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

ST has featured in the latest MedTech Sector report published by financial services and corporate advisory firm RSM. Turn to pages 16 and 17 where CEO Carlo Montagner discusses our business model, licensing strategy and future plans. Click on the magazine below to read the article.


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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.**

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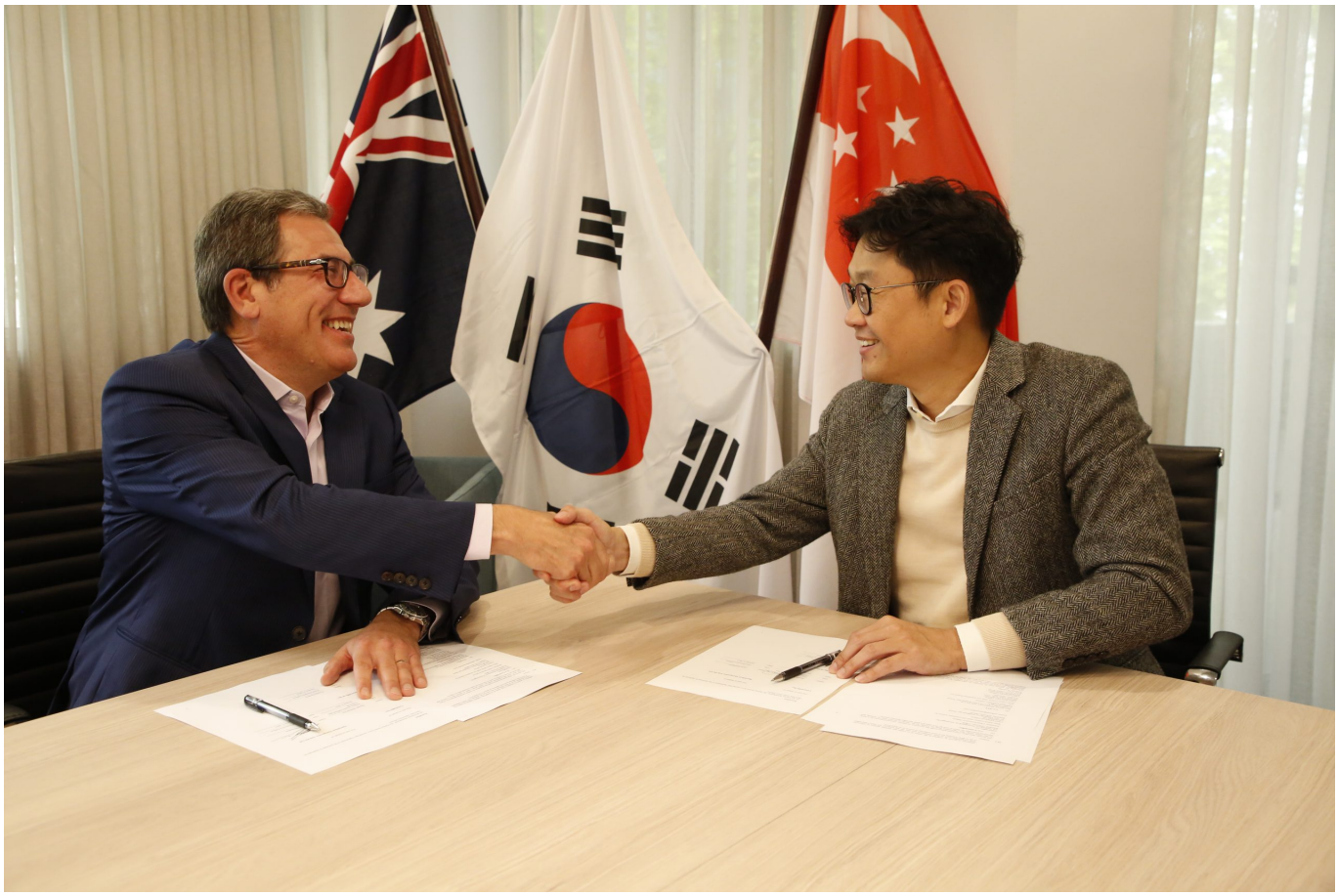
# Specialised Therapeutics Signs Exclusive License Agreement with CanariaBio for New Ovarian Cancer Therapy

- 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

**References:**


1. Brewer M, et al. Gynecol Oncol. 2020.156(3):523-529
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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.

