Transcript Details

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ReachMD
www.reachmd.com
info@reachmd.com
(866) 423-7849

Conference Coverage at ICCBH 2026: A New Frontier in Achondroplasia—Clinical Insights From the PROPEL 3 Trial of Oral Infigratinib in Children

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Dr. Ravi:
Hi, I'm Dr. Ravi, and it's great to be here with you from ICCBH 2026 in beautiful Montreal. And we've had some really interesting data that I presented yesterday in the late-breaking session, and that is the results from the PROPEL 3 study in young children with achondroplasia, age 3 to 18.

This was a double-blind, randomized, controlled pivotal study following children for 1 year who were either randomized to receive an oral medication called infigratinib, which is a tyrosine kinase inhibitor, or placebo. And the results have been very, very promising. It showed that in the treated children, they had a significant increase in their growth velocity and also in body proportionality in the youngest treated group, 3 to 8 years, and also that their arm span grew in commensurate fashion to their height.

So what does that mean? Well, it's very promising data, and is a big step forward towards regulatory approval of the first oral treatment for children with achondroplasia. We're hoping that the increases in height and proportionality will translate into improvements in functionality and decrease metaphysical complications for these children.

All of the children who have finished the trial will be continued on in an extension trial until they reach final adult height. So we will be able to directly measure how the improvements in proportionality and height translate to functional independence into the quality of their life and allowing basically kids to do what kids want to do.

So it's a really big step forwards. It could be a game changer in the treatment landscape for achondroplasia, and we hope that this publication in the *New England Journal of Medicine* will set the foundation for regulatory approvals, not only in the United States, but worldwide. So it's an exciting time. It's not mission accomplished, but it's a big step forward.

Thanks very much. And stay tuned for more.

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