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Ascendis Pharma announces top-line results for Ph3 heiGHt Trial

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Ascendis Pharma A/S, a biopharmaceutical company that utilizes its innovative TransCon technology to address unmet medical needs, has announced top-line results from its pivotal phase 3 heiGHt Trial were presented at the Endocrine Society's annual meeting, ENDO 2019, in New Orleans. Trial results demonstrated that TransCon Growth Hormone (hGH) administered once-weekly to children with pediatric growth hormone deficiency (GHD) had comparable safety and tolerability to daily Genotropin, with a significantly greater increase in annualized height velocity over the one-year study period. TransCon hGH has been designed to provide sustained release of unmodified hGH, the same growth hormone used in daily therapies, at a predictable rate over one week.

The heiGHt Trial findings were presented at ENDO 2019 by investigator Paul Thornton, M.B. B.Ch., MRCPI, a pediatric endocrinologist at Cook Children's Medical Center in Fort Worth, Texas, in an oral session.

"This is exciting news for all of those living with GHD, including our families at Cook Children's, because we have shown that a single once-weekly dose of TransCon hGH was just as safe and works as well or better than daily growth hormone," said Dr. Thornton. "Now, the heiGHt Trial has shown that children with GHD can grow effectively on one shot a week."

Pediatric GHD is a serious orphan disease characterized by short stature and metabolic abnormalities that affect overall physical and mental health. In GHD, the pituitary gland does not produce sufficient growth hormone, which is important not only for height but also for optimal bone, heart, muscle and brain development. As a result, children with GHD experience psychosocial challenges and poor quality of life, including impaired sleep and difficulty concentrating.

"Children have a short time to grow and a lifetime to live, which is why it is so important to help those with GHD have the best chance possible of growing up to achieve normal adult height and experience both good physical and mental health given the substantial psychosocial impact of the disease," said Mary Andrews, Chief Executive Officer and co-founder of the MAGIC Foundation, the global leader in endocrine health, advocacy, education, and support. "Children with GHD and their families

have waited years for a long-acting growth hormone therapy that could ultimately reduce the number of injections needed to help these children thrive. We are grateful for Ascendis' commitment to developing this new treatment option for pediatric GHD and making it a reality for patients and their families in the coming years."

Currently, in the United States and Europe, the only treatment option for pediatric GHD is a daily subcutaneous injection of hGH. While daily injections of hGH improve growth and metabolic effects, they can be associated with a high treatment burden. Patients receiving daily therapy may endure thousands of injections over the course of many years, which can lead to poor adherence and reduce overall treatment outcomes.