Building from a Solid Foundation: The Future of MedTech in Australia

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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.

Further enquiries:

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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.

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Patient Experience – Small Cell Lung Cancer

57 year old Lay Har has never been a smoker and has always considered herself relatively healthy, was shocked when she was diagnosed with Stage 4 Small Cell Lung Cancer in April this year, after experiencing some “discomfort” in her chest. She was scared by her diagnosis but has found support from fellow patients and the 365 Cancer Prevention Society.

Lay Har has shared her experience to mark Lung Cancer Awareness Month.

“For two days in a row, I felt some discomfort in a specific area of my chest, so I

went to a polyclinic to get an x-ray. The x-ray revealed a mass in my lungs, and I was scheduled for more scans at the hospital. It was after all these tests that I received the shocking diagnosis.

I remembered during those bad days, I was feeling scared. I was searching for more information about the cancer, when I stumbled upon the 365 Cancer Prevention Society's Facebook page. I made contact and was referred to one of their social service centres.

I was able to manage better with their help and support, which has included counselling, diet-related advice and regular check-ins from a care leader.

I was also able to speak with other patients going through similar experiences. They inspire me, because they say that overall the experience of going through has made them stronger.

These people give me hope to go on being courageous and to adopt a more positive mindset.

I am on targeted therapy, I take oral medication. I am exercising well, and eating a balanced diet.

Now I am focused on my treatment and hope I can do well and live well. I hope I can share my experience with the other cancer fighters.

It is important to keep a calm, positive attitude and stay busy with enjoyable activities.

Also remember to get enough rest.

Lung cancer can happen to anyone. It is a silent disease with almost no symptoms and is usually only detected in later stages.

I hope to see greater awareness of this cancer, so others can understand the risks.

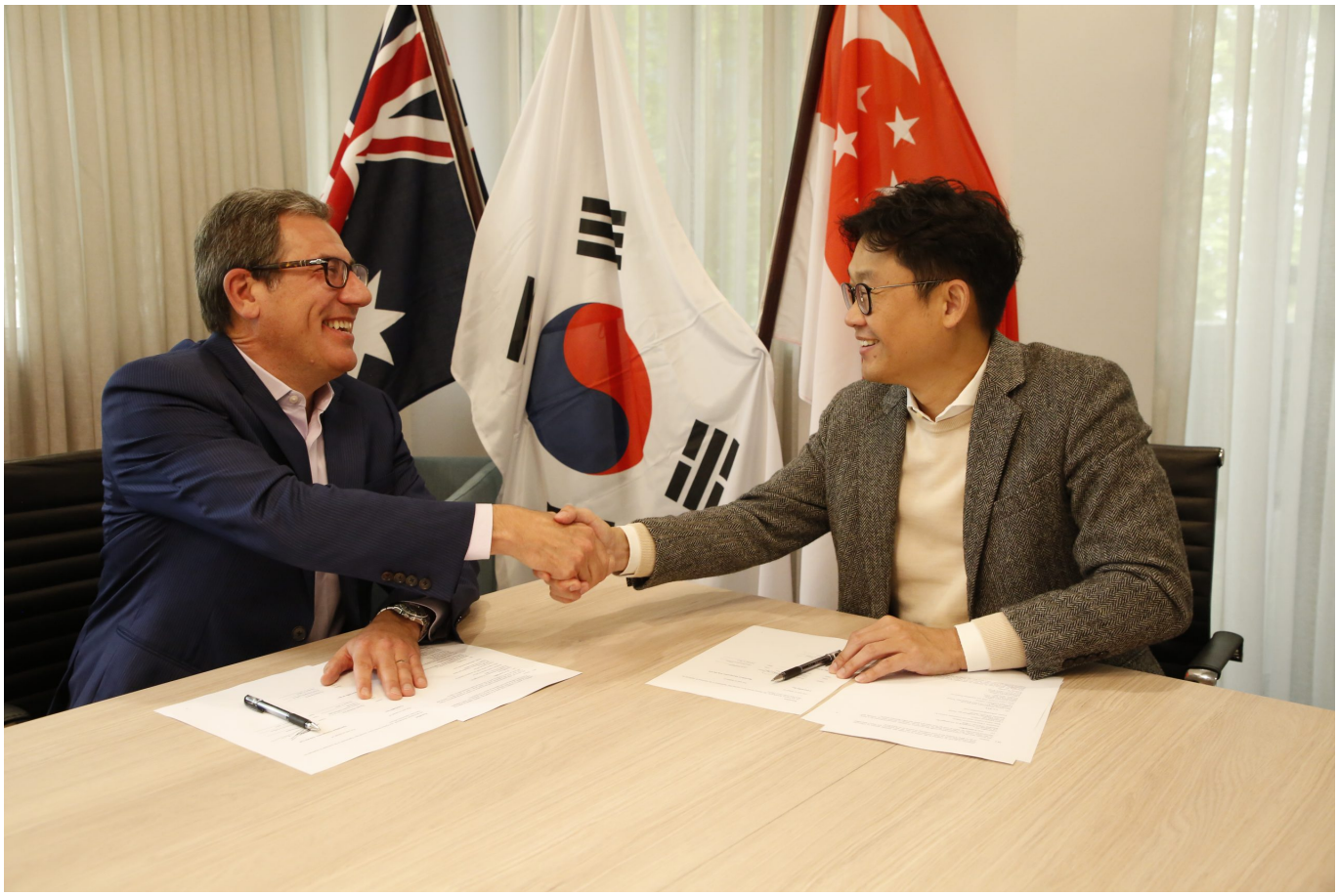
What gives me hope for the future?

