World-First Approval for Multiple Myeloma Drug Aplidin®

Singapore, 11 December 2018: Australian multiple myeloma patients will have world-first access to a new first-in-class drug developed to treat the disease, following approval by Australian regulatory authorities.

The drug, APLIDIN (plitidepsin) will be available to patients who have failed or are resistant to other therapies, after the Therapeutic Goods Administration (TGA) decision to approve APLIDIN before any other country.

Leading Australian myeloma clinicians are welcoming the decision, saying APLIDIN will provide another valuable treatment option for patients.

Alfred Hospital Head of the Malignant Haematology and Stem Cell Transplantation Service, Professor Andrew Spencer, said: "APLIDIN provides a chance for some myeloma patients to extend their lives.

"We now have another drug to offer patients who have relapsed after being treated with existing therapies.

"This is important, because once patients become resistant to standard therapies, there have been very limited treatment options."

And Peter MacCallum Cancer Centre and Royal Melbourne Hospital haematologist, Professor Jeff Szer, who was the Australian principal investigator on the pivotal APLIDIN registration study, said APLIDIN had been shown to be effective and well tolerated.

He commented: "More Australian myeloma patients were enrolled into the pivotal international trial of APLIDIN than anywhere else in the world.

"These patients in the Phase 3 study known as ADMYRE have now paved the way for others to have access to a new and novel therapy.

"This really means that some patients with advanced myeloma have the possibility of improved outcomes, when previous therapies have failed."

Specialised Therapeutics will continue providing APLIDIN to eligible Australian patients at no cost via a Compassionate Access Program, prior to national reimbursement.

Chief Executive Officer of Specialised Therapeutics Asia, Carlo Montagner, said Australian regulatory authorities should be commended for ensuring Australian myeloma patients have the first opportunity to access this cutting-edge therapy.

He commented: “It is not often that Australian patients are the first in the world to access new medicines. In this case, the TGA is at the forefront, with decision-makers recognising the great need that exists in multiple myeloma. This disease remains incurable and patients eventually run out of treatment options.

The company is pursuing opportunities to provide APLIDIN to myeloma patients across South East Asia.

Specialised Therapeutics Asia has exclusive rights to market and distribute APLIDIN in Australia, Singapore and 12 other South East Asian countries under the terms of an exclusive arrangement with European partner, PharmaMar.

APLIDIN was the first drug licensed by Specialised Therapeutics Asia for the broader SE Asian market.

PharmaMar President, José María Fernández Sousa-Faro, said: “This approval for an incurable disease, corroborates the work that the PharmaMar team has done over the years with APLIDIN®. Patients and the medical community will now have a new therapeutic alternative with a new mechanism of action, that is different from the products currently in use.”

Managing Director of PharmaMar´s Oncology Business Unit, Luis Mora, added: “The approval of Aplidin® is a very important step forward for the company. This increases PharmaMar’s presence with a second drug on the Australian market and, together with our partners, we are initiating procedures for other markets, such as South America, Mexico, Canada, Asia and Israel.”

Ends.

About APLIDIN® (plitidepsin)

Plitidepsin is an anticancer agent of marine origin, originally obtained from the ascidian *Aplidium albicans*. It specifically binds to the eEF1A2 and targets the non-canonical role of this protein, resulting in tumor cell death via apoptosis (programmed death). Plitidepsin is currently in clinical development for hematological cancers, including combination studies in relapsed or refractory multiple myeloma, and a Phase II study in relapsed or refractory angioimmunoblastic T-cell lymphoma.

About Multiple Myeloma in Australia

It is estimated that around 1800 Australians are diagnosed with MM every year and 1000 people die.[1] Fewer than 50% of patients survive five-years post diagnosis.[1]

MM accounts for between 10 and 15% of all haematological malignancies and is predominately a disease of the elderly, with median age at diagnosis 65-70 years.[2] This disease typically causes increased bone osteolysis resulting in pathological fractures, renal failure, hypercalcaemia, immune suppression, increased infection risk and bone marrow failure.[2]

Despite significant developments in frontline, maintenance and supportive therapy options, MM remains incurable, with treatment refractory relapse eventually occurring in all patients.[3]

About Specialised Therapeutics Asia

Headquartered in Singapore, Specialised Therapeutics Asia Pte Ltd (ST Asia) is an international biopharmaceutical company established to provide innovative specialist therapies and technologies to patients throughout South East Asia, as well as in Australia and New Zealand. ST Asia's existing product portfolio spans oncology, haematology, neurology, urology and ophthalmology. Additional

information can be found at www.stbiopharma.com

About PharmaMar

Headquartered in Madrid, PharmaMar is a world-leading biopharmaceutical company in the discovery and development of innovative marine-derived anticancer drugs. The company has an important pipeline of drug candidates and a robust R&D oncology program. PharmaMar develops and commercializes YONDELIS® in Europe and has three other clinical stage programs under development for several types of solid and hematological cancers PM1183, plitidepsin, and PM60184. PharmaMar is a global biopharmaceutical company with subsidiaries in Germany, Italy, France, Switzerland and the United States. PharmaMar fully owns three other companies: GENOMICA, Spain's leading molecular diagnostics company; Sylentis, dedicated to researching therapeutic applications of gene silencing (RNAI); and two other chemical enterprises, Zelnova and Xylazel. To learn more about PharmaMar, please visit us at www.pharmamar.com.

Further Inquiries

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References

1. Australian Institute of Health and Welfare (AIHW) 2017. Cancer in Australia 2017. Cancer series no. 101. Cat. No. 100. Canberra: AIHW
2. Palumbo A & Anderson K. Multiple Myeloma N Engl J Med 2011; 364: 1046-1060
3. Ludwig H et al. Survival and years of life lost in different age cohorts of patients with multiple myeloma. J Clin Oncol. 2010 Mar 20; 28(9):

1599-605

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Specialised Therapeutics to Collaborate with Pharmacy Phusion to Improve Patient Outcomes

Singapore, 2 November 2018: Independent pharmaceutical company Specialised Therapeutics (ST) has struck a new agreement with specialty

pharmacy services group Pharmacy Phusion to assist with the distribution and patient support for a new medicine that treats HER2 overexpressing early-stage breast cancer.

Under the terms of the agreement, patients prescribed this new breast cancer treatment by medical oncologists via the Therapeutic Goods Administration (TGA) Special Access Scheme will be contacted by a specialist pharmacist weekly for the first five weeks of treatment, and then followed up monthly for the duration of therapy.

ST Chief Executive Officer Mr Carlo Montagner said this new program was testament to the company's ongoing commitment to patient care.

"This is about reassurance," he said. "We want all patients who are being administered our products to feel supported, as well as to ensure treatment is properly initiated and managed so that the best therapeutic outcomes are achieved."

"This program is for a new medicine that ST is currently making available to medical oncologists in Australia at no cost under a strictly-controlled patient access program while undergoing regulatory evaluation by the TGA.

"We know many patients live in regional and remote communities and can encounter challenges when it comes to immediate support.

To this end, we have engaged a team of experienced pharmacists who will implement regular well-being calls to help our patients during therapy and address any other queries or concerns that may arise.

