

Analysis of direct costs of treating severe hemophilia A in Brazil

Margareth C Ozelo^{1,2}, Gabriela G Yamaguti-Hayakawa^{1,2}, Teresa Raquel de M Ramos³, José Carlos Thomaz Jr. ³, Mohit Jain⁴

¹Hemocentro Unicamp, Universidade de Campinas (Unicamp), Campinas, SP, Brasil; ²Departamento de Medicina Interna, Faculdade de Ciências Médicas, Universidade de Campinas (Unicamp), Campinas, SP, Brasil; ³BioMarin Farmacêutica do Brasil LTDA, São Paulo, Brasil; ⁴BioMarin Pharmaceutical, London, UK

EE51

Introduction

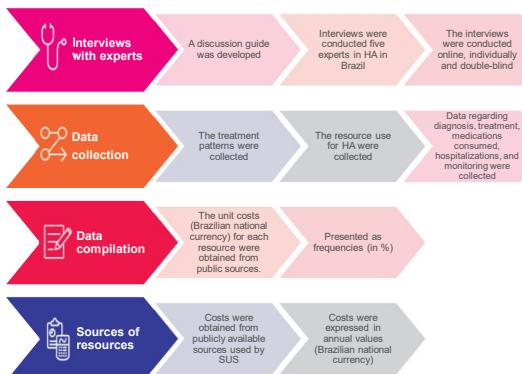
- In Brazil, hemophilia treatment centers hold and distribute all clotting factor concentrates nationwide, nonetheless the economic burden of hemophilia A (HA) management in the Public Healthcare System (Sistema Único de Saúde, SUS) needs to be further understood^{1,2}.
- The treatment of Hemophilia A is comprehensive and free at point of care in SUS, mainly through the replacement of the deficient coagulation factor, factor VIII (FVIII) concentrates. Currently, plasma-derived concentrates and standard half-life recombinant FVIII are available in Brazil^{3,4}.
- We aimed to estimate the direct costs related to the treatment of severe HA without inhibitors in SUS.

Methods

The opinion of a panel of experts

- A literature review was conducted to identify evidence on treatment patterns and local guidelines for people with severe hemophilia A (PwHA) for the construction of the discussion guide. The goal was to understand the natural history of the disease and its clinical characteristics, which allowed the establishment of the main parameters of cost items associated with HA management.
- This detailed costing approach involved measuring the use of health resources based on established clinical guidelines and supplemented by information collected via in-depth interviews with HA specialist physicians.
- A panel of experts was assembled to discuss the use of health resources and costs in the care and management of adults with severe HA without inhibitors. The selection of experts and the recruitment process were conducted blindly for both the study sponsor and the participating experts. Experts from all regions of the country were contacted, reflecting different levels of care and access to health in Brazil.
- Five interviews were conducted between June 2022 and May 2023, each lasting up to 2 hours.
 - The collected individual data were compiled, and data regarding diagnosis, treatment (consultations and exams), medications consumed, hospitalizations, and monitoring according to the public sources was presented as frequencies (in %).
 - Costs were obtained from publicly available sources used by SUS and were expressed in annual values (Brazilian national currency)^{5,6,7}.

Figure 1. Structure of the micro-costing



Results

- Data from 838 HA patients were analyzed
- The estimated direct annual cost of HA in PwHA was BRL 347,008.69 (USD 69,454.52*).
- Most of this cost (BRL 345,824.15 - 99.66%) was attributed to the consumption of hemophilia treatment (including consumption of FVIII, prophylactic and on demand use, and patient follow-up).
- The annual costs related to hospitalization, surgeries, and supplementary treatment were relatively low due to early diagnosis, improved access to treatment, and the emergence of new health technologies.

