PRESS RELEASE

First international Fibrodysplasia Ossificans Progressiva (FOP) Burden of Illness Survey reveals extent of social, economic, and quality of life impact of disease

Findings published in the Expert Review of Pharmacoeconomics & Outcomes Research demonstrate progressive loss of joint mobility and function is associated with a considerable, negative impact on quality of life for those living with FOP

Survey co-created by FOP community advisors in partnership with Ipsen

Paris, France. 5 September 2022 – Ipsen and the International FOP Association (IFOPA) today announced that *Expert Review of Pharmacoeconomics & Outcomes Research* has published the results of the first international Fibrodysplasia Ossificans Progressiva (FOP) Burden of Illness Survey[1] to investigate physical, quality of life (QoL), social and economic impacts of this ultra-rare disease on individuals and their family members. Recent research has enhanced knowledge on the clinical nature of FOP, but data on the multifaceted impacts are limited, despite being crucial for understanding the totality of the disease. This Burden of Illness survey was carried out in partnership with the IFOPA and Ipsen along with participation from national FOP organizations.

Results demonstrate that in FOP loss of joint mobility, largely due to the progressive and irreversible nature of heterotopic ossification (HO) or extra skeletal bone growth synonymous with the disease, is significantly associated with decreased physical functioning and ability to carry out activities of daily living (ADL). This, in turn, is associated with a severe, negative impact on QoL. There is a clear trend that mobility and joint function worsens over a patient's lifetime. As disability progressively increases, the way in which FOP is managed may change, with a greater focus on daily care and support, an increased reliance on assistance from family members, performing daily activities, and limitations in mobility restricting the ability to travel.

Results also suggest that loss of joint function leads to a negative financial impact. The accruing costs are underpinned by rising household expenses as the need for living adaptations, the likelihood of changes to career plans, and missed days of work for these individuals and their families increase.

"While research has increased our knowledge of the clinical aspects of FOP and the role of potential disease-modifying treatments, limited data exist on the holistic impact of FOP, including the impact on families and caregivers," said Steven Hildemann, Ipsen! Chief Medical Officer. *"*We are very grateful to our partner, IFOPA, and the other FOP patient organizations that informed the survey design and helped us identify survey participants, while also sharing invaluable insights that we hope to use to highlight what needs to be done to transform care for those living with FOP and their families."

"For the first time we have evidence-based data that clearly demonstrates how FOP impacts so many areas of everyday living, said Megan Olsen, IFOPA Board Chair, "This information is vital for identifying unmet needs, improving patient care, evaluating the benefits of new healthcare interventions, and improving support for the FOP community. We hope these data will help to increase awareness of this ultra-rare disease and further educate healthcare professionals and healthcare policymakers about the challenges of living with FOP."

About the Burden of Illness Survey
For patients with the most severe physical limitations (assessed at Level 4 using the patient-reported mobility assessment (PRMA*)), the mean EQ-5D-5L score was 0.05, which is close to the equivalent of mortality; in comparison, scores reported by people living with diabetes mellitus, cancer, multiple sclerosis, and cardiovascular disease are notably higher (ranging from 0.31 – 0.99)[2], showcasing the severe impact of FOP on patient's QoL.[1]

Progressing mobility limitations impede patients' ability to travel and patients with the most severe physical limitations (assessed at Level 4 using PRMA), half (50%) were unable to travel by plane and more than one-third (34.4%) unable to travel by car.[1] Nearly 60% of patients indicated financial costs limit or prevent their ability to travel entirely. Reduced or restricted travel could severely impact patients access to health outcomes, as many need to travel to centers of excellence to receive care.[1]

Results showed over 45% of primary caregivers experienced a mild to moderate impact on their health and psychological well-being.[1] Family members spent a mean average of eight hours each day helping to provide care or assistance to their relative with FOP. In fact, more than half of primary caregivers (51.3%) reported needing to adapt their careers to look after their relatives with FOP.[1]

In total, 219 patients and 244 family members from 15 countries completed the survey created by FOP community advisors and a team of researchers. The survey measured the impact of FOP on the physical functioning of patients, QoL of patients and family members, employment and ADLs, and utilization of healthcare services and living adaptations.[1]

The survey was sponsored by Ipsen. Available via an online platform in 11 languages, the survey was open to people of any age with FOP as well as their immediate family members. Age and gender demographics of patient participants were similar to those in the FOP Registry[3], in which more than one-third of all known individuals with FOP were enrolled (based on approximately 900 known individuals with FOP[4]).

About fibrodysplasia ossificans progressiva (FOP)
FOP is an ultra-rare genetic disorder with an estimated prevalence of 1.36 per million individuals.[5] The median age at time of FOP diagnosis is five years old.[6] It is characterized by HO,[7] which can be preceded by painful soft-tissue swellings or 'flare-ups'.[6] Flare-up episodes are a substantial contributor to the formation of new HO, which once formed is irreversible.[7] Disability is therefore cumulative and most people with FOP require a wheelchair by their third decade of life and require assistance to perform activities of daily living.[8,9] This loss of mobility, in addition to numerous life-limiting complications of FOP, leads to markedly shortened life expectancy.[7]

* *The PRMA scores each of 12 joints and three body regions as 0 (not limited at all), 1 (moderately limited), or 2 (extremely limited [cannot move at all]); total scores range from 0–30, with higher scores representing more severe limitations in mobility. PRMA total scores were categorized into four levels: Level 1, total score 0–6; Level 2, total score 7–12; Level 3, total score 13–18; and Level 4, total score ≥19.*

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