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The impact of burosumab on the clinical and economic burden of X-linked hypophosphatemia (XLH) in adult patients in France

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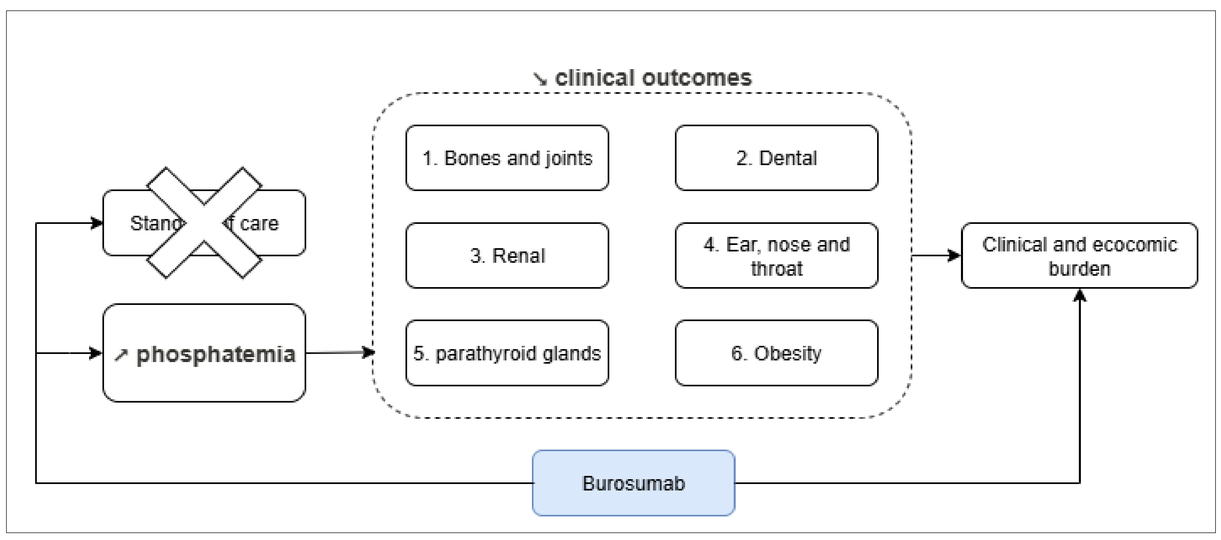
Objectives

X-linked hypophosphataemia (XLH) is a rare metabolic bone disorder characterized by increased synthesis of fibroblast growth factor 23 (FGF-23), which results in renal phosphate wasting with numerous manifestations affecting significantly quality of life (QoL) and physical capabilities. The management of XLH has evolved in the past years owing to the availability of burosumab a humanized anti-FGF-23 monoclonal antibody. This study aimed to evaluate the socio-economic impacts and cost-effectiveness of treating XLH adult patients with burosumab in France.

Method

A cost-utility model (*Figure 1*) was developed to estimate the clinical, including quality-adjusted life years (QALY), and cost burden of XLH over patients' lifetime. The model incorporated data from clinical trials, published studies, and real-world evidence to compare burosumab with oral phosphate supplements and active vitamin D treatment (standard of care), using cost inputs from the French National Health Insurance. Clinical outcomes included renal, dental, and bone complications, and their associated consequences. Costs covered both direct healthcare expenditures and indirect costs, such as work productivity loss.

