ESTIMATING THE BURDEN OF OSTEOGENESIS IMPERFECTA ON PATIENTS AND HEALTHCARE PAYERS: A HEALTH ECONOMIC MODELLING STUDY

EE101

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Background & objectives

Osteogenesis imperfecta (OI) is a serious genetic disorder causing fragile bones and skeletal deformities, leading to frequent fractures, severe chronic pain, and poor health-related quality of life.[1] OI also imposes a significant burden on payers due to increased healthcare resource utilization (HCRU). The objective of this study was to characterize the burden associated with OI in terms of life expectancy, quality-adjusted life years (QALYs) gained, and costs to healthcare payers in comparison with general population outcomes from a UK payer perspective.

Method

Increased fracture frequency over general population

OI causes a significant increase in annual fracture frequency over the UK general population. A *de novo* stochastic discrete event simulation model was developed to assess the impact of this increased rate over the general population. The model captures the potential impact of each fracture event on patient mobility and the presence of thoracic deformities. These probabilities are relative to the type of fracture (site, severity, and required therapy) and patient specific characteristics (age, sex, disease type) which are modelled individually for each patient.

Outcomes

A patient's loss of mobility and thoracic deformities are impactful comorbidities that require grading so are recorded and continually updated as patients progress through the model. As highlighted above relevant fracture events increase the likelihood of worsening these comorbidities, for example a severe hip fracture would have a high probability of impacting mobility, or severe spinal fracture impacting thoracic deformity levels. These cumulative characteristics impact various components of a patient's outcomes. Loss of mobility can lead to reduced health-related quality-of-life (HRQOL) and heightened risk of mortality. Similarly, thoracic deformities can lead to cardiovascular and pulmonary issues, which are also associated with reduced HRQOL and heighted risk of mortality.[2,3]

Costs applicable to the management of OI accrued over a patient's lifetime were estimated to characterise the total HCRU requirements of OI. These costs included acute fracture treatment, surgeries, carer employment and treatment acquisition.

Comparison against UK population norms

Estimated LYs, QALYs, and costs associated with OI patients were compared with estimated outcomes for the UK general population. Costs unrelated to OI were not considered in economic modelling.

Results

People with OI were estimated to achieve a total of 69.4 LYs, varying from 37.8 to 77.8 years, conditional on OI Type (**Table 1**). This represents a 11.6-year reduction in life expectancy for patients with OI versus the UK general population. Reduced HRQOL translated to total lifetime QALYs of 43.6 per patient with OI, or a 24.0 reduction in QALYs gained versus general population estimates. Total lifetime costs associated with OI management were estimated at £47,110 per patient; based on an estimated prevalence of 1:15,000, this corresponds to an annual cost of £3.05M to the UK NHS.

Figure 1 presents the differences in survival for people living with OI[4] in comparison with the general UK population. OI is associated with reduced life expectancy in all disease types, most notably Type III. Conversely, Type I represents the least change in survival, which also coincides with the lowest fracture frequency of the assessed types.

Figure 2 presents the differences in annual fracture rates of the simulated cohort of people with OI versus the general population.[5] Rates were disaggregated by sex to compensate for differing trends in fracture rate, with fracture incidence in OI estimated to be significantly higher than the general population, as anticipated. The difference in fracture rate between OI patients and the general population was estimated to narrow over time, with the difference smallest in people aged over 75 years old.

