Specialised Therapeutics Signs Exclusive Agreement with Ascendis Pharma A/S for Distribution and Commercialisation of Three Endocrinology Therapies in Australia and Select South-East Asia Countries

- Agreement between Ascendis Pharma A/S and ST is for the exclusive distribution and commercialisation of three Ascendis Pharma endocrinology therapies
- Two of the therapies are already internationally approved, the third is in development following successful Phase 2 data
- ST's exclusive distribution agreement covers Australia, New Zealand, Singapore, Malaysia, Brunei, Thailand and Vietnam

Singapore 8 January 2024: Independent biopharmaceutical company Specialised Therapeutics Asia Pte Ltd (ST) has added three new endocrinology therapies to its specialist portfolio, following an exclusive distribution agreement with Danish company Ascendis Pharma A/S (NASDAQ: ASND).

Under the terms of the agreement, ST will commercialise Ascendis Pharma's weekly injectable paediatric human growth hormone treatment **SKYTROFA™** (lonapegsomatropin), hypoparathyroidism treatment **YORVIPATH™** (palopegteriparatide) and investigational achondroplasia therapy **TransCon™ CNP** (navepegritide).

The agreement spans ST's key regions of Australia, New Zealand, Singapore, Malaysia, Brunei, Thailand, and Vietnam.

Two of the products included in this agreement are already internationally approved:

- Once-weekly SKYTROFA is a human growth hormone (hGH) approved in the United States ***for the treatment of paediatric patients aged >1 years weighing >11.5 kg with growth failure due to inadequate secretion of endogenous growth hormone (GH)***[1] and in the European Union ***for growth failure in children and adolescents aged from 3 to 18 years due to insufficient endogenous growth hormone secretion (growth hormone deficiency [GHD]).***[2]
- YORVIPATH is a first-in-class parathyroid hormone (PTH) replacement therapy to treat chronic hypoparathyroidism, a rare and potentially serious condition where the body produces no or abnormally low levels of PTH. It is approved in the European Union ***for the treatment of adults with chronic hypoparathyroidism.***[3]

The third product – TransCon CNP – is in development by Ascendis Pharma for the treatment of achondroplasia (ACH), the most common genetic form of skeletal dysplasia and resulting disproportionate short stature, following successful Phase 2 trial results.[4]

Australian endocrinologist Dr Veronica Preda noted that YORVIPATH would be the first specialist therapeutic option for Australian patients living with hypoparathyroidism.

“Hypoparathyroidism can seriously impact quality of life and has potentially life-threatening consequences,” Dr Preda said.

“To have an option that is able to treat the underlying cause of the disease, moving beyond standard oral calcium and active Vitamin D, is a great step forward.”

Announcing the partnership, ST Chief Executive Officer Carlo Montagner said this agreement was an important company milestone, signalling ST’s expansion into both endocrinology and paediatric medicine.

Mr Montagner commented: “We are delighted to have been selected as Ascendis Pharma’s exclusive partner for commercialising their portfolio in Oceania and

these South-East Asia countries and look forward to launching these critical endocrinology products in our regions as soon as possible.

“All three products are valuable inclusions to our broad therapeutic pipeline and our international business, as we continue to leverage our substantial experience commercialising specialist medicines across multiple regions.

“We look forward to working with endocrinologists across our territories to make these endocrine therapies available to all eligible patients who may benefit.”

Ascendis Pharma Executive Vice President and Chief Commercial Officer Camilla Harder Hartvig said ST had been selected to launch the endocrinology portfolio in these countries based on its strong track record commercialising specialist products in multiple regions.

“We are delighted to partner with Specialised Therapeutics to broaden the reach of our endocrinology rare disease portfolio, contributing to our shared goal of making a meaningful difference for patients facing unmet medical needs,” she said.

Ends.

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About Specialised Therapeutics

Founded in 2007, Specialised Therapeutics is the region’s largest independent specialty pharmaceutical company, providing new therapies and technologies to patients in Australia, New Zealand and across Southeast Asia. Headquartered in Singapore, ST partners with global pharmaceutical, biotech and diagnostic companies to bring novel healthcare opportunities to patients who are impacted by a range of diseases. ST has built a strong track record of success, navigating complex regulatory, reimbursement and commercialisation environments in its diverse regions. The ST mission is to provide specialty therapies where there is an unmet need. The company’s broad therapeutic portfolio currently includes novel

agents in oncology, haematology, CNS, neurology, endocrinology, ophthalmology and supportive care, although it is not confined to these areas. ST is a member of the World Orphan Drug Alliance (WODA).

Additional information: **www.stbiopharma.com**

About Ascendis Pharma

Ascendis Pharma is applying its innovative TransCon technology platform to build a leading, fully integrated biopharma company focused on making a meaningful difference in patients' lives. Guided by its core values of patients, science and passion, the company uses its TransCon technologies to create new and potentially best-in-class therapies. Please visit https://ascendispharma.com to learn more.

