Cost-Effectiveness Analysis of Turoctocog Alfa Pegol in Patients With Hemophilia A without Inhibitors in Colombia

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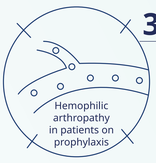


34.85%

Hemophilia severity

81.07% Severe
15.82% Moderate
3.11% Mild

Inhibitor Test[1]
72.37%

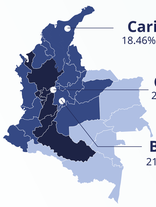
Colombian Context

With Inhibitors **5.97%**
Without Inhibitors **94.03%**

Distribution of patients with Hemophilia[1]

Prevalent cases of Hemophilia A according to region of Colombia[1]

2,953 patients with hemophilia in Colombia[1]



Hemophilia severity

688 **28.42%** Mild
455 **18.79%** Moderate
1.278 **52.79%** Severe

HemB *18.02% (532)* > **HemA** *81.98% (2.421)*

Context

Hemophilia is an **orphan disease**, X-linked genetic disorder that is linked to congenital bleeding disorders. The most prevalent form of this condition is hemophilia A, which is caused by a **deficiency in coagulation factor VIII (FVIII).** This disorder has a **significant economic impact** on healthcare payers, patients, caregivers, and society at large. The most severe complications of hemophilia include the development of inhibitors, arthropathies, and bleeding episodes, which can all have serious consequences for those affected[1]

Objective

To assess the cost-utility of turoctocog alfa pegol for the prophylactic treatment of severe hemophilia A patients without inhibitors in the Colombian health system.

Methods

Analysis model

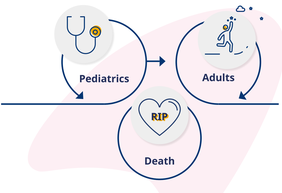


Figure 1. Markov model: Age estratification.[2]

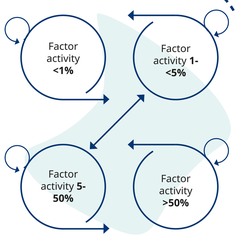


Figure 2. Markov model: health states within each stratum.[2]

A **Pharmacokinetic (PK) model** to predict FVIII levels for all treatments was adapted.

Patients can transit through four **health states** according to their factor VIII activity levels and, also, through three additional states depending on age stratification.

Cycle length was 28 days, and the time horizon was 70 years.

Deterministic and probabilistic sensitivity analyses were implemented.

PICO analysis

Population	Intervention	Comparators	Outcomes
Patients with hemophilia A **without inhibitors**, **pre-treated with prophylaxis.** Paediatric population (2 to 12 years) Adolescent/adult population (>12 years)	Turoctocog alfa pegol	**EHL:** Ruioctocog alfa pegol, Damactocog alfa pegol **SHL:** Octocog alfa[7], Simoctocog alfa, Moroctocog alfa, Turoctocog alfa, octocog alfa[18]	Light, moderate, or severe **bleeding** (life-threatening bleeding, gastrointestinal bleeding, intracranial, intra-abdominal, or intrathoracic bleeding, fractures) Quality-Adjusted Life Years (**QALYs**)[4,5]

Sources of information

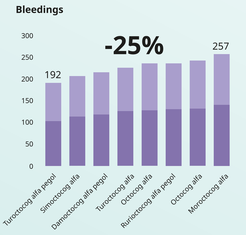
Data base	What is it?	Information extracted
INVIMA	The National Institute for Food and Drug Surveillance health authority entity	Dose Annual Bleeding Rate (ABR) [6-18]
ENSIN	Epidemological information from Colombia	Weight
FDA	Food & Drug Administration	Dose Annual Bleeding Rate (ABR) [6-18]
SISMED	Medicine prices information system	Institutional prices
ISS	Social Security Institute	Service rates (2001) + 30%
SOAT	Mandatory Traffic Accident Insurance	Service rates (2023)
WFH	World Federation of Hemofilia	Treatment protocol
SHL	Standart half-life	Molecules available in the colombian market: clinical information including dosage, treatment schemes based on label of local regulatory agency (INVIMA)
EHL	Extended half-life	

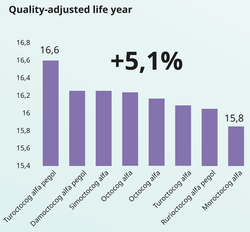
Results

On average, over 70 years, the total cost associated with hemophilia management **ranges from $901,829 USD with Moroctocog alfa to $1,538,066 USD with Octocog alfa.**

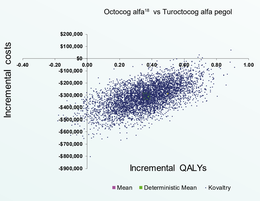
Turoctocog alfa pegol was a dominated alternative in comparation of Ruirioctocog alfa pegol, Octocog alfa[7], Damactocog alfa pegol, Octocog alfa.[18]

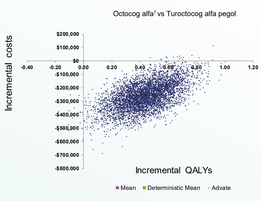
Bleedings rate and QALY comparison

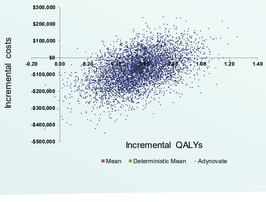


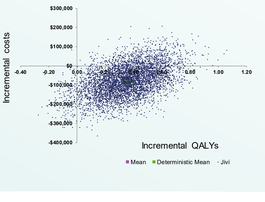


Probabilistic sensitivity analysis









Recommendations

Consider additional pharmacoeconomic studies that use local data as annual bleeding rates, dosage, and other health outcomes that are key drivers in the payer decision.

To address **sustainability concerns** within the healthcare system, it is crucial to adopt new therapeutic options that generate economic efficiencies and have a positive impact on patients health indicators.

Conclusion

Given the assumptions of the study, the use of turoctocog alfa pegol for hemophilia A patients without inhibitors in Colombia results in:


Lower treatment costs of spontaneous bleeds

+


Fewer total bleeds

+


More QALYs

=

The highest effectiveness was reported by Turoctocog alfa pegol with 16.60 QALY and costs of **$1,226,835.61** USD.

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