

# 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<sup>®</sup> (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<sup>1</sup>.

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 glioblastoma 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.”<sup>2</sup>

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.”<sup>3</sup>

“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.<sup>1</sup>**

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],  $p = 0.0003$ )<sup>1</sup>.

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**

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. Additional information can be found at [www.STAbiopharma.com](http://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

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# 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<sup>1</sup>.

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." <sup>2</sup>

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.<sup>3</sup> 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.<sup>1</sup>

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],  $p = 0.0003$ )<sup>1</sup>.

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.

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### **References**

1. Stummer W, Pichlmeier U, Meinel T, et al., Fluorescence-guided surgery with 5-aminovulnic 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

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# 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.<sup>1</sup>

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.<sup>1</sup>

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

## **About GLIOLAN<sup>®</sup>**

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.<sup>2</sup>

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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](http://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-aminovulnic acid for resection of malignant glioma: a randomised controlled multicentre phase III trial, *Lancet Oncol*, 2006;7:392-401
2. European Public Assessment Report

## Contacts

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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.<sup>1</sup>

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.<sup>2</sup>

A recent media report indicated 14 Victorians died from the infection during a 15-month period in 2010 and 2011.<sup>3</sup> 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.<sup>4</sup>

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.<sup>5</sup>

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.<sup>6</sup>

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.<sup>2</sup>

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.<sup>7,8</sup>

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<sup>®</sup>

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.<sup>1</sup>

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).<sup>1,7,8</sup>

## Contraindications and side effects:<sup>1</sup>

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 ( $\geq 1/10$ ) 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](http://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](http://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.<sup>7</sup>

## 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](http://www.specialisedtherapeutics.com.au)

## 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](http://www.optimerpharma.com).

OPTIMER and DIFICID are trademarks of Optimer Pharmaceuticals, Inc. All other trademarks are the property of their respective owners.

\* 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.
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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.<sup>1</sup>

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.<sup>1</sup>

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.<sup>1</sup>

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 x 10<sup>-10</sup>). Another endpoint assessed included time to treatment failure, which was significantly improved with the ABRAXANE combination compared to gemcitabine alone .<sup>1</sup>

“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 Zalcborg, 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 Zalcborg 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 women<sup>2</sup> 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  $\geq 3$  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).<sup>1</sup>

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<sup>1</sup>**

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<sup>2</sup> followed by 1000 mg/m<sup>2</sup> gemcitabine for 3 weeks followed by a week of rest) or gemcitabine alone (1000 mg/m<sup>2</sup> 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.<sup>2</sup>

## **About ABRAXANE<sup>®</sup>**

ABRAXANE is a solvent-free, nanoparticle chemotherapy treatment option for metastatic breast cancer.<sup>3</sup> 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*<sup>™</sup> 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.<sup>3-4</sup>

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.<sup>3-4</sup>

Anthracycline pre-treated patients in the study lived significantly longer.<sup>5</sup> The tolerability with ABRAXANE and solvent-based paclitaxel was comparable, despite the 49% greater dose of paclitaxel administered as ABRAXANE.<sup>3-4</sup> 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.<sup>3-4</sup>

## **Contraindications and side effects<sup>3</sup>:**

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 ( $\geq 1/10$ ) caused by ABRAXANE include; neutropenia, anemia, leucopenia, thrombocytopenia, lymphopenia, anorexia, peripheral neuropathy, hypoaesthesia, paraesthesia, 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](http://www.specialisedtherapeutics.com.au).

ABRAXANE<sup>®</sup> is a registered trademark of Celgene Corporation.

ABRAXANE<sup>®</sup> is distributed by STA under license from Celgene Corporation in Australia and New Zealand.

About Specialised Therapeutics Australia, Pty Ltd

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.

Currently STA markets two world leading cancer and cancer supportive care therapies, ABRAXANE<sup>®</sup> (nab-paclitaxel) and ALOXI<sup>®</sup> (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<sub>3</sub>) receptor antagonist. STA also has interests in the therapeutic areas of anti-infectives with the rights to commercialise DIFICID<sup>®</sup> (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](http://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

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**ABRAXANE®                      Demonstrates  
Significant                      Improvement                      in  
Progression-Free                      Survival**

# **Compared to Standard Chemotherapy in Advanced Melanoma Patients**

MELBOURNE, Australia - October 25, 2012 - Specialised Therapeutics Australia Pty Ltd today announced that abstracts for the upcoming Society for Melanoma Research meeting have been published online in the organization's official journal. The publication includes an abstract reviewing results from a phase III metastatic melanoma study with ABRAXANE® (nanoparticle albumin-bound paclitaxel).

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# **Helsinn Grants Specialised Therapeutics Australia (STA) Rights to Anamorelin, a First-in-Class Compound to Treat Cachexia-Anorexia Related to Non-Small Cell Lung Cancer (NSCLC)**

Lugano, Switzerland and Melbourne, Australia, October 15th, 2012 - Melbourne biopharmaceutical company Specialised Therapeutics Australia (STA) has been granted exclusive commercialisation rights to a new drug for the treatment of NSCLC cachexia-anorexia. This condition is a serious multifactorial disorder which involves muscle wasting and metabolic impairment and commonly affects patients with advanced cancer. STA has reached agreement with Swiss pharmaceutical company Helsinn Healthcare to in-license the novel ghrelin receptor agonist anamorelin for both Australia and New Zealand.

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# **GLIOLAN® Granted Orphan Drug Status by the Therapeutic Goods Administration**

**Melbourne, Australia April 2012:** A drug which aids neurosurgeons to better visualise and more completely remove malignant brain tumours has been granted orphan drug status by the Therapeutic Goods Administration (TGA).

