

Project Research Status

VoT2 – RNDs

Working Groups



Ataxia: final
Dystonia: final
PKU: final

Care pathway analysis Economic evaluation (cost csq analysis)



Case studies overarching theme: role of multidisciplinary, coordinated care (specialist centres/care coordinators) and its impact on health service use and patient outcomes

Academic partners



Ataxia & PKU: UCLH, UCL/University of Cambridge, UCD
Dystonia: University of Zagreb Medical School

Funding: We have received sponsorship from Takeda, Reata (ataxia case study), Ipsen (dystonia case study), and Biomarin (phenylketonuria case study).