Living with GIST – Sarah McGoram's Story

Canberra mother, wife and school teacher Sarah McGoram was diagnosed with GIST 25 years ago – and given just a year to live. She has defied all odds and now gives hope to other Australians living with the disease. Click on the video above to hear her story.

Channel 7: November 2021

The following tweet and story appeared on 7NEWS November 2021.

A new treatment is now within reach for sufferers of a rare and incurable cancer. The new tablet has been listed on the PBS, helping buy patients precious time. Click here to watch: https://youtu.be/bPUTlbcamVo
@AKunowski
#7NEWS

‘Great Relief’ for Australian Patients with New Therapy to Treat Rare GIST Cancers Listed on PBS

- *QINLOCK® (ripretinib) now PBS listed for Australian patients*
- *QINLOCK is the first new reimbursed therapy to treat advanced GIST in more than a decade*
- *Leading Australian oncologist says the listing will help to “buy patients more time”*
- *Data shows QINLOCK reduces risk of disease progression by 85%*[1, 2]

14 November, 2021: AUSTRALIAN cancer patients who have been diagnosed with rare gastrointestinal stromal tumours – GIST – will now have affordable access to a new therapy shown to improve survival, following its listing on the Pharmaceutical Benefits Scheme (PBS).[3]

The therapy, QINLOCK (ripretinib) is an oral medication and will be available to eligible patients on the PBS from **December 1**, in a listing described by cancer specialists and patient advocacy groups as “a great relief” for patients.

Leading Australian oncologist Professor John Zalcberg, who is a consultant medical oncologist at Alfred Health and is Head of Monash University’s Cancer Research Program in the School of Public Health, welcomed the reimbursement of QINLOCK, describing it as a “fantastic” result for patients and their families.

Professor Zalcberg commented: “It has been more than 10 years since a new therapy able to treat GIST has been listed on the PBS. QINLOCK is a therapy that can buy patients more time, but the market price of this therapy has meant that

until now, it has been out of bounds for most people. This PBS listing will be welcomed by many Australian patients and their families."

35-year-old Melbourne mother of two Renee Van Beelen was diagnosed with GIST five years ago, only eight weeks after giving birth to her second child. She has had her stomach removed and endured several cancer recurrences. She is relieved another therapy is now accessible.

"It can buy me time, at a price that we can afford for our family," she said. "We simply would not have been able to come up with thousands of dollars every month. Having QINLOCK listed on the PBS means the world to us, because it means I have another tool in my back pocket to help me watch my children grow up. All I want is to create moments with my family and knowing that this therapy is available on the PBS is a huge relief."

And Canberra teacher and mother Sarah McGoram, who was given a year to live 25 years ago, says this listing "will have a profound impact on my family."

Sarah, who has led a national lobby campaign fighting for QINLOCK to be funded, says: "QINLOCK being funded on the PBS can buy me time. It buys me a treatment option that was not otherwise there. That's all I want. I just want time with my family and time to fight this frustrating disease."

Rare Cancers Australia Chief Executive Officer Richard Vines said today was "a red-letter day" for the GIST community, as patients previously had no further treatment options after other therapies failed.

"QINLOCK offers hope, it offers time and it offers a future that otherwise they would not have had," Mr Vines said. "This has been a long time coming for GIST patients and it is a fantastic result."

QINLOCK belongs to a class of drugs known as tyrosine kinase inhibitors, or TKIs. It works by inhibiting key enzymes linked to tumour growth. It is now reimbursed "**for the treatment of patients with advanced metastatic or unresectable GIST who have progressed following treatment with imatinib and sunitinib.**" [3]

A pivotal Phase 3 clinical trial of QINLOCK – the INVICTUS study – demonstrated that QINLOCK was able to significantly reduce the risk of disease progression by

85% (hazard ratio of 0.15, p<0.0001) with a median progression-free survival of 6.3 months in patients administered QINLOCK, compared to 1.0 month in the placebo arm.[1] In addition, in a long-term follow up analysis, patients in the QINLOCK arm achieved a median overall survival of 18.2 months compared to 6.3 months in the placebo arm and QINLOCK reduced the risk of death by 58% (hazard ratio of 0.42).[1, 4]

QINLOCK is made available in Australia by independent pharmaceutical company Specialised Therapeutics (ST) under exclusive license from US based Deciphera Pharmaceuticals.

