Pfizer Doses First Participants as Part of Global Achondroplasia Phase 2 Development Program

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Today Pfizer announced that the first participants were dosed in the global Phase 2 multiple dose, randomized study to assesses the safety, tolerability, pharmacokinetics, and efficacy of recifercept in children with achondroplasia. Participants received a subcutaneous injection of the biologic recifercept at Antwerp University Hospital, Belgium, under the care of Professor Geert Mortier, and Hospital Vithas San José, Spain under Dr. Josep De Bergua.

“With our clinical program underway, starting simultaneously in Belgium and Spain, we look forward to advancing a potential novel differentiated medicine for children with achondroplasia who need it the most,” said Seng Cheng, Senior Vice President and Chief Scientific Officer of Pfizer’s Rare Disease Research Unit. “The initiation of the Phase 2 trial is a major step forward in our progress toward providing a potential therapy to address the varied functional, respiratory and neurological conditions associated with achondroplasia. We look forward to generating additional data as the program progresses.”

This Phase 2 study will enroll up to 63 children between 3 months and 11 years old with achondroplasia. Potential participants for this Phase 2 study will come from a subset of sites in an ongoing natural history study led by Pfizer, which seeks to understand the clinical and anthropometric characteristics of children with achondroplasia (www.clinicaltrials.gov; NCT03794609).

In addition to evaluating safety and tolerability, the phase 2 study will evaluate measurements of increase in height (growth above expected in reference population), assessments of potential impact on achondroplasia-related complications and changes in health-related quality of life. Participants will receive the study drug for 12 months. More

information about the trial and participating sites may be found at (www.clinicaltrials.gov NCT04638153). Further sites in addition to Antwerp Hospital and Hospital Vithas San José will soon begin enrolling in Europe, North America and Australia.

“We are pleased to partner with Pfizer to move this clinical study forward,” said Carmen Alonso, Director, Fundación ALPE Acondroplasia. “Children with achondroplasia lack options to address medical complications associated with their condition, and as a result this research has the potential to be lifechanging.”