ACHONDROPLASIA.EXPERT CONGRESS REVIEWS ICCBH 2026

Welcome to the ICCBH 2026 congress review

The 12th International Conference on Children's Bone Health (ICCBH) took place in Montréal, Canada, over 27–30 June 2026. Over 600 people from 44 countries attended the congress with learning objectives being to:

- Describe contemporary approaches to paediatric bone science
- Analyse disease mechanisms and evidence-based management strategies in paediatric bone disorder
- Apply current diagnostic, treatment, and long-term management principles to paediatric bone conditions
- Evaluate emerging research findings and innovations to inform clinical practice in paediatric bone health

This review covers selected content from the congress, including the 4th Achondroplasia Workshop on Obesity, and highlights key publications on the latest research in achondroplasia, including new data on vosoritide and novel therapeutic approaches.

We hope you find this review valuable.

The Achondroplasia Team

HIGHLIGHTS FROM THE 4TH ACHONDROPLASIA WORKSHOP ON OBESITY

Learn about the current understanding of obesity and its clinical implications for people with achondroplasia

▶ FIND OUT MORE

SCIENTIFIC PRESENTATIONS AT ICCBH

A summary of poster presentations on key topics in achondroplasia

▶ FIND OUT MORE

Abstracts can be found here

This congress review has been drafted using data from the posters presented at ICCBH.

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VOXZOGO® (vosoritide) is a C-type natriuretic peptide (CNP) analogue indicated for the treatment of achondroplasia in patients 4 months of age and older whose epiphyses are not closed. The diagnosis of achondroplasia should be confirmed by appropriate genetic testing. The latest aPI for VOXZOGO® can be found on the Achondroplasia.expert website, or by clicking here.

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HIGHLIGHTS FROM THE OBESITY IN ACHONDROPLASIA WORKSHOP

The content summarised below reflects the views presented by the individual speakers during the workshop. Where supporting references are not provided, the information represents the speakers' opinions and should be interpreted with appropriate caution.

The workshop, opened by Moira Cheung (London, UK), brought together international experts to discuss obesity in achondroplasia. It covered patient perspectives, obesity assessment, metabolic health, clinical consequences and treatment strategies. A consistent theme throughout the meeting was that; although obesity is common in people with achondroplasia; many aspects of its causes, health consequences and optimal management remain poorly understood.

Perspectives of individuals with achondroplasia and their families

Florian Innig (Germany) described the impact of obesity from the perspective of people with achondroplasia. Obesity can affect physical health, mental wellbeing and QoL, while increasing pressure on joints and mobility. Although maintaining a healthy weight is an important goal, many people with achondroplasia find it difficult to lose weight despite lifestyle changes.

Defining and understanding obesity

Several presentations focused on the challenges of defining obesity in achondroplasia. During a debate, Cathleen Raggio (New York, USA) highlighted that obesity is a major health concern in individuals with achondroplasia and that a high proportion of patients will classify as obese using BMI. It was hypothesised that the high prevalence of obesity in achondroplasia may be due to disease-specific biological factors such as FGFR3-related signalling, altered energy metabolism, and differences in fat cell development.

In contrast, Mike Bober (Wilmington, USA) argued that morbid obesity is no more common compared with the average-stature population. Although morbid obesity is associated with an increased risk of complications, type 2 diabetes (the most common obesity-related comorbidity) is not highly prevalent among people with achondroplasia. Dr Bober emphasised that BMI has not been validated for individuals with achondroplasia and proposed that the BMI threshold associated with an increased risk of obesity-related complications is higher in people with achondroplasia compared with average-stature populations. It was also noted that C-type natriuretic peptide (CNP) levels are elevated in achondroplasia and that CNP has been strongly correlated with cardiovascular risk factors in average stature populations.[1] This raised the question of whether factors beyond body weight may contribute to hypotension and cardiovascular outcomes. Audience polling following the debate supported this perspective, with most participants agreeing that obesity is more common in people with achondroplasia compared with the general population however, obesity-related morbidity is not.

