

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

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**Target conference:** ISPOR 2024 (Atlanta, Georgia, USA)

**Word count:** 297/300

### **Introduction**

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. 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.

### **Methods**

A *de novo* lifetime discrete event simulation model developed in Microsoft Excel was used to estimate health outcomes and HCRU in people with OI. Model cycles were defined by fracture incidence, which impacted patient mobility and the presence of thoracic deformities. Mortality was modelled using additional hazards applied to UK life tables. Fracture incidence, costs, and utility estimates were sourced from the published literature in OI and other proxy disease areas. Life years (LYs) and QALYs gained were compared with UK general population estimates.

### **Results**

People with OI were estimated to gain a total of 67.3 LYs, varying from 33.4 to 69.8 years, conditional on OI type. This represents a 19.1-year reduction in life expectancy for patients with OI versus the UK general population. Reduced quality-of-life translated to QALY gains of 28.7 per patient with OI, or a 38.9 reduction in QALYs gained versus general population estimates. Total lifetime costs associated with OI management were estimated at £130,554 per patient; based on an estimated prevalence of 1:15,000, this corresponds to an annual cost of £9.5M to the UK NHS.

### **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.