I hope there are new and better treatment options to help those who are diagnosed after me."

Specialised Therapeutics Signs Exclusive License Agreement with CanariaBio for New Ovarian Cancer Therapy

- First ovarian cancer therapy for ST oncology portfolio
- Phase 2 study demonstrated oregovomab in combination with chemotherapy improved progression free survival by ~30 months compared to chemotherapy alone[1]
- Phase 3 results expected in 2025
- Exclusive license for AU, NZ, Singapore, Malaysia, Brunei, Thailand and Vietnam

Singapore and Seoul, South Korea, 13 October 2023: Independent biopharmaceutical company Specialised Therapeutics Asia Pte Ltd (ST) has signed a license deal with Korea-based CanariaBio Inc., acquiring the exclusive license to a new monoclonal antibody therapy for patients with ovarian cancer in Australia, New Zealand and in select Southeast Asian countries.



The therapy, known as oregovomab, is currently in a pivotal phase III international clinical trial known as the FLORA-5 study.[2] This investigation is examining oregovomab in combination with chemotherapy agents carboplatin and paclitaxel for patients with advanced ovarian cancer.

Under the terms of the arrangement, ST will be responsible for all commercial, medical, regulatory and distribution activities for oregovomab in its key territories of Australia, New Zealand, Singapore, Thailand, Vietnam, Brunei and Malaysia. CanariaBio will be responsible for the manufacture and supply of oregovomab to ST.

Announcing the partnership, ST Chief Executive Officer Carlo Montagner said he was pleased CanariaBio had selected ST as a partner for this highly promising therapy.

“ST has a portfolio of anti-cancer therapies targeting multiple solid tumours with the exception of ovarian cancer, and now oregovomab becomes our first ovarian cancer agent,” Mr Montagner said.

“Despite great advances in recent years, there remains a high unmet need in all

our regions to treat this patient population. We look forward to working closely with our new partners at CanariaBio and pending the results of the pivotal Phase III registration study, making oregovomab available to eligible patients."

CanariaBio Chairman and CEO Michael Na said the company had selected ST for its regional expertise and strong track record commercialising oncology products. Carlo Montagner (Oct 11, 2023 12:11 GMT+11)

"Formalising this agreement is a pivotal moment for our program. This collaboration is more than just a deal – it's a shared commitment as we develop novel therapies to address unmet medical needs. At CanariaBio, we've always believed in the transformative power of partnerships, and teaming up with ST reinforces this belief." Oregovomab works by targeting and binding specifically to a surface protein known as CA-125 found on the surface of ovarian cancer cells, then activating the patient's own immune system to respond.[3]

In the Phase 2 study, the addition of oregovomab to chemotherapy yielded a median progression-free survival of 41.8 months compared with 12.2 months with standard chemotherapy alone (HR, 0.46, P=0.0027). The overall survival hazard ratio was 0.35.1 The Phase 3 FLORA-5 study is fully enrolled and ongoing. Final results are expected in 2025.

