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Long-term outcomes in haemophilia: The impact of the Pettersson score on cost-effectiveness modelling

Harrop, D., Kwarciak, A., Turner, P., Maruszczak, M.
MAP Patient Access, Cambridge, UK

Objectives

The Pettersson score (PS) is a radiological scoring system used to assess the severity of joint damage in individuals with hemophilia resulting from repeated joint bleeding episodes.[1] The PS has been used in several cost-effectiveness models (CEMs) and in published literature to capture the impact of long-term joint health in hemophilia patients. The purpose of this research is to assess the impact of the PS on long-term cost-effectiveness outcomes in hemophilia.

Methods

A landscaping review was conducted to assess the methods used in CEMs examining technologies in hemophilia. Seven CEMs met the criteria, three of which involved the PS. A de novo Markov model was then developed, informed by the approaches taken in the identified models, to estimate the cost and utility implications of the PS. The CEM used a bleed model encompassing five primary health states (no-bleeds, non-joint bleeds, joint bleeds, surgery, and death). Transition probabilities for bleeding events were calculated from annualized bleed rates (ABRs). An arthropathy sub-model, quantified by the PS and the number of accumulated joint bleeds, was included to capture long-term joint health. Patients began the model aged 12 and received treatment with hypothetical technologies (factor and mimetic prophylaxis, as well as on-demand therapy) using bleeding profiles informed by published results of existing technologies. The arthropathy health states captured quality of life impacts and monetary costs associated with deteriorating joint-health. The impact of the inclusion of the PS on model outcomes was assessed by estimating cost-effectiveness with and without the inclusion of the arthropathy sub-model.

Results

The inclusion of the PS was shown to have a limited impact on the long-term cost-effectiveness outcomes in hemophilia, with changes in the incremental net monetary benefit (INMB) of between 1-4% (Figure 2) for the comparison between factor mimetics and factor treatments (for INMB at a willingness to pay threshold of £30,000). Incremental costs and QALYs were estimated to be negligibly affected by the inclusion of the PS, with on-demand treatments showing the largest difference (Table 2). This is due to prophylactic treatments providing an effective preventative control for bleeds, with factor mimetics reducing the bleed rate sufficiently, so that patients would require 457-years in the model before requiring surgery.

Conclusion

While the PS is a useful clinical measure, we have shown it to have a limited impact on cost-effectiveness results. Prophylactic treatments control hemophilia sufficiently to limit long-term joint health problems, with any potential long-term effects on joint health being significantly diminished due to the impact of discounting in the model. The inclusion of the PS within the model can significantly increase complexity and uncertainty within the CEM, therefore its inclusion should be considered carefully.

