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CHANGE IN PATIENT-REPORTED OUTCOMES OF XLH REGISTRY PARTICIPANTS IN THE UK: A LONGITUDINAL STUDY

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Objectives

To characterise the change over time in pain, sleep, fatigue, anxiety, depression and health-related quality of life (HRQoL) for adults with X-linked hypophosphataemia (XLH).

Methods

Data were collected from participants diagnosed with XLH and registered in RUDY, a cohort of individuals with rare diseases in the UK. Seven instruments were analysed: EQ5D-5L and SF36 physical component score (PCS) and mental component score (MCS) as measures of HRQoL, PSQI of sleep quality, PainDETECT and SF-MPQ-2 of pain severity, FACIT-F of fatigue, and HADS measuring depression and anxiety. Participants were invited to submit questionnaires every six months and we used data reported between July 2014 and August 2019 for the analysis. Change in mean scores over four time-points (up to two years) was estimated using mixed-effects linear regression models controlling for gender, age (at registry in RUDY) and time of follow-up.

Results

The sample included 48 RUDY participants with XLH, mostly female (77%) and with a median age of 46 years (range 19-85). SF-MPQ-2 and PSQI showed a slight improvement in mean scores whilst all other instruments reported fluctuating means. Mixed effects models revealed statistically significant ($p<0.05$) time coefficients only for FACIT-F (b=-2.135, p=0.038) and HADS-Anxiety (b=0.314, p=0.031), both reporting slightly worsening scores over time. Within FACIT-F, the functional well-being subscale was found to be the clear driver of change (b=-0.754, p=0.003).

Conclusions

People with XLH report signals of deterioration in fatigue and anxiety over two years which could impact them significantly if sustained over longer periods of time. We did not find evidence of change in all other instruments, but individual-level trends suggest more research is needed to identify subgroups more likely to worsen as well as which patient characteristics may predict deterioration.

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