

Introduction

Hemophilia A is a congenital bleeding disorder disease that is characterized by missing or defective factor VIII. Its clinical manifestations are repeated spontaneous bleeding in patients, and the bleeding sites are concentrated in joints, muscles, and deep tissue bleeding. In severe cases, gastrointestinal bleeding and central nervous system bleeding may occur, and even life-threatening

Objective

The objective of the present study was to determine the clinical value, economic value, patient value and social value of prophylaxis in adult patients with hemophilia A compared with on-demand.

Methods

- **Database:** PubMed, EMBASE, CNKI, WanFang and VIP (Search Period: June 2002-June 2022).
- **Key words:** The following search terms were used for identifying relevant literature, 'Hemophilia A', 'prophyla*', 'safety', 'efficacy', 'cost-effectiveness', 'cost-utility', 'quality of life', 'QALY', 'utility' and 'productivity loss'. **(Table 1)**
- **Inclusion criteria:** The literature must meet the following criteria at the same time.
 - (1) the patient was diagnosed with hemophilia A;
 - (2) the patient received preventive treatment;
 - (3) the research type was clinical randomized controlled trial, real-world research, etc.
- **Exclusion criteria:** Articles with any of the following conditions in this study were excluded.
 - (1) duplicate articles and conference abstracts and academic papers;
 - (2) do not match the theme of hemophilia A;
 - (3) Animal
 - (4) Non-English and non-Chinese publications.

Table 1. Literature search terms

Evaluation Dimensions	Chinese search terms	English search terms
Clinical Value	“血友病”和“预防”和“回顾性 OR 前瞻性 OR 注册登记 OR RCT OR 安全性 OR 有效性 OR 疗效 OR 效果 OR 临床研究 OR 试验 OR 荟萃分析 OR Meta OR 真实世界 OR 系统综述”	("Hemophilia" OR "Factor VIII Deficiency") AND ("prophyla*") AND ("retrospective" OR "prospective" OR "RCT" OR "safety" OR "register" OR "meta" OR "efficacy" OR "effectiveness" OR "clinical study" OR "trial" OR "Real-world" OR "RWE" OR "systematic review")
Economic Value	“血友病”和“预防”和“成本效果 OR 成本效用 OR 成本效益 OR 最小成本 OR 经济学 OR 经济性 OR 预算影响 OR 卫生经济”	("Hemophilia" OR "Factor VIII Deficiency") AND ("prophyla*") AND ("cost-effectiveness" OR "cost-utility" OR "cost-benefit" OR "cost-minimization" OR "econom*" OR "budget impact")
Patient value	“血友病”和“预防”和“生命质量 OR QoL OR EQ-5D OR SF-36 OR SF-6D OR 效用 OR QALY OR DALY OR 生命年 OR 生活质量”	("Hemophilia" OR "Factor VIII Deficiency") AND ("prophyla*") AND ("quality of life" OR "QoL" OR "EQ-5D" OR "SF-36" OR "SF-6D" OR "utility" OR "QALY" OR "DALY" OR "life year")
Social value	“血友病”和“成本 OR 花费 OR 经济 OR 负担 OR 费用 OR 生产力 OR 误工 OR 医疗资源 OR 卫生资源”	("Hemophilia" OR "Factor VIII Deficiency")AND (cost OR burden OR fee OR expenditure OR productivity OR "work delay" OR resource)

Results

- A total of 62 literatures were included in the study through full-text reading, including 15 Chinese articles and 47 English articles.**(Figure 1)**
- **Clinical value:** Adult patients with prophylactic treatment, which can effectively reduce bleeding, protect joint function, and bring more significant clinical efficacy.^[1]
- **Economic Value:** Although prophylactic treatment increases drug costs, in the long run, it can reduce the number of bleeding patients, shorten the length of hospital stay, and save treatment costs caused by bleeding events, which is economically beneficial.^[2-4]
- **Patient value:** Adult patients, which treated with prophylaxis, can significantly improve the quality of life of adult patients, which is in line with the patient-centered medical concept.^[5]
- **Social value:** If adult patients can receive prophylaxis treatment, they can return to normal work and life while gaining clinical benefits, reducing the labor losses borne by families and society.^[6]