**References:**

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# ST to Commercialise New Anti-PD1 Antibody

Specialised Therapeutics Asia will partner with CTTQ-Akeso to commercialise a new immuno-oncology therapy in Australia, Singapore and across Southeast Asia.

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# Specialised Therapeutics Enters into a New Supply and Distribution Agreement with Incyte to Launch Two New Cancer Therapies

**Singapore, 22 October 2021:** Independent pharmaceutical company Specialised Therapeutics Asia Pte Ltd (ST) will partner with Incyte Biosciences International Sàrl, the Swiss-based affiliate of Incyte (NASDAQ:INCY), to launch and distribute two new medicines for its haematology and oncology portfolios, tafasitamab (sold as Monjuvi® in the United States and Minjuvi® in Europe) and pemigatinib (Pemazyre®).

Under the terms of the agreement, Incyte will be responsible for the development, manufacture and supply of both products and ST will be responsible for regulatory, distribution and local marketing related activities in Australia, New Zealand and Singapore.

Pemigatinib is approved in the United States, Europe and Japan for the treatment of adult patients with locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement that have progressed after at least one prior line of systemic therapy.

Tafasitamab in combination with lenalidomide is approved in the United States and Europe for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) who are not eligible for autologous stem cell transplant (ASCT).

ST Chief Executive Officer Mr Carlo Montagner said the new products were synergistic with the company's strong oncology and haematology portfolios, and the new agreement was further endorsement of ST's regional capabilities.

"We are proud to have been selected to partner with a world-leading biotech of Incyte's calibre and look forward to these important products in our key regions," he said.

“Both pemigatinib and tafasitamab address strong unmet needs in rare patient populations. We have extensive experience and a successful track record of working with clinicians and other stakeholders to bring innovative therapies to small patient populations where there is high unmet clinical need. Our teams look forward to working closely with Incyte to ensure all eligible patients have access to these therapies at the earliest opportunity.”

Incyte CEO Hervé Hoppenot said the latest collaboration and partnership provided an important strategic opportunity to further serve the global oncology community, offering innovative new medicines to patients with high unmet needs in Australia, New Zealand and Singapore.

“ST’s expertise in these regions, navigating complex regulatory channels to bring new therapies and technologies to patients with rare cancers, is complementary to our own commitment to positively impact the lives of patients with serious unmet medical needs,” he said. “We look forward to a successful and mutually beneficial partnership, working together with a shared goal of improving patient outcomes.”

Regulatory activities for both products are currently in progress.

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 South-East 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 Tafasitamab

Tafasitamab is a humanized Fc-modified cytolytic CD19 targeting monoclonal antibody. In 2010, MorphoSys licensed exclusive worldwide rights to develop and commercialize tafasitamab from Xencor, Inc. Tafasitamab incorporates an XmAb® engineered Fc domain, which mediates B-cell lysis through apoptosis and immune effector mechanism including antibody-dependent cell-mediated cytotoxicity (ADCC) and antibody-dependent cellular phagocytosis (ADCP).

In January 2020, MorphoSys and Incyte entered into a Collaboration and License agreement to further develop and commercialize tafasitamab globally. Monjuvi® is being co-commercialized by Incyte and MorphoSys in the United States. Incyte has exclusive commercialization rights outside the United States.

In the United States, Monjuvi® (tafasitamab-cxix) is approved by the U.S. Food and Drug Administration in combination with lenalidomide for the treatment of adult patients with relapsed or refractory DLBCL not otherwise specified, including DLBCL arising from low grade lymphoma, and who are not eligible for autologous stem cell transplant (ASCT). This indication is approved under accelerated approval based on overall response rate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

In Europe, Minjuvi® (tafasitamab) received conditional approval, in combination with lenalidomide, followed by Minjuvi monotherapy, for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) who are not eligible for autologous stem cell transplant (ASCT).

Tafasitamab is being clinically investigated as a therapeutic option in B-cell malignancies in several ongoing combination trials.

 Tafasitamab is co-marketed by Incyte and MorphoSys under the brand name Monjuvi® in the U.S., and marketed by Incyte under the brand name Minjuvi® in the EU.

