The economic impact of reducing bleeding with emicizumab in patients with hemophilia A and factor VIII inhibitors - Brazilian case

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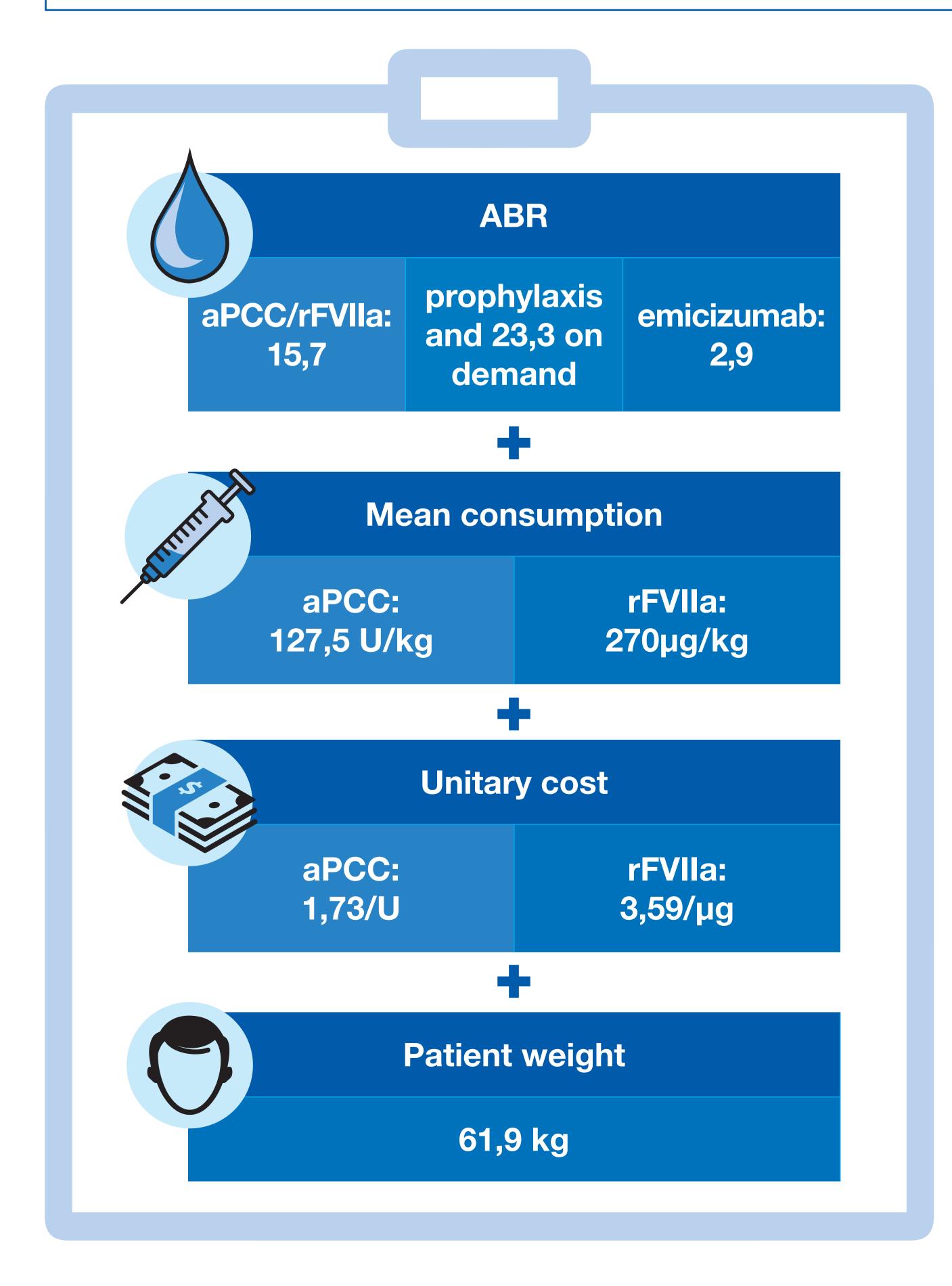
1. Techtrials Pesquisa e Tecnologia LTDA. 2. Produtos Roche Químicos e Farmacêuticos S/A

troduction

Hemophilia A is an X-linked hereditary bleeding disorder, caused by a deficiency in factor VIII (FVIII) clotting. Treatment aims at controlling bleeding through FVIII replacement, bypassagents, or emicizumab. FVIII treatments can lead to the development of FVIII inhibitors, a serious complication of the disease. This study aims to analyze the cost of treating bleeding, considering drug treatment from the perspective of the Brazilian public health system.