ST Chief Executive Officer Carlo Montagner said it was vital rare cancer patients were provided affordable access to specialist medicines.

“Without PBS reimbursement, most patients would be unable to afford this therapy,” he said. “We are celebrating the PBS listing of QINLOCK and look forward to seeing it make a difference to GIST patients and their families.”

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 growth usually begins in the connective tissue in the wall of the affected organ and grows outwards. The common location of GISTs are in the stomach (50 to 60%) and small intestines (30 to 40%) but can occur in any site in the digestive system. Other possible GIST sites are the oesophagus, rectum, and colon. GIST cases are rare and estimated to cause between 0.1% and 3% of GI cancer in Australia. The risk of GIST diagnosis increases with age, with GIST incidence peaking among people in their fifties and sixties.[5]

About QINLOCK (ripretinib)

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 to lock the

kinase in the inactive state, preventing downstream signalling and cell proliferation. This dual mechanism of action provides broad inhibition of KIT and PDGFRA kinase activity, including wild type and multiple primary and secondary mutations. Ripretinib also inhibits other kinases in vitro, such as PDGFRB, TIE2, VEGFR2, and BRAF.[2]

About Specialised Therapeutics

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 the INVICTUS Phase 3 Study

INVICTUS is a Phase 3 randomised, 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** once daily (n=85) or placebo (n=44). The primary efficacy endpoint was 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, $p<0.0001$).[1] Secondary endpoints as determined by independent radiologic review using modified RECIST included Objective Response Rate (ORR) and Overall Survival (OS). QINLOCK demonstrated an ORR of 9.4% compared with 0% for placebo ($p=0.0504$)[1]. In a long-term follow up of 19 months after the primary analysis,

QINLOCK also demonstrated a median OS of 18.2 months compared to 6.3 months in the placebo arm and reduced the risk of death by 58% (hazard ratio of 0.42).[4] The most common (>2%) grade 3 or 4 treatment related adverse events in the QINLOCK group included lipase increase (5%), hypertension (4%), fatigue (2%), and hypophosphataemia (2%); and in the placebo group, anaemia (7%), fatigue (2%), diarrhoea (2%), decreased appetite (2%), dehydration (2%), hyperkalaemia (2%), acute kidney injury (2%), and pulmonary oedema (2%).[1]

Further Enquiries

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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.

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.

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Meet the Specialist: Breast Cancer Oncologist Dr Khoo Kei Siong

Singapore breast cancer oncologist Dr Khoo Kei Siong says “changes in dietary habits, life style and reproductive patterns” are the most likely reasons breast cancer diagnoses are rising steadily in the region – with a more than three-fold increase since the late 1960s. But there is good news, with the prognosis for these women “improving significantly”.

New Therapy to Treat Advanced Small Cell Lung Cancer ZEPZELCA® (lurbinectedin) Approved in Singapore

Singapore, 22 September 2021: SINGAPORE patients with an aggressive form of lung cancer (metastatic small cell cung cancer) can now access a new therapy that may improve outcomes.

The drug ZEPZELCA (lurbinectedin) has been provisionally approved by

Singapore's Health Sciences Authority (HSA) "**for the treatment of adult patients with metastatic small cell lung cancer (SCLC) who have progressed after prior platinum-containing chemotherapy".**[1]

This means patients who have failed other existing treatment options will now have a further therapeutic option.

ZEPZELCA is the first new therapy approved by the HSA to treat second-line SCLC in more than two decades, and is the third oncology drug in Specialised Therapeutics (ST) portfolio to receive HSA approval.

The Singapore approval follows on from approvals by the US Food and Drug Administration (FDA) decision[2], as well as the Therapeutic Goods Administration (TGA) in Australia.[3]

"The new availability of ZEPZELCA will be welcomed by patients, families and the medical community, as we strive to improve patient outcomes for this disease," Professor Mitchell said.

"With this approval, we now have another option for patients who have progressed after prior platinum-based treatments. This provides an opportunity for them to continue treatment and potentially, improve outcomes."