"These pharmacists will consult as required with a patient's own medical oncologist. This is not about replacing the role of the doctor or other healthcare professional – it is about ensuring all patients have access to the right support exactly when they need it."

In addition to the formal calls, patients will also be able to telephone a pharmacist for advice at any time during business hours, seven days a week.

Pharmacy Phusion's Group Professional Manager Mark Silcock said the group works across a range of complex therapy areas, but all expert pharmacists engaged in customer support programs are experienced and uniquely positioned

to support patients prescribed new and often complex medicines.

“Pharma companies not only in Australia, but around the world can benefit from having an expert pharmacist team supporting their medications,” he said.

“Our specialist pharmacists have a deep understanding of the medicines they are discussing and how they might interact with other medicines.

“We find the primary role of the pharmacist in these programs is to provide support and reassurance, which ultimately leads to improved adherence.

“Time and time again, that is what patients want – it’s not just about the medicine, but about dosage and side effects.

“Our pharmacists take the time with each individual patient to help them understand what to expect and if they do experience any side effects, to manage them appropriately and efficiently.”
The Pharmacy Phusion customer support program takes effect from today.

Ends.

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Emma Power
STA Senior Corporate Affairs and Communications Manager
Phone: +61 419 149 525

About Specialised Therapeutics Asia

Specialised Therapeutics is an international, independent pharmaceutical company established to provide pioneering healthcare to patients throughout South East Asia, as well as in Australia and New Zealand.

ST collaborates with leading global pharmaceutical and diagnostic companies to bring novel, innovative and life changing therapies and technologies to patients affected by a range of diseases. ST remains committed to making new and novel therapies available to patients in its key regions of Australia, New Zealand and throughout South-East Asia, targeting diseases where there remains an unmet

medical 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.

Specialised Therapeutics Celebrates 10 Years and Unveils Expansion Plan

Melbourne, Australia 27 August 2018: Privately-held pharmaceutical company Specialised Therapeutics Australia will today mark its 10th anniversary, unveiling new Australian headquarters and a business plan to drive healthcare innovation over the next decade.

The company, which was founded ten years ago by pharmaceutical expats Carlo Montagner and Bozena Zembrzuski with a single chemotherapy product, has emerged as the largest privately-owned Australian specialty pharma company in the region, employing close to 50 employees, generating revenues of ~$30 million and with an expansive specialty drug portfolio spanning oncology, haematology, ophthalmology, supportive care and neurology.

Officially opening new Australian headquarters in Melbourne today, Chief Executive Officer Carlo Montagner attributed the company's success to a strategy of in-licensing mid-to late stage products for full commercialisation, but said the next 10-year plan included in-licensing earlier-stage drugs, steering them through full clinical development and globally commercialising these products. "This may require us to list a subsidiary company either on the ASX or on Singapore's SGX to co-fund compound development," he said.

"Our vision for the first 10 years was to build a profitable pharmaceutical company partnering with leading global biotech and pharmaceutical companies.

While we continue to invest aggressively to further expand our global partnerships and product pipeline into new therapeutic areas, it is now time to build on these solid foundations and execute the next stage of our company's development."

Federal Treasurer, Deputy Liberal Party leader and Member for Kooyong Josh Frydenberg MP will officially unveil the company's new headquarters, noting STA's role in cementing Victoria as a major pharmaceutical and biotech hub.

"This company is an Australian start-up success story," he said. "We know that as many as 90 per cent of start-ups fail to flourish after five years. STA is a stand-out in the pharmaceutical sector and continues to grow, providing employment and generating strong revenues."

Member for Kew and Shadow Education Minister Tim Smith MP commented:

"I am delighted that Specialised Therapeutics has chosen to set up their new headquarters in the eastern suburbs of Melbourne, specifically in my electorate of Kew. Small to medium enterprises are vitally important for our local economy and community.""

Mr Montagner said: "Bozena and I are extremely proud of what we have achieved in the past decade, which has laid the foundations for our ultimate vision: to build a global pharmaceutical company delivering specialist medicines to patients where there is an unmet clinical need."

Ends.

Media Inquiries:

Emma Power
STA Communications Manager
Phone: +61 419 149 525

About Specialised Therapeutics Australia

Specialised Therapeutics Australia is an independent, international pharmaceutical company providing new specialist medicines to patients in Australia, New Zealand and across South-East Asia. Dually headquartered in Melbourne, Australia and Singapore, STA and its affiliate company Specialised Therapeutics Asia Pte Ltd collaborates with leading global pharmaceutical, biotech and diagnostic companies to bring innovative specialist therapies and technologies to patients in its key regions.It's current portfolio includes products in oncology, haematology, supportive care, neurology and ophthalmology, but it is not confined to these therapeutic areas.

Specialised Therapeutics Asia Initiates Early Access Program for Neratinib

Singapore, 5 April 2018: Specialised Therapeutics Asia today announces the initiation of an early access program for neratinib, an extended adjuvant treatment for early-stage HER2-positive (HER2+) breast cancer.

Under this Special Access Program (SAP) select patients in Australia will be provided access to the medicine, where appropriate and when permitted by relevant regulatory authorities.

The SAP protocol allows for neratinib to be available to patients with HER2 overexpressing cancers.

In all cases, the patient must have a special clinical need that cannot be met by currently approved and available medicines.

Specialised Therapeutics' neratinib Special Access Program follows the signing of a key license agreement with Puma Biotechnology Inc. (NASDAQ:PBYI) in November 2017, providing exclusive rights to commercialise neratinib in Australia, New Zealand and in South East Asia.

Ends.

About Neratib[1]

Neratinib (NERLYNX™) is an irreversible tyrosine kinase inhibitor that blocks signal transduction through the epidermal growth factor receptors, HER1, HER2 and HER4.

Neratinib is the first HER2-targeted medication approved by the US Food and Drug Administration (FDA) as extended adjuvant treatment for early-stage HER2-positive (HER2+) breast cancer, for patients who have previously been treated with the medicine trastuzumab following surgery (i.e., adjuvant trastuzumab-based therapy).

Extended adjuvant therapy is the next step of treatment that follows adjuvant therapy (treatment after surgery) to further reduce the risk of breast cancer returning.

Neratinib is an oral tablet and works by binding to multiple receptors inside the cancer cell, blocking signals that tell cancer cells to grow and multiply.

About Special Access Programs

Special Access Programs enable pharmaceutical companies a means of providing ethical access to off-label or unapproved medicines to assist patients where there is an unmet medical need. Enrolment in any access program is only provided following request from an appropriate medical professional. Special Access Programs are strictly overseen to ensure full compliance, and are opened when no alternative treatment options are available.

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 remains an unmet medical need. 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

Further enquiries: ST Asia Communications Manager Emma Power is available on +61 419 149 525.

