Hypophosphatasia (HPP) Patient and Caregiver Disease Burden, Quality of Life, and Treatment Experience: A Mixed Methods Study Concept

Authors:	Helmy R., Laramee P., Staub D., Seefried L, Padidela R.
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Contact:	Remon Helmy (remon.helmy@alexion.com)

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Abstract body word count: 281/300

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Authors (author's last name, followed by first initial) institution(s), city, state, country	**Helmy R.** Alexion Pharma GmbH, Baar, Zug, Switzerland. **Laramee P.** Alexion Pharma, Barcelona, Catalonia, Spain. **Staub D.** Alexion Pharma GmbH, Baar, Zug, Switzerland. **Seefried L.** Musculoskeletal Center Wuerzburg, University of Würzburg, Würzburg, Bavaria, Germany. **Padidela R.** Department of Paediatric Endocrinology, Royal Manchester Children's Hospital, Manchester, United Kingdom. Faculty of Biology, Medicine & Health, University of Manchester, Manchester, United Kingdom.
Abstract (do not indent; must include OBJECTIVES, METHODS, RESULTS, CONCLUSION unless a Conceptual Papers submission) **Abstract has a 300 maximum word count**	**OBJECTIVES**: Quantitatively and qualitatively describe disease burden and treatment experience of symptomatic hypophosphatasia (HPP) patients across various ages and disease burden levels, and the burden on their caregivers, for asfotase alfa-untreated or recently initiated [<6 months] patients, and for asfotase alfa-experienced [≥6 months] patients; and to measure quality of life among caregivers. **METHODS**: This is an observational, cross-sectional, mixed methods study designed to use quantitative measurement and qualitative interviews with patients with HPP and their caregivers. Study has 2 components: online survey of approximately 50 patients with HPP and 30-50 caregivers using validated measures; qualitative interview of approximately 20-30 patients with HPP and 10 caregivers. Interviews will be conducted using semistructured interview guides. Patients across a span of ages with a range of HPP manifestations, asfotase alfa treatment status, and country income levels (and corresponding healthcare systems) will be included. Patients and caregivers will be recruited from Canada, China, Colombia, France, Saudi Arabia, Turkey, and the United Kingdom. HPP patient advocacy groups in each country will be the primary recruitment resource. **RESULTS**: Quantitative analysis of survey data will present demographic, quality of life, physical, emotional and functional data of patients and caregivers, including health utility estimates for

	caregivers. Semi-structured qualitative interviews will add depth using thematic analysis of disease impact and treatment experience. **CONCLUSION:** To the best of our knowledge, this is the first mixed methods study on HPP patient disease burden and treatment experience, and the first study measuring HPP burden on caregivers and their quality of life. It is expected to contribute to better understanding of the wider disease impact and to help decision making with regards to resource allocation for treatments that reduce the burden of HPP patients and, correspondingly, their caregivers.