XmAb® is a trademark of Xencor, Inc.

# About Pemigatinib

Pemigatinib (Pemazyre®) is a kinase inhibitor indicated in the United States for the treatment of adults with previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or other rearrangement as detected by an FDA-approved test. This indication is approved under accelerated approval based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

In Japan, Pemazyre is approved for the treatment of patients with unresectable biliary tract cancer (BTC) with a fibroblast growth factor receptor 2 (FGFR2) fusion gene, worsening after cancer chemotherapy.

In Europe, Pemazyre is approved for the treatment of adults with locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement that have progressed after at least one prior line of systemic therapy.

Pemazyre is a potent, selective, oral inhibitor of FGFR isoforms 1, 2 and 3 that, in preclinical studies, has demonstrated selective pharmacologic activity against cancer cells with FGFR alterations.

Pemazyre is marketed by Incyte in the United States, Europe and Japan. Incyte has established various license or distribution agreements for Pemazyre in certain geographies and retains all other rights to develop and commercialize pemigatinib outside of the United States.

Pemazyre is a trademark of Incyte.

## Further Enquiries

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# New Drug for Gastrointestinal Stromal Tumours (GIST) to be Launched in Australia, New Zealand and South East Asia Following Distribution Agreement

**Singapore, 06 November 2020:** A NEW therapy to treat advanced gastrointestinal stromal tumours (GIST) will be available to patients in Australia, New Zealand and in some parts of South East Asia, following an exclusive distribution agreement.

Independent pharmaceutical company Specialised Therapeutics Asia (STA) has signed an agreement with US-based Deciphera Pharmaceuticals, Inc. (NASDAQ: DCPH) to commercialise the switch-control tyrosine kinase inhibitor QINLOCK (ripretinib) in key regions, including Australia, New Zealand, Singapore, Malaysia and Brunei.

The therapy was one of the first approved by Australia's Therapeutic Goods Administration (TGA) earlier this year under Project Orbis, which enables concurrent review of oncology products by international regulators, including the TGA, FDA and Health Canada.

It is indicated **"for the treatment of adult patients with advanced gastrointestinal stromal tumours (GIST) who have received prior**

**treatment with three or more kinase inhibitors, including imatinib"**.

QINLOCK has also been approved by the US Food and Drug Administration (FDA) and Health Canada (HC) for the fourth-line treatment of GIST.

The TGA approval was based on efficacy results from the pivotal global Phase 3 INVICTUS study in patients with advanced GIST as well as combined safety results from INVICTUS and the Phase 1 study of QINLOCK. In INVICTUS, QINLOCK demonstrated a median progression-free survival of 6.3 months compared to 1.0 month in the placebo arm and significantly reduced the risk of disease progression or death by 85% (hazard ratio of 0.15; 95% CI 0.09-0.25; $p<0.0001$). In addition, QINLOCK demonstrated a median overall survival of 15.1 months compared to 6.6 months in the placebo arm and reduced the risk of death by 64% (hazard ratio of 0.36; 95% CI 0.21-0.62).[1]

One of the INVICTUS study authors, Professor John Zalcberg who holds the Tony Charlton Chair of Oncology and is Head of the Cancer Research Program in the School of Public Health at Monash University as well as a consultant medical oncologist at Alfred Health, described QINLOCK as an important new agent in the GIST treatment armamentarium, noting it was the first TGA approved fourth-line therapy to treat the disease.

"QINLOCK represents another step forward to improve outcomes for patients who are affected by this rare cancer," Professor Zalcberg said.

"This is an area of high unmet need because of the poor prognosis of patients whose tumours continue to grow on prior treatment.

"We are further encouraged by data demonstrating that QINLOCK is well-tolerated, with patient-reported outcomes (PROs) suggesting that patients who received QINLOCK therapy in the INVICTUS study were able to maintain their quality of life in contrast to the fact that quality of life deteriorated in patients not receiving QINLOCK."

STA Chief Executive Officer Carlo Montagner said QINLOCK would bolster the company's already-robust oncology portfolio, and was synergistic with its mission to address areas of unmet clinical need.

"We are thrilled to introduce this valuable therapy to patients with GIST in our

region, working in collaboration with our new international partner, Deciphera Pharmaceuticals," Mr Montagner said.