Julie Hoover-Fong (Baltimore, USA) reinforced these messages by highlighting the limitations of conventional obesity assessment tools. Dr Hoover-Fong proposed that obesity should be evaluated using a combination of anthropometric, metabolic, functional and psychological measures rather than BMI alone, and emphasised the need to identify individuals at greatest risk of morbidity and mortality through large-scale validation studies.

Metabolic health and cardiovascular risk

James Betts (Bath, UK) presented data on energy balance and metabolic regulation in achondroplasia, demonstrating that conventional biomarkers of cardiometabolic health were not substantially elevated in people with achondroplasia.[2] Although physical activity was limited in people with achondroplasia, basal metabolic rate was not low, and there was no evidence of excessive energy intake. In addition, individuals with achondroplasia appeared to exhibit distinct patterns of glucose and lipid metabolism.[2] Participants with achondroplasia spent approximately 2 hours per day with glucose levels above 7.8 mmol/L, the upper limit of the recommended range for people without diabetes. By comparison, a previous study demonstrated that healthy individuals of average stature typically spend 30 to 60 minutes per day above this threshold.[3] Participants with achondroplasia also presented higher resting metabolic requirements than those with other forms of restricted growth (+1544 kJ/day).[2]

Svein Fredwall (Nesodden, Norway) discussed the metabolic consequences of obesity and presented data from a Norwegian study (N=49).[4] Findings showed that the prevalence of cardiovascular risk factors in adults with achondroplasia was similar to or lower compared with the general population. Several predictive variables were found to be associated with obstructuve sleep apnoea (OSA) in these patients, including hypertension, age >40 years, and BMI >30 kg/m^2. The reduced life expectancy reported in adults with achondroplasia may be associated with cardiovascular disease, however, additional comorbidities are also likely to contribute to this observation.[5]

Clinical consequences of obesity

The workshop highlighted several important obesity-related complications. Josef Milerad (Stockholm, Sweden) reviewed the respiratory consequences of obesity, emphasising the impact of sleep-disordered breathing (SDB) on cognitive development, behaviour, blood pressure, growth and quality of life. Early identification, weight management and appropriate ventilatory support were identified as important strategies to reduce these risks.

Stuart MacKenzie (Wilmington, USA) discussed the orthopaedic consequences of obesity, noting that excess weight increases

biomechanical stress on the spine and lower limbs, exacerbating spinal stenosis, osteoarthritis and other musculoskeletal complications. Obesity was also associated with an increased risk of postoperative complications and poorer surgical outcomes.

Management strategies

Penny Ireland (Brisbane, Australia) presented the challenges people with achondroplasia face when participating in physical activity, including altered biomechanics, muscle weakness, reduced endurance and neurological complications. However, Dr Ireland emphasised that appropriately adapted exercise, including walking, swimming, cycling and resistance training, remains an important component of maintaining health. Clinicians were encouraged to tailor exercise advice to the preference of the individual, to help them increase their participation in exercise. When giving advice, it is important to consider anatomical and functional characteristics of individuals with achondroplasia.

The workshop concluded with a discussion of medical and surgical approaches to weight management. Klaus Mohnike (Magdeburg, Germany) and Julie Hoover-Fong reviewed the limited evidence available in achondroplasia. While bariatric surgery has demonstrated meaningful weight loss in case reports, potential nutritional complications require careful consideration. New pharmacological treatments for obesity, including GLP-1 receptor agonists, may offer promising alternatives, although further evidence is needed to establish their safety profile and effectiveness in this population.