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, ophthalmology and supportive care, although it is not confined to these areas. Additional information: www.stbiopharma.com

About CanariaBio Inc. CanariaBio Inc. is a clinical-stage biopharmaceutical company dedicated to the development and commercialization of innovative cancer biotherapeutics. CanariaBio's technology platform includes a portfolio of tumor antigen-specific monoclonal antibodies targeting CA-125, MUC1, PSA, and HER2/neu.

About Oregovomab

Oregovomab is a murine monoclonal antibody directed to the tumor-associated antigen CA-125 that stimulates a host cytotoxic immune response against tumor cells expressing CA-125, a biomarker commonly found in ovarian cancer (OC). In a randomized Phase 2 clinical trial, oregovomab demonstrated a significant improvement in progression-free and overall survival in advanced OC treatment when administered simultaneously with first-line chemotherapy. This promising schedule is currently being investigated in a Phase 3 trial.

About FLORA-5 Phase 3 Study

The Phase 3 clinical trial called FLORA-5/GOG-3035, is a double-blind, placebo-controlled, multicentre clinical study comparing the safety and efficacy of oregovomab versus placebo when administered in combination with specific cycles of a standard six-cycle chemotherapy regimen (paclitaxel and carboplatin)

for the treatment of newly diagnosed patients with advanced epithelial ovarian, fallopian tube or peritoneal carcinoma, in conjunction with optimal debulking surgical resection. The primary and secondary endpoints, for both the adjuvant and neoadjuvant cohorts of this trial, are progression free survival and overall survival, respectively. The FLORA-5 trial is being conducted in collaboration with the Gynecologic Oncology Group Foundation in the US and IQVIA (a clinical research organization). Greater China area clinical trials are conducted in collaboration with OncoVent, a Shenzhen Hepalink Pharmaceuticals Group Company in China, which is also the commercialization license holder of oregovomab for China. Information on the clinical trial can be found on www.clinicaltrials.gov

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Breast Cancer Awareness Month: Meet the Specialist

Consultant Clinical Oncologist Dr Aqilah Othman says an ageing population, fewer and later pregnancies, a lack of physical activity, obesity and poor dietary habits may be contributing to a rising incidence of new breast cancer cases across Southeast Asia.

To mark Breast Cancer Awareness Month 2023, the Sunway Medical Centre oncologist discusses the incidence of breast cancer in Malaysia as well as the risk and fear of recurrence.

Q: How common is breast cancer in Malaysia?

A: According to the Malaysia National Cancer Registry Report 2012-2016 there were 13,485 breast cancer cases, with an approximate lifetime risk of 1 in 20 women.[1]

The number of breast cancer cases is projected to spike in the near future, and we believe that this could be due to an ageing population, as well as other risk factors including not having children or late pregnancies, a lack of physical activity, obesity, and poor diets, with the consumption of highly processed foods and unregulated supplements.

Q: What are the chances of a cancer recurrence following the original breast cancer diagnosis?

A: Up to 10% of women with early breast cancer will have a recurrence after treatment.[2]

The risk is higher for those with genetic mutations or those who have had suboptimal treatment. The biology of the tumour itself – for example, if it is HER2 positive or triple negative – also increases the risk of recurrence. We know that between 30 and 50% of HER2 positive breast cancer patients will relapse within 10 years without HER2 targeted treatment.

Q: Is the fear of recurrence almost universal and how can this be addressed?

A: Many women who have been treated for breast cancer are afraid that the cancer is still there, or that it will relapse. This is normal and I think it is a reasonable worry.

However, if this fear becomes an obsession or interferes with normal life activities then this is a serious problem that needs to be addressed.

Women should be assured that they have done whatever they can within their limits by pursuing and completing all recommended treatments prescribed by

their oncologists, as well as modifying other risk factors, for example, increasing physical activity, adopting a healthier diet and lifestyle and paying attention to their mental and emotional wellbeing.

Some identified distraction strategies like work, exercise, meditation or praying can help to curb negative thoughts as soon as they start.