References:

1. NERLYNX® (neratinib) US Product Information (approved) https://www.accessdata.fda.gov/drugsatfda_docs/label/2017/208051s000lbl.pdf

Federal Government Rejects Funding Bid for Novel Breast Cancer Test That May Spare Women from Chemotherapy

Oncotype DX® breast cancer assay may spare thousands of women from chemotherapy
Medical Services Advisory Committee has now rejected five funding applications for Oncotype DX

Melbourne, Australia, 4 October 2017: THE Federal Government's peak advisory committee for Medicare funding has rejected calls from doctors, patients and the pharma industry to fund a novel breast cancer test that may spare thousands of Australian women from enduring unnecessary chemotherapy.

The Health Department's Medical Services Advisory Committee (MSAC) recommended against funding the expensive Oncotype DX breast cancer assay for Australian women – despite it being reimbursed and freely available to women in many other countries, including the United States, Canada, the United Kingdom and throughout Europe.

This genetic test identifies those women who could safely avoid chemotherapy, by analysing the activity of specific cancer genes taken from a single sample of tumour tissue. It is suitable for breast cancer patients who have hormone receptor positive, HER2 negative, early stage breast cancer, which is a common form of breast cancer affecting thousands of Australian women.

The test provides a prognosis of the likelihood the cancer will recur. It is also able to provide medical teams with predictive information, identifying tumours that would be more sensitive to chemotherapy.

Specialised Therapeutics Australia has made the test available in Australia since 2014 to those women who are able to afford the $4500 out of pocket cost. Since

2014, more than 1,000 men and women diagnosed with breast cancer have paid for an ODX test allowing them and their medical team to make a more informed decision about their treatment.

In the US, Canada, the UK and Europe, the Oncotype DX test is reimbursed, widely available and consistently shown to be cost-effective. It has spared many patients from enduring unnecessary and debilitating chemotherapy.

Respected Australian surgical oncologist and specialist breast surgeon, Professor Bruce Mann said he was “very disappointed” by the decision, noting the test had been shown to change treatment decisions in many cases. He said that most frequently, it enabled patients to avoid chemotherapy. But sometimes, test results indicated that chemotherapy was the best treatment path.

“Many breast cancer patients simply cannot afford the high costs of this test and so are making treatment decisions without all potentially available information,” Professor Mann said.

“Having access to funded tests would allow limited health resources to be directed towards those who will benefit most.”

Australian breast surgeon Miss Jane O’Brien said that while the test frequently helped identify those women who could avoid unnecessary chemotherapy, it was also able to identify those for whom chemotherapy should be recommended.

“Without Oncotype, some patients may face the prospect of being under-treated,” she said.

“I have had patients who have taken the test and been advised to proceed with chemotherapy, when perhaps medical oncologists would have been confident in recommending anti-hormone therapy alone, based on the standard criteria that we have historically used. I think it is a great pity this test is not widely funded for all appropriate Australian patients.”

The Oncotype DX breast cancer assay measures the expression of 21 cancer-related genes to provide a Recurrence Score® result, a number between 0 and 100.

A low Recurrence Score result is associated with a better prognosis and the

likelihood that there would be little to no benefit in being treated with chemotherapy. Conversely, a high result would indicate a poorer prognosis, however chemotherapy is likely to be effective and reduce the risk of recurrence.

The Oncotype DX breast cancer assay is suitable for women diagnosed with hormone-receptor positive, HER-2 negative breast cancer. The test is performed on tumour tissue removed during original surgery and patients are advised to have the test soon after surgery and before commencing follow up treatment.

The Oncotype DX test was developed by Genomic Health, Inc. (NASDAQ: GHDX) a world leading provider of genomic-based diagnostic tests that optimise treatment for early stage cancer. The company is based in California in the USA.
The Oncotype DX breast cancer assay is made available in Australia by international biopharmaceutical company Specialised Therapeutics Australia at a cost of $4,500.

Specialised Therapeutics' Chief Executive Officer Mr Carlo Montagner said he was dismayed and frustrated by the latest MSAC decision, which follows five funding applications for Oncotype DX in Australia.

"This simply means that Australian women continue to be at a disadvantage," he said. "This test is widely available and reimbursed for women in most developed countries, including the United States and the United Kingdom.

"It seems that in Australia, only the 'haves' of our society can benefit from this cutting edge technology. What a pity, in this age of personalised medicine and especially at a time when the Government has acknowledged a commitment to innovation. Our belief in this technology is validated by clinical data and the experience of doctors and patients from around the world. We are lagging behind."

Specialised Therapeutics Australia will now seek to meet with health department authorities to reconsider the funding application.

Ends.

About the Specialised Therapeutics Group

The Specialised Therapeutics (ST) group of companies collaborates with leading global pharmaceutical and diagnostic companies to bring novel, innovative and life changing healthcare solutions to patients affected by a range of diseases in Australia, New Zealand and throughout South East Asia. ST is committed to making new and novel therapies available to patients around the world, with a broad therapeutic portfolio spanning oncology, hematology, urology and ophthalmology. Further information can be found at www.STAbiopharma.com

About Oncotype DX®

The Oncotype DX portfolio of breast, colon and prostate cancer tests applies advanced genomic science to reveal the unique biology of a tumour in order to optimise cancer treatment decisions. The company's flagship product, the Oncotype DX Breast Recurrence Score® test, has been shown to predict the likelihood of chemotherapy benefit as well as recurrence in invasive breast cancer. With more than 800,000 patients tested in more than 90 countries, the Oncotype DX tests have redefined personalised medicine by making genomics a critical part of cancer diagnosis and treatment. To learn more about Oncotype DX tests, visit www.OncotypeIQ.com or www.MyBreastCancerTreatment.org.

About Genomic Health

Genomic Health, Inc. (NASDAQ: GHDX) is the world's leading provider of genomic-based diagnostic tests that help optimise cancer care, including addressing the overtreatment of the disease, one of the greatest issues in healthcare today. With its Oncotype IQ® Genomic Intelligence Platform™, the company is applying its world-class scientific and commercial expertise and infrastructure to lead the translation of clinical and genomic big data into actionable results for treatment planning throughout the cancer patient journey, from diagnosis to treatment selection and monitoring. The Oncotype IQ portfolio of genomic tests and services currently consists of the company's flagship line of

Oncotype DX® gene expression tests that have been used to guide treatment decisions for more than 800,000 cancer patients worldwide. Genomic Health is expanding its test portfolio to include additional liquid- and tissue-based tests, including the recently launched Oncotype SEQ® Liquid Select™ test. The company is based in Redwood City, California, with international headquarters in Geneva, Switzerland. For more information, please visit, www.GenomicHealth.com and follow the company on Twitter: @GenomicHealth, Facebook, YouTube and LinkedIn.

Brain Surgery Breakthrough: New Zealand Neurosurgeon Pioneers NZ-First Technique

Auckland, New Zealand, 31 May 2017: A 33 year old Wellington mother of two has become the first New Zealand patient to be treated with a novel brain cancer visualisation drug that 'lights up' tumours during surgery to enable more complete removal of the malignant tissue.

GLIOLAN® (aminolevulenic acid: ALA) is taken as a drink three hours prior to surgery and works by causing cancerous tissue in the brain to fluoresce. This enables surgeons to more clearly see and better remove highly aggressive brain tumours known as glioblastoma multiforme, or GBM.

