Update for Achondroplasia Associations

New Drug Application Submitted for Vosoritide

BioMarin has announced the submission of a New Drug Application (NDA) to the US Food and Drug Administration (FDA) for vosoritide, an investigational medicine for achondroplasia. An investigational medicine is a drug that is being studied to see if it is safe and effective to treat a particular condition. Vosoritide has not been approved for use or determined to be safe or effective. BioMarin has submitted data that will now be reviewed by regulators who decide whether to approve the drug to be marketed in the United States.

Over 500 children with achondroplasia from 8 countries have enrolled in BioMarin clinical studies. These children and their families have been crucial to the ongoing research into achondroplasia and the safety and efficacy of vosoritide. We are incredibly grateful to everyone who participates in our clinical studies.

For additional information on BioMarin clinical studies:

- Visit www.clinicaltrials.gov and type in the study code "BMN 111"
- For inquiries or to provide feedback from advocacy organisations, please contact patientadvocacy@bmrn.com
- Contact BioMarin Medical Information at medinfoeu@bmrn.com or toll free at: 00800 742 46627