Take-home message

The workshop highlighted that despite obesity being widely recognised as an important comorbidity in achondroplasia, substantial knowledge gaps exist in the understanding of obesity in this population. BMI as a tool for measuring obesity has limitations, particularly in people with achondroplasia, due to altered body composition and disproportionate body size; therefore, its clinical interpretation requires caution. Further research is needed to clarify the impact of obesity, develop validated assessment tools, and establish evidence-based approaches to prevention and treatment of obesity for people with achondroplasia.

SUMMARY OF POSTER PRESENTATIONS ON KEY TOPICS IN ACHONDROPLASIA

THERAPEUTIC ADVANCES AND EMERGING TREATMENTS

Vosoritide is a CNP analogue indicated for the treatment of achondroplasia in patients 4 months of age and older whose epiphyses are not closed. The diagnosis of achondroplasia should be confirmed by appropriate genetic testing.[6]

The rate of non-responders over the first 2 years of vosoritide treatment was investigated by Soucek *et al.* at the Czech Reference Centre for Rare Bone Diseases. A total of 43 children aged 0.76–14.6 years were included in this prospective, longitudinal, observational study. Clinically significant response to vosoritide was defined as an increase in height Z-score of >0.2 per year. Six children did not complete the first year of vosoritide treatment and two children were cross-border participants who were treated in their home country before finishing the first year and were therefore excluded from the current analysis. Of the remaining 35 children, four were classified non-responders at Year 1: two had sufficient growth at Year 2, one had the treatment withdrawn due to funder-established lack of efficacy at Year 1, and one did not show sufficient growth at Year 2. The authors concluded that approximately one in 10 children do not show response to vosoritide at Year 1 based on height Z-score of >0.2 per year, although most children do respond within this parameter by the end of Year 2. ▸ [P172]

BMN 333 is an investigational, biodegradable treatment designed to advance the therapeutic approach established with vosoritide by extending its half-life through time-dependent release. ▸ [P84 and P158]

Preclinically, BMN 333 has demonstrated sustained vosoritide release, minimised haemodynamic effects, and enabled higher CNP exposure to promote skeletal growth. Savarirayan *et al.* reported the BMN 333 clinical development programme.

Phase 1 evaluated BMN 333 safety and pharmacokinetics in healthy adults. The study demonstrated that BMN 333 was well-tolerated with no dose-limiting toxicities identified in healthy adults. The Phase 2/3 study, ASPEN, will be the first head-to-head superiority trial of BMN 333 versus vosoritide in treatment-naïve children with achondroplasia. Phase 2 will evaluate once-weekly doses of BMN 333 (150, 250, 350 µg/kg) versus once-daily vosoritide over

52 weeks in children aged ≥2 to <11 years. Phase 3 will assess the superiority of BMN 333 versus vosoritide for improving growth in children aged ≥2 to <18 years receiving once-weekly BMN 333 or once-daily vosoritide over 52 weeks. Phase 2 recruitment was planned for early 2026. In summary, ASPEN will evaluate the safety profile, efficacy, and superiority of BMN 333 versus vosoritide for improving growth in children with achondroplasia. ▸[P84]

Wendt *et al.* described the development of BMN 333. In the pharmacokinetic (PK) analysis of BMN 333, 48 wild-type mice received a single SC injection at 630 or 1900 µg/kg. Findings showed dose-dependent, sustained exposure to released vosoritide and longer half-life compared with mice dosed with vosoritide (10 hours versus ~15 minutes, respectively). Growth studies in 3-week-old wild-type mice treated for 5 weeks with either vosoritide or BMN 333 showed significant increase in femur length at higher daily doses of BMN 333 (80, 300, or 1000 µg/kg Q2D, or 500, or 1600 µg/kg QD). In analyses of 52 2-year-old cynomolgus monkeys receiving 75, 200, or 350 µg/kg QW for 26 weeks, half-life was ~60 hours. In juvenile mice and monkeys, BMN 333 showed consistent vosoritide release, leading to dose-dependent increases in endochondral bone growth without haemodynamic effects. ▸[P158]