About SKYTROFA (lonapegsomatropin)

SKYTROFA™ (lonapegsomatropin, developed as TransCon™ hGH) is a prodrug of somatropin, designed to provide sustained release of unmodified somatropin. The unmodified, unbound somatropin released from lonapegsomatropin has the same 191 amino acid sequence and size as endogenous GH.[5] TransCon hGH is approved and marketed as SKYTROFA (lonapegsomatropin-tcgd) in the United States[1] and as SKYTROFA (lonapegsomatropin) in the European Union[2] as a once-weekly treatment for children and adolescents with GHD.

SKYTROFA single-use, prefilled cartridges are manufactured in nine dosage strengths, allowing for convenient dosing flexibility. They are designed for use only with the SKYTROFA Auto-Injector and may be stored at room temperature for up to six months. The recommended dose of SKYTROFA for treatment-naïve patients and patients switching from daily somatropin is 0.24 mg/kg body weight, administered once weekly.[1,2] The dose may be adjusted based on the child's weight and insulin-like growth factor-1 standard deviation score (IGF-1 SDS).[2]

TransCon hGH was studied in over 300 children with GHD across the Phase 3 program, which consisted of the Height Trial[5] (for treatment-naïve patients), the Flight Trial[6] (for treatment-experienced patients), and the Enlighten Trial[7] (a long-

term extension trial). Patients who completed the Height or Flight Trials were able to continue in Enlighten, with some on lonapegsomatropin treatment for over four years.

Ascendis Pharma is also conducting the ongoing global Phase 3 Foresight Trial of TransCon hGH in adults with GHD.[8]

About Paediatric Growth Hormone Deficiency

Paediatric GHD is a serious orphan disease caused when the pituitary gland does not produce enough GH. Children with GHD are not only characterised by short stature; they also may experience metabolic abnormalities, psychosocial challenges, and an overall poor quality of life. For decades, the standard of care for GHD has been a daily subcutaneous injection of hGH to improve growth and overall endocrine health.

About YORVIPATH (palopegteriparatide)

YORVIPATH™ (palopegteriparatide, developed as TransCon™ PTH) is a once-daily prodrug with sustained release of active PTH approved by the European Union as a PTH replacement therapy for the treatment of adults with chronic hypoparathyroidism. Treatment should be initiated and monitored by physicians or qualified healthcare professionals experienced in the diagnosis and management of patients with hypoparathyroidism.[3]

TransCon PTH met all primary and key secondary endpoints in the Phase 3 Pathway Trial, *demonstrating a response rate of 78.7% compared to 4.8% for control (p-value <0.0001) for the primary composite endpoint, and statistically significant improvements compared to control on all key secondary endpoints, which included measures evaluating patient-reported disease symptoms and impacts.*[9]

About Hypoparathyroidism

Hypoparathyroidism is an endocrine disease caused by insufficient levels of PTH, the primary regulator of calcium/phosphate balance in the body, acting directly on bone and kidneys and indirectly on intestines. Hypoparathyroidism is considered chronic if it persists >6 months following surgery. Individuals with

hypoparathyroidism may experience a range of severe and potentially life-threatening short-term and long-term complications, including neuromuscular irritability, renal complications, extra-skeletal calcifications, and cognitive impairment.[9]

About TransCon CNP (navepegritide)

TransCon™ CNP (navepegritide) is an investigational long-acting prodrug of C-type natriuretic peptide (CNP), designed to provide continuous exposure of CNP at safe, therapeutic levels, via a single, weekly subcutaneous dose, for the treatment of children with ACH.[4]

The Phase 2 Accomplish Trial, a randomised, double-blind, placebo-controlled, dose-escalation trial evaluating the safety and efficacy of once-weekly TransCon CNP compared to placebo in prepubertal children with ACH aged 2 to 10 years old, met its primary objectives, and demonstrated that TransCon CNP at 100 µg/kg/week was superior to placebo for the primary efficacy endpoint of annualised growth velocity (AGV) at 52 weeks[4].

All 57 randomised children completed the blinded portion of Accomplish and are currently continuing in the open label extension at the 100 µg/kg/week dose[4]. Ascendis Pharma recently confirmed that these 57 clinical trial patients have all completed one year of treatment with TransCon CNP at 100 µg/kg/week, and announced that TransCon CNP is the first investigational product to demonstrate improvements in health-related quality of life and disease impacts in children with ACH.[10]

About Achondroplasia

Achondroplasia is the most common genetic form of skeletal dysplasia and resulting disproportionate short stature, caused by a genetic mutation in the fibroblast growth factor receptor 3 (FGFR3). This leads to an imbalance between the stimulatory and inhibitory signaling pathways involved in regulating bone growth. People living with ACH may experience serious complications and comorbidities due to inhibited skeletal development. Complications may include sleep apnoea and respiratory problems, chronic back and leg pain from lower spine impingement, and sudden infant death from compression of the brain stem.

Chronic ear infections due to eustachian tube problems can lead to hearing loss and speech delay. Children with ACH may also experience social and emotional challenges.

Reference

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