The drug will now be reimbursed for New Zealand patients at District Health Boards (DHB) hospitals from tomorrow, **1 June**, following PHARMAC's decision to

fund GLIOLAN for newly diagnosed, untreated patients.

It is expected around 100 NZ brain cancer patients a year will now benefit from this cutting-edge medicine, which has been shown to almost double the rate of complete resection and six-month progression-free survival in patients with GBM[1].

The first patient operated on using GLIOLAN is Wellington mother of two Alice Chambers-Smith, who was diagnosed with a brain tumour just weeks ago after moving back to NZ from England with her young family late last year.

Her doctors – who suspected her cancer may be gliolblastoma multiforme – were able to access GLIOLAN on a compassionate basis prior to the public reimbursement.

The young mother, who has a 3 year-old daughter and 6 year-old son, said she hoped GLIOLAN would enable her doctors to remove as much of her cancer as possible.

“I just want to do every single possible thing I can to be the tiny statistic that doesn’t lose this battle,” she said.

“I think the PHARMAC decision to make this technology available can only be a good thing.”

Leading New Zealand neurosurgeon Mr Kelvin Woon was the first neurosurgeon to use the technology in New Zealand. “GLIOLAN provides a great opportunity for NZ patients who are affected by these highly malignant tumours,” he said.

“We are pleased to be pioneering this operation at the Wellington Regional Hospital as we endeavour to improve outcomes for patients with these aggressive brain tumours.

“Although not curative, GLIOLAN helps us to better visualise what can be poorly-defined tumour margins, which limits our ability to resect the tumour macroscopically.

“Using GLIOLAN, we can more clearly see what is brain tissue and what is tumour. This gives us the confidence to be more aggressive and strive for maximum resection. This is important, because the evidence points to maximum (complete macroscopic) resection and increases the chances of extending overall survival.” [2]

GLIOLAN is given to patients as a drink prior to surgery. The drug is preferentially taken up by the malignant tumour tissue.

During surgery, a neurosurgical microscope fitted with a specialised blue operating light is used, which causes cancerous tissue containing the drug to glow fluorescent pink whilst normal brain tissue appears blue. This enables neurosurgeons to better visualise these tumours and more completely remove them, whilst sparing the neighbouring healthy brain tissue.

The drug is made available in New Zealand by international biopharmaceutical company Specialised Therapeutics Ltd, an affiliate of Specialised Therapeutics Asia (ST Asia).

Chief Executive Officer Mr Carlo Montagner applauded the PHARMAC decision to enable GLIOLAN to be used in complex neurosurgery cases for eligible patients.

“In this region and around the world, these patients have typically had a very poor prognosis,” he said.

“With current standard chemotherapy and radiation treatment, these patients have a median overall survival of 12, maybe 15 months.[3]

“GLIOLAN has been shown to help GBM patients survive longer without tumour progression compared to standard surgical procedures. Any drug or technology that enables patients additional time with their families is extremely valuable.”

International studies have shown that the use of GLIOLAN during brain tumour surgery has nearly doubled the rate of achieving a complete resection of the main tumour bulk, which in turn has resulted in a doubling of the number of patients without progression of their brain cancer six months after surgery.[1]

The pivotal Phase III study published in The Lancet Oncology Medical Journal reported complete resection of malignant brain tumour tissue in 65% of patients receiving GLIOLAN compared to 36% of patients in the study’s control arm (difference between groups 29% [95% CI 17–40], p<0·0001). Six-month progression-free survival was achieved in 41% of patients receiving GLIOLAN compared to 21% of patients who were operated on without the use of the drug (difference between groups 20% [95% CI 9·1–30·7], p=0·0003)[1].

GLIOLAN was first approved in Europe in 2007 and is marketed by medac GmbH

in Europe, Africa, South America and Asia (excepting Japan and Korea). Around 500 Australian patients have been operated on using GLIOLAN since 2012.

About GLIOLAN®

The active substance in GLIOLAN, aminolevulinic acid (ALA), is a photoreceptive compound which is absorbed by cells in the body, where it is converted by enzymes into fluorescent chemicals, particularly protoporphyrin IX (PPIX). Since glioma cells take up more of the active substance and convert it more rapidly into PPIX, higher levels of PPIX accumulate in the cancer cells than in normal tissue. When illuminated under blue light of a specific wavelength, the PPIX in the tumour glows an intense red, while the normal brain tissue appears blue. This enables the surgeon to see the tumour more clearly during brain surgery and to remove it more accurately, sparing healthy brain tissue.

Like all medications GLIOLAN may cause side effects. GLIOLAN should not be used in patients with hypersensitivity to ALA or porphyrins, or in cases of acute or chronic porphyria, or in pregnancy. Cardiac disorders, gastrointestinal disorders and skin and subcutaneous disorders are all reported as being uncommon.

About the Specialised Therapeutics Group

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ophthalmology. Additional information can be found at www.STAbiopharma.com

References

1. Stummer W, Pichlmeier U, Meinel T, et al., Fluorescence-guided surgery with 5-aminovulinec acid for resection of malignant glioma: a randomised controlled multicentre phase III trial, Lancet Oncol, 2006;7:392-401
2. S.J. Hentschel and Sawaya, R. Optimizing Outcomes with Maximal Surgical Resection of Malignant Gliomas. Cancer Control 2003; Vol 10: 109-112
3. Stupp R, et al. N Engl J Med 2005; 352: 987-996

Brain Tumour Visualisation Drug GLIOLAN to be Listed on NZ Hospital Medicines List from 1 June

Singapore, Melbourne and Auckland, 28 April 2017: A NOVEL drug which 'lights up' malignant brain tumours to help surgeons more thoroughly resect the cancer tissue will be widely available to New Zealand patients from **1 June**, after a leading neurosurgeon applied for its reimbursement.

The drug, GLIOLAN (aminolevulinic acid HCl), assists neurosurgeons to more completely remove malignant brain tumours (gliomas) by causing them to become fluorescent during surgery.

It is expected around 100 NZ brain cancer patients a year will be operated on using this cutting-edge technology, which has been demonstrated to improve complete resection rates and almost double six-month progression free survival in patients with the most serious form of brain tumours, Glioblastoma Multiforme, or GBM[1].

It will be made available to newly diagnosed, untreated patients who are eligible for fluorescence-guided surgery.

GLIOLAN will be reimbursed subject to the following hospital restrictions:

- Patient has newly diagnosed, untreated, glioblastoma multiforme
- Treatment to be used as adjuvant to fluorescence-guided resection
- Patient's tumour is amenable to complete resection

Leading New Zealand neurosurgeon Dr Kelvin Woon made an application to PHARMAC seeking reimbursement and ensuring GLIOLAN's broad accessibility.

He has described the PHARMAC decision to list GLIOLAN on the hospital medicines list as "a big step forward".

"This is a great opportunity for NZ patients who are affected by these highly malignant tumours," he said.

"Although not curative, GLIOLAN helps us to better visualise what can be poorly-defined tumour margins, which limits our ability to resect the tumour macroscopically.