Figure 1: Model Structure

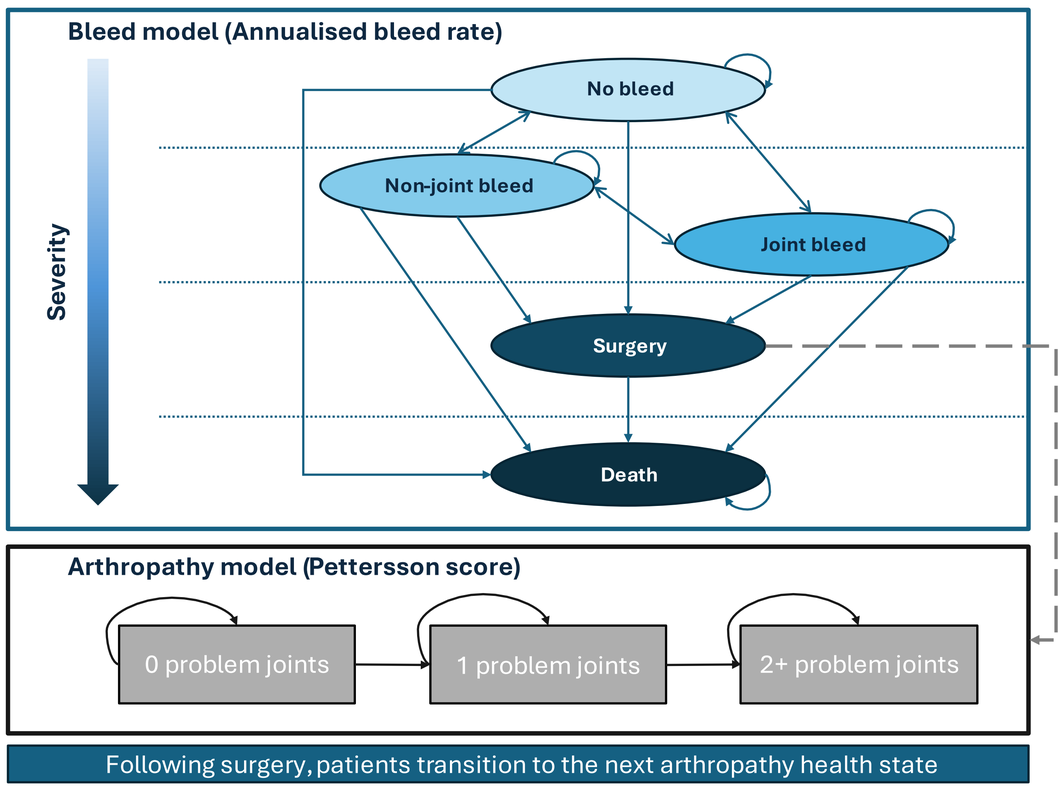


Table 1: Inputs: Annualized Bleed Rates by Technology and Pettersson Score Bleed Requirements

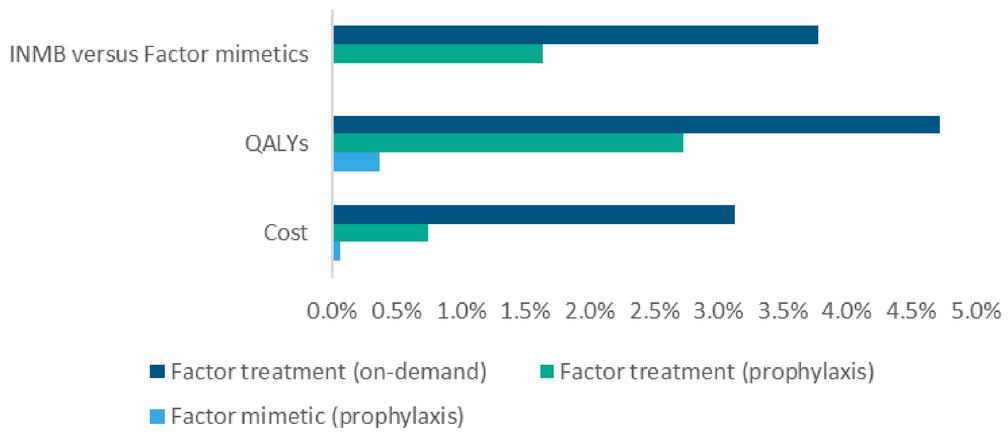
Technology	Factor mimetic (prophylaxis)	Factor treatment (prophylaxis)	Factor treatment (on-demand)
ABR	**1.15**	**8.02**	**38.20**
ANJBR	0.89	6.20	29.52
AJBR	0.26	1.82	8.68
Number of joint bleeds to increase PS			12.6[2]
Increase in PS required to trigger surgery			28.0[3]

Abbreviations: ABR, Annualized bleed rate; AJBR, Annualized joint bleed rates; ANJBR, Annualized non-joint bleed rate; PS, Pettersson score

Table 2: Results: Bleeds and Required Surgeries by Technology

Technology	Factor mimetic (prophylaxis)	Factor treatment (prophylaxis)	Factor treatment (on-demand)
Total Bleeds	**75**	**506**	**2,023**
Non-joint Bleeds	17	119	530
Joint Bleeds	58	387	1,493
Total Surgeries	**0.15**	**1.01**	**4.82**

Figure 2: Percentage Differences in Cost-effectiveness Outcomes by Technology



Abbreviations: INMB, Incremental net monetary benefit; QALY, quality-adjusted life year

References:
1. Pettersson H, Ahlberg A, Nilsson IM. A radiologic classification of hemophilic arthropathy. Clin Orthop Relat Res. 1980(149):153-9.
2. Fischer K, van Hout BA, van der Bom JG, Grobbee DE, van den Berg HM. Association between joint bleeds and Pettersson scores in severe haemophilia. Acta radiologica (Stockholm, Sweden : 1987). 2002;43(5):528-32.
3. Earnshaw SR, Graham CN, McDade CL, Spears JB, Kessler CM. Factor VIII alloantibody inhibitors: cost analysis of immune tolerance induction vs. prophylaxis and on-demand with bypass treatment. Haemophilia : the official journal of the World Federation of Hemophilia. 2015;21(3):310-9