**INTRODUCTION**

Hemophilia B (HB) is a rare disease caused by congenital Factor IX (FIX) deficiency. HB requires lifelong 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 consequent 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 HB can vary significantly from patient to patient. Also, the type of the clotting factor in use (standard half-life [SHL] vs extended half-life [EHL]) greatly impact associated costs.

**OBJECTIVE**

The literature review was conducted to study costs describing costs and resource utilization associated with HB in the US.

**METHODS**

A systematic literature review was performed by searching relevant electronic databases (MEDLINE, PubMed, and Tufts Cost-Effectiveness Analysis Registry) to identify full-text studies published within the period of 03/2009-03/2019. Additionally, a manual search for abstracts from relevant conferences was performed (conference period 01/2018-03/2019).

Studies were included in the review using predefined criteria with respect to: population (HB) and study type (budget impact model, cost study, burden of illness study, healthcare resource utilization associated with disease management can vary significantly from patient to patient. Also, the type of the clotting factor in use (standard half-life [SHL] vs extended half-life [EHL]) greatly impact associated costs.

**RESULTS**

- Three studies presented total direct medical cost of HB management. Median cost ranged from $722,792 (Armstrong et al. 2014 [10]), through $23,256 (Armstrong et al. 2014 [10]), to $51,814 (Chen CX et al. 2017 [3]), to $311,928 for severe/moderate and $181,070 for severe HB in the US were included.
- Variation in cost across studies could be due to disease severity (severe, moderate, and mild), treatment regimen (prophylaxis and on-demand), and other factors.
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**LIMITATIONS**

- Differences in the methodological approach in terms of cost aggregation (including differences in the way costs were defined by individual studies) restricted comparison of results across studies.
- Differences in patient clinical profiles may influence the required treatment regimen, therefore differences in clinical profiles between SHL/EHL groups could account for some of the differences in costs among included studies.
- Expected rise in the number of HB patients switching from SHL to EHL could generate substantial burden to the payer base for FIX prophylaxis.

**CONCLUSION**

- This systematic literature review found substantial economic burden associated with HB in the US. Mean annual clotting factor cost of HB management was found to range from $203,660 to $107,935 for a patient on SE, lowest cost from $49,400 to $102,654 for those on SHL.
- The significant costs and health resources utilized by patients highlighted onerous medical needs remaining in HB management.

**REFERENCES**

1. Blankenship et al. Change in factor cost s and units consumption by people with hemophilia A and B after switching from a standard half-life product to extended half-life product. In: NHF 2018, Orlando, US.
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