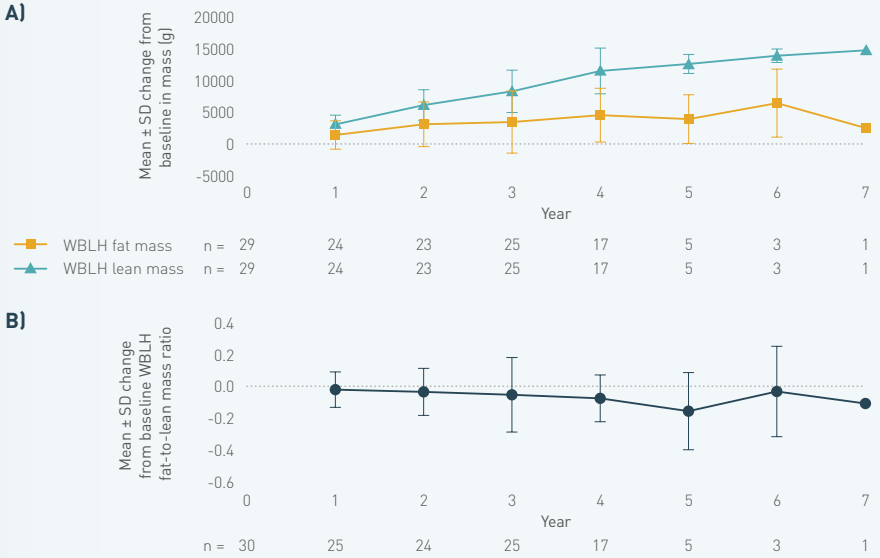
TREATMENT OUTCOMES: GROWTH AND BEYOND

New data demonstrated the sustained benefits of long-term vosoritide treatment on body composition and endochondral bone growth in children and adolescents with achondroplasia. ▸[LB20, P82]

Mohnike *et al.* presented the long-term impact of vosoritide on body composition using whole body less head (WBLH) dual X-ray absorptiometry in participants aged ≥5 and <15 years at treatment initiation in the Phase 3 CANOPY ACH-3 and -EXT studies. Of 119 children and adolescents enrolled in ACH-EXT, 58 received vosoritide and 61 received placebo. After 52 weeks, improvements in WBLH lean mass were observed in participants receiving vosoritide versus untreated children, with treated adolescents having the largest decrease in WBLH fat mass and fat-to-lean mass ratio (see figure opposite). The BMI of treated children decreased over time compared with untreated children, and after 6 years of treatment, a positive correlation was seen between reduced WBLH fat-to-lean mass and reduction in BMI and BMI Z-score. There was no correlation between fat-to-lean-mass and height Z-score, suggesting that improvements in body composition were driven not only by gains in linear height. ▸[LB20]

Engelke *et al.* compared radial and tibial parameters, including bone mineral density, of 30 children with achondroplasia who completed 10 years of vosoritide treatment with published age- and sex-stratified curves from an average stature population. Distal and proximal volumes of interest (VOIs) were positioned in the radius and tibia in children aged ≥5 to <15 years from the CANOPY ACH-2 and -EXT studies; bone mineral density (BMD) at each VOI was determined and plotted against

Change from baseline in WBLH a) fat mass or lean mass and b) fat-to-lean mass ratio in adolescents over time



Mohnike K, *et al.* [LB20]