Support from family, friends, support groups and therapists can also help, but I find that many times it is the acceptance of the diagnosis by the individuals themselves, knowing that they are not the only ones and interacting with others who are in similar situations is effective.

What are the median survival rates of breast cancer in Southeast Asia?

A: There are big differences within and between each Southeast Asian country, however it is known that there are more late presentations, less treatment uptake and adherence leading to poorer outcomes in this region, compared to our western counterparts.

In some regions there is simply a lack of resources to identify and treat cancer optimally.

On the other hand, there are regions that have underutilised state of the art facilities and have less than ideal uptake of early detection programmes or timely treatment.

Q: Why is this the case?

A: Barriers include the inability to recognise or acknowledge symptoms, poor decision-making skills, lack of self and body empowerment, belief in alternative therapies and perceived cancer fatalism. Some patients believe that death is inevitable when cancer is diagnosed, and this can lead to delayed treatment even when diagnosed early.

Q: Why should women in Southeast Asia be vigilant so cancer is detected

early?

A: Early detection and timely access to optimal treatments can save lives and improve outcomes. There is still a long way to go in integrating the psychosocial and cultural beliefs of the local population. We must continue to dispel myths, build awareness and address these to close the cancer gap.

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World Orphan Drug Alliance Welcomes Specialised Therapeutics

- ***SEA's largest independent specialty pharma company Specialised Therapeutics (ST) has joined global pharma consortium committed to collaborating to provide new specialist medicines for rare diseases***
- ***ST to represent WODA in Australia, New Zealand and across Southeast Asia (ANZSEA)***
- ***World Orphan Drug Alliance (WODA) now spans 152 countries.***
- ***WODA offers a 'one-stop' solution for biotech companies seeking to commercialise products across global markets.***

Dubai, Moscow, Ljubljana, Sao Paulo, Zurich, Shanghai, Singapore,

September 8, 2023 – Independent biopharmaceutical company Specialised Therapeutics (ST) has joined World Orphan Drug Alliance (WODA), an international consortium of pharmaceutical companies, established to improve patient access to new treatments for rare diseases.

WODA operates by identifying biotech and pharmaceutical companies with new therapies for treating rare diseases that may not be available to patients in many regions and providing these companies with the opportunity to commercialise their novel therapies in member countries.

WODA Chairman Patrick Jordan commented: *"It's truly inspiring and exciting to witness the alliance's expansion. WODA's commercial presence has now extended to an impressive number of 152 markets covered by nine like-minded pharmaceutical companies, providing extensive global outreach with a local focus. Our members are experienced and high-performance commercialisation companies, each being an expert in their own region."*

He added: *"Through seamless collaboration among our members, we provide our partners with a single platform for full commercialisation of medicines, tailored to both partner and product needs."*

Specialised Therapeutics CEO Carlo Montagner said WODA's mission to address the unmet medical needs of local communities with novel therapies strongly aligned with ST's vision.

"We firmly believe that patients in our regions should have access to the same innovative treatments as patients have in larger markets like the US and Europe," Mr. Montagner commented.

"We now look forward to working with our WODA peers to ensure timely and equitable access to new therapies that may improve outcomes. I am confident that our WODA membership will further expand our capabilities, enabling us to provide additional therapies where there is an unmet need."

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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, ophthalmology and supportive care, although it is not confined to these areas.

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

About WODA

The World Orphan Drug Alliance (WODA) is a global alliance of commercial distributors focused on providing access to treatments for rare diseases and specialty medicines in complex and underserved markets around the world. WODA aims to provide comprehensive support to pharmaceutical and biotech companies with rare disease, oncology, and highly specialized therapeutics portfolio, starting from Named Patient Programs through to full commercialization.

Additional information**: www.woda-alliance.com**

About other WODA members

- **EffRx Pharmaceuticals** is a Switzerland based company focused on the late-stage development and commercialization of prescription medications for niche and orphan indications.
- **Medis** based in Slovenia is the commercialization partner of choice for innovative pharmaceutical and biotech companies seeking strong business growth in Central and Eastern Europe.
- **Orpharm** is a Moscow based full-service distributor covering Russia and the Commonwealth of Independent States (CIS).
- **OrphanDC** based in Brazil acts as a partner for biotech companies in

Latin America. They focus on supporting their clients from the clinical development stage throughout the product lifecycle.

