

Reimbursement Recommendation

Vosoritide (Voxzogo)

Indication: Vosoritide is indicated to increase linear growth in patients with achondroplasia who are 4 months of age and older whose epiphyses are not closed. The diagnosis of achondroplasia should be confirmed by appropriate genetic testing.

Sponsor: BioMarin Pharmaceutical (Canada) Inc.

Final recommendation: Do not reimburse

Summary

What Is the Reimbursement Recommendation for Voxzogo?

Canada's Drug Agency (CDA-AMC) recommends that Voxzogo not be reimbursed by public drug plans for increasing linear growth in patients with achondroplasia (ACH) who are 4 months of age and older whose epiphyses are not closed.

Why Did CDA-AMC Not Recommend Reimbursement?

The Canadian Drug Expert Committee (CDEC) concluded that it is uncertain whether Voxzogo demonstrates acceptable clinical value compared with best supportive care alone in patients 4 months of age and older with ACH whose epiphyses are not closed. Given that Voxzogo is expected to be an added treatment to best supportive care, acceptable clinical value refers to added value versus best supportive care alone.

Evidence from 2 double-blind, randomized controlled trials (RCTs) showed that Voxzogo increased annualized growth velocity in patients younger than 18 years, and increased height z scores in patients aged 5 years to younger than 18 years compared with placebo at 52 weeks. However, there was little to no demonstrated benefit in patient-important outcomes at 52 weeks, including body proportionality, health-related quality of life, or sleep apnea, and the clinical significance of the observed height gains for improving patient-important outcomes remains uncertain. Long-term extension studies suggested sustained effects on height, but interpretation was limited by the lack of a comparator group. Overall, it remains uncertain whether Voxzogo improves long-term functional outcomes, ACH-related complications, and the need for surgery or supportive care.

CDEC further reviewed evidence from 6 sponsor-submitted studies, including the Natural History Integrated Comparative Analysis, 3 real-world evidence studies, and 2 additional analyses of the 2 double-blind RCTs that intend to address gaps in the evidence. However, due to important methodological limitations of the studies, CDEC was unable to draw conclusions on the comparative effects of Voxzogo. CDEC concluded that the results from these studies did not meaningfully address the key evidence gap remaining from the 2 RCTs and their long-term extension studies, which is the absence of comparative evidence versus placebo beyond 52 weeks. The submitted studies primarily provided additional evidence on growth outcomes but evidence for the long-term outcomes identified as important by patients and clinicians was either uncertain or unavailable.

Summary

CDEC considered there to be significant unmet need based on potential challenges with evidence generation associated with the rarity of ACH and the severity of ACH despite available treatment options. However, even when taking the significant unmet clinical need into account, CDEC was unable to conclude that Voxzogo addresses the unmet need with an acceptable level of certainty in clinical value.

CDEC acknowledged that there is a significant unmet nonclinical need given that current supportive treatments are mainly available in tertiary pediatric care centres. This can create barriers to accessing care for patients living in rural areas or for those with fewer resources who may face travel-related burdens. However, CDEC was unable to conclude that Voxzogo addresses this significant unmet nonclinical need and health inequity because the submitted evidence did not demonstrate that Voxzogo could reduce the need for specialized services or supportive treatments.

Based on all the preceding considerations, CDEC recommended that Voxzogo not be reimbursed.

Review Background

- **Disease background:** Achondroplasia (ACH) is a rare, progressive, genetic disorder in which a variant in the *FGFR3* gene causes impaired endochondral bone formation. ACH is typically diagnosed before or shortly after birth based on clinical characteristics, which include disproportionate short stature, long-bone shortening affecting the upper and lower extremities, and macrocephaly. ACH complications may involve the skeletal (e.g., spinal stenosis, bowed legs, kyphosis, back pain), respiratory system (e.g., sleep apnea), neurological system (e.g., hydrocephalus), dentition (crowded teeth), and metabolism (e.g., obesity, cardiovascular disease), often stemming from constricted bone growth, especially at the skull base and spine. In North America, the estimated prevalence is 4 per every 100,000 individuals.
- **Indication and reimbursement request:** Vosoritide (Voxzogo) has been approved by Health Canada to increase linear growth in patients with ACH who are 4 months of age and older whose epiphyses are not closed. The diagnosis of ACH should be confirmed by appropriate genetic testing. The sponsor is seeking reimbursement for this patient population.
- **Drug under review:** Vosoritide is a stabilized version of naturally occurring C-type natriuretic peptide that counteracts the effects caused by the *FGFR3* gene variant present in ACH, thereby promoting endochondral bone growth. It is administered by subcutaneous injection based on the patient's weight at a recommended dose of 15 mcg/kg to 30 mcg/kg.
- **Treatment costs:** At the submitted price of \$950.00 per vial (regardless of strength), the annual cost of vosoritide is expected to be \$346,988 per patient, based on the Health Canada–recommended dosage.

