# Therachon Receives EMA and FDA Orphan Drug Designation for TA-46 for Achondroplasia

BASEL, Switzerland--(<u>BUSINESS WIRE</u>)-- Therachon AG, a biotechnology company focused on rare genetic diseases, announced today that the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) have granted Orphan Drug Designation to TA-46 for the treatment of patients with achondroplasia, the most common form of short-limbed dwarfism.

"We are pleased that both the EMA and FDA have granted orphan drug designation to our lead pipeline program, TA-46," said Luca Santarelli, M.D., Chief Executive Officer of Therachon. "The granting of orphan drug designation by both regulatory bodies highlights the global unmet medical need for new therapies for patients with achondroplasia. We are committed to translating the promise of TA-46 into a novel medicine for children with achondroplasia and look forward to start our clinical program later this year."

The EMA orphan drug designation is a status assigned to a medicine intended for use against a rare condition. Prevalence of the condition in the European Union (EU) must not be more than five in 10,000. EMA orphan drug designation benefits include protocol assistance, reduced EU regulatory filing fees and ten years of market exclusivity. Under the U.S. Orphan Drug Act, the FDA's Office of Orphan Products Development provides special status to encourage the development of drugs for diseases affecting fewer than 200,000 people in the U.S. Orphan drug designation provides up to seven years of market exclusivity if the product is approved in the U.S. and offers various development incentives, including tax credits related to clinical trial expenses, an exemption from the FDA-user fee and FDA assistance in clinical trial design.

### **About TA-46**

TA-46 directly modulates the function of the fibroblast growth factor receptor 3 (FGFR3). The mutations of FGFR3 that cause achondroplasia result in excessive activation of this receptor, which slow down normal bone growth. TA-46 prevents the excessive activation of FGFR3 by binding to its natural ligands and preventing them from over-activating the mutated FGFR3. TA-46 has not shown any dose-limiting safety or tolerability issues in safety studies to date. TA-46 will be evaluated in children who would receive weekly subcutaneous injections to help restore normal bone growth.

## **About Achondroplasia**

Achondroplasia is a rare, genetic condition which affects about one in 15,000 children and is caused by a genetic mutation of the FGFR3, which stunts child bone growth. This condition is accompanied by life-altering neurological, orthopedic and ear, nose and throat complications. Children are typically diagnosed at birth, but increasingly parents are finding out via prenatal ultrasounds. Currently, the only available treatment option for achondroplasia is limb lengthening surgery, an extremely invasive surgical procedure that addresses height but not specific achondroplasia-associated complications.

### **About Therachon**

Therachon is a global biotechnology company focused on developing medicines for rare, genetic diseases that currently have no available treatments. The company's lead pipeline candidate, TA-46, is a novel protein therapy in development for achondroplasia, the most common form of short-limbed dwarfism. Therachon is committed to translating the promise of its science into new treatments for patients with high unmet medical needs. For more information, visit <a href="https://www.therachon.com">www.therachon.com</a>.

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