- **Vector Pharma** is a Dubai based full-service distributor covering Middle East, North Africa and Turkey.
- The Greater China region is covered by **RareStone Group**, which aims to become the leading company supporting the rare disease community in China.
- Founded in 1921, **CTS** is among Israel's leading pharmaceutical companies and is well-known for its strong capabilities in local access, distribution, and marketing of high-end therapies.
- **Path Pharma** is a full-service distributor in Greece, Cyprus, and Malta. Founded by industry experts, Path Pharma has expertise in local market access, medical support, and marketing and sales, with focus on rare diseases, highly specialized therapeutics, and oncology.

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Specialised Therapeutics Acquires Commercialisation Rights to New

Oral MND Therapy

Singapore and Tilburg, Netherlands, August 28 2023: Independent biopharmaceutical company Specialised Therapeutics Asia Pte Ltd (ST) will partner with Netherlands based biotechnology company Treeway BV to commercialise a new therapy to treat Amyotrophic Lateral Sclerosis (ALS) – the most common form of Motor Neurone Disease (MND) – in Australia and New Zealand.

The therapy is known as TW001 and is a unique oral formulation of edaravone which works by reducing the oxidative damage associated with neuron death in ALS.[1] TW001 is currently being evaluated in the pivotal ADORE phase III registration study at almost 40 global sites.[2]

Australian neurologist Associate Professor Susan Mathers said around 2000 people were living with MND at any one time in Australia, and an oral therapy like edaravone presented the opportunity for patients to be managed at home.

Associate Professor Mathers commented: “Better disease modifying therapies are urgently needed to slow and potentially halt this disease. Oral therapies like edaravone present the opportunity for a simple to manage therapy which can be taken at home and monitored through each person’s local health care provider.”

And key patient advocacy body MND Australia is also welcoming the potential for this new oral treatment option.

Executive Director, Research Gethin Thomas commented: “Oral edaravone would complement the recent approval of intravenous edaravone in Australia and broaden the patient base able to access treatment.”

Under the terms of the licensing agreement, ST will be responsible for all marketing, regulatory and distribution activities of TW001 for ALS/MND in Australia and New Zealand.
Announcing the partnership, ST Chief Executive Officer Carlo Montagner said TW001 was the first central nervous system (CNS) therapy to be included in the company’s therapeutic portfolio and the arrangement was further endorsement of ST’s regional capabilities and focus on making available in this region unique

therapies that would otherwise not be accessible.

“We are delighted to partner with Treeway as this promising treatment progresses through the final stages of the pivotal global registration ADORE study,” he said. “We look forward to working with the wider MND community, who are determined to access new therapies to treat this terrible disease. “While there is still no cure for MND, we remain hopeful that new therapies such as TW001 may help to slow disease progression and improve outcomes.”

Treeway CEO Inez de Greef commented: “This important therapy has shown very encouraging results in all studies to date. We look forward to further results from the ADORE study and then working with ST to make our therapy available for all eligible patients in Australia and New Zealand who may benefit. ST is focussing on bringing new therapies to the market for diseases with a high medical need and therefore ST fits well as a licensing partner for Treeway.”

Ends.

About Specialised Therapeutics

Headquartered in Singapore, Specialised Therapeutics Asia Pte Ltd (ST) is an international biopharmaceutical company established to commercialise new therapies and technologies to patients throughout Southeast Asia, as well as in Australia and New Zealand. ST and its regional affiliates collaborate with leading global pharmaceutical and diagnostic companies to bring novel, innovative and life-changing healthcare solutions to patients affected by a range of diseases. Its mission is to provide therapies where there is an unmet need. The company’s broad therapeutic portfolio currently includes novel agents in oncology, haematology, neurology, ophthalmology and supportive care. Additional information can be found at www.stbiopharma.com.

About Treeway

Treeway is a clinical-stage biotechnology company with a mission to develop therapies to cure ALS and other neurodegenerative diseases. Founded in 2012 by two ALS patients, Treeway is commited to developing the neurodegenerative

disease drugs of tomorrow. Treeway's research and development portfolio has a strong focus on ALS and Alzheimer's Disease and is continuously looking to expand the therapeutic targets within the neurodegenerative diseases arena.