Highlights of Input From Interested Parties

The patient group The Chandler Project noted the following regarding impacts of the disease, unmet needs, and important outcomes:

- ACH causes disproportionate growth and shortened limbs that could cause respiratory and neurological complications and reduce quality of life. The burden of emotional distress caused by limitations in independence, motor skill difficulties, and financial strain driven by medical needs further strain quality of life.
- Currently available therapeutic interventions primarily involve symptom management and supportive care that could include surgeries. There is a need for new treatments that target the underlying cause of ACH to increase growth and reduce the risk of health complications associated with ACH and reduce the need for best supportive therapies (e.g., orthopedic corrective surgery or invasive limb lengthening). They hoped new treatments could reduce spinal and leg deformities, improve mobility, prevent breathing difficulties, reduce spinal stenosis, and improve the foramen magnum size.

The clinician group Canadian Skeletal Dysplasia Group and the clinical experts consulted by Canada's Drug Agency (CDA-AMC) noted the following regarding unmet needs arising from the disease and place in therapy for the drug under review:

- There is an unmet need for new treatments that address the underlying cause of ACH to prevent life-threatening morbidities (e.g., foramen magnum stenosis, hydrocephalus, spinal stenosis), minimize the number of surgeries and surgical morbidity, alter body proportions, enhance functional status, and decrease the need for occupational therapy and physiotherapy.
- The anticipated place in therapy would be first-line treatment for patients who are 4 months of age and older whose epiphyses are not closed. The clinical experts noted that vosoritide is not a cure for ACH and it could be used in combination with best supportive care (e.g., surgery, physiotherapy), as needed.

The participating public drug programs raised potential implementation issues related to relevant comparators, considerations for initiation, renewal, discontinuation, and prescribing of therapy; care provision issues; and system and economic issues.

Person With Lived Experience

Two parents of a young child in Ontario presented their family's journey with ACH, describing a premature birth followed by severe breathing, feeding, and neurological complications. Their child has required prolonged hospitalization, multiple surgeries, a tracheostomy, and ongoing ventilatory and feeding support. They expressed that their hope for treatments for ACH, such as Voxzogo, is to potentially influence the underlying pathway of the condition in ways that might lessen future complications, not only impact height. During the Q&A, they emphasized goals for improved airway stability, fewer surgeries, and greater independence, and highlighted the challenges many families face navigating complex care systems, especially those with fewer resources or living far from specialized centres.

Note: The perspectives shared by people with lived experience who present to the committee reflect their individual experiences and are not necessarily representative of all people with the same condition or course of treatment. Their insights provide valuable context about what a patient, support person, or caregiver might go through with this condition or treatment, helping to inform the committee's deliberations. These narratives complement other forms of evidence and input and should be considered as 1 element contributing to a broader understanding of the condition and treatment under review.

Recommendation

With a vote of 13 to 2, the Canadian Drug Expert Committee (CDEC) recommends that vosoritide not be reimbursed to increase linear growth in patients 4 months of age and older with ACH whose epiphyses are not closed.

Rationale for the Recommendation

Clinical Value

Based on the totality of the presented clinical evidence, CDEC concluded that it is uncertain whether vosoritide demonstrates acceptable clinical value compared with best supportive care alone in patients 4 months of age and older with ACH whose epiphyses are not closed. Given that vosoritide is expected to be an added treatment to best supportive care, acceptable clinical value refers to added value versus best supportive care alone.