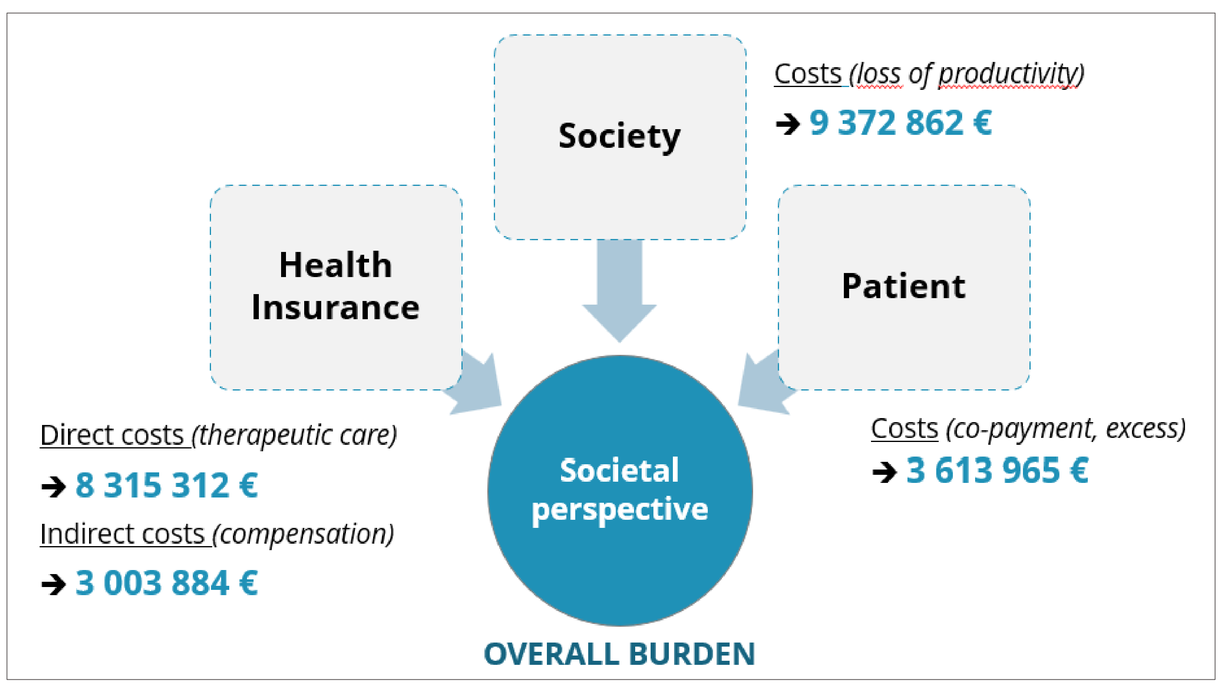
Figure 1. Model diagram



Results

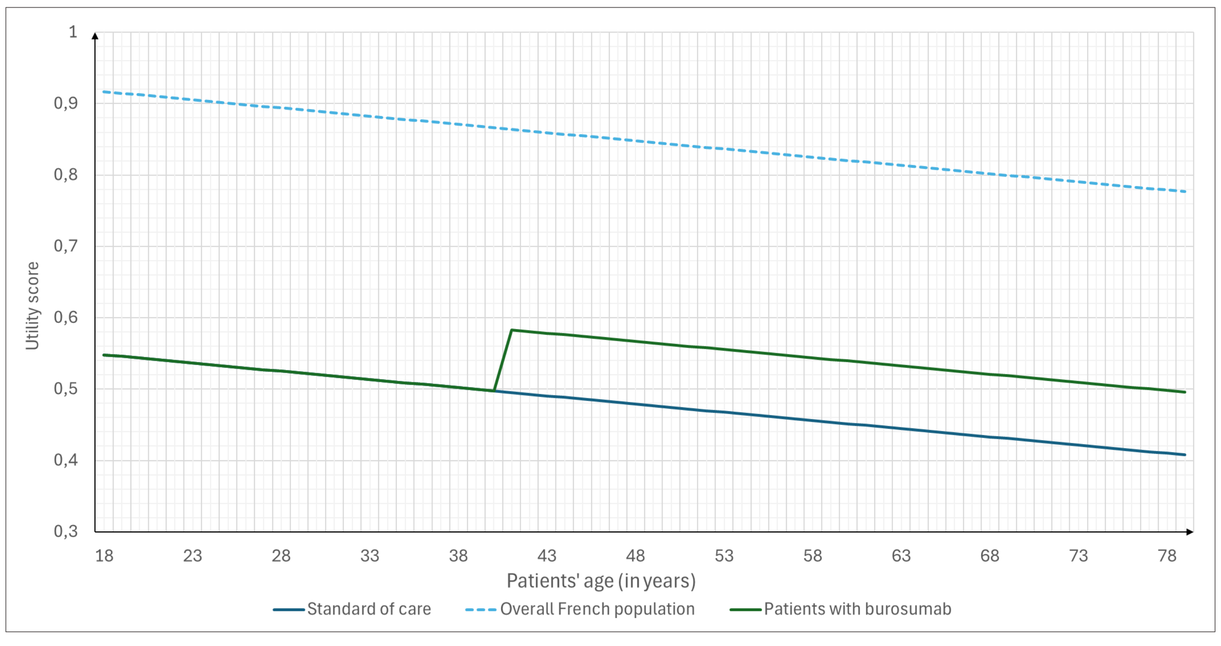
For the adult XLH population in France (1,900 patients), the annual economic burden would be estimated at €24.3 million (*Figure 2*), driven by 8 renal, 42 bone, and 446 dental complications.