Figure 1: Survival in OI and the general population

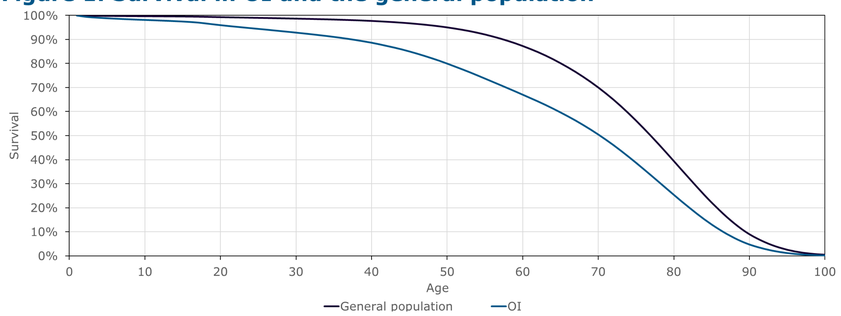
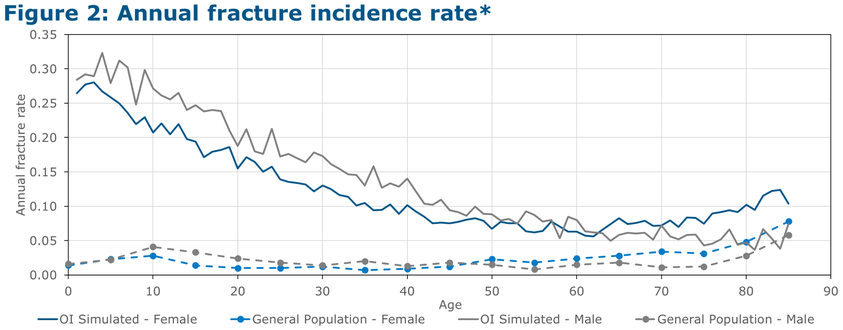


Figure 2: Annual fracture incidence rate*



The 'Annual fracture incidence rate' presented in **Figure 2 is calculated as the total number of fractures occurring within the population over a one-year period, divided by the total person-years of observation. This method yields the rate of fractures per person-year, providing insight into the frequency of fractures within the studied population.*

Figure 3 presents the differences in aggregate life years and cumulative QALY by disease type. This corresponds to an estimated mean utility of 0.66, 0.52, 0.58 for OI Types I, III, IV respectively. Overall, this represented a utility score of 0.63 for OI patients weighted by Type prevalence. This represents a significant reduction in estimated HRQOL versus the general population with an average utility score of 0.83.

Figure 3: Cumulative LYs and QALYs by OI Type

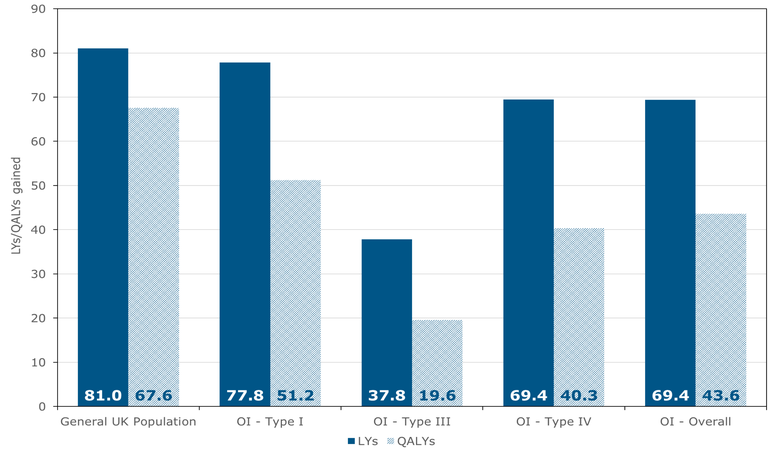


Table 1: Estimated costs and health outcomes

Population	Cumulative LY	Incremental LYs	Cumulative QALYs	Incremental QALYs	OI costs
General UK Population	81.0	-	67.6	-	-
OI - Type I	77.8	-3.2	51.2	-16.3	£36,600
OI - Type III	37.8	-43.2	19.6	-48.0	£76,570
OI - Type IV	69.4	-11.6	40.3	-27.3	£54,610
OI - Overall	69.4	-11.7	43.6	-24.0	£47,110

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

Conclusions

OI results in significant reductions in both LYs and QALYs in comparison with general population estimates. There is a significant unmet need for new treatments in this patient population that could help ameliorate the burden imposed by OI.

References

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