The HSA approval of ZEPZELCA has been granted following collaboration with the US FDA via the 'Project Orbis' initiative, due to the high unmet clinical need in SCLC. It is based on monotherapy clinical data from an open-label, multi-centre, single-arm study in 105 adult platinum-sensitive and platinum-resistant patients with SCLC who had disease progression after treatment with platinum-based chemotherapy.[6]

The data, which appeared in *The Lancet Oncology* May 2020 issue, demonstrated that in patients with relapsed SCLC, ZEPZELCA provided an ORR of 35% and a median duration of response of 5.3 months as measured by investigator assessment (30% and 5.1 months respectively, as measured by an independent review committee (IRC)).[6]

The provisional approval is the subject of a further confirmatory study in more than 700 patients with 2L SCLC. This study is expected to be completed in 2025.

ZEPZELCA is being made available in Singapore by independent pharmaceutical company Specialised Therapeutics under exclusive license from its international partner PharmaMar.

Specialised Therapeutics Chief Executive Officer Mr Carlo Montagner said lung cancer was the third most common cancer in Singapore, representing more than 22% of all cancer deaths. SCLC represented between 10 – 15% per cent of all lung cancer diagnoses.[7,8]

“We are delighted to be able to provide a new therapy option in Singapore for patients with this difficult to treat cancer,” he said.

“While patients may initially respond to traditional chemotherapy, they often experience an aggressive recurrence that is historically resistant to treatment.

“We expect that this therapy may now be an option for up to 100 Singapore patients every year and look forward to making a difference for these patients and their families.”

PharmaMar president José María Fernández Sousa-Faro, PhD, said the company was delighted South-East Asian patients would now be provided access to ZEPZELCA.

“We are pleased to bring a new treatment choice to relapsed SCLC patients. “The accelerated approval of ZEPZELCA underscores its potential to fill an unmet need in this often-overlooked SCLC community.”

ZEPZELCA has been available in Singapore via an Early Access Program since July 2020.

Ends.

About SCLC in Singapore

SCLC represents a serious condition. It is a particularly aggressive type of lung cancer related to smoking that represents approximately 10-15% of all lung cancers, accounting for more than 275,000 new cases worldwide every year.[9,10]

SCLC is characterised by rapid growth, early dissemination that is often asymptomatic and with acquired resistance to drugs. SCLC is staged into limited-stage or extensive-stage disease. Limited-stage disease is potentially curable with aggressive therapy consisting of concurrent chemoradiotherapy, prophylactic cranial irradiation, and occasionally, surgery.[11,12] However, nearly two-thirds of SCLC patients have extensive-stage disease at diagnosis, which is not curable, and patients are treated with palliative intent, with a median survival of 7 to 11 months after diagnosis and with less than 5% survival at 2 years.[13,14]

Lung cancer is the third most common cancer in Singapore and represents 22.3% of all cancer deaths. Between 2014 and 2018, approximately 7,945 new cases of lung cancer were diagnosed in Singapore, with 10-15% of these classified as SCLC (between 150 and 240 new SCLC cases annually).7,8 While the age-standardised incidence rate of all lung cancer has been in decline since the 1970's (mid-50's per 100,000 in 1968 to mid-30's per 100,000 in 2017), the five-year relative survival has seen a moderate increase to approximately 10% in 2017. Globally, the prognosis of patients with SCLC is dismal with a 5-year survival rate of less than 5% and an average overall survival period of only 2–4 months for patients not receiving any active treatment.[11,12]

Modern studies, including those of recent immunotherapies, suggest that between 40-60% of patients that receive front-line therapy will be clinically eligible for second-line therapy.[15-18] This suggests that, based on 2014-2018 lung cancer incidence figures from the Singapore Cancer registry8, between 60 to 100 patients would be eligible for second-line SCLC treatment in Singapore.

About ZEPZELCA® (lurbinectedin)

ZEPZELCA is an alkylating drug that binds guanine residues within DNA. This triggers a cascade of events that can affect the activity of DNA binding proteins, including some transcription factors, and DNA repair pathways, resulting in disruption of the cell cycle and eventual cell death.[1]

ZEPZELCA or injection 4 mg is a prescription medicine used to treat adults with a kind of lung cancer called small cell lung cancer (SCLC) that has spread to other parts of the body (metastatic) and who have received treatment with

chemotherapy that contains platinum, and it did not work or is no longer working. ZEPZELCA is approved based on response rate and how long the response lasted. Additional studies will further evaluate the benefit of ZEPZELCA for this use.

