Abstract Submission

27. Thalassemias

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SYSTEMATIC LITERATURE REVIEW OF HEALTH-RELATED QUALITY OF LIFE BURDEN IN PATIENTS ACROSS THE SPECTRUM OF THALASSEMIA

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Background: Recent systematic literature reviews (SLRs) have highlighted the health-related quality of life (HRQoL) burden in transfusion-dependent thalassemia (TDT), particularly β-thalassemia (β-thal). However, less is known about HRQoL in non–transfusion-dependent thalassemia (NTDT) and alpha-thalassemia (α-thal).

Aims: To conduct an SLR on the real-world HRQoL burden across the entire spectrum of thalassemia and report evidence gaps.

Methods: Searches (2010 to September 2021) were conducted in MEDLINE, Embase, Cochrane Database of Systematic Reviews, Health Technology Assessment Database, NHS Economic Evaluation Database, and EconLit for real-world studies reporting on HRQoL, utilities, and patient-reported outcomes in adult and pediatric patients with thalassemia. Conference abstracts from 2017 to September 2021 were also searched. References were screened at title/abstract and then full text by two independent reviewers. Data extraction was undertaken by one reviewer and validated by a second independent reviewer. Results were grouped by genotype (β-thal/α-thal) and transfusion phenotype (TDT/NTDT) to support comparisons.

Results: A total of 2,541 records were screened at title/abstract, 441 at full-text and 7 references were identified through other sources. A total of 126 publications reporting on 116 study populations were included. Most evidence was published since 2015 (n=95) and sample sizes ranged from 22 to 1240. The number of publications by transfusion phenotype (TDT/NTDT) and thalassemia genotype (β-thal/α-thal) is shown in Table 1. Overall, most TDT/NTDT studies were conducted in β-thal or did not report genotype. Four studies included patients with α-thal but did not report HRQoL results separately for this subgroup. Across 52 studies reporting on TD β-thal, most showed that both adult and pediatric patients have reduced HRQoL. One study found EQ-VAS utility values to be poorer in adults than in children with TDT (mean [SD]: 75.04 [17.86] for >18 years vs. 84.97 [15.16] for ≤18 years; P<0.001). Four studies in pediatric patients noted the negative impact of TDT on caregivers' HRQoL. Very few studies assessed HRQoL for NTDT patients vs healthy controls, and none were in adult population. One pediatric study found NTDT patients had worse total and domain PedsQOL scores (all P<=0.05) than healthy controls. Several studies compared NTDT and TDT patients. Using the SF-36, one study found significantly poorer HRQoL in NTDT than in TDT adults (mean total score [SD]: 66.5 [16.1] vs. 75.8 [18.8]; P=0.021). A prospective, non-interventional study found significantly lower SF-36 mental component scores at baseline in NTDT compared to TDT adults (mean [SD]: 47.4 [8.4] vs. 51.6 [7.2]; P <0.01), and NTDT patients had worse HRQoL change from baseline at 24 weeks than TDT patients. In pediatric patients, one study found poorer HRQoL in TDT compared to NTDT (mean PedsQL total score [SD]: 42.25 [20.61] vs. 67.76 [18.31]; P=0.0001). However, both NTDT and TDT patients had poorer HRQoL than healthy controls (83.45 [15.6]); P<0.0001).

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Table 1 Number of publications per reported thalassemia genotype and transfusion phenotype

Thalassemia genotype	Transfusion phenotype					
	TDT only	NTDT only	Mixed (TDT vs. NTDT) *	Mixed (TDT and NTDT) †	Unspeci-fied	Total
β-thal only	52	2	6	4	16	80
α-thal only	0	0	0	0	0	0
Mixed (β-thal vs. α-thal) *	0	0	0	0	0	0
Mixed (β-thal and α-thal) †	1	0	1	2	0	4
Unspecified	24	0	2	2	14	42
Total	77	2	9	8	30	126

* Publications reporting results separately for these subpopulations

† Publications including these subpopulations without reporting results separately

≤10 publications identified
11-49 publications identified
≥50 publications identified

Summary/Conclusion: This SLR highlights the negative impact of thalassemia on HRQoL. Adding to previously published SLRs, it shows limited evidence is available for NTDT patients; however, studies suggest that HRQoL burden may be worse in NTDT than TDT patients, particularly in adults. Characterizing the HRQoL burden of α-thal is a clear evidence gap. High-quality, real-world studies in NTDT and α-thal are needed to better understand the HRQoL burden and the potential impact of new treatments on HRQoL.

Keywords: Quality of life, Thalassemia, Transfusion