Modelling the Cost of Equivalent trough Level across Gene Therapy and Factor IX Replacement Therapy in Haemophilia B

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Background: Haemophilia is a genetic disorder caused by the deficiency of the body to produce FVIII (haemophilia A) or FIX (haemophilia B) clotting factors. Current standard of care in haemophilia is regular infusions with factor replacement therapy, with novel treatments seeking to minimise the administration burden of treatment while providing greater protection against bleeding episodes and joint damage.

Aims: The aim of this analysis is to quantify the consumption and cost of factor IX replacement therapy required to achieve a trough level equivalent to the hypothetical steady-state expression of a gene therapy over various alternative scenarios.

Methods: Time to FIX trough level (IU/dL) equivalent to hypothetical gene therapy expression was calculated based on published clinical trial outputs, assuming 33.8 hours for standard half-life (SHL) treatment and 82.1 hours for extended half-life (EHL) products. Gene therapy expression was modelled in 30, 50 and 100 IU/dL scenarios. The dose frequency required to maintain each trough level was modelled in order derive annualised factor consumption (Figure 1). Cost of achieving an equivalent trough level was calculated using cost per IU in three markets (Germany, Canada and the USA) and annualised. The unit costs for each market were €1.27, CAN$0.90 and USD$1.64 for SHL and €1.41, CAN$1.85 and US$3.14 for EHL.

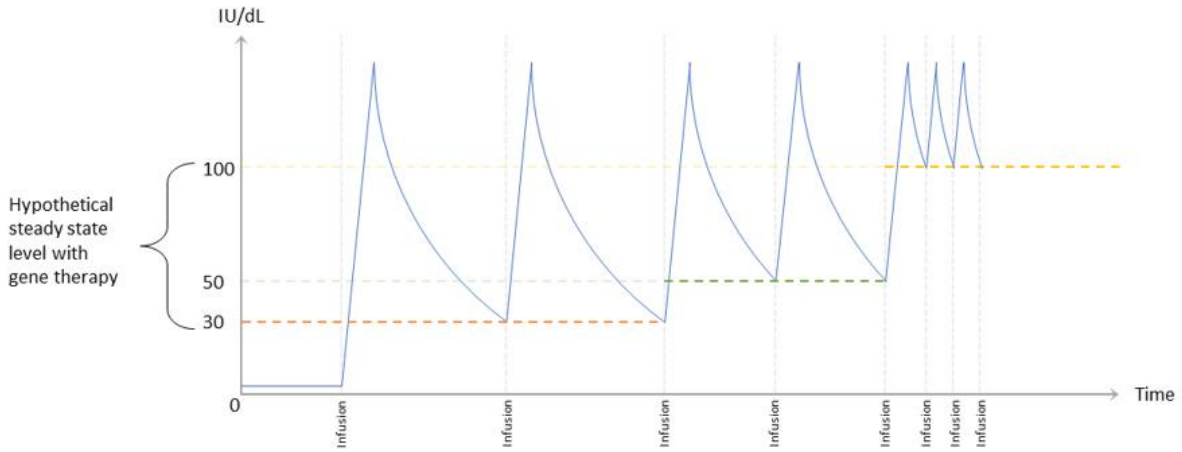
Results: The annual cost of factor product to maintain a FIX trough level equivalent to the hypothesised gene therapy expression scenarios varied from €907,370 to €15,648,929 in Germany, CA$1,029,837 to CA$11,089,792 in Canada and from $1,747,605 to $20,187,119 in the USA (Table 1). This was across assumed expression of 30, 50 and 100IU/dL.

Conclusions: This analysis demonstrates the magnitude of cost required to achieve equivalent trough level across factor replacement therapy and a hypothetical steady-state gene therapy in haemophilia B patients in Germany, Canada and the USA.

	SHL Yearly Cost			**EHL Yearly Cost**		
Country	30 (IU/dL)	50 (IU/dL)	100 (IU/dL)	30 (IU/dL)	50 (IU/dL)	100 (IU/dL)
Germany (€)	1,974,807	3,430,172	15,648,929	907,370	1,576,070	7,190,254

Canada (CA$)	1,399,470	2,430,831	11,089,792	1,029,837	1,788,791	8,160,717
US (US$)	2,547,501	4,424,922	20,187,119	1,747,605	3,035,530	13,848,517

[Table 1: Annualised cost across three markets, Canada, Germany and the USA]



[Figure 1: Infusion frequency diagram]

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