U.S. FOOD & DRUG ADMINISTRATION

FDA Advisory Committee Meeting on Achondroplasia

March 22, 2018 Silver Spring, MD

An opportunity to provide patient input to support development of potential pharmaceutical treatments for achondroplasia

What
On March 22, 2018, the U.S. Food and Drug Administration (FDA) will conduct a public advisory committee meeting to discuss the major objectives of a phase 3 drug development program indicated for the treatment of children with achondroplasia.

When
The meeting's open public session will take place on March 22, 2018, from 10:30 a.m. to 5:30 p.m. The opportunity for open public comment will span approximately one hour from 1:00 p.m. to 2:00 p.m.

Where
FDA White Oak Campus, 10903 New Hampshire Avenue, Silver Spring, MD 20993-0002

How
You can submit input to FDA through the open public comment period at this meeting or through the public docket in writing.

What is FDA interested in learning from patients?

We are interested in hearing anything that is important to you related to achondroplasia and the development of potential treatments. The following questions may be helpful as you prepare your oral or written comments, on behalf of yourself (as an individual with achondroplasia) or your loved one.

- What health effects of achondroplasia have the most significant impact on you/your loved one's health, functioning, and well-being? How these effects change over time through childhood and into adulthood.
- What would you/your loved one see as the possible goals of pharmaceutical treatments? In other words, what types of benefits would you like to see in your/your loved one's health and functioning?
- What considerations would factor into patients and family's decisions to whether to start a new treatment, and at what age to start treatment?
- If you had the opportunity for you/your child to participate in a clinical trial studying an experimental treatment for achondroplasia, what considerations would factor most into your decision?

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www.fda.gov

How can individuals, families, and others provide input?

Register to Provide Oral Comments at the Meeting
Anyone interested in making formal oral presentations at the meeting should contact Marieann Brill (Marieann.Brill@fda.hhs.gov or 240-402-3838) and briefly describe the type of input you would like to present.

Provide Written Comments to the Public Docket
For anyone interested in providing written comments: comments received to the public docket by March 8, 2018 will be provided to the committee for their consideration at the meeting. Don't worry if you miss the March 8 deadline. FDA will receive and consider comment submitted to the docket until March 23, 2018.

Are there other resources for this meeting?

Frequently Asked Questions
Answers to commonly asked questions including information regarding special accommodations due to a disability, visitor parking, and transportation may be accessed at https://www.fda.gov/AdvisoryCommittees/AboutAdvisoryCommittees/ucm408555.htm.

Background Materials
FDA intends to make background material available to the public no later than 2 business days before the meeting. If FDA is unable to post the background material on its website prior to the meeting, the background material will be made publicly available at the location of the advisory committee meeting, and the background material will be posted on FDA's website after the meeting. Background material is available at https://www.fda.gov/AdvisoryCommittees/Calendar/default.htm.

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The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, and products that give off electronic radiation, and for regulating tobacco products.

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