Treeway, Therapy development inspired by patients

www.treeway.nl

About ALS[3]

Amyotrophic Lateral Sclerosis (ALS), the most frequent motor neuron disease, is a progressive neurodegenerative disease of motor neurons in the brain and spinal cord, resulting in progressive paralysis, with death typically within 2 to 5 years of diagnosis.

ALS is a rare disease that typically occurs in people between 40-70 years old, slightly more men than women. It is caused by a multitude of factors: 10-15% of cases may have a genetic/family link, while 85-90% are considered sporadic, with no known cause.

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The following story appeared on 7NEWS August 2023.

When nature delivers new medicine: Our soft tissue sarcoma therapy Yondelis® (trabectedin) has this month been listed on the Pharmaceutical Benefits Scheme for patients with advanced liposarcoma and leiomyosarcoma, but you may be surprised where scientists originally sourced this novel compound.

Global Sarcoma Therapy Now Listed on Pharmaceutical Benefits Scheme

- *YONDELIS® (trabectedin) now PBS listed for Australian patients*
- *Listing described as "wonderful news" for patients living with rare lipo and leiomyo sarcomas*
- *YONDELIS® (trabectedin) demonstrates 45% reduction in risk of disease progression or death versus dacarbazine*[1]

Singapore, 31 July 2023: AUSTRALIAN cancer patients who have been diagnosed with rare soft tissue sarcomas will now have affordable access to a global therapy shown to improve survival, following its listing on the Pharmaceutical Benefits Scheme (PBS).

The therapy YONDELIS® (trabectedin) is a novel anti-tumour agent originally derived from the sea squirt and will be available to eligible patients on the PBS from **August 1.**

It is used extensively around the world and has been shown to improve progression-free survival for patients with liposarcoma and leiomyosarcoma when used after anthracycline-based therapy.[1]

Until today, some patients have paid up to $50,000 to access YONDELIS treatment.

News of the PBS listing is being welcomed by oncologists and the Australian sarcoma community, who say it will alleviate cost of treatment pressures for those patients whose disease has progressed.

Medical oncologist and Scientific Advisory Committee member and Lead of the ANZSA National Sarcoma Database Dr Susie Bae, said YONDELIS has been available in Europe since 2007 for patients with advanced soft tissue sarcoma, and Australian patients had waited many years for reimbursed access.

“This milestone means patients don’t need to worry about not being able to afford or miss out on an active drug that can potentially buy precious time with their loved ones, by providing disease control and keeping symptoms at bay for longer,” Dr Bae said.

Melbourne patient advocate and mother of two Karen Lurati – herself diagnosed with liposarcoma six years ago – said this listing provided new hope for other patients.

“A PBS listing for YONDELIS is so exciting for those people who may not have been able to afford the treatment before,” she said. “Rare cancers don’t often get (Government) funding or attention. To now have this therapy on the PBS is great

progress.

“Patients often feel that they have to go overseas and spend enormous amounts of money on treatments that may not be available in Australia. This can be frustrating and financially crippling. So, for patients to have access to a global therapy in their own country is wonderful news.”

And Rare Cancers Australia (RCA) Chief Executive Richard Vines said he was “delighted with this outcome”, describing the listing as “great news” for patients living with an L-sarcoma.

“For too long, sarcoma patients have been unable to access all therapies which may provide benefit,” he said. Today’s announcement means they can access a PBS funded medicine instead of having to try and find tens of thousands of dollars – if not more – to self-fund a treatment that may give them more time.”

YONDELIS is marketed in Australia by independent pharmaceutical company Specialised Therapeutics, under an exclusive license arrangement with international partner PharmaMar.

ST Chief Executive Officer Carlo Montagner said the PBS listing was a significant milestone for the company.

He commented: “We acquired the YONDELIS rights in 2019 following requests from key oncology groups and doctors, who had been importing the product at great cost and with complex logistics for those patients diagnosed with these rare cancers.

“This PBS listing is the culmination of a substantive effort by our team together with the oncology community to achieve full regulatory approval and a PBS listing.

“We look forward to continuing our work with the sarcoma community.”

Ends.

