

From Pain to Patient Preferences: Patient Engagement in Early HTA for Repurposing Losartan in Osteogenesis Imperfecta

Dalma Hosszú ^{1,2*}, Eve Hewitt ³, Zsuzsa Réka Pozsár ^{1,4,5}, Claudia Fuchs ⁶, Judith Cohen ⁷, Nick J Bishop ⁸, Donald C Lo ⁹, Zoltán Kaló ^{1,4,5}, Antal Tamás Zemplényi ^{1,10,11}

¹ Syreon Research Institute, Budapest, Hungary

² Institute of Psychology, University of Pécs, Pécs, Hungary

³ Beacon for Rare Diseases, Cambridge, United Kingdom

⁴ Center for Health Technology Assessment, Semmelweis University, Budapest, Hungary

⁵ Center for Pharmacology and Drug Research & Development, Semmelweis University, Budapest, Hungary

⁶ EURORDIS – Rare Diseases Europe, Paris, France

⁷ Faculty of Health Sciences, University of Hull, Hull, United Kingdom

⁸ School of Medicine and Population Health, The University of Sheffield, Sheffield, United Kingdom

⁹ European Infrastructure for Translational Medicine (EATRIS), Amsterdam, The Netherlands

¹⁰ Faculty of Pharmacy Center for Health Technology Assessment and Pharmacoeconomic Research, University of Pécs, Pécs, Hungary

¹¹ Center for Pharmaceutical Outcomes Research, University of Colorado Anschutz Medical Campus, Aurora, US



BACKGROUND

- Patient engagement is essential for aligning drug development with unmet needs, and meaningful outcomes, supporting access decisions and early identification of non-viable projects. REMEDI4ALL is an EU-funded initiative for drug repurposing, embedding early health technology assessment (eHTA) and patient engagement to guide losartan's development in osteogenesis imperfecta (OI). While fracture reduction is a key clinical outcome in trials and is targeted with treatments, patients indicated that their unmet needs were not fully represented by this outcome. This work aimed to define a disease-specific, patient-informed unmet need list for OI.
- An unmet patient need is a problem or challenge that people with OI still face because current treatments or support don't fully address what matters in daily life, which can have a serious impact on overall quality of life.

METHODS

- An in-person multi-stakeholder workshop organised by REMEDI4ALL - involving five patients (including one paediatric), one caregiver, one patient representative, one clinician, and one developer - in 2024 explored patient preferences of treatments in OI using an initial, non-disease specific element list.
- The first session laid the foundation for a workshop with 31 OI patients of varying ages and severity held at the Osteogenesis Imperfecta Federation Europe (OIFE) annual meeting in June 2025.
- After presenting collected elements of unmet need (shown in Table) from patient-informed, current OI literature (1.-4.), participating patients expressed their unmet needs in facilitated group discussions.
- Subsequently, written outputs were synthesised into domains and elements by the Health Economics and Outcomes Research (HEOR) team.

| Factor | Mild OI | Moderate OI | Severe OI | Children | Adolescents | Adults | Elderly |
|------------------------|----------------------------------|--|--|---------------------------|--------------------------|------------------------|------------------------|
| Pain | Chronic, but less severe | Persistent and debilitating | Persistent and debilitating | Emerging issue | Increasing | Major issue | High impact |
| Fatigue | Common | Severe | Severe | Moderate | High impact | High impact | High impact |
| Fracture Burden | Lower | High, frequent surgeries | Very high, frequent surgeries | Very high | High | Moderate | High |
| Mobility Needs | Walking aids, occasional support | Wheelchair use, rehabilitation needs | Wheelchair dependence, intensive rehab | Moderate | Increasing need | High impact | Severe |
| Mental Health Needs | Anxiety, hearing loss | Depression, social isolation | Depression, social isolation | Emerging | High concern | High concern | Aging-related anxiety |
| Employment Challenges | Some impact | High unemployment, workplace barriers | High unemployment, workplace barriers | – | – | Major concern | Retirement concerns |
| Dental & Hearing Needs | Hearing aids, mild dental issues | Severe malocclusion, dentinogenesis imperfecta | Severe malocclusion, dentinogenesis imperfecta | Early dental intervention | Orthodontic needs | Ongoing dental care | Hearing loss worsening |
| Financial Concerns | Moderate | High due to disability | High due to disability | – | – | High financial burden | Cost of caregiving |
| Specialized Care Needs | Moderate | High (surgery, ortho, rehab) | High (surgery, ortho, rehab) | Pediatric OI care | Transition to adult care | Multidisciplinary care | Assisted living |

Table: Literature-informed list and grouping of elements of unmet need in osteogenesis imperfecta

RESULTS

- Twenty distinct unmet need elements were identified and grouped into three domains: Therapeutic Gaps (4), Clinical Care Gaps (8), Supportive and Mental Health Care Gaps (8) (shown in Figure.)
- Chronic pain, fatigue have again emerged as the most reported unresolved burden in all severity and age groups.
- Mental health challenges were consistently mentioned across workshops, reflecting its impact on daily functioning and overall quality of life.
- These elements cover a broad range of patient-centric value drivers that go beyond conventional clinical endpoints.

