The Economic Burden of β-Thalassaemia, Sickle Cell Disease, and Myelodysplastic Syndrome: A Literature Review

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Background

Individuals who suffer from specific anaemias or hemoglobinopathies often receive regular red blood cell (RBC) transfusions as part of their therapy. These individuals can be at a higher risk of experiencing transfusional iron overload (TIO), particularly if they experience transfusion dependence (TD), where RBC transfusions occur more frequently than every eight weeks[1]. β-thalassaemia, sickle cell disease (SCD) and myelodysplastic syndrome (MDS) are examples of conditions that can predispose individuals to TD and subsequent TIO.

Whilst the health-related quality of life (HRQoL) impacts and direct costs associated with regular blood transfusions may be a burden to people with β-thalassaemia, SCD or MDS, there are also indirect costs to consider. In particular, people with symptomatic β-thalassaemia, SCD or MDS may experience presenteeism (reduced performance while at work due to uncontrolled diseases or health risks), absenteeism (health-related productivity loss due to sick-leave), and other non-work related productivity losses[2]. Understanding and quantifying indirect costs are crucial to gain an insight into the overall economic burden of disease.

The objective of this literature review was to identify studies exploring the economic burden of β-thalassaemia, SCD and MDS.

Methods

Searches were performed in PubMed (including MEDLINE) and the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) database.

Search strategies were designed using a combination of high-level Medical Subject Heading (MeSH) terms and free text terms. Search terms (Table 1) were identified from a pragmatic search of available literature, as well as discussions between members of the study team. Initial search strategies were designed and piloted using the PubMed database, with relevant amendments made following discussions between members of the study team. The final search strategies were then adapted as required for use in the ISPOR presentation website databases.

Results

Searches returned 96 results (63 from PubMed and 33 from ISPOR), of which 30 were ultimately included. Thirteen studies were available as full-text studies, and 17 studies were ISPOR abstracts. Of the full-text studies (Table 2), two studies were literature reviews[7,8], with one review focusing solely on studies published in the United States (US)[7]. The second literature review identified studies from both the US and the United Kingdom (UK)[8]. Six of the remaining studies were carried out in the US[3,4,6,11,12,14], with one study each conducted in Iran[15], Italy[5], Japan[10], Greece[9] and Sri Lanka[13] (Figure 1).

Three full-text studies focused on MDS[3,6,10], four on β-thalassaemia[5,12,13,15], four on SCD[4,8,9,11], one on SCD and β-thalassaemia[14], and one on β-thalassaemia, SCD and MDS[7]. Only four full-text studies investigated the impact on indirect costs[5,11,13,15], with three of these studies focusing on β-thalassaemia[5,13,15], and one focusing on SCD[11] (Table 2).

Results confirmed the significant burden of all three conditions with respect to medication, inpatient and outpatient costs. Higher medical costs were reported in individuals requiring regular transfusions, with costs driven both by the infusions and subsequent iron chelation therapy (ICT). Patients with β-thalassaemia, SCD and MDS experienced absenteeism, presenteeism, and significant treatment-related burdens that impacted their HRQoL and finances. The conclusions presented in the 17 ISPOR abstracts were broadly aligned with those from the full-text studies.

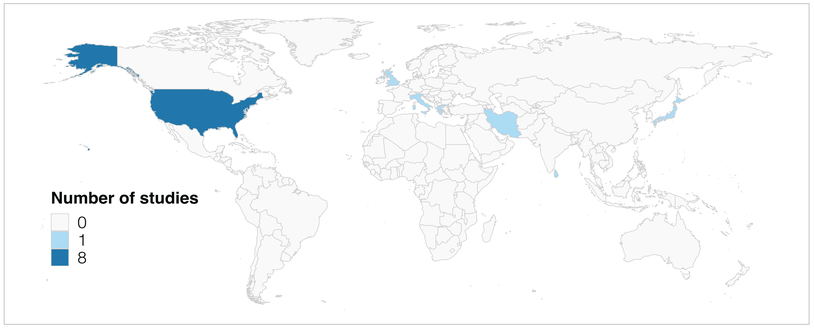
Table 2. Peer-reviewed economic burden studies of β-thalassaemia, SCD or MDS

Study	Year	Country	Condition(s)	Costs reported
Frytak *et al.*[3]	2009	US	MDS	Direct
Kauf *et al.*[4]	2009	US	SCD	Direct
Scalone et al.[5]	2008	Italy	β-thalassaemia	• Direct • Indirect
Goldberg *et al.*[6]	2012	US	MDS	Direct
Zhang *et al.*[7]	2011	US	• β-thalassaemia • SCD • MDS	Direct
Nietert *et al.*[8]	2002	• US • UK	SCD	Direct
Tsolakidis *et al.*[9]	2021	Greece	SCD	Direct
Tsutsué *et al.*[10]	2022	Japan	MDS	Direct
Holdford *et al.*[11]	2021	US	SCD	Indirect
Weiss *et al.*[12]	2019	US	β-thalassaemia	Direct
Reed-Embleton *et al.*[13]	2020	Sri Lanka	β-thalassaemia	• Direct • Indirect
Vekeman *et al.*[14]	2016	US	• β-thalassaemia • SCD	Direct
Esmaeilzadeh *et al.*[15]	2016	Iran	β-thalassaemia	• Direct • Indirect

Table 1. Search terms used to identify published studies investigating the economic burden of β-thalassaemia, SCD or MDS

#	Search term	PubMed Hits
1	(beta-Thalassemia[MeSH] OR MDS[MeSH] OR Anemia, Sickle Cell[MeSH] OR "sickle cell anemia"[tiab] OR "sickle cell anaemia"[tiab])	57,187
2	(“economic burden”[tiab] OR “cost of illness”[tiab] OR “burden of illness”[tiab] OR “burden of disease”[tiab])	28,578
3	#1 AND #2	63
4	Limit #3 to studies published in English	63

Figure 1. Economic burden studies of β-thalassaemia, SCD or MDS by country



Nietert et al. reported data from both the UK and the US settings

Conclusion

β-thalassaemia, SCD and MDS pose a significant economic burden to healthcare systems, with transfusions and iron chelation therapies forming a significant proportion of these costs; however, studies exploring the indirect costs (particularly related to MDS) are scarce and more research is required for the burden to be effectively characterised. Future studies could also further explore the impact of specific treatments on the economic burden of β-thalassaemia, SCD and MDS.

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