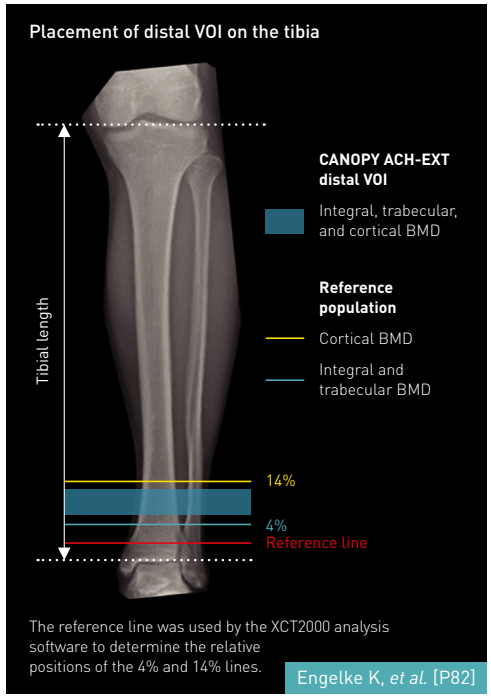
Correlation between change from baseline in WBLH fat-to-lean mass ratio and BMI, BMI Z-score, or height Z-score at year 6

	Full analysis set (N=119)
Correlation parameters for change from baseline in WBLH fat-to-lean mass ratio vs	
Change from baseline in BMI	n = 43
Pearson's r	0.489
P-value	0.0009
Change from baseline in BMI Z-score (average stature-referenced)	n = 43
Pearson's r	0.429
P-value	0.0041
Change from baseline in height Z-score (average stature-referenced)	n = 43
Pearson's r	−0.064
P-value	0.6858

Each correlation was based on participants with data available at year 6. BMI and height Z-scores were referenced to age- and sex-matched reference data for average-stature children (CDC 2000 dataset).[7]

Mohnike K, *et al.* [LB20]

age- and sex-stratified reference curves from an average-stature population to assess the potential impact of vosoritide on BMD over time. The distal tibia volumetric BMD of the integral, trabecular and cortical compartments increased with age in the children treated with vosoritide in line with that of the average-stature reference curves. Despite different anatomical positions of the VOIs in the average-stature population, it was determined that in children from CANOPY-EXT, lower trabecular BMD is expected given the more proximal location of the VOI. Lower cortical BMD could be explained by partial volume effects due to the thinner cortex given the more proximal location of the VOI. Results for the radius were similar but were not shown in the poster. This analysis is the first to demonstrate sustainable endochondral bone growth over time in children with achondroplasia treated with vosoritide. ▸[P82]



NOVEL APPROACHES TO MEASURING CLINICAL IMPACT

The need for clinically relevant outcome measures in achondroplasia, including assessments of obesity, mobility, muscle function, and treatment-related changes beyond height gain was highlighted.

As discussed above, the assessment of obesity in achondroplasia is challenging due to disproportionate short stature. Lanzafame *et al.* aimed to assess which anthropometric measure best reflect adiposity and its functional impact in people with achondroplasia. Overall, 24 participants aged 9–18 years were included. Anthropometric measurements included weight, BMI, waist and hip circumference and waist-to-height ratio; these measurements were referenced to dual-energy X-ray absorptiometry (DXA)-derived fat mass.

Findings demonstrated that all anthropometric measures were significantly associated with DXA-derived fat mass after adjustment for age and sex, confirming their validity as markers of overall adiposity in achondroplasia. BMI and waist-to-height ratio were associated with reduced performance on the 6-minute walking test, suggesting that body proportionality measures better reflect impact on mobility. BMI was the only anthropometric measure independently associated with the Skeletal Dysplasia Mobility and Fatigue Score, although the effect size was small. These findings support the use of these anthropometric measures to assess obesity-related functional burden in achondroplasia; however, larger studies are needed to refine risk stratification and outcome prediction. ▸[P123]