Figure 2. XLH overall economic burden



The impact of the disease on quality of life is significant: the difference between the two curves (*blue dotted curve and full blue curve*) represents an average loss of 22.9 QALYs (*Figure 3*). Starting treatment at the age of 40 could lead to an increase of 3.4 QALYs, representing a 15% higher quality of life compared to individuals who do not receive treatment. Moreover, the earlier patients are treated, the greater the improvement in their quality of life.

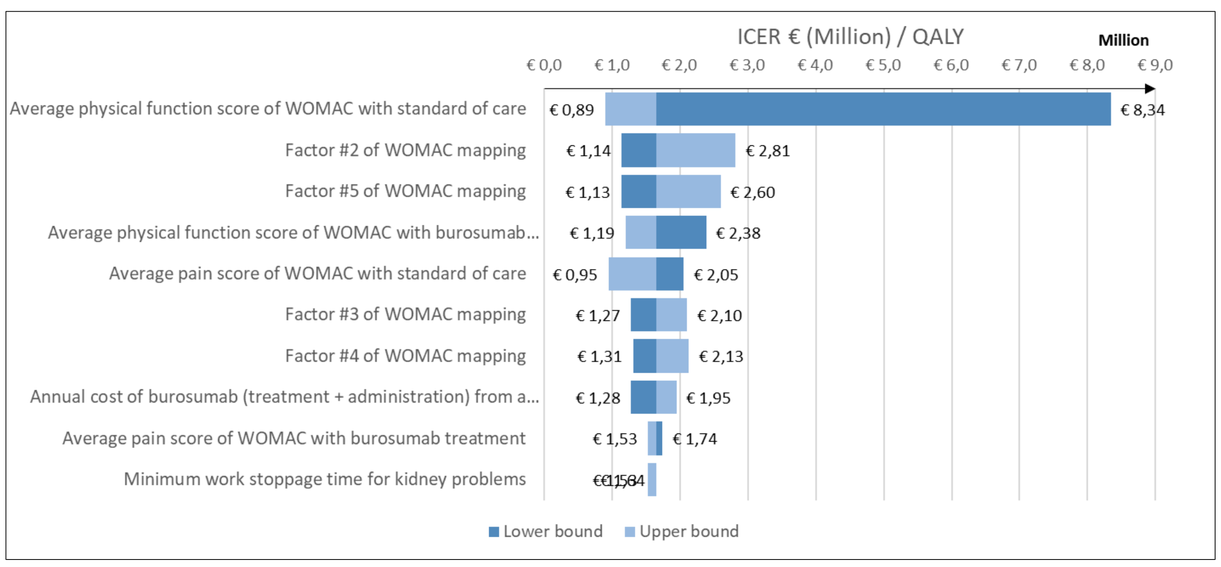
Figure 3. Comparison of QoL between patients in the general population and patients with XLH treated at the age of 40.



Among burosumab-eligible patients (950 individuals), burosumab could reduce XLH-related complications by 49%, preventing 146 acute complications annually and generating €1.2 million in cost savings from reduced complications.

Uncertainty was examined using deterministic analysis (*Figure 4*). The parameters with the greatest impact on the results include the physical score of WOMAC for both standard of care and burosumab, factors 2, 3, 4, and 5 of the WOMAC mapping, as well as the average pain score of WOMAC for standard of care.

Figure 4. Deterministic sensitivity analysis on the 10 most influential parameters



Conclusion

Burosumab could reduce XLH-related complications, improve QoL, and prevent acute events among eligible patients, demonstrating clinical and socio-economic benefits for adult XLH patients in France. It would appear to be a cost-effective option, aligning with the latest published ICER benchmarks for rare diseases in France.

References

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