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FDA explores therapeutic goals in achondroplasia

by from the Food and Drug Administration's Division of Pediatric and Maternal Health, Division of Metabolism and Endocrinology Products, and the Office of Pediatric Therapeutics

The Food and Drug Administration (FDA) is exploring the development of drugs to treat achondroplasia, an inherited short-stature skeletal dysplasia.

Children with achondroplasia are at risk for multiple complications related to abnormal bone growth, including spinal stenosis, sleep apnea, recurrent ear infections, and speech and motor developmental delays. Supportive therapies aim to prevent or treat complications of achondroplasia, but no targeted treatment exists.

The FDA held a joint meeting of the Pediatric Advisory Committee and the Endocrinologic and Metabolic Drugs Advisory Committee to discuss drug development programs. Key topics of discussion included clinically meaningful outcomes of drug therapy, age of patients who might benefit most from drug therapy, and study design and duration.

During a public hearing, 32 speakers testified, including parents and children with achondroplasia, stakeholders in the pharmaceutical industry, and patient advocacy groups. Parents and children with achondroplasia described challenges, such as the need for recurrent surgeries and medical appointments, pain, poor stamina, social stigma, issues with self-image, lack of autonomy, and the cost of adaptive equipment.

The advisory committees agreed that growth velocity, as a potential primary study endpoint, would be an objective measure of treatment effect. The committees noted that a randomized, placebo-controlled study of at least two years would be needed to provide clinically significant efficacy data for growth velocity. The committees also acknowledged that improvement of the many complications of achondroplasia should be evaluated as key secondary endpoints.

Involvement of younger patients in clinical studies was thought to be desirable because they have the greatest likelihood of benefiting from treatment. The importance of enrolling older patients to identify safety concerns also was recognized.

The FDA plans to incorporate the advisory committees' recommendations and the views expressed during the public hearing into discussions of clinical study design with the aim of improving the lives of patients and their families.

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