Therachon Announces the Start of a Natural History Study in Children with Achondroplasia

BASEL, Switzerland, June 21, 2018 – Therachon AG, a clinical-stage biotechnology company focused on rare diseases, today announced the start of "Dreambird", a natural history study of children with achondroplasia, the most common form of dysproportionate short stature in humans.

The "Dreambird' study will prospectively assess the burden of complications of achondroplasia in approximately 200 children across multiple sites in Europe, Canada and the United States. The study will pave the way to future interventional studies with TA-46 studies and also support the development of key biomarkers of bone tissue growth.

"Knowledge of the natural history of achondroplasia and identification of molecular biomarkers of bone growth are essential to build the scientific foundation for the forthcoming clinical development program of TA-46" said Christian Meyer, M.D., Ph.D., Chief Medical Officer. "We are grateful to the children and their families who will participate in this study."

"I'm excited to support Therachon's efforts to find a treatment for children with achondroplasia," said Professor Geert Mortier, clinical geneticist and international coordinating investigator, based at the Antwerp University Hospital, Belgium. "This study will provide additional clinical data to inform future endpoints in clinical trials and regulatory reviews with TA-46. We look forward to working together to develop TA-46 for the achondroplasia community."

TA-46 is being developed as a weekly subcutaneous drug for children and adolescents living with the condition. Enrollment in the natural history study is a pre-requisite for participation in the forthcoming interventional studies with TA-46.

Achondroplasia is a rare, genetic condition which affects approximately one in 15,000 children and is caused by a genetic mutation of the FGFR3 gene, which hinders child bone growth. Currently, the only available treatment option for achondroplasia is limb-lengthening surgery, an extremely invasive surgical procedure that addresses height but not achondroplasia-associated complications.

Details of the study will be available shortly on www.clinicaltrials.gov.

Therachon is currently conducting a Phase 1 randomized, placebo-controlled, double-blind trial to evaluate the safety, tolerability and pharmacokinetics of single and multiple increasing doses of TA-46 in approximately 70 male and female adult healthy volunteers in the Netherlands to enable clinical trial applications in early 2019.

About Therachon
Therachon is a clinical-stage global biotechnology company focused on developing medicines for rare diseases with significant unmet need. The company's lead pipeline candidate, TA-46, is a novel protein

therapy in development for achondroplasia, the most common form of dysproportionate short stature in humans. Therachon is committed to translating the promise of its science into new treatments for patients with high unmet medical needs. For more information, visit www.therachon.com.

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