



# Key4OI - Development and implementation of a Standard set of Outcome measures for Osteogenesis Imperfecta

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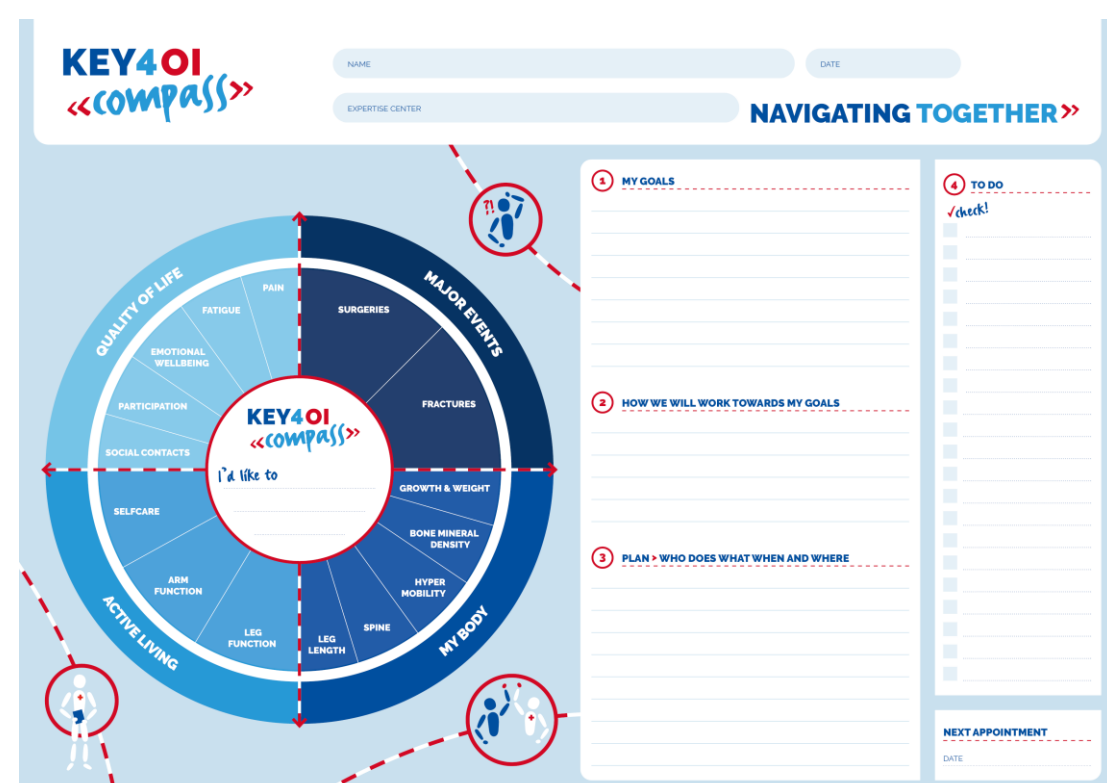
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## Background and aim:

Osteogenesis Imperfecta (OI) is a genetic disorder also known as 'brittle bone disease'. The clinical manifestation of OI shows a wide variation. Therefore, care for patients with OI requires an interdisciplinary approach. The effectiveness of particular interventions and treatment protocols of interdisciplinary teams is not clear due to a non-standardized and wide variation of patient outcomes thus making the comparison of outcome measures available in the literature difficult. It is only by agreeing on a common, standard set of outcome measures for the comprehensive appraisal of OI that comparisons across interdisciplinary treatment centers for OI will be possible in the future.



## Methods:

The Key4OI international interdisciplinary working group of 27 members used a consensus-driven modified Delphi approach to develop a set of global outcome measures for patients with OI. The International Classification of Functioning, Disability and Health (ICF), was used to define domains and organize the outcomes from the literature search.

After reviewing the outcomes extracted from the literature, trials and registries, the working group agreed on a final selection of domains and their definition (ICF definition as well as a lay description). These domains were then presented to the focus groups who prioritized the outcome domains by taking into account the items important to the OI community. All content was collected and analyzed and final domains were determined. A consensus of appropriate measuring instruments for each domain was reached with Delphi rounds. The entire approach was in line with the International Consortium for Health Outcomes Measurement ICHOM methodology

