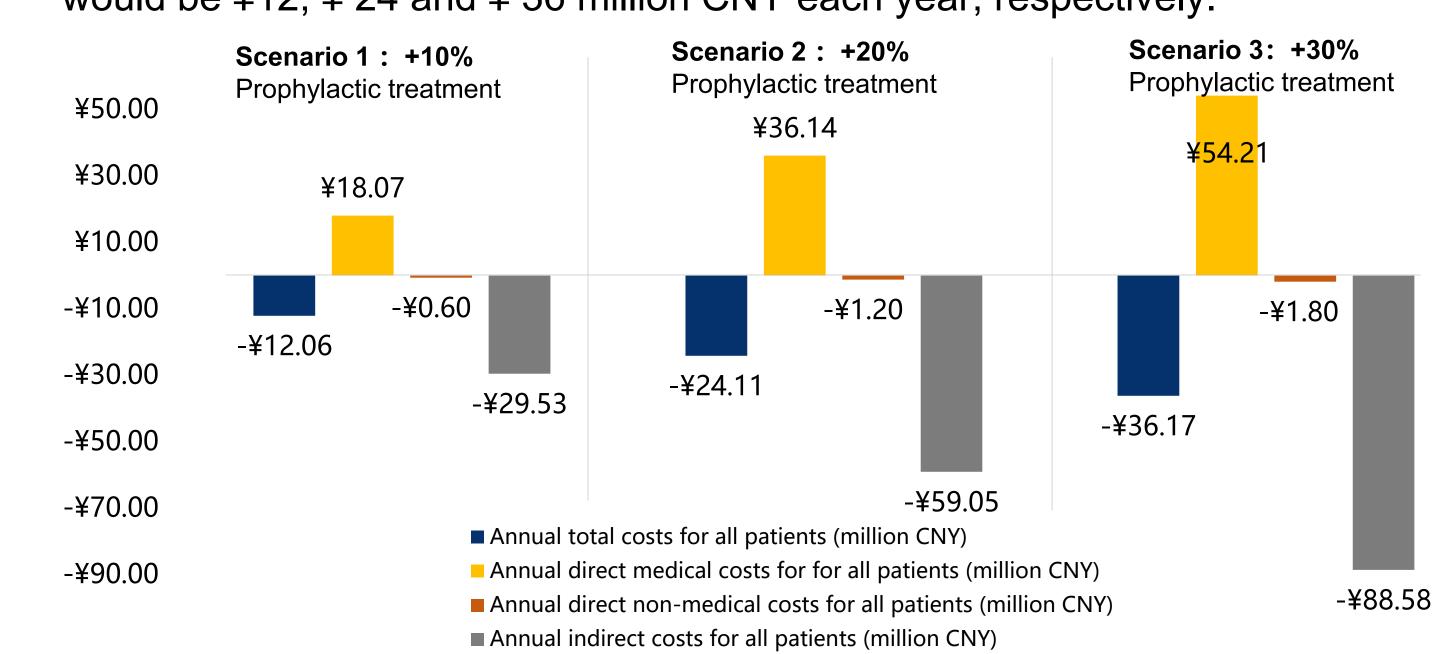


Figure 2. Incremental cost savings under each scenario

Conclusions

Prophylactic treatment for adult patients with haemophilia A was a cost-saving strategy compared with on-demand treatment in the participating 5 centers from societal perspective. These results suggest that prophylaxis should be considered to be implemented into clinical practice across China.

Disclosures

This study was sponsored by Bayer Healthcare Company Ltd, China. RH, NH and LB are Bayer employees. BT, AL, JX, JS and SH have no conflicts of interest to disclose.

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