Evidence from 2 double-blind, randomized controlled trials, including a phase III trial in patients aged 5 years to younger than 18 years (Study 111-301; N = 121) and a phase II trial in patients younger than 5 years (Study 111-206; N = 75), demonstrated that treatment with vosoritide resulted in an increase in annualized growth velocity (AGV) compared to placebo at 52 weeks in patients with ACH whose epiphyses were not closed. In patients aged 5 years to younger than 18 years, height z score also increased at 52 weeks. However, compared to placebo, vosoritide appeared to have little to no difference in upper-to-lower body segment ratio, health-related quality of life (HRQoL), and sleep apnea-hypopnea index score (assessed in Study 111-206 only), which are among the goals of therapy, at 52 weeks in the trials. Furthermore, the clinical importance of the demonstrated increased AGV or height is uncertain. Direct comparative evidence was limited to 52 weeks. There was no direct evidence to show that, compared to placebo, vosoritide treatment when initiated in patients without epiphyseal closure could lead to a final adult height that would result in clinically meaningful improvements in functional outcomes. Additionally, no literature supporting AGV as a surrogate for functional outcomes was submitted.

In their long-term extension (LTE) studies (Study 111-302 for up to 364 weeks; Study 111-208 for up to 286 weeks), the effect of vosoritide on height appeared maintained; however, there were minimal changes from baseline in upper-to-lower body segment ratio and HRQoL (assessed in Study 111-301 only) in patients receiving vosoritide. The long-term evidence was overall uncertain due to the lack of a comparator group because it is uncertain whether the observed changes could be attributed to vosoritide treatment alone or to changes expected with age. Ultimately, it is uncertain whether, compared with placebo, vosoritide could improve long-term outcomes highlighted as important to patients and clinicians, including functional outcomes, medical complications (e.g., foramen magnum stenosis, spinal stenosis) and surgeries associated with ACH, and the need for best supportive care therapies. These outcomes were either not assessed in the trials or were reported descriptively with no formal statistical analysis.

CDEC further reviewed evidence from 6 sponsor-submitted studies, including the Natural History Integrated Comparative Analysis, 3 real-world evidence studies, and 2 additional analyses of the 111-301 and 111-206 studies that intended to address gaps in the evidence. However, due to important methodological limitations of the studies, CDEC was unable to draw conclusions on the comparative effects of vosoritide. CDEC concluded the results from these studies did not meaningfully address the key evidence gap remaining from the 111-301 and 111-206 trials and their LTE studies, which is the absence of comparative evidence versus placebo beyond 52 weeks, particularly for the previously mentioned long-term outcomes identified by patients and clinicians as important.

Further information on the committee's discussion regarding clinical value is provided in the Summary of Deliberation section.

Considering Significant Unmet Clinical Need

Due to challenges with evidence generation associated with the rarity of ACH and the severity of ACH despite available treatment options, CDEC considered there to be significant unmet need as described in the recommendation framework in the Procedures for Reimbursement Reviews. However, even when taking the significant unmet clinical need into account, CDEC was unable to conclude that vosoritide addresses the unmet need with an acceptable level of certainty in clinical value.

Further information on the committee's discussion around unmet clinical need is provided in the Summary of Deliberation section.

Considering Significant Unmet Nonclinical Need or Health Inequity

CDEC acknowledged that there is a significant unmet nonclinical need in the patient population under review given that current supportive treatments are mainly available in tertiary pediatric care centres. This can create barriers to accessing care for patients living in rural areas or for those with fewer resources who may face travel-related burdens. However, CDEC was unable to conclude that vosoritide addresses this significant unmet nonclinical need and health inequity because the submitted evidence did not demonstrate that vosoritide could reduce the need for specialized services or supportive treatments.

Further information on the committee's discussion around unmet nonclinical need is provided in the Distinct Social and Ethical Considerations domain in the Summary of Deliberation section.

Developing the Recommendation

Due to the uncertainty in clinical value, CDEC could not recommend to reimburse vosoritide based on clinical value alone. Therefore, they also considered whether vosoritide addresses a significant unmet clinical need with an acceptable level of certainty in clinical value. CDEC was not able to recommend reimbursement even after taking this into account. Finally, they considered whether vosoritide addresses a significant unmet nonclinical need or health inequity. CDEC was unable to conclude that vosoritide addresses a significant unmet nonclinical need or health inequity to a degree that overcomes the uncertainty in clinical value and potential risks. Based on all the preceding considerations, CDEC recommended that vosoritide not be reimbursed.