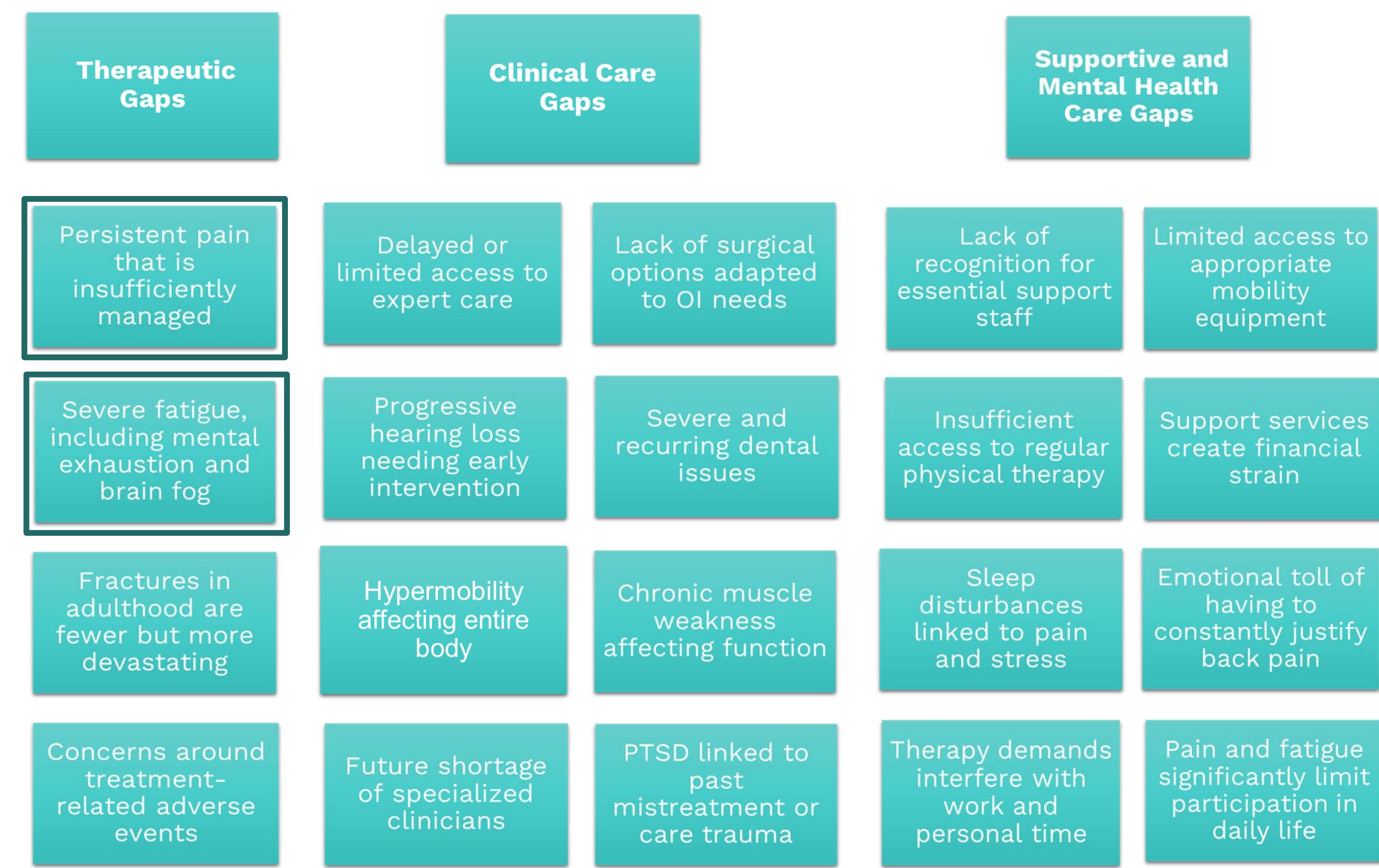


Figure: Unmet patient need elements in osteogenesis imperfecta

IMPLEMENTATION

The identified unmet needs directly informed how treatment benefits were communicated in patient-support materials, including revisions to the patient information leaflet to reflect priorities such as pain and fatigue.

This input contributed to a shift in the target product profile: while fracture reduction remained the central target for drug action, greater emphasis was placed on pain and fatigue reduction being leading therapeutic benefits. Incorporating patient perspectives early provided insights into enrolment barriers and communication needs, improved evidence generation strategies, and will support better alignment between value propositions and patient priorities.

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