Socio-Economic Burden Among Children and Adults With Achondroplasia in Europe and Latin America

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Zhang Y[1], Dee A[1], Maman K[2], Butt T[2], Adepu S[3], dos Santos J[4], Pahwa A[5]

[1]BioMarin Pharmaceutical, San Rafael, CA, USA; [2]BioMarin, London, United Kingdom; [3]ICON, Hyderabad, India; [4]ICON, Lisbon, Portugal; [5]Icon Clinical Research, Bengaluru, India.

Background

- Objective: To estimate the socio-economic burden among children and adults with achondroplasia using observational real-world studies.

Methods

- This was a cross-sectional analysis of data pooled from LIAISE (Lifetime Impact of Achondroplasia Study in Europe) and LISA (Lifetime Impact Study in Achondroplasia in South America) studies.
  - LIAISE[1] was a multinational, observational, retrospective, cross-sectional study of individuals with achondroplasia conducted across six countries at 13 sites in Europe and enrolled 195 individuals age ≥5 years old.
  - LISA[2] was a multinational, observational, retrospective, cross-sectional study of individuals with achondroplasia conducted across three countries at four sites in Latin America and enrolled 172 individuals age ≥3 years old.
- Descriptive analyses were used to characterize the socio-economic burden and regression analyses to explore the association of demographic and clinical characteristics with1 the socioeconomic factors.
- For cost data, financial variables were adjusted for inflation from the date of data collection to 2023 values and converted to US Dollars using 2022 purchasing power parity (PPP) rates for each country.

Table 1. Descriptive Analysis of Demographic and Clinical Characteristics (Overall, Children and Adults)

	Overall (N=356)	Children (N=203)	Adults (N=153)
Age in years, mean (SD)	21.5 (16.5)	9.9 (3.9)	36.9 (13.9)
Female, n (%)	190 (53.4)	96 (47.3)	94 (61.4)
Height z-score*, mean (SD)	-5.5 (1.5)	-5.2 (1.6)	-6.2 (1.1)
Region, n (%)			
- Europe	184 (51.7)	110 (54.2)	74 (48.4)
- Latin America	172 (48.3)	93 (45.8)	79 (51.6)
Marital status, n (%)			
- Single	86 (56.2)	-	86 (56.2)
- Married or living as couple	58 (37.9)	-	58 (37.9)
- Divorced/ separated	6 (3.9)	-	6 (3.9)
- Widowed	2 (1.3)	-	2 (1.3)
- Missing	1 (0.7)	-	1 (0.7)
Wheelchair use, n (%)			
- Yes	20 (5.6)	12 (5.9)	8 (5.2)
- No	321 (90.2)	176 (86.7)	145 (94.8)
- Missing	15 (4.2)	15 (7.4)	0 (0)
Use of walking aid(s), n (%)			
- Yes	23 (6.5)	9 (4.4)	14 (9.2)
- No	304 (85.4)	169 (83.3)	135 (88.2)
- Missing	29 (8.1)	25 (12.3)	4 (2.6)

*Height z-score is height relative to the average for a participants age and gender, expressed in standard deviations

Results

- The analysis sample comprised of 356 subjects: 184 from Europe and 172 from Latin America with a mean height z-score of -5.5. (Table 1)
- 153 were adults and 203 were children (age <18 years at enrollment)

Table 2. Descriptive analysis of employment status (Adults)

	Adults (N=153)
Current employment status, n (%)	
- Paid employment or self-employed	86 (56.2)
- Unemployed	18 (11.8)
- Other	36 (23.5)
- Missing	13 (8.5)
Currently unemployed – feel this is due to achondroplasia, n (%)[a]	12 (66.7)
Employed - Need additional support in workplace, n (%)[b]	47 (54.7)
Days of work missed due to achondroplasia or associated conditions (last 12 months), mean (SD)	18.7 (72.7)

[a]of 18 unemployed, excluding missing; [b]of 86 employed or self employed, excluding missing.

Socio-economic impact

- In the overall population, 12.1% required walking assistance (5.6% used a wheelchair and 6.5% used a walking aid).
- Among adults, 37.9% were married or living as couple, and 38.4% and 69.8% reported the condition had impacted education and employment status respectively.
- Only half of the adults (56.2%) were employed or self-employed and 66% of unemployed adults felt it was due to their achondroplasia. (Table 2)
- Of those in employment, an average of 18.7 days of work were missed due to achondroplasia in the past 12 months, and 54.7% reported that they needed additional support at the workplace.
- Adjusting for inflation and PPP, disability benefits received per month [median (IQR), in US dollars] for adults and children were: $915 ($330, $17,392) and $566 ($328, $951), and social benefits received per month were $372 ($0, $628) and $412 ($222, $842) respectively.

Association with height

- After adjusting for clinical characteristics, a more severe height z-score was associated with higher spending on personal assistance among adults ($p<0.05$) and, similarly, with higher spending on travel to hospital ($p<0.05$). (Table 3)
- Additionally, in Latin America, a more severe height z-score was associated with higher impact of achondroplasia on a person's employment status ($p<0.05$).

Table 3. Multivariable Regression Analysis: Association Between Demographic and Clinical Characteristics with Finances (Adults)

	B	Intercept	P value	Point estimate (95% CI)
Amount spent for personal assistance, n=25 ($)				
Gender: Female vs. Male (ref)	**2.91**	**-2.49**	**<0.0001***	**2.91 (1.56, 4.26)**
Height z-score	**-1.27**	**-2.49**	**0.0048***	**-1.27 (-2.15, -0.39)**
Limb-lengthening surgery: Yes vs. No (ref)	-	-	-	-
Study LIAISE vs. LISA (ref)	-0.71	-2.49	0.3909	-0.71 (-2.34, 0.91)
Amount spent for travel to hospital appointments, n=30 ($)				
Gender: Female vs. Male (ref)	2.38	-5.23	0.1458	2.38 (-0.83, 5.59)
Height z-score	**-2.04**	**-5.23**	**0.0461***	**-2.04 (-4.05, -0.04)**
Limb-lengthening surgery: Yes vs. No (ref)	3.41	-5.23	0.1632	3.41 (-1.39, 8.21)
Study LIAISE vs. LISA (ref)	-2.71	-5.23	0.0580	-2.71 (-5.52, 0.09)

Because of the skewness in cost data, generalized linear model (GLM) with gamma distribution and log link function were used to model all cost outcome variables

Conclusions

- Achondroplasia has serious impacts on social and economic status, and these impacts should be considered in the management of the condition.
- Effects on education and employment were notable, and particularly high rates of absence from work were observed.
- Some of socio-economic factors were significantly associated with height z-score.

References

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