Because CDEC recommended that vosoritide not be reimbursed, further deliberation was not required on whether reimbursement conditions should be added to address important economic considerations, health system impacts, or social and ethical considerations, or to ensure clinical value is realized.

Summary of Deliberation

CDEC considered all domains of value of the deliberative framework before developing its recommendation: clinical value, unmet clinical need, distinct social and ethical considerations, economic considerations, and impacts on health systems. For further information on the domains of value, refer to [Expert Committee Deliberation at Canada's Drug Agency](#).

The committee considered the following key discussion points, organized by the 5 domains of value.

The sponsor requested a reconsideration of the initial draft recommendation not to reimburse vosoritide to increase linear growth in patients with ACH who are 4 months of age and older whose epiphyses are not closed. There were 3 issues outlined by the sponsor in the request for reconsideration that were discussed by CDEC.



Clinical Value

- **Appropriate comparator:** During the initial and reconsideration meetings, CDEC discussed that there are currently no pharmacological therapies approved for the treatment of ACH. Patients are currently managed by best supportive care, which may include surgical interventions, such as foramen magnum decompression, occupational therapy to support environmental adaptation, and physiotherapy as needed. CDEC considered best supportive care alone as an appropriate comparator of vosoritide.
- **Efficacy of vosoritide versus placebo in patients aged 5 years to younger than 18 years:** During the initial and reconsideration meetings, CDEC discussed evidence from 1 phase III, double-blind, randomized controlled trial (Study 111-301). The trial demonstrated that treatment with vosoritide resulted in an increase in AGV and height z score in children aged 5 years to younger than 18 years with ACH whose epiphyses were not closed. For AGV and height z score at 52 weeks, the estimated between-group difference in least squares mean change from baseline was 1.57 cm/year (95% confidence interval [CI], 1.22 cm/year to 1.93 cm/year) and 0.28 standard deviation (SD) score (95% CI, 0.17 SD score to 0.39 SD score), respectively. There appears to be little to no difference between groups at 52 weeks in upper-to-lower body segment ratio and HRQoL as assessed by the Quality of Life in Short Stature Youth caregiver and patient self-reported total scores. There was no evidence for the effect of vosoritide on sleep apnea-hypopnea index score, so it is unknown whether vosoritide has any impact on this outcome. In the ongoing 111-302 LTE study, increases in AGV from baseline were maintained through week 312; however, interpretation beyond 260 weeks is limited due to reduction in sample sizes. Consistent changes in height z score were also observed from baseline to week 260; however, interpretation of these changes is limited due to the reduced sample size. Minimal changes from baseline were observed in upper-to-lower body segment ratios and HRQoL during the LTE study. No definitive conclusions on the long-term effects of vosoritide can be made in the absence of a randomized comparator because it is not possible to determine the extent to which the observed effects can be attributed to vosoritide.

