

Systematic Literature Review on the Value of Hemophilia Treatment in Japan

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OBJECTIVES

- The emergence of new modalities, including gene therapies, has raised interest in the multidimensional value of hemophilia treatments.
- This study aimed to collect a wide range of evidence on the value of hemophilia treatment in Japan, mainly from the health economic and quality of life perspectives, through a systematic literature review.

METHODS

- The target databases used were PubMed (since 1990) and two Japanese literature databases, Ichushi-Web and CiNii.
- We developed a search strategy that included search terms related to “hemophilia”, “cost”, and “quality of life” and selected evidence from any study design for Japanese hemophilia patients, excluding those related to efficacy and safety outcomes.
- Articles that qualitatively or narratively described the value of hemophilia treatment were included, as well as quantitative analysis.
- Table 1 presents PICOS criteria for inclusion and exclusion of articles.

Table 1. PICOS criteria for inclusion and exclusion of studies

Category	Inclusion	Exclusion
Population	Japanese patients with hemophilia A and B	<ul style="list-style-type: none">Patients without hemophiliaNon-Japanese patients
Intervention	Any types of hemophilia treatment	Interventions not aiming to treat hemophilia
Comparator	NA	NA
Outcome	<ul style="list-style-type: none">Economic outcomesQOL related and patient reported outcomesOther outcomes demonstrating the value of hemophilia treatment (excluding efficacy and safety)	NA
Study design	NA	NA