"Because we can more clearly see what is brain tissue and what is tumour, it gives us the confidence to be more aggressive and strive for maximum resection. This is important, because the evidence points to maximum (complete macroscopic) resection and increases the chances of overall survival." [2]

GLIOLAN is given to patients as a drink prior to surgery. The drug is preferentially taken up by the malignant tumour tissue.

During surgery, a neurosurgical microscope fitted with a specialised blue operating light is used, which causes cancerous tissue containing the drug to glow fluorescent pink whilst normal brain tissue appears blue. This enables

neurosurgeons to better visualise these tumours and more completely remove them, whilst sparing the neighbouring healthy brain tissue.

The drug is made available in New Zealand by international biopharmaceutical company Specialised Therapeutics Ltd, an affiliate of Specialised Therapeutics Asia (ST Asia).

Chief Executive Officer Mr Carlo Montagner said several NZ hospitals had already upgraded operating theatre equipment to enable the use of GLIOLAN and neurosurgeons were preparing to use this technology as soon as the PHARMAC approval and listing takes effect.

“We are delighted to be able to provide another tool for NZ neurosurgeons to use in complex brain tumour cases,” he said.

“In this region and around the world, these patients have a very poor prognosis. With current standard chemotherapy and radiation treatment, these patients have a median overall survival of 12, maybe 15 months.[3] GLIOLAN has been shown to help GBM patients survive longer without tumour progression compared to standard surgical procedures. Any drug or technology that enables patients additional time with their families is extremely valuable.”

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GLIOLAN was first approved in Europe in 2007 and is marketed by medac GmbH in Europe, Africa, South America and Asia (excepting Japan and Korea). Around 500 Australian patients have been operated on using GLIOLAN since 2012.

GLIOLAN will be available to purchase from May 12 from ST's New Zealand distributor, Healthcare Logistics (HCL).

About GLIOLAN®

The active substance in GLIOLAN, aminolevulinic acid (ALA), is a photoreceptive compound which is absorbed by cells in the body, where it is converted by enzymes into fluorescent chemicals, particularly protoporphyrin IX (PPIX). Since glioma cells take up more of the active substance and convert it more rapidly into PPIX, higher levels of PPIX accumulate in the cancer cells than in normal tissue. When illuminated under blue light of a specific wavelength, the PPIX in the tumour glows an intense red, while the normal brain tissue appears blue. This enables the surgeon to see the tumour more clearly during brain surgery and to remove it more accurately, sparing healthy brain tissue.

Like all medications GLIOLAN may cause side effects. GLIOLAN should not be used in patients with hypersensitivity to ALA or porphyrins, or in cases of acute or chronic porphyria, or in pregnancy. Cardiac disorders, gastrointestinal disorders and skin and subcutaneous disorders are all reported as being uncommon.

GLIOLAN will be available to purchase from May 12 from ST's New Zealand distributor, Healthcare Logistics (HCL).

About the Specialised Therapeutics Group

The Specialised Therapeutics group of companies collaborates with leading global pharmaceutical and diagnostic companies to bring novel, innovative and life changing healthcare solutions to patients affected by a range of diseases in Australia, New Zealand and throughout South East Asia. ST is committed to making new and novel therapies available to patients around the world, with a broad therapeutic portfolio spanning oncology, hematology, urology and ophthalmology. Additional information can be found at www.STAbiopharma.com

For all inquiries, please phone Specialised Therapeutics Asia

Communications Manager Emma Power on +61 149 149 525

References

1. Stummer W, Pichlmeier U, Meinel T, et al., Fluorescence-guided surgery with 5-aminovulinec acid for resection of malignant glioma: a randomised controlled multicentre phase III trial, Lancet Oncol, 2006;7:392-4012.
2.S.J. Hentschel and Sawaya, R. Optimizing Outcomes with Maximal Surgical Resection of Malignant Gliomas. Cancer Control 2003; Vol 10: 109-1123
3. Stupp R, et al. N Engl J Med 2005; 352: 987-996

Specialised Therapeutics Australia Receives Therapeutic Goods Administration Approval for Brain Tumour Visualisation Drug – GLIOLAN®

Melbourne, Australia and Hamburg, Germany, November 2013: A novel drug which assists neurosurgeons to better visualise and remove malignant brain tumours has been approved by the Therapeutic Goods Administration (TGA).

Until now, GLIOLAN (aminolevulinic acid HCl) has only been available via the Federal Government's Special Access Scheme (SAS). It will now be made widely available for use by neurosurgeons to treat patients with high grade glioma, specifically glioblastoma multiforme (GBM), which are tumours that typically have a very poor prognosis.

GLIOLAN is indicated in adult patients for **visualisation of malignant tissue during surgery for malignant gliomas that are glioblastoma multiforme (GBM) on preoperative imaging, and who are intended for resection of the tumour**.

GLIOLAN causes brain tumours (gliomas) to become fluorescent and glow during surgery. This enables neurosurgeons to better visualise these tumours and more completely remove them. GLIOLAN is given to the patient as a drink three hours before surgery. During surgery, a modified neurosurgical microscope fitted with a specialised blue operating light is used, which causes cancerous tissue to glow fluorescent red whilst normal brain tissue appears blue.

Melbourne bio-pharmaceutical company Specialised Therapeutics Australia Pty Ltd (STA) in-licenses the drug from German partner photonamic GmbH and Co. KG.

Announcing the TGA approval, STA chief executive officer Mr Carlo Montagner said GLIOLAN had already been used to treat over 100 Australian patients via the SAS and a number of hospitals have been quick to upgrade neurosurgical microscopes with fluorescence capability.

“We are pleased with the positive response from neurosurgeons since GLIOLAN was made available via the SAS and this approval from the TGA is an extremely positive outcome,” he said.

“It has always been our intention to make this high class compound available to all patients who may benefit. Brain tumour surgery using GLIOLAN has been widely adopted throughout Europe and we expect a similar uptake in Australia to improve outcomes for all GBM patients.”

The chief executive officer of photonamic Mr Ulrich Kosciessa said: “The approval in Australia is another milestone in our global development of GLIOLAN, which is now registered in more than 30 countries world wide.

“GLIOLAN was developed to provide neurosurgeons with an effective tool to increase radicality of brain tumour resection without compromising safety for the patients. We are pleased that our partner STA has successfully been able to achieve an approval from the TGA.”

International studies have shown that use of GLIOLAN during brain tumour surgery has nearly doubled the rate of achieving a complete resection, which in turn has resulted in a doubling of the number of patients without progression of their brain cancer six months post surgery.[1]

The pivotal Phase III registration study published in The Lancet Oncology medical journal reported complete resection of the malignant brain tumour tissue was achieved in 65% of patients receiving GLIOLAN, compared to 36% of patients in the control arm. This resulted in 6-month progression-free survival being achieved in 41% of patients receiving GLIOLAN compared to 21.1% of patients who received surgery without the use of the drug.[1]

Brisbane neurosurgeon, Lindy Jeffree, has used GLIOLAN in 36 patients since the drug was first made available via the SAS. She regards fluorescence guided surgery as an important tool in helping surgeons distinguish parts of a tumour which would otherwise be invisible to the naked eye.

