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ONLINE JOURNAL OF HEMATOLOGY & MEDICINE

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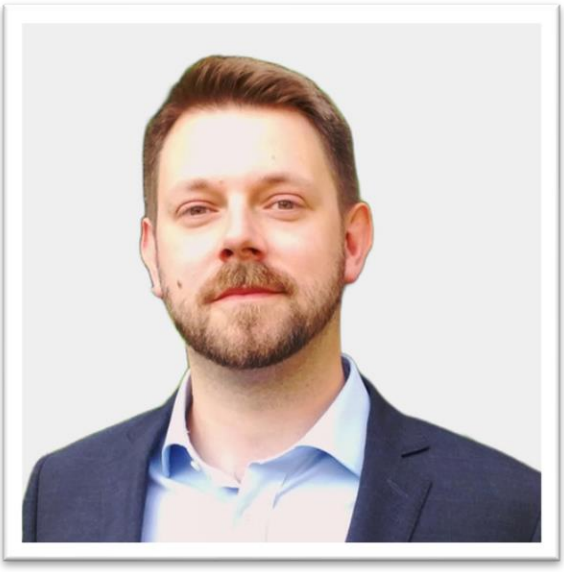
COST OF HAEMOPHILIA TREATMENT IN THE ERA OF NEW EXTENDED HALF-LIFE PRODUCTS AND NON-FACTOR REPLACEMENT THERAPY.

The cost associated with a clinical condition can be categorized into three main categories: direct, indirect, and intangible. Direct costs are items that are more closely related to the disease and its treatment (e.g. drug treatment, hospitalizations, surgery, physiotherapy), indirect costs that are related to productivity variation due to the disease and its management, and intangible costs that refer to the consequences of a disease and of a medical treatment on individuals' quality of Life. Haemophilia is a condition with significant burdens and costs associated with all these three categories; however, the focus has always been on the direct costs associated with the drug treatment.



The average cost of prophylaxis treatment of haemophilia patients has ranged from €180,000 to more than €800,000 based on the presence and absence of inhibitors and on patient's characteristics. Recently the availability of new treatments has had a significant impact on clinical outcomes and patients' quality of life but also on the associated costs. The first non-factor replacement therapy approved for haemophilia A with inhibitor has been associated with a significant cost reduction of these patients due to the lower cost of this treatment compared to the bypassing agents. Furthermore, the availability of different ended half-life products has also reported a potential reduction of FXII and FIX IU consumption with overall lower costs also due to the lower price per IU associated with these new products. The availability of more efficient and effective treatment has created a situation of a potential improvement of haemophilia management and patients' quality of life with no impact on overall budget or even a budget reduction. Specific real life data are needed to assess if this potential impact will be realized. A better understanding of the value created could be reached assessing changes in costs and the associated clinical variations and outcomes reported by patients.