WeeFIM, Pediatric Functional Independence Measure; WPAI, Work Productivity and Activity Impairment; BPI-SF, Brief Pain Inventory – Short Form; NHP, Nottingham Health Profile; QoLISSY, Quality of Life in Short Stature Youth; PedsQL, Pediatric Quality of Life Inventory Questionnaire; APPT, Adolescent Pediatric Pain Tool; EQ-5D-5L, EuroQol 5-dimension QoL questionnaire; NHP: Nottingham Health Profile; QOLISSY: quality of life of short-stature youth; WeeFIM: paediatric functional independence measure; BPI, Brief Pain Inventory; NHP, Nottingham Health Profile; QOLISSY, Quality of Life in Short Stature Youth; PedsQL, Pediatric Quality of Life Inventory Questionnaire; APPT, Adolescent Pediatric Pain Tool.

Limitations of the existing data on health-related quality of life (HRQoL) in achondroplasia patients in Latin America: 67.5% of adolescent subjects completed the APPT, reporting a mean (SD) number of pain sites of 1.9.

Results

Demographics

Patients: 226 parents, 172 children, 209 adolescents, 88 adults. Median age of participants was 10 years (range 1-69 years).

Characteristics at Time of Enrollment

Overall: EAS=172

Participates per country, n (%)

- Brazil: 94 (54.5%)
- Argentina: 37 (21.5%)
- Colombia: 41 (23.8%)

Gender, n (%)

- Male: 81 (47.1%)
- Female: 91 (52.9%)

Age (years)

- Median (25%, 75% Percentile): 16.0 (8.0, 31.5)
- Age subgroup (years, n, %)
  - 3-5: 20 (11.6%)
  - 6-10: 35 (20.3%)
  - 11-15: 26 (18.0%)
  - 16-20: 16 (9.3%)
  - 21-30: 25 (14.5%)
  - 31-40: 22 (13.4%)
  - 41+: 24 (14.0%)

Limb lengthening per time enrollment, n (%)

- Yes: 12 (7.0%)
- No: 164 (93.0%)

Time since limb lengthening (years)

- Median (25%, 75% Percentile): 6.4 (2.6, 10.2)

Conclusions

- Most of the adult participants reported mild pain.
- The LISA study provides the largest set of data to date from Latin America on the lifetime impact of achondroplasia.
- The study addresses the gaps in knowledge about clinical and socioeconomic burden of illness, HRQoL, psychosocial impact, and healthcare resource use for individuals with achondroplasia in Latin America.
- Despite the diversity of patients and sites across the 3 countries that participated of LISA, assessments of WeeFIM, BPI-SF and pain questionnaires were similar.
- LISA data demonstrates that subjects experience significant burden of illness across multiple domains, which impact the HRQoL.
- Limitations of the existing data on health-related quality of life (HRQoL) in achondroplasia patients in Latin America:
  - retrospective data; observational and retrospective design
  - Retrospective data review revealed gaps in medical records (with exception to Argentina), and due to the small number of sites involved in the study, generalization of results to a larger population should be made with caution.

References