She commented: "It makes it much easier to distinguish tumour from normal brain tissue, which has undoubtedly assisted during some complex surgical procedures. Our aim is to provide optimal patient benefit. Using GLIOLAN to see tumour tissue more clearly enables better and more thorough resection which can make a big difference to a patient's response to subsequent treatment and ultimately to survival."

"I am extremely pleased to see this drug being made more widely available to improve surgical outcomes for patients with GBM around the country."

The approval by the TGA approval brings the number of countries where GLIOLAN is registered to 31, including 27 in the EU as well as Japan, Korea and Taiwan. GLIOLAN was first approved in Europe in 2009 and is marketed by medac in Europe, Africa, South America and Asia (except Japan and Korea).

- Novel drug which improves visualisation and resection of malignant brain tumours now widely available
- Twice as many patients are without progression of brain cancer six months after surgery with GLIOLAN

- To date over 100 Australian patients have been treated with GLIOLAN via the Federal Government's Special Access Scheme

The following Australian hospitals currently perform fluorescence-guided resection of brain tumours using GLIOLAN:

1. Royal Brisbane and Woman's Hospital, Queensland
2. The Wesley Hospital, Queensland
3. The Mater Private Hospital, Queensland
4. Princess Alexandra Hospital, Queensland
5. Prince of Wales Hospital, New South Wales
6. Newcastle Private Hospital, New South Wales
7. Calvary Hospital, Tasmania
8. The Royal Melbourne Hospital, Victoria
9. St Vincent's Private Hospital, Victoria

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About Specialised Therapeutics Australia

Specialised Therapeutics Australia Pty Ltd (STA) is a biopharmaceutical company dedicated to working with leading pharmaceutical companies worldwide to provide acute care therapies for high unmet medical needs to people living in Australia and New Zealand. The STA therapeutic portfolio and pipeline at present encompasses oncology and infectious diseases. STA also has interests in the therapeutic areas of respiratory, dermatology, endocrinology and central nervous system (CNS). Additional information can be found at www.specialisedtherapeutics.com.au

About photonamic GmbH and Co KG

photonamic GmbH and Co KG was established in 2003 to develop photosensitisers in the field of fluorescence guided diagnostics and photodynamic therapy. photonamic has developed ALA for the fluorescence guided resection of glioblastoma (GLIOLAN) and for the photodynamic therapy of skin lesions (ALACARE). Both products are approved in Europe and will further be developed for the global market. photonamic is based in Hamburg, Germany.

References:

1. Stummer W, Pichlmeier U, Meinel T, et al., Fluorescence-guided surgery with 5-aminovulinec acid for resection of malignant glioma: a randomised controlled multicentre phase III trial, Lancet Oncol, 2006;7:392-401
2. European Public Assessment Report

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New ‘Superbug’ Antibiotic Approved for Use in Australia

MELBOURNE, Australia – April 26, 2013 – An effective new antibiotic designed to specifically treat the common superbug* infection *Clostridium difficile*-associated diarrhoea will be available to patients in Australia from 14th May 2013.

Melbourne biopharmaceutical company Specialised Therapeutics Australia Pty Ltd (STA) has received Therapeutic Goods Administration (TGA) approval to market the drug DIFICID (fidaxomicin) in Australia. Until now, it has only been available in Australia under the Special Access Scheme.

DIFICID is indicated for the treatment of confirmed *Clostridium difficile* (CDI) infections in adults.[1]

The macrocyclic antibiotic therapy, taken in tablet form, is regarded as a breakthrough treatment to help fight serious CDI, which typically develops in patients following broad-spectrum antibiotic use. CDI targets the large intestine, causing diarrhoea which can range from moderate & debilitating to severe & life-threatening. It is extremely common in hospitals and aged care facilities as older patients are particularly vulnerable, and can be fatal.[2]

A recent media report indicated 14 Victorians died from the infection during a 15-month period in 2010 and 2011.[3] According to data generated by the Quebec provincial hospitalisation database, there were 7004 cases of *C. difficile* across Quebec from April 1st 2003 to March 31st 2004, and 1270 people died after

contracting CDI.[4]

Medical experts say Australian infection rates have at least doubled in recent years in major public hospitals, but concede the incidence of CDI is under reported.

STA Chief Executive Officer Mr Carlo Montagner is excited about the valuable treatment alternative DIFICID offers Australian patients who contract CDI.

“DIFICID is a potentially life saving drug for this extremely serious infection plaguing public hospitals and the wider community,” he said. “Unfortunately, it is estimated that almost 30% of patients can have a recurring infection. DIFICID is the only approved drug on the market which studies have shown will lower the risk of that infection returning.”

DIFICID is the first in a new class of antibiotics which are minimally absorbed by the bloodstream and have been shown to fight CDI while leaving healthy gut flora untouched.[5]

Hypervirulent strains of *C. difficile*, including the PCR ribotype 027 strain recently identified in Australia, have been associated with epidemic spread and high rates of severe disease and death.[6]

Risk factors for CDI include exposure to antimicrobial drugs, gastric acid-suppressive therapy, advanced age, prolonged hospitalisation, cancer chemotherapy, co-morbidity and immuno- suppression. Although most cases have been in hospital inpatients, increasing numbers of community-associated cases are now being reported.[2]

Leading Australian CDI expert Professor Thomas Riley from The University of Western Australia, acknowledged that studies had demonstrated patients treated with DIFICID were significantly less likely to develop recurrent infections.[7,8]

He regarded DIFICID as an important new treatment alternative, with infection rates of *C. difficile* climbing substantially in public hospitals around the country.

“Introducing DIFICID to Australia basically means we have another drug in the arsenal to treat this infection. Until now, we have had only two drugs available.

“Fewer recurrences will help contain the spread of the illness. Most importantly, DIFICID will benefit individual patients, who become weaker and more vulnerable with each recurrent infection, enormously.”

STA licenses DIFICID for the Australian market from US-based Optimer Pharmaceuticals. Optimer Chief Executive Officer & Chairman of the Board, Dr Henry McKinnell, said he was confident DIFICID would provide a valuable new treatment option for an unmet medical need in Australia. “With the recent approval in Australia, fidaxomicin is now approved by four regulatory agencies, broadening access to patients in need across the globe,” said Dr. Henry McKinnell. “CDI infections represent a global healthcare challenge, and we believe an innovative drug like DIFICID that can deliver a substantial clinical improvement over existing therapies is an important new option that should be widely available to patients.”

About DIFICID®

Fidaxomicin is a novel antibiotic agent and the first of a new class of antibacterials called macrocycles. Fidaxomicin is bactericidal against *C difficile* in vitro, inhibiting RNA synthesis by RNA polymerases.[1]

DIFICID was studied for the treatment of CDI in two randomised Phase III studies and was found to have equivalent efficacy to vancomycin. Notably, DIFICID was associated with significantly greater improvements in the rate of sustained clinical response and significantly lower rates of CDI recurrence (than vancomycin).[1,7,8]

Contraindications and side effects:[1]

Like all medications, DIFICID may cause side effects. DIFICID should not be used in patients who are hypersensitive to any ingredient in the formulation or component of the container. As there is minimal systemic absorption of DIFICID, it should not be used for the treatment of systemic infections. Most common side

effects (≥1/10) caused by DIFICID include nausea, constipation and vomiting.