Methods

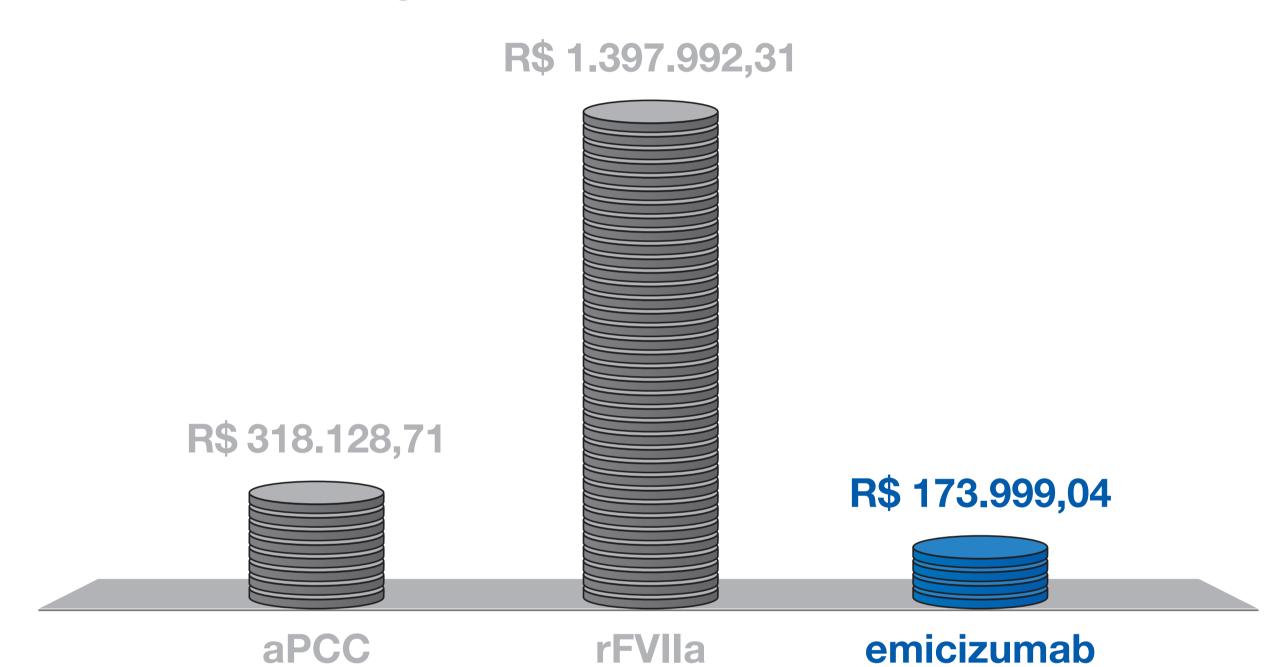
This study used annual bleeding rates (ABR), local guidelines regimens and prices.^{2, 3, 4, 5} We considered an average adult patient weighing 61,9kg. The ABR used, was: bypass agents 15,7 in prophylaxis and 23,3 on demand treatment and for 2,9 emicizumab. A mean consumption of 270 mgc/kg of recombinant activated factor VII (rFVIIa) or 127,5 U/kg of activated prothrombin complex concentrate (aPCC) was considered for the treatment of each bleeding. The unit cost of aPCC was R\$ 1,73/U and R\$ 3,59/µg of rFVIIa. The costs of immunotolerance treatment were not computed.