- Efficacy of vosoritide versus placebo in patients younger than 5 years:** During the initial and reconsideration meetings, CDEC discussed evidence from the phase II, randomized, double-blind, placebo-controlled trial (Study 111-206) in children younger than 5 years of age with ACH. The trial reported that treatment with vosoritide increased AGV but showed little to no difference in height z score, upper-to-lower body segment ratio, sleep apnea-hypopnea index score, and HRQoL compared to placebo. However, these results were uncertain because the trial was not designed to be confirmatory of efficacy. Additionally, the sample size was small, there is an increased risk that prognostic balance between groups was not achieved, and effect estimates were frequently affected by imprecision. The efficacy and safety of vosoritide for up to 286 weeks in the LTE study (Study 111-208) appeared consistent with that of the parent trial (Study 111-206); however, there is uncertainty due to the open-label design and lack of a comparator group.
- Clinical importance of increased growth:** During the initial and reconsideration meetings, CDEC discussed the clinical importance of increased growth, which is 1 of the outcomes identified as important in the patient input received for this review. CDEC noted that, although the 111-301 and 111-206 trials demonstrated that vosoritide, compared to placebo, resulted in an increase in height, based on AGV, in pediatric patients with ACH, the comparative evidence was limited to 52 weeks. CDEC considered clinical expert input that the potential for AGV results to be clinically meaningful is based on the assumption that if patients started the treatment as early as possible, they may achieve a final adult height that would result in clinically meaningful improvements in functional outcomes identified as important to patients. They discussed the importance of clear evidence demonstrating the relationship between increased growth parameters and functional outcomes. They acknowledged that although some individuals within the ACH community would value impacts on height in and of itself, others may only be willing to accept these impacts if paired with demonstrable improvements in long-term outcomes (discussed in further detail subsequently). The committee therefore considered it important to evaluate whether increases in growth parameters translate into meaningful improvement in patient-important outcomes. However, direct assessment of the effect of vosoritide treatment relative to placebo on final adult height and how this relates to patient-important outcomes were not evaluated in the trials submitted. Also, the evidence submitted by the sponsor did not include any literature supporting AGV as a surrogate for functional outcomes. Overall, CDEC noted that the clinical importance of increasing AGV or height is uncertain in patients without epiphyseal closure.
- Efficacy of vosoritide on long-term outcomes:** During the initial and reconsideration meetings, CDEC discussed that in addition to increasing growth, input from patients and clinicians indicated that there is an unmet need for effective and safe treatment options that target the underlying cause of ACH and improve long-term outcomes, including reducing spinal and leg deformities, reducing complications (e.g., foramen magnum stenosis and spinal stenosis, sleep apnea) and surgeries associated with ACH, improving mobility and HRQoL, and reducing the need for best supportive care. CDEC noted that it is uncertain if vosoritide can address this unmet clinical need. Clinical expert input indicated that although vosoritide acts on the signalling pathway affected by the *FGFR3* mutation, it does not modify or correct the underlying genetic defect itself. Also, CDEC noted that the comparative effect of vosoritide versus placebo on the long-term outcomes highlighted as important by patients

and clinicians is unknown because there is no direct comparative evidence between vosoritide and placebo beyond 52 weeks. Spinal and leg deformities, functional outcomes, complications and surgeries associated with ACH, and the need for best supportive care were either not assessed in the trials or were reported descriptively with no formal statistical analysis. Overall, CDEC noted that there is insufficient evidence to conclude that vosoritide could improve the long-term outcomes of patients with ACH relative to placebo, which is inclusive of best supportive care and the natural history of ACH.

- Relationship between growth and long-term outcomes:** During the reconsideration meeting, CDEC discussed the preceding considerations regarding clinical importance of increased growth and long-term outcomes alongside feedback received from patients and clinicians that growth is not a cosmetic outcome but a clinically meaningful outcome that may be associated with improvements in functioning, HRQoL, caregiver burden, and future complications. CDEC acknowledged these perspectives and recognized the importance of these outcomes. However, the committee noted that the available evidence primarily demonstrated effects on growth and height, and that the relationship between these outcomes and long-term patient-important outcomes remains uncertain. In particular, there was limited evidence from the submission to support assumptions that growth gains would be sustained through skeletal maturity and translate into meaningful improvements in functioning, HRQoL, morbidity, complications, or the need for corrective surgery. CDEC further noted that evidence was lacking regarding the degree of height gain required to achieve clinically meaningful improvements in patient-important outcomes, and whether the gains observed with vosoritide would be sufficient to achieve such benefits.
- Certainty of the evidence:** During the initial and reconsideration meetings, CDEC discussed the Grading of Recommendations Assessment, Development and Evaluation (GRADE) assessment of selected outcomes from the 111-301 trial and noted that the certainty of the outcomes of AGV, height z score, and upper-to-lower body segment ratio were considered high. The certainty of HRQoL scores were considered low and very low at 52 weeks due to concerns with imprecision and with potential prognostic balance and imprecision, respectively.
- Harms findings versus placebo:** During the initial meeting, CDEC noted that evidence from the phase III (111-301) and phase II (111-206) trials suggested that there were no notable safety signals relative to placebo, and the safety profile of vosoritide appears to be manageable, although less common harms and those occurring with longer duration of treatment are not known. CDEC noted that the safety of vosoritide for up to 364 weeks in the 111-302 LTE study and up to 286 weeks in the 111-208 LTE study appeared consistent with that of parent trials (Study 111-301, Study 111-206); however, the results are uncertain due to the open-label design and lack of a comparator group.
- Evidence gaps:** During the initial meeting, CDEC discussed 3 additional studies submitted by the sponsor to address gaps in the evidence. CDEC noted that the evidence from the Natural History Integrated Comparative Analysis was too uncertain to draw any conclusions on the comparative effects of vosoritide versus the natural history cohort. This uncertainty was mostly attributed to a high risk of confounding, small sample size, and imprecision in the estimates. Evidence from 2 real-world