About the Phase II Monotherapy Trial

The Phase 2 trial of ZEPZELCA was an open-label, single-arm study, which enrolled a total of 105 SCLC patients at 26 hospitals in six European countries and the U.S.[6] In the trial, platinum-sensitive and platinum-resistant patients were treated with ZEPZELCA 3.2 mg/m^2, administered as a 60-minute IV infusion repeated every 21 days until disease progression or unacceptable toxicity. The primary endpoint, ORR, was 35% and the median duration of response was 5.3 months as measured by investigator assessment (30% and 5.1 months respectively, as measured by an IRC).[6] ZEPZELCA was discontinued in 1.9% of patients and was delayed in 30.5% of patients due to an adverse reaction. Dose reductions for an adverse reaction occurred in 25% of patients.[6]

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 which takes its inspiration from the sea to discover molecules with antitumor activity. It is a

company that seeks innovative products to provide healthcare professionals with new tools to treat cancer. Its commitment to patients and to research has made it one of the world leaders 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. With subsidiaries in Germany, Italy, France, Switzerland, Belgium, Austria and the United States. PharmaMar wholly owns other companies: GENOMICA, a molecular diagnostics company; Sylentis, dedicated to researching therapeutic applications of gene silencing (RNAi). To learn more about PharmaMar, please visit us at www.pharmamar.com.

Further Enquiries

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New Therapy to Treat Advanced Small Cell Lung Cancer Approved for Australian Patients

Singapore, 14 September 2021: AUSTRALIAN patients with an aggressive form of lung cancer (metastatic Small Cell Lung Cancer) can now access a new therapy that may improve outcomes.

The therapy, ZEPZELCA™ (lurbinectedin) has been approved by the Therapeutic Goods Administration (TGA) "for the treatment of patients with metastatic small cell lung cancer (SCLC) that has progressed on or after prior platinum-containing therapy".[1]

This means patients who have failed other existing treatment options will now be able to access another line of therapy.

ZEPZELCA is the first new therapy approved by the TGA to treat second-line SCLC in more than two decades.

Australian lung cancer oncologist Professor Paul Mitchell from the Olivia Newton-John Cancer and Wellness and Research Centre said SCLC was particularly aggressive and more than two-thirds of patients were diagnosed with extensive stage disease. He said fewer than 5% of these patients currently survived more than five years post diagnosis. [3,4]

"The new availability of ZEPZELCA will be welcomed by patients, families and the medical community, as we strive to improve patient outcomes for this disease," Professor Mitchell said.

"With this approval, we now have another option for patients who have progressed after prior platinum-based treatments. This provides an opportunity for them to continue treatment and potentially, improve outcomes."

The TGA approval of ZEPZELCA has been granted under a provisional regulatory pathway. The US Food and Drug Administration (FDA) and Australia's Therapeutic Goods Administration (TGA) collaborated via 'Project Orbis' to accelerate availability to Australian patients.

ZEPZELCA's approval is based on clinical data from an open-label, multi-centre, single-arm phase II study in 105 adult patients with SCLC who had disease progression after treatment with platinum-based chemotherapy.[2]

The data, which appeared in *The Lancet Oncology* May 2020 issue, demonstrated that in patients with relapsed SCLC, ZEPZELCA provided an Overall Response Rate (ORR) of 35% and a median duration of response of 5.3 months as measured by investigator assessment (30% and 5.1 months respectively, as measured by an independent review committee (IRC).[2]

The provisional approval is the subject of a further confirmatory study in more than 700 patients with 2nd line SCLC including some Australian sites. This study is expected to be completed in 2025.

ZEPZELCA is being made available in Australia by the independent pharmaceutical Company, Specialised Therapeutics (ST), under exclusive license from international partner, PharmaMar.

ST Chief Executive Officer Mr Carlo Montagner said the approval of ZEPZELCA would potentially make a difference for around 400 Australian patients annually who had run out of treatment options.