"STA will expedite access to this important medicine, with a Patient Access Program to open in Q1 2021. This will provide subsidised access for appropriate patients at the earliest opportunity, as we file for additional regulatory approvals in other key markets, including New Zealand, Singapore and Malaysia."

Deciphera President and Chief Executive Officer Mr Steve Hoerter commented: "We are committed to ensuring QINLOCK's global commercial availability and are proud to be executing on our plan to deliver this important medicine to patients with advanced GIST worldwide.

"We look forward to collaborating with STA as we bring a much-needed therapeutic option to patients living in locations where we do not anticipate setting up our own commercial activities near term."

A submission to have QINLOCK reimbursed for eligible Australian patients has been lodged with the Pharmaceutical Benefits Advisory Committee in November for consideration at the March 2021 meeting. If successful, QINLOCK could be reimbursed for Australian patients in the latter half of 2021.

**Ends.**

**Further inquiries: STA Senior Manager Communications and Corporate Affairs Emma Power +61 419 149 525.**

# About Specialised Therapeutics Asia

Specialised Therapeutics Asia Pte Ltd (ST Asia) is an international biopharmaceutical company established to provide pioneering healthcare solutions to patients throughout South East Asia, as well as in Australia and New Zealand.

ST Asia 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. ST Asia is committed to

making new and novel therapies available to patients around the world targeting diseases where there remain unmet medical needs. STA's broad therapeutic portfolio currently includes novel agents in oncology, haematology, neurology, ophthalmology and supportive care.

Additional information can be found at www.STAbiopharma.com

# About Deciphera Pharmaceuticals

Deciphera is a biopharmaceutical company focused on discovering, developing and commercializing important new medicines to improve the lives of people with cancer. Deciphera is leveraging its proprietary switch-control kinase inhibitor platform and deep expertise in kinase biology to develop a broad portfolio of innovative medicines. In addition to advancing multiple product candidates from the company's platform in clinical studies, QINLOCK is Deciphera's FDA-approved switch-control kinase inhibitor for the treatment of fourth-line gastrointestinal stromal tumor (GIST). QINLOCK is also approved for fourth-line GIST in Canada and Australia. For more information, visit www.deciphera.com and follow the company on LinkedIn and Twitter (@Deciphera).

# About GIST

Gastrointestinal stromal tumor (GIST) is a cancer affecting the digestive tract or nearby structures within the abdomen, most often presenting in the stomach or small intestine. GIST is the most common sarcoma of the gastrointestinal tract, with approximately 4,000 to 6,000 new GIST cases each year in the United States and a similar incidence rate in European and other countries. Most cases of GIST are driven by a spectrum of mutations. The most common primary mutations are in KIT kinase, representing approximately 80% of cases, or in PDGFRα kinase, representing approximately 6% of cases. Current therapies are unable to inhibit the full spectrum of primary and secondary mutations, which drives resistance and disease progression. Estimates for 5-year survival range from 48% to 90%,

depending on the stage of the disease at diagnosis.

# About the INVICTUS Phase 3 Study

INVICTUS is a Phase 3 randomized, double-blind, placebo-controlled, international, multicenter clinical study evaluating the safety, tolerability, and efficacy of QINLOCK compared to placebo in patients with advanced GIST whose previous therapies have included imatinib, sunitinib, and regorafenib. Patients were randomized 2:1 to either 150 mg of QINLOCK or placebo once daily. The primary efficacy endpoint is progression-free survival (PFS) as determined by independent radiologic review using modified Response Evaluation Criteria in Solid Tumors (RECIST). The median PFS in the study was 6.3 months compared to 1.0 month in the placebo arm and significantly reduced the risk of disease progression or death by 85% (hazard ratio of 0.15, $p<0.0001$). Secondary endpoints as determined by independent radiologic review using modified RECIST include Objective Response Rate (ORR) and Overall Survival (OS). QINLOCK demonstrated an ORR of 9.4% compared with 0% for placebo ($p=0.0504$). QINLOCK also demonstrated a median OS of 15.1 months compared to 6.6 months in the placebo arm and reduced the risk of death by 64% (hazard ratio of 0.36).