evidence studies in populations treated with vosoritide and assessing additional outcomes, including 6-minute walk score and spinal and lower limb alignment outcomes, was reviewed; however, interpretation of findings was limited by study design, lack of a comparator, small sample sizes, and single-centre settings. As well, skeletal alignment measures are known to change with age as part of the natural history of achondroplasia, making it difficult to distinguish treatment effects from expected developmental changes. CDEC felt the results from these studies did not meaningfully address the key evidence gap remaining from the 111-301 and 111-206 trials and their LTE studies, which is the absence of comparative evidence versus placebo beyond 52 weeks, particularly for long-term outcomes identified by patients as important (e.g., complications and surgeries associated with ACH, need for best supportive care therapies, functional outcomes, HRQoL).

During the reconsideration meeting, CDEC further examined new evidence from 3 studies in patients with ACH identified in the reconsideration request that aimed to assess the effects of vosoritide beyond growth outcomes. CDEC noted that the absence of a comparator in the Reincke et al. (2026) study limits the committee's ability to determine whether changes in muscle performance parameters (2-legged jump test, 6-minute walking test), could be attributed to vosoritide treatment, natural history of the condition, or other factors. Studies by White et al. (2026) and Irving et al. (2026) provided additional information on skeletal morphology (tibial bowing angle, fibula-to-tibia ratio, ankle joint-to-distal fibula plate distance) and spinal morphology (interpedicular distance, spinal canal width, thoracolumbar kyphosis angle), respectively. While CDEC acknowledged clinical expert input that the studies suggested possible directional improvements in certain skeletal and spinal morphology measures, the committee noted that the evidence remained highly uncertain because most estimates were imprecise, follow-up was limited to 52 weeks, and the relationship between these measures and long-term patient-important outcomes (functional outcomes, HRQoL, pain, mobility, the need for corrective surgery, or ACH-related complications) had not been established. Furthermore, the study by White et al. was at high risk of bias due to selective outcome reporting because analyses were not prespecified and no a priori statistical hypotheses were reported. The study by Irving et al. was not designed to confirm efficacy and was limited by a small sample size and increased risk of prognostic imbalance between treatment groups. CDEC acknowledged clinical expert input that generating evidence on morbidity and HRQoL outcomes in ACH may be challenging, and that submitted evidence may represent the best currently available evidence for vosoritide. Nevertheless, CDEC considered the new evidence insufficient to address the gaps in evidence regarding long-term patient-important outcomes.

- **Clinical value:** During the initial and reconsideration meetings, based on all the preceding considerations, the committee was unable to determine whether vosoritide provides added clinical value compared with best supportive care alone in the population under review.



Unmet Clinical Need

- **Input on unmet clinical need:** Patients and clinicians reported that currently available therapeutic interventions primarily involve symptom management and best supportive care that could include

surgeries. Patients highlighted the need for new treatments that target the underlying cause of ACH to increase growth and reduce the risk of health complications associated with ACH, reduce the need for best supportive care, reduce spinal and leg deformities, improve mobility, prevent breathing difficulties, reduce spinal stenosis, and improve the foramen magnum size. The clinician group and the clinical experts consulted by CDA-AMC identified an unmet need for new treatments targeting the underlying cause of ACH to prevent life-threatening morbidities (e.g., foramen magnum stenosis, hydrocephalus, spinal stenosis), minimize the number of surgeries and surgical morbidity, alter body proportions, enhance functional status, and decrease the need for occupational therapy and physiotherapy.

