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