RESULTS

1. Characteristics of the identified articles

Figure 1. Flow diagram and article selection process

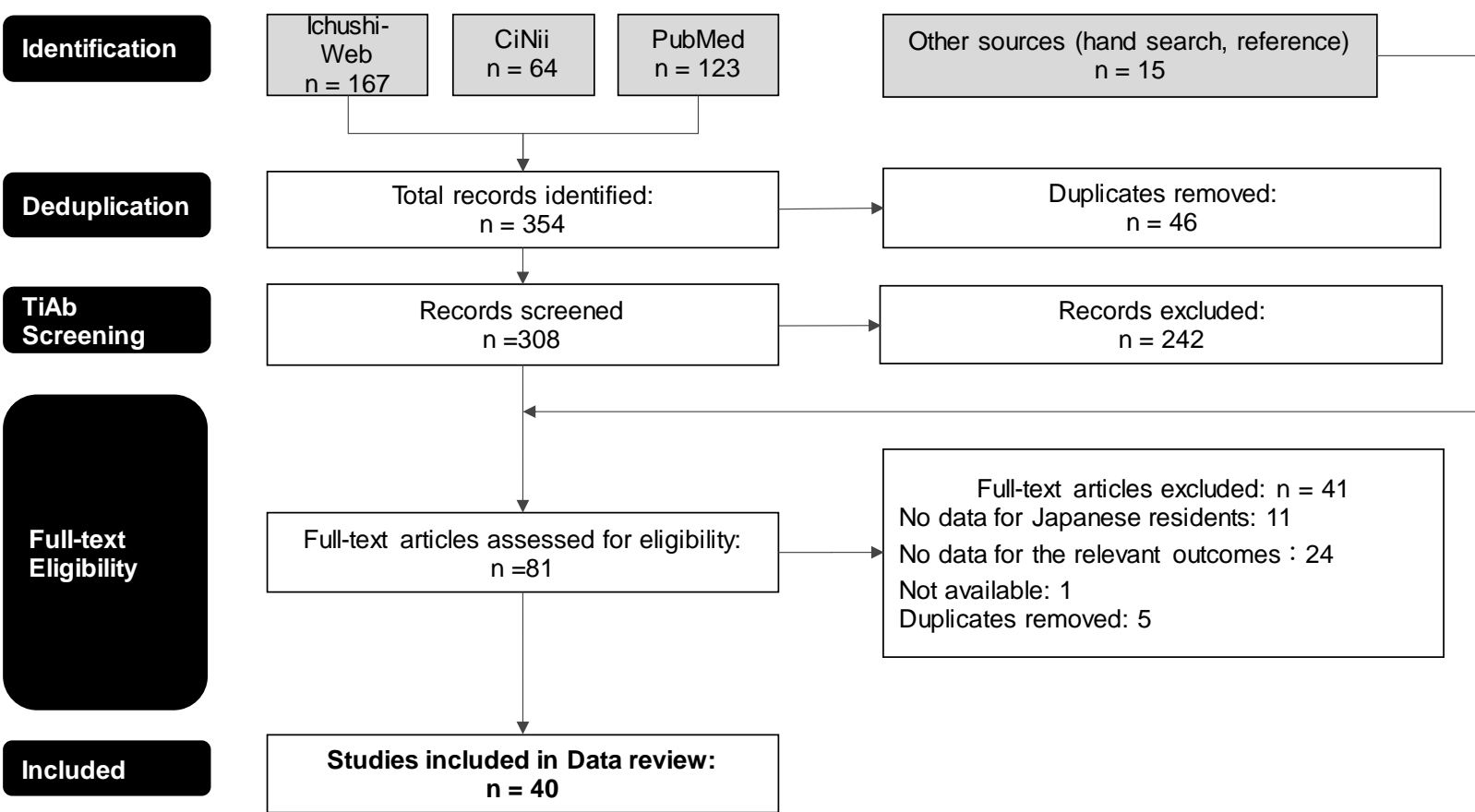
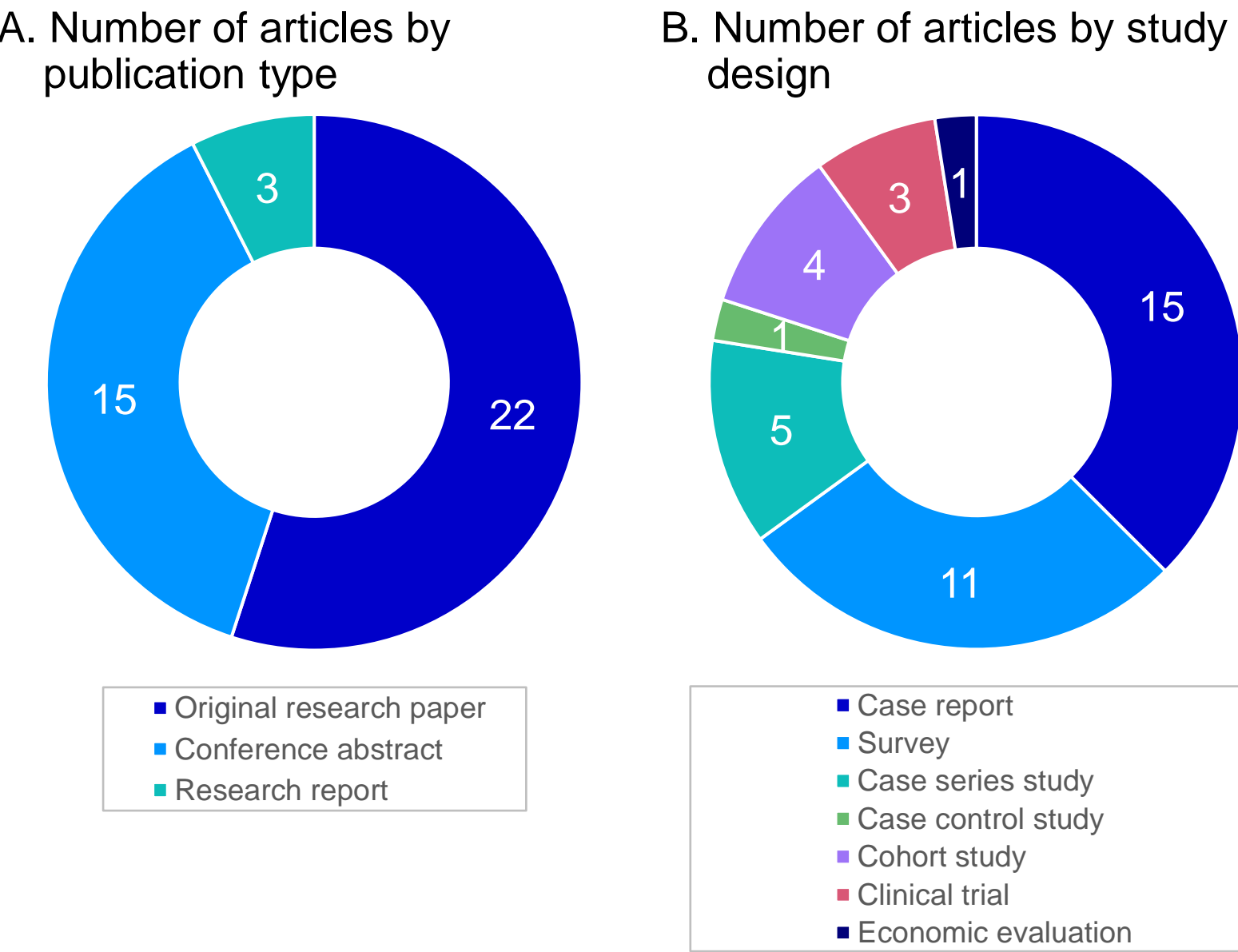


Figure 2. Publication type and study design of the articles



2. Hemophilia treatment featured in the identified articles

- Of the 40 articles identified, 14 focused on specific drugs for hemophilia, including 6 on emicizumab, a new drug for hemophilia A.
- Specific types of therapies were featured in 7 articles such as prophylaxis, concentrate and ITT.

- For surgery, 4 articles focused on TKA and THA and 3 on synovectomy.
- Eight articles measured QOL and other PROs for hemophilia patients in general, without focusing on specific treatments.