- **Severity of the disease:** During the initial and reconsideration meetings, CDEC acknowledged input from patients and clinicians that ACH is a rare, progressive genetic disorder associated with significant symptoms that impact daily function and HRQoL. ACH complications could include musculoskeletal, respiratory, neurological, dental, and metabolic issues, often stemming from constricted bone growth, especially at the skull base and spine.
- **Availability of treatment options:** During the initial and reconsideration meetings, CDEC discussed that no therapy addressing the underlying cause of ACH is available. Available therapy is limited to best supportive care, which may include surgical interventions, occupational therapy, and physiotherapy, as needed.
- **Significant unmet clinical need:** During the initial meeting, due to challenges with evidence generation associated with the rarity of ACH and the severity of ACH despite available treatment options, CDEC considered there to be significant unmet need as described in the recommendation framework in the Procedures for Reimbursement Reviews. During the reconsideration meeting, CDEC further considered feedback from patients and clinicians, and clinical expert input which emphasized the substantial disease burden, the lack of effective alternative treatments, and the difficulties in generating evidence on morbidity and HRQoL outcomes for ACH. The committee continued to acknowledge that a significant unmet clinical need exists but upheld their initial conclusion that, even when taking the significant unmet clinical need into account, the committee was unable to conclude that vosoritide addresses the unmet need with an acceptable level of certainty in clinical value.



Distinct Social and Ethical Considerations

- **Input on unmet nonclinical need:** During the initial and reconsideration meetings, CDEC acknowledged the caregiver burden, psychological impact, and occupational impact of ACH despite best supportive therapies that were identified by patients and clinicians. CDEC also noted that there is considerable variability and disparity in access to care for ACH across the country. Current supportive treatments are mainly available in tertiary pediatric care centres. The need for travel for specialist visits can be a burden for some patients and their caregivers.
- **Equity considerations:** During the initial and reconsideration meetings, CDEC noted that many individuals with ACH regularly experience discrimination and social stigma due to their stature. They

acknowledged that while some individuals may consider demonstrated height gains with vosoritide as equity enhancing, there are others who would not choose to pursue height-altering interventions. They instead suggested that social stigma and functional and infrastructural accommodation are the issues in need of resolution.

- **Significant unmet nonclinical need or health inequity:** During the initial and reconsideration meetings, CDEC acknowledged that there is a significant nonclinical need in the patient population under review. As previously mentioned, current supportive treatments are mainly available in tertiary pediatric care centres. This can create barriers to accessing care for patients living in rural areas or for those with fewer resources who may face travel-related burdens. CDEC was unable to conclude that vosoritide addresses a significant unmet nonclinical need or health inequity to a degree that overcomes the uncertainty in clinical value and potential risks.
- **Ethical implications:** During the initial and reconsideration meetings, CDEC discussed the potential of causing unintentional social harm if vosoritide were recommended for reimbursement. Specifically, they noted concerns that funding decisions may unintentionally signal that short stature is a health problem requiring correction, creating risks of stigmatization or undermining identities for those who view short stature associated with ACH as a meaningful aspect of their identity. They acknowledged that this is particularly challenging given that the evidence evaluated for this review is focused on height-related outcomes, yet potential downstream impacts of vosoritide on medical complications or functional outcomes remain undemonstrated.



Economic Considerations

- **Deliberation on economic considerations:** During the initial meeting, the committee reviewed the economic considerations for vosoritide; however, the recommendation not to reimburse meant that further deliberation on the considerations was not required.



Impacts on Health Systems

- **Deliberation on impacts on health systems:** During the initial meeting, the committee considered the impacts on health systems when implementing vosoritide; however, the recommendation not to reimburse meant that further deliberation on measures to address these impacts was not required.