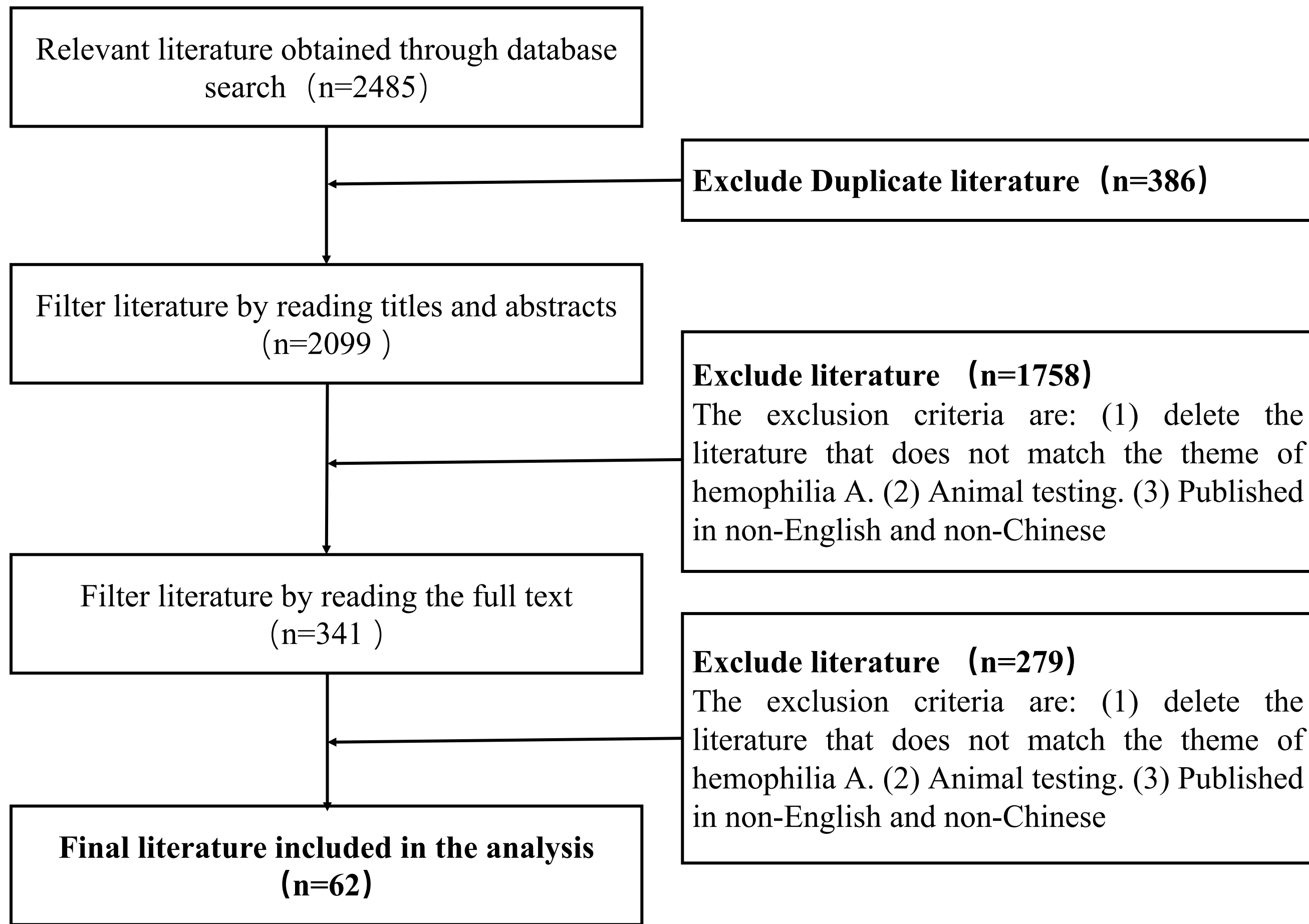


Figure 1. The result literature search

Conclusions

The review indicated that the core value of prophylaxis in adult patients with hemophilia A. Future research is needed to construct a value-based Multi-Criteria Decision Analysis framework approved by experts, which provides assessment basis for decision making by multiple stakeholders.

References

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