Wang *et al.* undertook a scoping review of the literature to establish the methods used to quantify functional endpoints beyond anthropometry in children with achondroplasia receiving growth-promoting therapies. A total of 39 publications (31 study reports and 8 protocols) were included in this review following a systematic search for English-language studies published up to 8th April 2026 in Medline, Embase, Web of Science Core Collection, and ClinicalTrials.gov databases and registries. The 39 publications represented 33 distinct studies encompassing 7,981 individuals with achondroplasia aged <18 years, receiving either growth hormone (13/33), vosoritide (14/33), recifercept (3/33), navepegritide (2/33), meclizine (1/33), infigratinib (1/33), and growth hormone + vosoritide (1/33). Clinical imaging endpoints were used in 80% of studies (27/33) including limb length and lower extremity alignment, spinal deformity, canal dimensions, and cranial outcomes. Imaging modalities included radiographs, MRI, and computed tomography, with questionnaire-based outcomes reported in 8/33 studies to assess HRQoL, functional independence, and achondroplasia-specific patient and caregiver-reported outcomes. Functional assessments including the 6-minute walk test, jumping mechanography and polysomnography were used in three studies. One recent study included 3-dimensional facial surface imaging to quantify changes in facial structure. In summary, standardising endpoints that extend beyond anthropometry and proportionality may be valuable in ongoing research into growth-modifying treatments to comprehensively capture their impact on key features of skeletal dysplasia and effects on daily living. ▸[LB33]

The same group of authors, in a separate study, also conducted a scoping review of methods for quantifying linear growth and anthropometric measures in trials of growth-modulating therapies in children and adolescents ⩽18 years with achondroplasia. A total of 36 peer-reviewed publications from 30 trials were identified following a search for studies up to 16th March 2026 in Medline, Embase, Web of Science Core Collection, and ClinicalTrials.gov databases or registries. The publications comprised 7,784 children receiving growth hormone (12/30 studies), vosoritide (10/30), recifercept (3/30), navepegritide (2/30), meclizine (1/30), infigratinib (1/30), and combined growth hormone + vosoritide (1/30). Linear growth outcomes were assessed using change in height, height Z-score, growth velocity, growth velocity Z-score, and proportion of patients with growth velocity ⩾6 cm/year. The most frequently assessed anthropometric measures were upper-to-lower segment ratio, sitting height-to-height ratio, and arm span. Other parameters captured in the studies analysed were BMI, weight, arm span-to-height ratio, head circumference, sitting height, arm span-to-height differences, lower limb-to-height ratio, and change in bone age relative

to chronological age. The authors emphasised the importance of identifying the minimal clinically important differences in linear growth parameters to enable evaluation of functional endpoints and their relationship to growth outcomes. ▸ [LB34]

Muscle function in achondroplasia has not been explored in detail. Chinoy *et al.* reported findings from a retrospective study that analysed muscle function parameters in children with achondroplasia compared with normative data. From Dec 2024 to Sept 2025, children attending annual clinical visits had their muscle function measured as part of physiotherapy assessments. Leonardo mechanography was used to measure the 2-legged jump, multiple 1-legged hop and grip force. Z-scores were calculated by Leonardo software, accounting for body mass, age and sex. Mean Z-scores were compared against the normative reference mean using two-sided one-sample t-tests. Findings demonstrated that 15 children had significantly reduced Z-scores for jumping power (-2.99 [95% CI -3.63, -2.34], p<0.001), jumping efficiency (-2.61 [95% CI -3.27, -1.95], p<0.001), hopping force (n=9, 95% CI -2.33 [-3.49, -1.18], p=0.002) and grip force (n=7, -3.33 [95% CI -3.71, -2.96], p<0.001).

These data suggest that children with achondroplasia may have reduced dynamic muscle function. Further studies are needed to confirm these findings and to assess the impact on daily functioning and physical activities for people with achondroplasia. ▸ [P113]

OPTIMISING CLINICAL MANAGEMENT AND SURVEILLANCE

Recognising the multisystem nature of achondroplasia, researchers have explored strategies to improve clinical surveillance and streamline the management of associated complications across the lifespan.

The lifelong and multisystem impacts of achondroplasia necessitate a proactive, multidisciplinary approach to the clinical care of affected individuals across all stages of life.[8] Haysom *et al.* reported findings from a retrospective two-cycle audit including children with achondroplasia under the care of Royal Manchester Children's Hospital. Monitoring practices were assessed across key domains (general patient information and auxology, neurological, sleep/respiratory, audiology, orthopaedic, MRI, psychosocial, occupational therapy and dental). Two audits were conducted from June 2023 to August 2024 and from September 2024 to December 2025.

