ESTIMATING THE BENEFITS OF REDUCED FRACTURE INCIDENCE FOR PATIENTS LIVING WITH OSTEOGENESIS IMPERFECTA AND FOR HEALTHCARE PAYERS

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Background & objectives

Osteogenesis imperfecta (OI) is characterized by frequent fractures caused by the malformation of type I collagen. Fractures cause severe chronic pain and can result in loss of mobility and skeletal deformities, negatively impacting patients' health-related quality-of-life (HRQOL).[1] Managing the acute and chronic impact of frequent fractures also imposes a significant cost on healthcare payers. The objective of this study was to estimate the benefits to patients and healthcare payers of reduced fracture incidence in OI.

Methods

A *de novo* lifetime stochastic discrete event simulation model developed in Microsoft Excel was used to estimate health outcomes and healthcare resource utilization (HCRU) in people with OI. Model cycles were defined by time to next fracture, determined by annual fracture frequency. Each fracture event has a possibility to impact patient mobility and the presence of thoracic deformities in the individual patient.

Outcomes

Patient's mobility status and the presence of thoracic deformities were recorded and continually updated as patients progressed through the model. Fracture events increased the likelihood of reduced mobility and increased risk of thoracic deformity.[1] These cumulative characteristics impacted components of patient's health outcomes; loss of mobility can lead to reduced HRQOL and heightened risk of mortality. Similarly, thoracic deformities were also associated with reduced HRQOL and increased mortality risk. [2,3]

Assessing impact of reducing fracture frequency

The developed model was used to assess the impact of an assumed reduction in fracture frequency on both patient benefits accrued, and costs incurred by healthcare payers. The model captures the impact of direct improvements in HRQOL and reduced costs from reducing fracture rate, but also captures the additional benefits accrued from reducing the progression of associated disease characteristics.

The model was used to evaluate four cohorts of patients. The cohorts are differentiated solely by their annual fracture frequency[4], with one cohort reflecting the current natural history of OI based on published literature, and the others experiencing reductions in fracture frequency of 30%, 50% and 60%. Costs incurred and health state utilities were based on previously published estimates.

The model was used to evaluate health outcomes and costs incurred over a lifetime horizon.

Results

Patients with OI were estimated to have a life expectancy of 69.2 life years (LYs); this translated to total QALYs gained of 43.4 per patient (**Table 1**).

Figure 1 & 2 present the differences in simulated patient life years[5] and annual QALYs gained between the four cohorts. Patient survival is improved as a result of maintained mobility arising from reducing fracture rate, however patient HRQOL is also consistently improved by reduced fracture incidence, and consequently more QALYs are gained over a patient's life. Representing a utility value of 0.63 in the natural history population, versus 0.69, 0.73, and 0.77 for reductions of 30%, 50%, and 60% respectively. Reducing fracture incidence by an 30%, 50% and 60% improved patient outcomes, with estimated LYs increasing to 71.3, 71.7, and 72.1, respectively, or an improvement of 2.15, 2.55, and 2.92 versus the modelled natural history cohort. (**Figure 1**) A greater difference was observed in QALY gains, with reductions in fracture incidence resulting in QALY gains of 49.0, 52.5, and 55.2 when fracture incidence is reduced by 30%, 50%, and 60%. (**Figure 2**) Patients are estimated to lose mobility at a slower rate with reduced fracture rates with median time maintaining full mobility being 16 years in the modelled natural history, versus 24, 37, and 57 years when fracture incidence is reduced by 30%, 50%, and 60%. (**Figure 3**)

Fracture incidence in the natural history cohort resulted in estimated total lifetime costs of £46,860, with reduction in fracture incidence resulting in a saving of £12,000, £20,600, and £25,850 per patient, respectively.

Table 1: Estimated costs and health outcomes

	Simulated			Difference to Baseline		
	Life Years	QALYs	Costs	Life Years	QALYs	Costs
Natural History OI	69.16	43.39	£46,860			
30% Reduction	71.31	49.02	£34,834	2.15	5.63	-£12,026
50% Reduction	71.71	52.45	£26,265	2.55	9.06	-£20,595
60% Reduction	72.08	55.20	£21,010	2.92	11.81	-£25,850

Abbreviations: OI, osteogenesis imperfecta; QALY, quality-adjusted life year

Figure 1: Patient survival

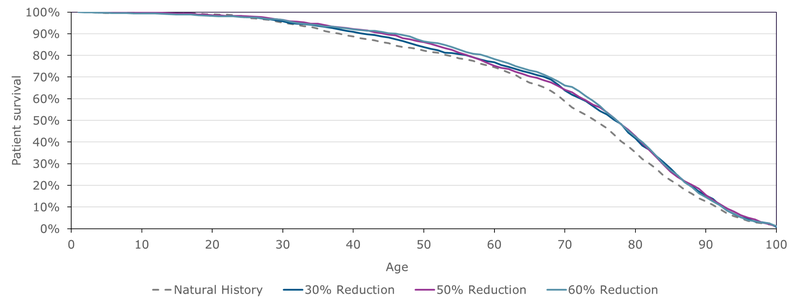


Figure 2: Patient QALYs

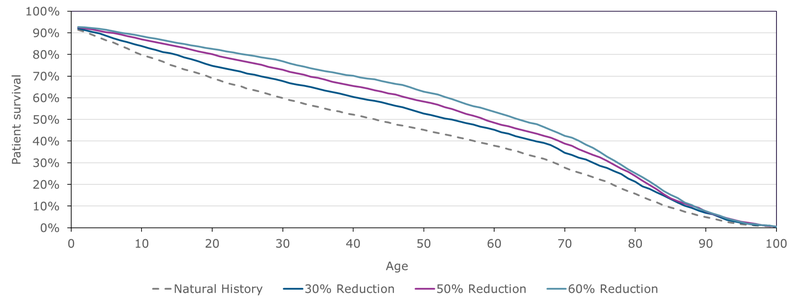
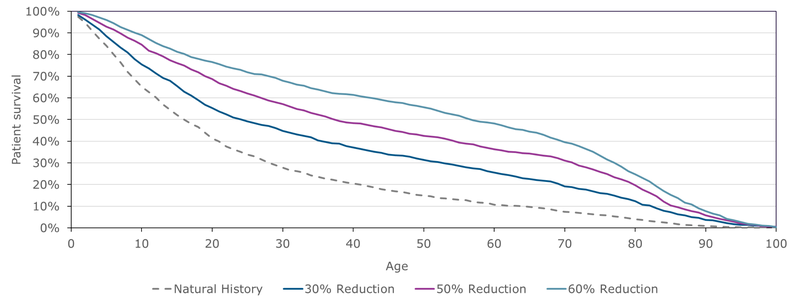


Figure 3: Patients maintaining full mobility



Conclusion

Fractures have a substantial impact on both people living with OI and payers. New interventions targeted at reducing the risk of fracture in OI have the potential to significantly reduce the burden of OI for both patients and healthcare payers.

References

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0056_SET_4-24_NPM. April 2024

Presented at ISPOR 2024, 5-8 May, Atlanta, GA, USA

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