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About Specialised Therapeutics Asia

Headquartered in Singapore, Specialised Therapeutics (ST) is an international biopharmaceutical company established to commercialise new therapies and technologies to patients in Australia, New Zealand and across South-East Asia. ST and its regional affiliates collaborate with leading global pharmaceutical and diagnostic companies to bring novel, innovative and life-changing healthcare solutions to patients affected by a range of diseases. Our mission is to provide therapies that would otherwise not be available to communities in our regions. The company's broad therapeutic portfolio currently includes novel agents in oncology, haematology, neurology, ophthalmology and supportive care. Additional information can be found at www.stbiopharma.com

About YONDELIS® (trabectedin)

YONDELIS® (trabectedin) is a novel, multimodal, synthetically produced antitumor agent, originally derived from the sea squirt*, Ecteinascidia turbinata*. The anti-cancer medicine works by preventing tumor cells from multiplying and is approved in 76 countries in North America, Europe, South America and Asia for the treatment of advanced soft-tissue sarcomas as a single-agent, and in 69 countries for relapsed ovarian in combination with doxorubicin HCl liposome injection.

The approval was based on the results of a pivotal phase 3, randomised, open-label controlled study which evaluated YONDELIS versus dacarbazine in over 500 patients with unresectable or metastatic liposarcoma (LPS) or leiomyosarcoma (LMS) previously treated with an anthracycline and at least one additional chemotherapy regimen. LPS and LMS are subtypes of soft tissue sarcoma (STS) and represent more than 35% of all STS cases.[3]

The median progression-free survival (PFS) among the YONDELIS treatment group was 4.2 months compared to 1.5 months in the dacarbazine treatment group, representing a 45% reduction in the risk of disease progression or death with YONDELIS (HR=0.55; 95% CI: 0.44 – 0.70; $p<0.001$).[1]

Among the 340 patients who received YONDELIS and were included in the safety analysis in the randomised trial, the most common (≥20%) adverse reactions were nausea (73%), fatigue (67%), vomiting (44%), constipation (36%), decreased appetite (34%), diarrhoea (34%), dyspnoea (25%), peripheral oedema (24%) and headache (23%). The most common (≥20%) laboratory abnormalities were neutropenia (49%), increased alanine transaminase (ALT) (45%), anaemia (39%), increased aspartate aminotransferase (AST) (35%), thrombocytopaenia (30%) and increased blood alkaline phosphatase (20%).[1]

About Soft Tissue Sarcoma

Soft tissue sarcoma is a rare type of cancer that forms as a painless lump (tumour) in any one of the soft tissues connecting all the organs and body structures – including fat, muscle, nerves, deep skin tissue, blood vessels and the tissue surrounding joints (synovial tissue). Soft tissue sarcomas commonly develop in the thigh, shoulder and pelvis and may sometimes develop in the abdomen or chest.[6]

Metastatic or locally advanced STS is generally considered incurable, with the mainstay of treatment being systemic chemotherapy. For some patients with limited disease burden however, long-term remission can be achieved through a multimodality approach involving medical, surgical and radiation therapy.[4]

About PharmaMar

PharmaMar is a biopharmaceutical company focused on the research and development of new oncology treatments, whose mission is to improve the healthcare outcomes of patients afflicted by serious diseases with our innovative medicines. The Company is inspired by the sea, driven by science, and motivated by patients with serious diseases to improve their lives by delivering novel medicines to them. PharmaMar intends to continue to be the world leader in marine medicinal discovery, development and innovation.

PharmaMar has developed and now commercializes Yondelis® in Europe by itself,

as well as Zepzelca® (lurbinectedin), in the US; and Aplidin® (plitidepsin), in Australia, with different partners. In addition, it has a pipeline of drug candidates and a robust R&D oncology program. PharmaMar has other clinical-stage programs under development for several types of solid cancers: lurbinectedin, ecubectedin, PM534 and PM54. It also has a preclinical and clinical program in virology. Headquartered in Madrid (Spain), PharmaMar has subsidiaries in Germany, France, Italy, Belgium, Austria, Switzerland and The United States. PharmaMar also wholly owns Sylentis, a company dedicated to researching therapeutic applications of gene silencing (RNAi). To learn more about PharmaMar, please visit us at www.pharmamar.com.

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