Vosoritide : Adis Evaluation

Clinical Considerations

- First precision therapy approved for achondroplasia
- Significantly increases annualized growth velocity and height Z-score relative to placebo, without worsening upper to lower body segment ratio
- Growth-promoting effects maintained for up to 60 months of treatment
- Generally well tolerated

Plain Language Summary

Background and rationale

- Achondroplasia is the most common form of skeletal dysplasia that is characterized by short stature with disproportionate bone growth
- The condition is caused by a gain-of-function mutation in the gene for fibroblast growth factor receptor 3 (FGFR3), which is a negative regulator of bone growth
- With increased understanding of the pathogenesis of achondroplasia, vosoritide (Voxzogo®), a modified recombinant human C-type natriuretic peptide (CNP) analogue, has been approved as the first precision therapy for the treatment of achondroplasia in patients aged ≥ 2 years with open epiphyses in the EU and those aged ≥ 5 years with open epiphyses in the USA

Clinical findings

- In children with achondroplasia, daily subcutaneous administration of vosoritide was associated with growth-promoting effects
- Vosoritide is generally well tolerated, with most adverse events being mild in severity

Conclusion

Vosoritide is a valuable, effective and generally well-tolerated treatment option for children with achondroplasia

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