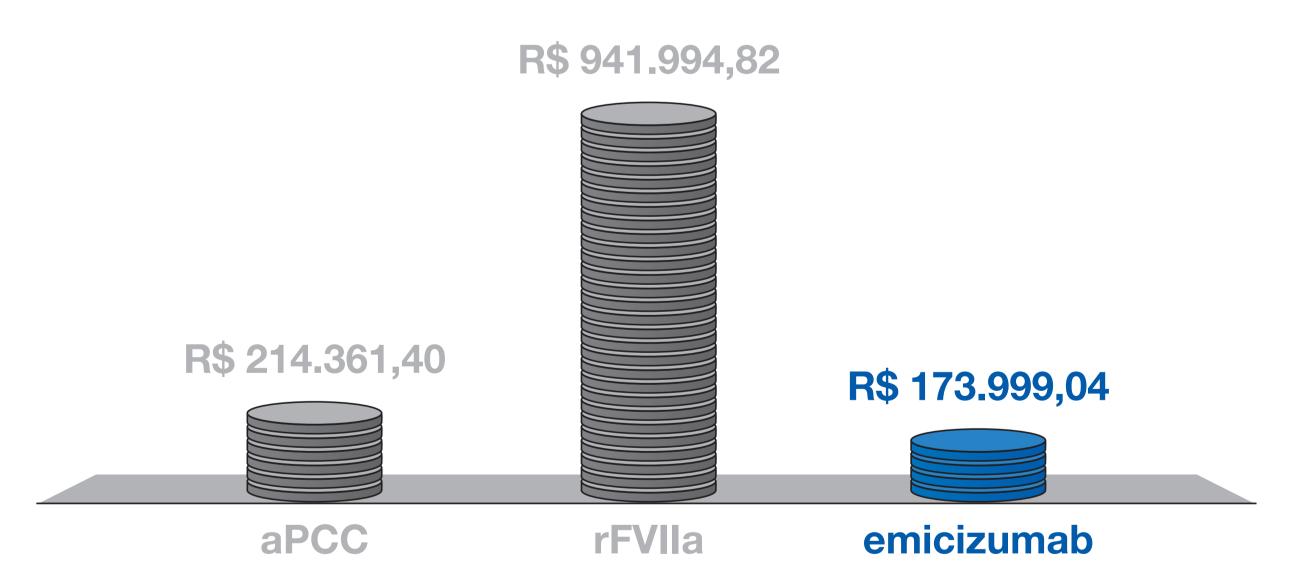
Results

The annual cost of prophylaxis drug treatment of bleeding in an average patient with Bypass was R\$214,361.40 with CCPa and R\$ 941,994.82 with rFVIIa. In patients treated with emicizumab, the cost was R\$ 173,999.04 with the use of rFVIIa to control bleedings. The cost is more than 5 times lower in patients treated with emicizumab, which represents an 81.5% reduction in the average annual cost of drug treatment for bleeding. In the on-demand scenario, the cost is about 8 times lower when emicizumab is used.

Bleeding treatment cost on demand



Bleeding treatment cost with prophylaxis



Conclusion

Emicizumab represents an opportunity in terms of effectiveness with a reduction of more than 80% in the number of bleeds/year, with savings thavvt can range from R\$174,765.98 (prophylactic aPCC) to R\$1,223,993.27 (rFVIIa on demand) for every average patient treated with this technology. These data suggest the opportunity to optimize the allocation of resources to technologies that offer greater sustainability for the public health care Brazilian system.

References: 1. Srivastava A, Santagostino E, Dougall A, Kitchen S, Sutherland M, Pipe SW, et al. WFH Guidelines for the Management of Hemophilia, 3rd edition. Haemophilia; 26 Suppl 6:1-158 (2020). 2. Instituto Brasileiro de Geografia e Estatística (IBGE). [Internet]. Available from: https://www.ibge.gov.br/estatisticas/sociais/populacao/9109-projecao-da-populacao.html?=&t=resultados. 3. OLDENBURG, J. et al. Emicizumab Prophylaxis in Hemophilia A with Inhibitors. N Engl J Med; 377:809-818 (2017). 4. MAHLANGU, J. et al. Emicizumab Prophylaxis in Patients Who Have Hemophilia A without Inhibitors N Engl J Med; 379:811-822 (2018). 5. Banco de Preços em Saúde (BPS). [Internet]. 2021. Available from: http://bps.saude.gov.br/visao/consultaPublica/relatorios/geral/index.jsf.