For further information regarding DIFICID and potential side effects, physicians should review the DIFICID Approved Product Information available from www.specialisedtherapeutics.com.au/index.php?q=clinician-resources.html and patients should consult their prescribing physician or the DIFICID Consumer Medicine Information available in the pack or via www.specialisedtherapeutics.com.au/index.php?q=dificid.html

About CDI

CDI has become a significant medical problem in hospitals, long-term care facilities and the community. CDI is a serious illness resulting from infection of the inner lining of the colon by *C. difficile*, which produces toxins that cause inflammation of the colon, severe diarrhoea and, in the most serious cases, death. Patients typically develop CDI following the use of broad-spectrum antibiotics which disrupt normal gastrointestinal (gut) flora, possibly allowing *C. difficile* to enter the gut and flourish. Older patients in particular are at risk for CDI, potentially because of a weakened immune system or the presence of underlying disease. Approximately two-thirds of CDI patients are 65 years of age or older. Historically, approximately 20 – 30% of CDI patients who initially respond to treatment experience a clinical recurrence.[7]

About Specialised Therapeutics Australia

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About Optimer Pharmaceuticals

Optimer Pharmaceuticals, Inc. is a global biopharmaceutical company focused on developing and commercialising innovative hospital specialty products that have a positive impact on society. Optimer developed DIFICID (fidaxomicin) tablets, an FDA-approved macrolide antibacterial drug for the treatment of *Clostridium difficile*-associated diarrhoea (CDAD) in adults 18 years of age and older and is commercializing DIFICID in the US and Canada. Optimer also received marketing authorisation for fidaxomicin tablets in the European Union, where its partner, Astellas Pharma Europe, is commercialising fidaxomicin under the trade name DIFICLIR™. The company is exploring marketing authorisation in other parts of the world where *C. difficile* has emerged as a serious health problem. Additional information can be found at www.optimerpharma.com.

* Superbug is a common term to describe a bacterium that is resistant to multiple antibiotics.

References:

1. DIFICID Approved Product Information
2. Cheng AC, Ferguson JK, Richards MJ, et al. Australasian Society for Infectious Diseases guidelines for the diagnosis and treatment of *Clostridium difficile* infection. Med J Aust 2011; 194: 353-358.
3. The Age, Saturday 26 May 2012.
4. Eggertson, L. CMAJ 2004; 171: (11) 1331-1332
5. Duggan ST. Fidaxomicin: In *Clostridium Difficile* Infection. Drugs 2011
6. Stuart R, Marshal C. *Clostridium difficile* infection: a new threat on our doorstep. Med J Aust 2011; 194: 331-332
7. Louie TJ, Miller MA, Mullane KM et al. Fidaxomicin versus Vancomycin for *Clostridium difficile* Infection N Engl J Med 2011;364:422-31.

8. Cornelly OA, Crook DW, Esposito R, et al. Fidaxomicin versus vancomycin for infection with *Clostridium difficile* in Europe, Canada, and the USA: a double-blind, non-inferiority, randomised controlled trial. Lancet 2012

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ABRAXANE® Plus Gemcitabine Improves Survival in Phase III Study of Patients with Advanced Pancreatic Cancer

MELBOURNE, Australia – January 23, 2013 – Australian biopharmaceutical company Specialised Therapeutics Australia announces that a phase III clinical trial of world leading breast cancer drug ABRAXANE® (nanoparticle albumin-bound paclitaxel) in combination with current standard of care gemcitabine in patients with advanced pancreatic cancer has demonstrated substantially improved survival times, with double the number of patients surviving two years.[1]

The MPACT (Metastatic Pancreatic Adenocarcinoma Clinical Trial) investigation involved 861 treatment naïve patients internationally.

Researchers found those patients treated with ABRAXANE plus gemcitabine had a statistically significant improvement in overall survival compared to patients receiving gemcitabine alone .[1]

Moreover, ABRAXANE plus gemcitabine demonstrated a 59% increase in one-year survival (35% vs. 22%, p=0.0002) and demonstrated double the rate of survival at two years (9% vs. 4%, p=0.02) as compared to gemcitabine alone.[1]

ABRAXANE plus gemcitabine also demonstrated statistically significant improvements in key secondary endpoints compared to gemcitabine alone, including a 31% reduction in the risk of progression or death with a median progression-free survival (PFS) of 5.5 vs. 3.7 months (HR 0.69, P=0.000024) and an overall response rate (ORR) of 23% compared to 7% (response rate ratio of 3.19, $p=1.1 \times 10^{-10}$). Another endpoint assessed included time to treatment failure, which was significantly improved with the ABRAXANE combination compared to gemcitabine alone .[1]

“The past few decades have brought us very few treatment advances for patients with advanced pancreatic cancer, which is both deadly and incredibly difficult to treat with success,” said Daniel D. Von Hoff, M.D., F.A.C.P., Lead Principal Investigator of the MPACT study and Chief Scientific Officer for Scottsdale Healthcare’s Virginia G. Piper Cancer Centre Clinical Trials and Physician-In-Chief for TGen. “The fact that ABRAXANE plus gemcitabine demonstrated an overall survival benefit, and also did so at one and two years, is a significant step forward in offering potential new hope for our patients.”

Professor John Zalcberg, Chief Medical Officer and Executive Director of Cancer Medicine at the Peter MacCallum Cancer Centre in Melbourne, said the evidence strongly supported using ABRAXANE in combination with gemcitabine as a new standard of care to treat appropriate patients, many of whom were not diagnosed until the disease was metastatic.

While acknowledging that this advance could not be seen as a cure for pancreatic cancer, Professor Zalcberg said the 59% increase in the number of patients who

lived beyond 12 months was very encouraging.

“We are extremely encouraged by the results of this study involving ABRAXANE and regard this outcome as a significant breakthrough in terms of the future management of this disease,” he said.
“In addition to treating women with metastatic breast cancer with ABRAXANE in the appropriate setting, we look forward to its approval in Australia for treating patients with advanced pancreatic cancer.”

Specialised Therapeutics Australia (STA) Chief Executive Officer Mr Carlo Montagner said the positive data paved the way for Australian patients with advanced pancreatic cancer to access more effective treatment options.

He commented: “In Australia, pancreatic cancer is the fourth most common cause of death from cancer for both men and women2 and very few treatment options exist for this group of patients. We are extremely pleased to demonstrate that ABRAXANE is capable of prolonging survival for patients with advanced pancreatic cancer and we hope to have ABRAXANE approved by the Australian Therapeutic Goods Administration (TGA) in the latter half of 2014.”

The most common grade ≥ 3 treatment-related adverse events in the study for ABRAXANE plus gemcitabine vs. gemcitabine alone were neutropenia (38% vs. 27%), fatigue (17% vs. 7%), and neuropathy (17% vs. 1%). In the ABRAXANE plus gemcitabine arm, the median time to neuropathy improvement was 29 days. There was no difference in serious life threatening toxicity (4% in each arm).[1]

Further details of the study will be highlighted in a late-breaking oral presentation by Dr. Daniel D. Von Hoff:

Abstract: LBA #148: Final results of a randomized phase III study of weekly nab-paclitaxel plus gemcitabine versus gemcitabine alone in patients with metastatic adenocarcinoma of the pancreas. Friday, January 25th between 2:00 to 3:30 pm PST at the American Society of Clinical Oncology’s (ASCO) 2013 Gastrointestinal Cancers Symposium in San Francisco, CA.