## Results:

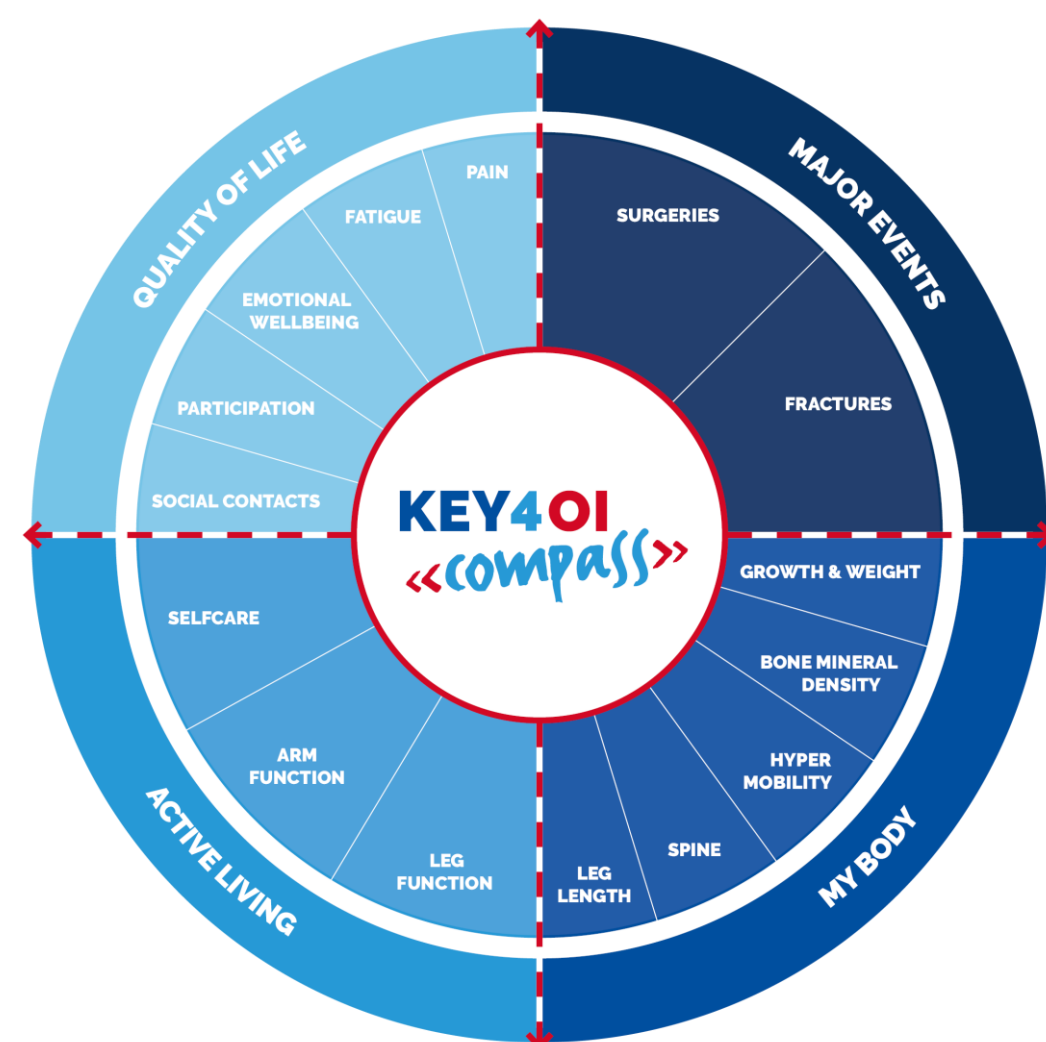
More than 400 different outcome measures were identified in our literature search. After three Delphi rounds, 24 domains were selected. After the focus group sessions, the number of domains were reduced to 15. A consensus was reached on the measuring instruments to cover these domains for both children and adults.

## Conclusion :

The Key4OI project resulted in a standard set of outcome measures focused on the needs and wishes of individuals with OI and their families. This outcome set will enable healthcare teams and systems to compare and to improve their care pathways and quality of care worldwide. Further studies are needed to evaluate the implementation of this standardized outcome set.

## Implementation and take home message:

- Implementation has started** in 5 different countries (6 hospitals): China, Norway, USA, Canada and the Netherlands (Isala Zwolle and UMC Utrecht). Various other countries will start implementation in 2022.
- The Key4OI Outcome set is helpful for **multidisciplinary teams in expert centers and the individual person with OI**, as it establishes a strong standard for what domains require attention in order to improve quality of life for people with OI. Over time it will lead to comparable data across locations, enabling **healthcare improvement and research for OI**.
- Key to success** (1) Involving some of the best **experts** worldwide (medical field and patient experts), (2) Collaborating with **patient organizations** every step of the way (3) Using **ICHOM processes and advice** to develop the outcome was critical to achieve this result efficiently and effectively.



## Participating Healthcare providers



## Participating Patient organisations

