Update for Achondroplasia Associations

The European Medicines Agency Committee for Medicinal Products for Human Use gives a positive response to a treatment for achondroplasia.

What is the European Medicines Agency (EMA)?

The EMA is responsible for the approval and regulation of medicines across all European Union member states and the European Economic Area (EEA). The EMA's role is to ensure that all medicines available on the EU market are safe, effective, and of high quality.

What is the Committee for Medicinal Products for Human Use (CHMP)?

The CHMP is responsible for conducting the initial assessment of EU-wide marketing authorisation applications made by medicine developers and recommends whether or not a medicine should be granted marketing authorisation.

The CHMP determines whether the medicine meets the necessary quality, safety, and efficacy requirements and that it has a positive risk-benefit balance through an evaluation of the scientific data. The CHMP will give a positive or negative opinion on the medicine based on this scientific assessment.

If the medicine receives a positive opinion, the EMA will send a recommendation to the European Commission (EC) to approve the medicine. The EC will grant a license, called marketing authorisation, so that the company can market the medicine in all EU and EEA countries. The EMA takes approximately one year to evaluate a medicine and the EC grants marketing authorisation approximately two months after receiving the positive CHMP opinion.

The EMA regularly exchanges views on ongoing medicines' assessments with other regulatory agencies such as the US FDA, Health Canada and the Japanese regulatory authorities.

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For additional information:

- For information on BioMarin clinical studies, visit www.clinicaltrials.gov and type in the study code “BMN 111”
- For information and resources about living with achondroplasia, please visit www.achondroplasia.com
- For inquiries or to provide feedback from advocacy organizations, please contact patientadvocacy@bmrn.com
- Contact BioMarin Medical Information at medinfoeu@bmrn.com

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