"We are delighted to be able to provide a new therapy option for patients with this difficult to treat cancer," he said.

"While patients may initially respond to traditional chemotherapy, they often experience an aggressive recurrence that is historically resistant to treatment.

"Our mission has always been to provide therapies in areas of unmet need and SCLC is certainly one of these areas. We look forward to making a difference for these patients and their families."

PharmaMar president José María Fernández Sousa-Faro, PhD, said the Company was delighted Australian patients would now be provided access to ZEPZELCA.

“We are pleased to bring a new treatment choice to relapsed SCLC patients. “The accelerated approval of ZEPZELCA underscores its potential to fill an unmet need in this often-overlooked SCLC community.”

ZEPZELCA is currently available in Australia via a Special Access Program.

Commercial supplies of ZEPZELCA will commence early 2022.

Ends.

About Small Cell Lung Cancer (SCLC)

SCLC is a particularly aggressive type of lung cancer that represents approximately 10-15% of all lung cancers,[3] accounting for more than 275,000 new cases worldwide every year. In Australia, around 1,900 patients are diagnosed annually with the disease,[4] which is characterised by rapid growth, early dissemination that is often asymptomatic and with acquired resistance to drugs[2]. SCLC is staged into limited-stage or extensive-stage disease. Limited-stage disease is potentially curable with aggressive therapy consisting of concurrent chemoradiotherapy, prophylactic cranial irradiation, and occasionally, surgery. However, nearly two-thirds of SCLC patients have extensive-stage disease at diagnosis, which is not curable, and patients are currently treated with palliative intent, with a median survival of 7 to 11 months after diagnosis and with less than 5% survival at 2 years.[5,6]

About ZEPZELCA™ (lurbinectedin)

ZEPZELCA also known as PM1183, is an alkylating drug that binds guanine residues within DNA. This triggers a cascade of events that can affect the activity of DNA binding proteins, including some transcription factors, and DNA repair pathways, resulting in disruption of the cell cycle and eventual cell death.[1]

ZEPZELCA 4 mg is a prescription medicine used to treat adults with a kind of lung cancer called small cell lung cancer (SCLC) that has spread to other parts of

the body (metastatic) and who have received treatment with chemotherapy that contains platinum, and it did not work or is no longer working. ZEPZELCA is approved based on response rate and how long the response lasted. Additional studies will further evaluate the benefit of ZEPZELCA for this use.

About the Phase II Monotherapy Trial

The Phase II trial of ZEPZELCA was an open-label, single-arm study, which enrolled a total of 105 SCLC patients at 26 hospitals in six European countries and the U.S.[2] In the trial, platinum-sensitive and platinum-resistant patients were treated with ZEPZELCA 3.2 mg/m^2, administered as a 60-minute IV infusion repeated every 21 days until disease progression or unacceptable toxicity. The primary endpoint, ORR, was 35% and the median duration of response was 5.3 months as measured by investigator assessment (30% and 5.1 months respectively, as measured by an IRC).[2] Serious adverse reactions in ≥3% of patients included pneumonia, febrile neutropenia, neutropenia, respiratory tract infection, anaemia, dyspnoea, and thrombocytopenia. ZEPZELCA was discontinued in 1.9% of patients and was delayed in 30.5% of patients due to an adverse reaction. Dose reductions for an adverse reaction occurred in 25 percent of patients.[2]

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 which takes its inspiration from the sea to discover molecules with antitumor activity. It is a company that seeks innovative products to provide healthcare professionals with new tools to treat cancer. Its commitment to patients and to research has made it one of the world leaders 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. With subsidiaries in Germany, Italy, France, Switzerland, Belgium, Austria and the United States. PharmaMar wholly owns other companies: GENOMICA, a molecular diagnostics company; Sylentis, dedicated to researching therapeutic applications of gene silencing (RNAi). To learn more about PharmaMar, please visit us at www.pharmamar.com.

Further Enquiries

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Specialised Therapeutics Asia to Re-introduce Global Sarcoma Drug to Patients in Singapore, Malaysia and Brunei

Singapore, 9 September 2021: A globally regarded sarcoma therapy that has been shown to improve progression free survival[1] is now being re-introduced in key regions of South-East Asia by independent pharmaceutical company Specialised Therapeutics Asia (STA).