Sources of Information Used by the Committee

During the initial meeting, to make its recommendation, the committee considered the following information (links to the full documents for the review can be found on the [project web page](#)):

- the CDA-AMC review of the clinical and pharmacoeconomic evidence submitted by the sponsor as well as relevant ethical issues related to vosoritide (refer the Main Report and Supplemental Material document)

- the sponsor's comments on the draft report and the CDA-AMC responses
- patients' perspectives gathered by 1 patient group, The Chandler Project (refer to the Patient and Clinician Group Input document)
- input from 1 clinician group, Canadian Skeletal Dysplasia Group (refer to the Patient and Clinician Group Input document)
- input from public drug programs that participate in the reimbursement review process (refer to the Supplemental Material document)
- input from a panel of 4 clinical specialists with expertise in the management of ACH consulted by CDA-AMC.

Special thanks: CDA-AMC extends our special thanks to the family who presented directly to CDEC, and to the organizations representing the community of those living with ACH, including The Hospital for Sick Children and particularly Lucie Dupuis.

General note: CDA-AMC makes every attempt to engage with people with lived experience as closely to the indication under review as possible; however, at times, CDA-AMC is unable to do so and instead engages with individuals with similar treatment journeys to ensure lived experience perspectives are included and considered in Reimbursement Reviews. CDA-AMC is fortunate to be able to engage with individuals who are willing to share their treatment journeys with CDEC.

Request for Reconsideration

The sponsor filed a request for reconsideration of the draft recommendation for vosoritide to increase linear growth in patients with ACH who are 4 months of age and older whose epiphyses are not closed. In their request, the sponsor identified the following issues:

- The sponsor requested that CDEC reconsider their viewpoint that it is uncertain whether vosoritide demonstrates acceptable clinical value compared with best supportive care alone and to issue a recommendation to list vosoritide with conditions under the "significant unmet clinical need" framework based on the totality of the evidence submitted. The sponsor stated that the evidence package for vosoritide is robust and comprehensive, particularly for a rare disease.
- The sponsor requested that CDEC issue a recommendation to list vosoritide with conditions under the "significant unmet clinical need" framework. The sponsor noted that in the draft recommendation, the rarity and severity of ACH despite available treatment options were acknowledged by CDEC and that there is significant unmet need for ACH.
- The sponsor requested that CDEC reconsider how growth outcomes are interpreted in the assessment of clinical value and recognize the improvements in growth parameters provide objective evidence of treatment effect on the underlying pathophysiology of achondroplasia. The sponsor noted that the longer-term functional outcomes related to growth should be appropriately contextualized

and accepted given vosoritide demonstrated consistently positive effects within the constraints of the current maturity of the data for a rare disease.

In the meeting to discuss the sponsor's request for reconsideration, CDEC considered the following information:

- information submitted as part of the sponsor's request for reconsideration
- information from the initial submission related to the issues identified by the sponsor
- new information provided by the sponsor to address an important clear gap in the evidence identified by CDEC
- feedback from 3 clinical specialists with expertise in diagnosing and treating patients with ACH
- feedback on the draft recommendation from 4 patient groups, The Chandler Project, Canadian Organization for Rare Disorders, Fundación ALPE Acondroplasia (with European Skeletal Dysplasia Alliance SDA) and Alianza Iberoamericana de Displasias Esqueléticas, and the Little Legs Big Hearts Foundation
- feedback on the draft recommendation from 1 clinician group, Canadian Skeletal Dysplasia Group
- feedback on the draft recommendation from the public drug program that participate in the reimbursement review process
- feedback on the draft recommendation from the sponsor.

All feedback received in response to the draft recommendation is available on the CDA-AMC [project web page](#).

CDEC Information

Members of the Committee

Dr. Peter Jamieson (Chair), Dr. Kerry Mansell (Vice-Chair), Sally Bean, Daryl Bell, Dan Dunsky, Dr. Ran Goldman, Dr. Trudy Huyghebaert, Dr. Dennis Ko, Dr. Christine Leong, Dr. Alicia McCallum, Dr. Srinivas Murthy, Dr. Nicholas Myers, Dr. Krishnan Ramanathan, Dr. Marco Solmi, Carla Velastegui, Dr. Edward Xie, and Dr. Peter Zed.

Initial meeting date: February 25, 2026

Regrets: One expert committee member did not attend.

Conflicts of interest: None

Reconsideration meeting date: July 22, 2026

Regrets: One expert committee member did not attend.

Conflicts of interest: None



Canada's Drug Agency
L'Agence des médicaments du Canada
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