These results are from an investigational study. ABRAXANE is not approved for the treatment of advanced pancreatic cancer. Following TGA review and approval, STA will seek to have ABRAXANE included on the Pharmaceutical Benefits Scheme (PBS) for the reimbursement of ABRAXANE for advanced

pancreatic cancer.

About the MPACT Study[1]

In the MPACT (**M**etastatic **P**ancreatic **A**denocarcinoma **C**linical **T**rial) study, a Celgene-sponsored, open-label, randomised, international study of 861 patients with metastatic pancreatic cancer were randomised to receive either ABRAXANE plus gemcitabine (125 mg/m^2 followed by 1000 mg/m^2 gemcitabine for 3 weeks followed by a week of rest) or gemcitabine alone (1000 mg/m^2 administered weekly for 7 weeks followed by a week of rest followed by cycles of weekly administration for 3 weeks followed by one week of rest).

The primary endpoint for the study is improvement in overall survival. Secondary endpoints were progression-free survival, and overall response rate determined by independent radiological review. Other endpoints included progression-free survival, overall response rate determined by investigator and the safety and tolerability of this combination in this patient population.

About Advanced Pancreatic Cancer

Advanced pancreatic cancer is a difficult-to-treat cancer with the lowest survival rates among all cancer types. Across all patients with pancreatic cancer, relative 5-year survival is 6% and is less than 2% for those with advanced disease. There are two main types of pancreatic cancer – adenocarcinomas, which accounts for approximately 90% of all pancreatic cancer, and neuroendocrine tumors. Pancreatic cancer is relatively uncommon with new cases accounting for only 2.1% of all newly diagnosed cancers. However, pancreatic cancer is the fourth most common cause of cancer death for men and women in the United States and Australia, and the ninth most commonly diagnosed cancer in Australia.[2]

About ABRAXANE®

ABRAXANE is a solvent-free, nanoparticle chemotherapy treatment option for metastatic breast cancer.[3] In Australia, ABRAXANE is currently listed on the PBS for the treatment of metastatic breast cancer and HER2 positive breast cancer in combination with trastuzumab.

ABRAXANE is approved for metastatic breast cancer in over 40 countries including the U.S., Canada, European Union, Japan and China, and more than 500,000 cancer patients have received ABRAXANE therapy in the past five years.

In Australia, ABRAXANE has been granted orphan drug designation by the Therapeutic Goods Administration for the treatment of pancreatic cancer. Orphan drug status is granted to drugs used to treat relatively rare diseases such as pancreatic cancer and may allow for priority evaluation by the TGA.

ABRAXANE is currently in various stages of investigation for the treatment of the following cancers: metastatic melanoma, bladder, ovarian, and expanded applications for breast cancer.

Developed using the proprietary *nab*™ technology platform, ABRAXANE is a nanoparticle protein-bound chemotherapy agent. ABRAXANE combines paclitaxel with albumin, a naturally-occurring human protein, to deliver the drug and eliminates the need for solvents in the administration process. Nanoparticle technology allows ABRAXANE to deliver a 49% higher dose compared to regular solvent-based paclitaxel without compromising safety and tolerability.[3-4]

In a randomised phase III study of metastatic breast cancer patients, ABRAXANE demonstrated nearly double the overall tumour response rate compared to solvent-based paclitaxel. [3-4]

Anthracycline pre-treated patients in the study lived significantly longer.[5] The tolerability with ABRAXANE and solvent-based paclitaxel was comparable, despite the 49% greater dose of paclitaxel administered as ABRAXANE.[3-4] Neutropenia was lower with ABRAXANE compared to solvent-based paclitaxel, although there was an increase in incidence of grade 3 peripheral neuropathy with ABRAXANE.

However the median time to improvement, from grade 3 peripheral neuropathy to grade 2 or lower, was 22 days. No adverse events were reported that were not already known for paclitaxel.[3-4]

Contraindications and side effects[3]:

Like all medications, ABRAXANE may cause side effects.
ABRAXANE should not be used in patients who have baseline neutrophil counts of $<1.5 \times 10^9$ /L.
In patients who have exhibited hypersensitivity reactions to paclitaxel or albumin, patients should not be treated with ABRAXANE.

ABRAXANE is contraindicated during pregnancy and lactation.

Most common side effects (≥1/10) caused by ABRAXANE include; neutropenia, anemia, leucopenia, thrombocytopenia, lymphophenia, anorexia, peripheral neuropathy, hypoaesthesia, paraethesia, nausea, diarrhoea, vomiting, constipation, stomatitis, alopecia, rash, arthralgia, myalgia, fatigue, asthenia, pyrexia.

For further information regarding ABRAXANE and potential side effects, physicians should review the ABRAXANE Product Information and patients should consult their oncologist or the ABRAXANE Consumer Medicine Information available on www.specialisedtherapeutics.com.au.

About Specialised Therapeutics Australia, Pty Ltd
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Currently STA markets two world leading cancer and cancer supportive care

therapies, ABRAXANE® (nab-paclitaxel) and ALOXI® (palonosetron HCl) respectively, and has recently licensed two new agents from the Helsinn Group. Firstly Anamorelin, which is a novel ghrelin receptor agonist for the treatment of anorexia-cachexia in NSCLC, and a fixed-dose combination product (in both oral and intravenous forms) containing netupitant, a neurokinin-1 (NK1) receptor antagonist, combined with Aloxi, a serotonin-3 (5-HT_3) receptor antagonist. STA also has interests in the therapeutic areas of anti-infectives with the rights to commercialise DIFICID® (fidaxomicin) for the treatment of Clostridium difficile infections, respiratory, dermatology, endocrinology and central nervous system (CNS). Additional information can be found at www.specialisedtherapeutics.com.au

- ABRAXANE plus gemcitabine demonstrated highly statistically significant and clinically meaningful results across primary and key secondary endpoints and patient subgroups
- ABRAXANE plus gemcitabine patients showed 59% higher chance of survival at one year; survival rates doubled at two years
- A new standard of care for patients with advanced pancreatic cancer
- Oral Presentation Scheduled for Friday, January 25th at ASCO's Gastrointestinal Cancers Symposium Annual Meeting

References:

1. Von Hoff DD et al. Abstract: LBA #148: Final results of a randomized phase III study of weekly nab-paclitaxel plus gemcitabine versus gemcitabine alone in patients with metastatic adenocarcinoma of the pancreas. ASCO GI 2013

2. Cancer in Australia. An Overview 2012. Australian Institute of Health and Welfare (AIHW)

3. Abraxane Product Information

4. Gradishar WJ et al. J Clinical Oncology 2005;23:7794-7803

5. Vukelja SJ et al. ASCO 2008, Abstract 1082