





Meeting outcomes: Gaps in knowledge and next steps



Guiding principles

- The importance of an ongoing collaborative approach
- Proceeding with caution for patient safety during move into human interventional studies
- Need for a pediatric focus during drug development
- Ensuring access to clinical trials and new developments internationally



Gaps: Disease knowledge

- Phenotypic heterogeneity is not explained
(e.g., head flare-ups / scalp-lump profile, flares that occur before diagnosis, major trauma that results in no flare-up)
- Understanding influence of genetics and epigenetics
- Understanding role of immunology
- Understanding neurological factors in HO



Gaps: Tools and models

- Better understanding the predictive value of our animal models
- Need a widely accessible mouse model
- Need animal models of FOP pathophysiology during periods of growth
- Need for biospecimens



Gaps: Interventions

- Understanding how potential therapeutics could combine at different stages of FOP/HO development
- Understanding the therapeutic potential of available drugs and/or getting negative results if already available
- Need for observational data on exercise within the registry



Next Steps

- Registry data on head flares
- Registry data on exercise
- Immunology workshop
- IFOPA to consider a mouse model RFP
- IFOPA to consider assessment of available drugs and/or getting negative results



Next Steps

- Genetics project?
- Improving awareness of presenting profiles amongst clinicians
- Improving FOP searchability using patient and clinician targeted terms online
- Strategy for working with pharma in presentations to regulatory bodies