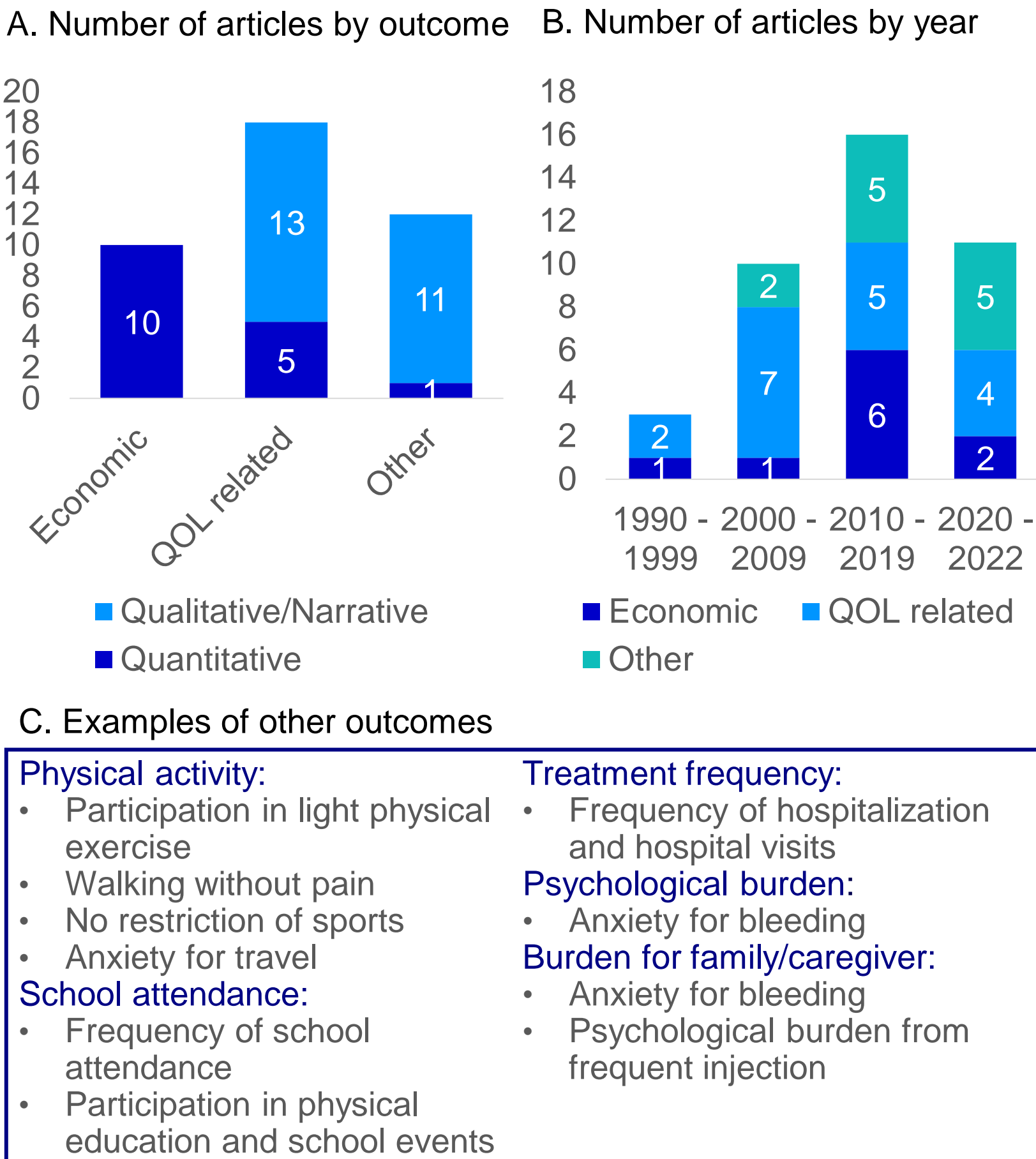
Table 2. Hemophilia treatment featured in the articles

Category	Treatment	# of articles
Drug	EHL	4
	aPCC	3
	concizumab	1
	emicizumab	6
Therapy	Prophylaxis/concentrate/ITT	7
Surgery	TKA/THA	4
	Synovectomy	3
Other	Physiotherapy	1
	CV port	1
	Comprehensive medical care	1
	Personalized medicine	1
General	-	8
Total		40

3. Publication year and outcome of the identified articles

- Ten articles focused on economic outcomes, 18 on QOL related outcomes and 12 on other outcomes (Figure 3-A).
- Other outcomes included physical activities in daily life, school attendance and frequency of hemophilia treatment (Figure 3-C).
- All economic outcomes were assessed quantitatively, while QOL related and other outcomes were assessed qualitatively or narratively for the 13 and 11 articles, respectively.
- Figure 3-B shows when the identified articles were published; 3 from 1990 to 1999, 10 from 2000 to 2009, 16 from 2010 to 2019 and 11 from 2020 to 2022.
- The number of articles has increased over the years, which was attributed to increased economic and other outcomes.

Figure 3. Publication year and outcome of the articles



CONCLUSION

- With the development of new therapies for hemophilia since 2010, there has been a growing body of evidence in Japan focusing on the broader value of hemophilia treatment in addition to the traditional outcomes of treatment cost and QOL.
- However, since most of the articles only discussed the value of hemophilia treatment qualitatively or narratively, more quantitative evidence is strongly required in the future.
- Limitations of this study were as follows: 1) Fifteen of the identified articles were conference abstracts, so the study details were not available; 2) The quality of the identified articles was not examined; 3) Synthesis was not performed in any form.