Figure 2. Profile of specialists and PwHA

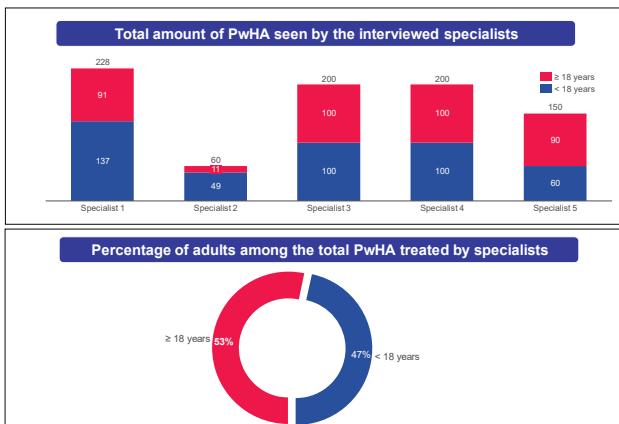
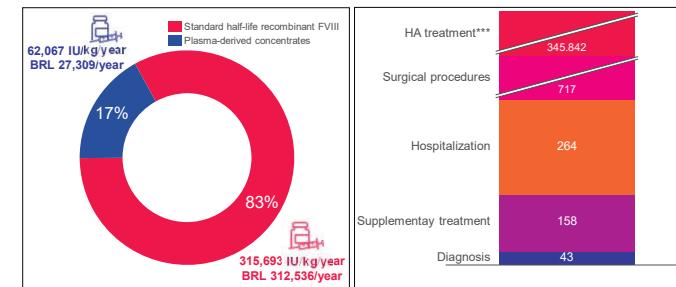


Figure 3. Coagulation FVIII consumption and weighted annual costs of treatment with PwHA**



**Sum of weighted average reported healthcare resource usage adjusted for individual costs in each category.

*** Price (in BRL) including consumption of FVIII, prophylactic and on demand use as well as and patient follow-up. Supplementary treatment cost was related to the use of tranexamic acid and blood transfusion.

Conclusions

- This study provided an annually estimated cost for an adult PwHA within the Brazilian public healthcare system context in BRL 347,008. Within this model of analysis, FVIII replacement therapy appeared as the main factor in the economic burden, as it represented 98% of the total cost.
- The FVIII replacement therapy in adult PwHA can be influenced by the level of treatment individualization and optimization, adherence to prophylaxis, regional disparities and other external factors, such as the pandemic COVID-19, which may have impacted the real-world data collection¹.
- With medical expertise development and efforts towards the uninterrupted implementation of the public policies regarding severe hemophilia A treatment in Brazil, the cost of treatment tends to increase. In this vein, it is critical to seek opportunities to optimize the resources, as exploring treatment alternatives that both substitute the need for continuous treatment and provide a better care for the PwHA by reducing bleeding rates and increasing the health-related quality of life.

References

- World Federation of Hemophilia. World Federation of Hemophilia Report on the Annual Global Survey 2022. 2023;(October).
- Boadas A, Ozelo MC, Solano M, Berges A, Ruiz-Saez A, Linares A, et al. Haemophilia care in Latin America: Assessment and perspectives. Haemophilia. 2018;24(6):e395-401.
- Ministério da Saúde (Brasil). Manual de Hemofilia. 2nd ed. Secretaria de Atenção à Saúde. Brasília; 2015. 1-82 p.
- Maglione CA da S, Pereira ACP, Fernandes RA, Sato-kuwabara Y, Loze PM. Statements about hemophilia A in Brazil: an expert Delphi panel. 2022;55(21):96-103.
- Brasil. Ministério da Saúde. SIGTAP - Sistema de Gerenciamento da Tabela de Procedimentos, Medicamentos e OPM do SUS. 6. Ministério da Saúde do Brasil. Sistema de Informações Hospitalares do SUS (SIH/SUS) [Internet]. 2023. 7. Ministério da Saúde do Brasil. BPS - Banco de Preços em Saúde [Internet]. 2023 [cited 2023 Oct 31]. Available from: <https://bps.saude.gov.br/login.jsf>

Acknowledgements

Funding for this study was provided by BioMarin Farmacêutica do Brasil, LTDA. Medical writing support was provided by IQVIA and funded by BioMarin Farmacêutica do Brasil, LTDA.

Authors would like to thank Maiara Araujo and Bruce Kano for support with the analysis. They were IQVIA employees at the time of the study.

Disclosures

Margareth C Ozelo has received consulting fees of Pfizer, research grant, travel support and speaker fees from BioMarin Pharmaceutical Inc, participated as clinical investigator trials sponsored by BioMarin, Pfizer, Novo Nordisk, Roche, Sanofi, Spark and Takeda. Gabriela G Yamaguti-Hayakawa has received consulting fees from Pfizer, Roche, Novo Nordisk and BioMarin, travel support from BioMarin and speaker fees from BioMarin, Pfizer, Novo Nordisk, Roche, Sanofi, Spark and Takeda. Teresa Raquel de M Ramos and José Carlos Thomaz Jr are employees of BioMarin Farmacêutica do Brasil LTDA and company's stockholders.

*The value in BRL was converted to USD based on the current exchange rate (04/01/2024) of the dollar according to Central Bank of Brazil (BACEN).