The compound YONDELIS® (trabectedin) will be available to advanced sarcoma patients via their treating oncologists in Singapore, Malaysia and Brunei, following a license agreement between STA and its international pharmaceutical partner, PharmaMar S.A.

YONDELIS has been approved in Singapore, Malaysia and Brunei since 2009 and was previously provided to patients in these regions under a separate pharmaceutical arrangement with former product licensee, Janssen Products, L. E. Full marketing authorisation has now been formally transferred to STA.

YONDELIS – which has been shown to improve progression-free survival when used subsequent to anthracycline-based therapy for patients with unresectable or metastatic liposarcoma (LPS) or leiomyosarcoma (LMS)[1] – is approved in Singapore and has been available to patients in the United States since 2015,[2]

and in Europe since 2007.[3]

Malaysian sarcoma specialist Dr Aminudin Rahman Bin Mohd Mydin, Consultant Clinical Oncologist at the KPJ Damansara Specialist Hospital, welcomed the renewed availability of YONDELIS for appropriate patients in South East Asia.

Dr Aminudin commented: “This is exciting news. YONDELIS is an established therapy that has already been extensively used globally to treat advanced sarcoma patients in South East Asia and globally.

“We expect that the reintroduction of this important therapy in key South East Asian regions will provide a new treatment option for many of our patients, as we strive to provide new therapy options and improve outcomes.”

STA Chief Executive Officer Mr Carlo Montagner said YONDELIS had been previously available to South East Asian patients and is recognised as a global standard of care therapy.

Mr Montagner commented: “Our company has a strong and extensive foundation in oncology and this product is a valuable inclusion to our therapeutic portfolio. We look forward to working with the sarcoma community in multiple regions of South East Asia to improve access to YONDELIS and ensure that it is considered as a new therapy option for all appropriate patients.”

STA markets YONDELIS under an exclusive license arrangement with an international partner, PharmaMar.

Ends.

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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 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.[5]

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.[5]

Further Enquiries

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Oncotype DX®– Maxine Gladwin's Experience

Maxine Gladwin was 47 and a single mum to three teenage daughters when she was diagnosed with breast cancer. When her oncologist advised chemotherapy treatment, she was devastated – fearing it would impact her health, her job and her ability to provide for her family. With support from her family, a sample of Maxine's tumour was tested, using the Oncotype DX® Breast Recurrence Score Test. Maxine explains what happened next.

"I was 47 years old when I was diagnosed with breast cancer in November 2014.

After a mastectomy, my oncologist recommended chemotherapy treatment, along with hormone therapy.

When my family raised concerns about chemotherapy, my oncologist told me about the Oncotype DX test. The cost of this test was well out of my reach, as I am a single parent with three teenage daughters.

My mother was with me at the consultation and she insisted on paying for it, because we were hoping I could avoid chemotherapy if it was safe.

It was such a relief when I got the results. I had a 3% chance of recurrence and I would not be any better off having chemo.

My Dad cried with relief when he heard. Because I am the sole breadwinner, I was terrified of losing my job and I really thought this could happen if I was missing work for treatment.

I had contacted Centrelink prior to receiving my results and was advised that my entitlements would not even cover our rent, so our family could have been homeless.

Two of my girls were doing HSC at the time and moving them away from their school, family and friends would have been hugely disruptive.

Without Mum at my appointment, I would have just gone with chemotherapy.

It's terrible knowing that many women are going through unnecessary treatment.

I consider myself lucky. I am happy to report that five years after my diagnosis, I have had no cancer recurrence. My oldest daughter is married and is a manager for a travel agency, my two younger girls are nearly finished university degrees – one is studying to be a nurse and the other a social worker.

I don’t know if any of this would have happened if I had just proceeded through treatment. Having an Oncotype test and getting the results I did, really changed my life.”

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BREAST Issue #1

Introducing BREAST, a magazine produced by ST for breast cancer patients. Thanks to all patients, families and clinicians who provided their stories and insights. Click on the image below to view as a flipbook. We look forward to your feedback.