**Effects of Burosumab (KRN23), a Fully Human Anti-FGF23 Monoclonal Antibody, on Functional Outcomes in Children With X-Linked Hypophosphatemia (XLH): Results From a Randomized, Open-label Phase 2 Study**

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**INTRODUCTION**

At baseline, 34 patients had high phosphate concentrations [48, 40, 8.5 (1.87)] and 20 of the 24 Fast serum phosphate ≥ 14) Patients (KRN23), a Fully Human Anti-FGF23 monoclonal antibody, was administered subcutaneously every two weeks (Q2W) or every four weeks (Q4W) (Table 1). Key endpoints:

- **Rickets**: Total Thacher Rickets Severity Score (RGS) and Radiographic Global Improvement of Rickets (RGS-C) (Figure 3)
- **Safety**: Adverse events

**RESULTS**

After 40 weeks of treatment, the LSM increase (95% CI) was 1.53 (1.00, 2.05) and 0.96 (0.43, 1.49) at baseline 

- **Safety**: There were no significant changes in laboratory parameters, vital signs, or physical examination findings, and no serious adverse events were reported. Five patients reported injection site reactions, including perifollicular erythema and injection site pain and tenderness.

- **Efficacy**: Burosumab was associated with a significant improvement of Global Functioning Score (Figure 6) and a near significant improvement of Pain/Comfort score (Figure 5). Figure 7 shows the improvements in the POSNA-PODCI Global Functioning score (Figure 7). Other significant improvements were observed in the Sports/Physical Functioning, Pain/Comfort, and Radiographic Global Improvement of Rickets (RGS-C) (Figure 3).

**DISCUSSIONS**

- **At baseline**: 23 of the 51 (45%) evaluable patients had substantial functional impairment (PODSNA-PODCI Global Functioning score ≤ 60). The mean baseline PODSAN-PODCI Global Functioning score among those with functional impairment at baseline was 32.6 ± 12.4, and 102 patients had normal vision (100/120) implants.

- **Safety**: There were no deaths or infectious complications reported during the study period.

**CONCLUSIONS**

- **At baseline**: 23 of the 51 (45%) evaluable patients had substantial functional impairment (PODSNA-PODCI Global Functioning score ≤ 60). The mean baseline PODSAN-PODCI Global Functioning score among those with functional impairment at baseline was 32.6 ± 12.4, and 102 patients had normal vision (100/120) implants.

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