# About QINLOCK (ripretinib)Specialised

QINLOCK is a switch-control tyrosine kinase inhibitor that was engineered to broadly inhibit KIT and PDGFRα mutated kinases by using a dual mechanism of action that regulates the kinase switch pocket and activation loop. QINLOCK inhibits primary and secondary KIT mutations in exons 9, 11, 13, 14, 17, and 18 involved in GIST, as well as the primary exon 17 D816V mutation involved in systemic mastocytosis, or SM. QINLOCK also inhibits primary PDGFRα mutations in exons 12, 14, and 18, including the exon 18 D842V mutation, involved in a subset of GIST.

QINLOCK is approved by the U.S. FDA for the treatment of adult patients with

advanced GIST who have received prior treatment with three or more kinase inhibitors, including imatinib. It is also approved by Health Canada for the treatment of adult patients with advanced GIST who have received prior treatment with imatinib, sunitinib, and regorafenib and by the Australian Therapeutic Goods Administration for the treatment of adult patients with advanced GIST who have received prior treatment with three or more kinase inhibitors, including imatinib.

Deciphera Pharmaceuticals is developing QINLOCK for the treatment of KIT and/or PDGFRα-driven cancers, including GIST, and maintains global development and commercial rights except for select geographies. Deciphera Pharmaceuticals has an exclusive license agreement with Zai Lab (Shanghai) Co., Ltd. for the development and commercialization of QINLOCK in Greater China (Mainland China, Hong Kong, Macau, and Taiwan). Deciphera Pharmaceuticals has an exclusive distribution agreement with Specialised Therapeutics Asia (STA) for the commercialization of QINLOCK in Australia, New Zealand, Singapore, Malaysia and Brunei.

- • Specialised Therapeutics Asia (STA) to make QINLOCK® (ripretinib) available to appropriate patients in Australia, New Zealand, Singapore, Malaysia and Brunei following exclusive distribution agreement
- • QINLOCK is already approved by the Therapeutics Good Administration (TGA) and US Food and Drug Administration (FDA)
- • In the INVICTUS study, QINLOCK reduced the risk of disease progression by 85% in advanced GIST patients who have received three prior therapies[1]

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# Specialised Therapeutics Signs Exclusive Deal for New Haematology Drug

**Singapore, 18 December, 2019:** Independent pharmaceutical company Specialised Therapeutics Asia (STA) has signed an exclusive license deal with US-based Onconova Therapeutics (NASDAQ: ONTX), securing commercialisation rights to a new therapy for the treatment of Myelodysplastic Syndrome (MDS).

The drug, known as rigosertib, is currently in a Phase 3 clinical trial to assess the efficacy and safety of IV rigosertib in higher-risk MDS (HR-MDS) patients who have progressed on, failed to respond to, or relapsed after first-line treatment. The trial is over 90% enrolled and has clinical trial sites open in Australia.

STA Chief Executive Officer, Mr Carlo Montagner, said patients with high-risk MDS had limited treatment options following currently available first-line treatment.

“There is no currently approved treatment following failure of standard chemotherapy with hypomethylating agents. Patients are left with the option of entering clinical trials if available, or supportive care,” he said.

“If approved, rigosertib would address a clear unmet medical need and may be a valuable inclusion to the STA therapeutic portfolio.”

“We are delighted to enter into this collaboration with Onconova and look forward to the results of the ongoing phase III INSPIRE trial of intravenous (IV) rigosertib.”

MDS includes a group of diseases which impact the production of normal blood cells in the bone marrow. MDS is more common in elderly people, with 90% of patients diagnosed over age 60, although it can present at any age.[1]

Onconova Therapeutics' President and Chief Executive Officer Dr Steven Fruchtman commented: "We are pleased to partner with Specialised Therapeutics Asia, which has a strong track record of commercialising new products in oncology and haematology across Australia and New Zealand, We look forward to working together and following a successful readout of the ongoing INSPIRE Trial, potentially providing rigosertib as a new therapeutic option for patients diagnosed with MDS."

# About Specialised Therapeutics Asia

Headquartered in Singapore, Specialised Therapeutics Asia Pte Ltd (STA) is an international biopharmaceutical company established to commercialise new therapies and technologies throughout South East Asia, as well as in Australia and New Zealand. STA 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 Onconova Therapeutics, Inc.

