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POSTER #27

Economic burden of hemophilia B in the US: a systematic literature review

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ABSTRACT

Background: Hemophilia B (HB) is a rare disease caused by congenital Factor IX (FIX) deficiency. HB requires life-long management to prevent or manage bleeding and associated morbidity. Although HB affects only a small portion of the population, it is associated with high overall cost and imposes a significant financial burden on individuals, payers, and society in general. Due to variation in patient clinical characteristics and treatment choice, cost and healthcare resource utilization associated with disease management can vary significantly from patient to patient.

Aims: To review published direct costs and healthcare resource utilization associated with the management of HB in the US.

Methods: A systematic literature review was conducted by searching electronic databases (e.g. MEDLINE, Tufts CEA registry) to identify full-text studies (March 2009–March 2019). Additionally, a manual search for abstracts from relevant conferences was performed (from 2016). Studies were included in the review using pre-defined inclusion/exclusion criteria for population, study type, language (English), and location (US). Publications consisting of budget impact analysis, cost, burden of disease, healthcare resource utilization, and economics evaluations were included.

Results: Of 693 titles and abstracts screened, a total of 17 studies evaluating cost and resource utilization in patients with HB in the US were included. Data sources for these studies included: medical records ($n = 5$), insurance claims databases ($n = 10$), and surveys ($n = 2$). Reported cost and resource use varied across studies depending on severity of the disease, treatment regimen, and product type: extended (EHL) or standard half-life (SHL). The cost of FIX replacement therapy constitutes the majority of costs in HB management. Among patients with severe or moderate HB, reported mean annual cost of FIX ranged from $187,070 to $925,864 with an average of $560,801. Annual cost of EHLs could exceed more than twice the cost of SHLs. For example, mean annual cost of EHL FIX was $921,291 vs $478,096 for SHL FIX. Rates of healthcare resource utilization were also substantial for patients with HB and include hospitalizations, emergency room visits, and physician visits.

Conclusions: This systematic literature review found significant economic burden associated with HB in the US. The substantial costs and health resources utilized by patients highlight unmet needs remaining in HB.

KEYWORDS
Hemophilia; haemophilia; burden; cost; resource

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