Findings demonstrated that the introduction of a multidisciplinary team (MDT) clinic was associated with monitoring improvements across all domains. Most notable improvements were observed in dental health (34% to 74%), psychosocial assessment (71% to 100%) and sleep/respiratory monitoring (36% to 100%). In summary, this audit has demonstrated quality improvements due to the addition of an MDT. ▸ [P135]

Optimal timing for first and follow-up MRI in infants with achondroplasia has not been established, despite current literature supporting its use to screen for foramen magnum stenosis (FMS). Snow *et al.* evaluated the natural history of FMS in infants with achondroplasia undergoing early MRI and follow-up scans to establish the optimal timing for imaging. A total of 71 infants were evaluated between 2020 and 2025 in a retrospective cohort study. Inclusion criteria included genetic confirmation of achondroplasia and first MRI performed before the age of 3 months; 38 infants were excluded from analysis due to not meeting these criteria. The remaining 24 infants were analysed and stratified into two groups based on timing of follow-up MRI at <50 weeks (n=13) or ≥50 weeks (n=11). In the <50 weeks group, mean follow-up screening was at 40.2 weeks, and Achondroplasia Foramen Magnum Score (AFMS) remained stable in 61.5%, progressed in 30.8% and improved in 7.7%. In the ≥50 weeks group, mean follow-up screening was at 63.7 weeks; AFMS remained stable in 63.6% and progressed in 36.4%. Median AFMS remained unchanged in the <50 weeks group (AFMS1), while in the ≥50 weeks group median AFMS increased from AFMS1 to AFMS2, with a statistically significant difference seen between scans (p=0.03). The authors concluded that while the proportion of infants with progressing AFMS did not differ significantly whether they received the follow-up scan before or after 50 weeks, the significant increase in severity of AFMS if scanned after 50 weeks suggest that earlier follow-up may detect progression sooner and enable earlier intervention. ▸ [LB3]

EPIDEMIOLOGY AND NATURAL HISTORY

New epidemiological data provided updated insights into the global and regional birth prevalence of achondroplasia.

Epidemiology studies in achondroplasia are limited and vary widely globally. Raveendran *et al.* conducted a systematic literature review (PubMed and Embase) and meta-analysis to estimate the birth prevalence of achondroplasia globally and by region. Birth prevalence estimates were standardised to reflect rates per 100,000 live births.

The literature review identified 55 studies. The estimated pooled global birth prevalence of achondroplasia was 3.7 per 100,000 live births (95% CI, 3.1, 4.2) with considerable study heterogeneity (I2 = 79.7%). Estimated pooled birth prevalence varied by region, with studies conducted within the Middle East and North Africa reporting the highest estimates (23.1 per 100,000 live births [95% CI, 13.4, 32.8]) and Northern America the lowest (2.8 per 100,000 live births [95% CI, 1.8, 3.8]). Despite study heterogeneity, these findings provide an updated foundation for understanding global and regional prevalence patterns of achondroplasia. ▸ [P68]

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LIST OF ABBREVIATIONS

AFMS – Achondroplasia Foramen Magnum Score
BMD – bone mineral density
CDC – Centers for Disease Control and Prevention
CNP – C-type natriuretic peptide
FMS – foramen magnum stenosis
GLP-1 – glucagon-like peptide-1
LDL-C – low-density lipoprotein cholesterol
MDT – multidisciplinary team
OSA – obstructive sleep apnoea
PK – pharmacokinetic
Q2D – every other day
QD – once daily
QW – every week
SC – subcutaneous
SDB – sleep disordered breathing
VOI – volumes of interest
WBLH – whole body less head

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