Onconova Therapeutics, Inc. is a Phase 3-stage biopharmaceutical company discovering and developing novel small molecule drug candidates to treat cancer, with a focus on Myelodysplastic Syndromes (MDS). Using a proprietary chemistry platform, Onconova has created a pipeline of targeted agents designed to work against specific cellular pathways that are important in cancer cells. Advanced clinical trials with the Company's lead compound, rigosertib, are aimed at what the Company believes are unmet medical needs of patients with MDS. Onconova has conducted trials with two other research compounds and has a pre-clinical program with a CDK4/6 and Ark5 inhibitor, ON 123300.

For more information, please visit http://www.onconova.com.

# About Myelodysplastic Syndromes

MDS is a group of blood disorders that affect bone marrow function, whereby the bone marrow cells appear dysplastic and their capacity to produce cells is defective. As a result, patients with MDS have low blood cell counts and require frequent blood transfusions. In approximately one-third of patients, higher-risk MDS can progress to acute myeloid leukaemia (AML).

The Leukemia Foundation of Australia estimates that an incidence of between four to five per 100,000 of the population. However, in patients over the age of 60, this increases to anything from 20 to 50 per 100,000.[1]

# About Rigosertib

Rigosertib, Onconova's lead candidate, is a proprietary Phase 3 small molecule. A key publication in a preclinical model described rigosertib's ability to block cellular signaling by targeting RAS effector pathways (Divakar, S.K., et al., 2016: "A Small Molecule RAS-Mimetic Disrupts RAS Association with Effector Proteins to Block Signaling." Cell 165, 643). Onconova is currently in the clinical development stage with oral and IV rigosertib, including clinical trials studying single agent IV rigosertib in second-line higher-risk MDS patients (pivotal Phase 3 INSPIRE trial) and oral rigosertib plus azacitidine in first-line and refractory higher-risk MDS patients (Phase 2). Patents covering oral and injectable rigosertib have been issued in the US and are expected to provide coverage until at least 2037.

# About the INSPIRE Phase 3 Clinical Trial

The INternational Study of Phase 3 IV RigosErtib, or INSPIRE, clinical trial was

finalised following guidance received from the U.S. Food and Drug Administration and European Medicines Agency. INSPIRE is a global, multi-center, randomised, controlled study to assess the efficacy and safety of IV rigosertib in higher-risk MDS (HR-MDS) patients who had progressed on, failed to respond to, or relapsed after previous treatment with a hypomethylating agent (HMA) within nine cycles over the course of one year after initiation of HMA treatment. This time frame optimises the opportunity to respond to treatment with an HMA prior to declaring treatment failure, as per NCCN Guidelines. Patients are randomised at a 2:1 ratio into two study arms: IV rigosertib plus Best Supportive Care versus Physician's Choice plus Best Supportive Care. The primary endpoint of INSPIRE is overall survival. The trial continued beyond the pre-specified interim analysis and is nearing its conclusion. Full details of the INSPIRE trial, such as inclusion and exclusion criteria, as well as secondary endpoints, can be found on clinicaltrials.gov (NCT02562443).

- • STA secures exclusive commercialisation rights for AU and NZ
- • Rigosertib is a promising new compound to treat Myelodysplastic Syndrome, which has limited treatment options

# Further Inquiries

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# STA to market sarcoma drug in Australia, New Zealand and SE Asia Following License Deal

**Singapore and Melbourne, Australia, 14 October 2019:** Independent pharmaceutical company Specialised Therapeutics Asia (STA) has signed a new license deal, enabling it to provide a global advanced sarcoma therapy to patients in Australia, New Zealand and throughout SE Asia.

Under the terms of the agreement, STA will provide the marine-derived compound YONDELIS (trabectedin) to patients throughout Australia, New Zealand and in South East Asia under exclusive license from Spanish company PharmaMar.

YONDELIS – which has been shown to improve progression-free survival when used as second-line therapy for patients with unresectable or metastatic liposarcoma (LPS) or leiomyosarcoma (LMS) [1] – is already approved and has been available to patients in the United States since 2015,[2] and in Europe since 2007.[3]

Until now, it has not been available in Australia and New Zealand, although it is currently provided to patients in Singapore, Malaysia and Brunei via a previous pharmaceutical arrangement. Former product licensee Janssen will continue to distribute YONDELIS in Singapore, Malaysia and Brunei until marketing authorisation is formally transferred to STA.

Announcing the new deal, STA Chief Executive Officer Mr Carlo Montagner said some Australian patients and their doctors had previously sought to access YONDELIS from international sources, at great difficulty and expense.

“We are delighted to provide this important therapy to patients in Australia, New Zealand and in South-East Asia,” he said.

“We will be immediately seeking approval from the Therapeutic Goods

Administration (TGA) and in the interim, will ensure YONDELIS is available to appropriate patients via a Special Access Program."

Associate Professor Jayesh Desai, Medical Oncologist at the Peter MacCallum Cancer Centre in Melbourne, Australia, and Deputy-Chair of the Australia New Zealand Sarcoma Association (ANZSA) said the availability of YONDELIS in Australia would be greatly appreciated by the sarcoma community.

"Sarcoma is a relatively rare cancer and treatment options are limited for Australian patients with advanced disease," Associate Professor Desai said.

"YONDELIS has been shown to provide a 45% reduction in the risk of disease progression or death versus dacarbazine in patients who have failed prior therapies,[1] and has been a global standard of care. We welcome news that Australian patients will soon be provided access to this therapy that is already providing benefit to sarcoma patients around the world."

Specialised Therapeutics will now seek formal regulatory approval to market YONDELIS in Australia from the Therapeutic Goods Administration (TGA) and subsequent reimbursement via the Pharmaceutical Benefits Scheme (PBS).

In the interim, a Special Access Program will be opened on November 1 to ensure YONDELIS is available at the earliest opportunity to eligible patients.

PharmaMar President, José María Fernández Sousa-Faro, commented: "This new license arrangement is the third we have struck with STA, and is a strong endorsement of their capabilities in these key marketing regions of Australia, New Zealand and South-East Asia.

"Patients and the medical community will now be provided the opportunity to readily access YONDELIS, which is already recognised as a global standard of care. We look forward to seeing sarcoma patients benefit with improved outcomes."

Ends.

# About Specialised Therapeutics Asia

Headquartered in Singapore, Specialised Therapeutics Asia Pte Ltd (STA) is an international biopharmaceutical company established to commercialise new therapies and technologies to patients throughout South East Asia, as well as in Australia and New Zealand. STA 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 PharmaMar

Headquartered in Madrid, PharmaMar is a biopharmaceutical company focused on oncology and committed to research and development, taking its inspiration from the sea to discover molecules with antitumor activity. It is a company seeking innovative products to provide health care professionals with new tools to treat cancer. Its commitment to patients and to research has made it a world leader in the discovery of antitumor drugs of marine origin.

PharmaMar has a pipeline of drug candidates and a robust R&D oncology program. It develops and commercializes YONDELIS® in Europe and has other clinical stage programs under development for several types of solid cancers: lurbinectedin (PM1183), PM184 and PM14.

# 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 DOXIL®/CAELYX® (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.[4] The median PFS among the YONDELIS treatment group was 4.2 months (n=345; 95% confidence interval (CI): 3.0 – 4.8 months), while the median PFS in the dacarbazine treatment group was 1.5 months (n=173; 95% CI: 1.5 – 2.6 months), 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 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%), peripheral oedema (24%), dyspnoea (25%) and headache (23%). The most common (≥20%) laboratory abnormalities were neutropenia (49%), increased alanine transaminase (ALT) (45%), thrombocytopaenia (30%), anaemia (39%), increased aspartate aminotransferase (AST) (35%) 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.[5]

It is estimated around 1500 new cases of STS will be diagnosed in Australia every year, with more men than women typically affected.[6] Median survival from diagnosis has increased from 5.80 years in 1985-1989 to 8.18 years in 2010 – 2014.[7]The outcome of patients with metastatic disease is poor with a median overall survival (OS) estimated to be between 12 and 18 months.[8,9]

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.[10]

- YONDELIS® (trabectedin) is a globally recognised treatment for patients with advanced soft tissue sarcoma as second-line therapy and beyond, but has been difficult for Australians to access
- YONDELIS demonstrates 45% reduction in risk of disease progression or death versus dacarbazine[1]
- Specialised Therapeutics now filing for TGA approval
- YONDELIS to be made available in Australia via Special Access Program to open **November 1**.

# Further Inquiries

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