The drug, Gliolan, is currently in-licensed by Melbourne biopharmaceutical company, Specialised Therapeutics Australia (STA) and is currently only available to neurosurgeons via the federal government's Special Access Scheme (SAS).

Gliolan has been granted orphan drug designation for photodynamic diagnosis of gliomas that are glioblastoma multiforme (GBM) (malignant) on preoperative imaging, and intended for gross macroscopic resection of all visible tumour. STA will lodge an application for TGA approval later this year. Orphan drug designation also means TGA application fees are waived.

STA chief executive officer, Mr Carlo Montagner, said orphan drug status is an important milestone as the company progressed plans to register the drug with the TGA.

"After we submit our documentation for registration by the TGA, approval for Gliolan could take 12 to 18 months. We look forward to making this product broadly available to patients as it has been shown to significantly improve outcomes in glioma patients."

Gliolan is administered to patients three hours prior to surgery and causes cancerous tissue to glow fluorescent red during brain surgery. This enables improved visualisation of the boundary between healthy and diseased brain tissue, and aids the surgeon to more thoroughly remove the tumour. International studies have shown the use of Gliolan during surgery has nearly doubled the rate of achieving a complete resection, which has resulted in a

doubling of the number of patients without progression of their brain cancer six months after their surgery.<sup>1</sup>

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.<sup>1</sup>

Gliolan has been accessed via the SAS and used in five brain tumour (high grade glioma) operations to date in Australia, at the Royal Melbourne Hospital and the Wesley Hospital in Brisbane.

The drug has been approved for use in 29 countries since 2007, including the United Kingdom, France, Germany, and Korea. Gliolan is used in adult patients with malignant glioma. The active substance in Gliolan, 5-aminolevulinic acid, is a photoreceptive compound which is predominantly absorbed by highly proliferative cells in the body and converted by enzymes into fluorescent chemicals, particularly protoporphyrin IX (PPIX).<sup>2</sup>

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 which enables the surgeon to visualise the tumour more clearly during brain surgery and to remove it more completely and accurately, sparing healthy brain tissue.<sup>2</sup>

#### References:

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

# About Gliolan<sup>®</sup>

The active substance in Gliolan is 5-aminolevulinic acid. It 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.<sup>2</sup>

Like all medications Gliolan may cause side effects. Gliolan should not be used in patients with hypersensitivity to 5-ALA or porphyrins, 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 Specialised Therapeutics Australia, Pty Ltd

Specialised Therapeutics Australia Pty Ltd (STA) was established to identify, develop and commercialise innovative anti-cancer and other specialised therapies for the Australasian market. Currently STA markets two world leading cancer and cancer supportive care therapies, ABRAAXANE<sup>®</sup> (nanoparticle albumin-bound paclitaxel) and ALOXI<sup>®</sup> (palonosetron) respectively. Based in Melbourne, Australia, the privately held company is currently negotiating the rights to several more important therapeutic agents for release in Australasia and other regional markets.

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# Specialised Therapeutics Australia Extends Collaboration with Swiss Helsinn Group

**Melbourne, Australia and Lugano, Switzerland, 10 August 2011:** Melbourne bio-pharmaceutical company Specialised Therapeutics Australia plans to further expand its oncology portfolio, to include a new product for the prevention of chemotherapy-induced nausea and vomiting (CINV).

The Australian company has signed a letter of intent with its Swiss partner, Helsinn Group, to in-license Helsinn's new compound for the prevention of chemotherapy induced nausea and vomiting. The arrangement covers the development of a fixed-dose combination product (in both oral and intravenous forms) containing netupitant, a neurokinin-1 (NK<sub>1</sub>) receptor antagonist, and Aloxi® (palonosetron), a serotonin-3 (5-HT<sub>3</sub>) receptor antagonist.

This further collaboration follows the successful Australian launch in November last year of the second generation 5-HT<sub>3</sub> antagonist, Aloxi®, which is listed on the Pharmaceutical Benefits Scheme (PBS).

Aloxi® has been available internationally after being registered by Helsinn Group in the USA in 2003 and Europe in 2005, and is indicated for the prevention of nausea and vomiting induced by cytotoxic chemotherapy. It is successfully marketed in over 50 countries, with annual sales in 2010 in excess of \$500M worldwide.

Under the terms of the agreement, Helsinn will manufacture the new product in the group's plant located in Ireland and will also be responsible for the supply of the product for clinical and commercial use in Australia.

STA will be responsible for regulatory/clinical development and commercial activities within Australia and New Zealand. It is anticipated approval submissions will be lodged with the Therapeutic Goods Administration in 2014 following the successful completion of the Phase III registration program.

STA chief executive officer Mr Carlo Montagner said his company would pay an

upfront payment to Helsinn, as well as milestone and royalty payments.

Given the promising data from the phase I and II studies, he said he was optimistic this new product would further establish both STA and Helsinn as market leaders in oncology patient supportive care.

Riccardo Braglia, CEO of Helsinn Group said the company is very proud that the existing successful collaboration with STA for Aloxi® is now extending to netupitant-palonosetron fixed dose combination. He added that the strength of the two companies will enable Australian patients to have additional treatments for CINV now and in the future.

Ends.

For further information please contact Emma Power at Monsoon Communications on (03) 9620 3333 or 0419 149 525.

## **About Netupitant**

Netupitant is a highly selective NK<sub>1</sub> receptor antagonist, an antiemetic that works by blocking the action of Substance P, an endogenous neurotransmitter contained in high concentrations in the vomiting centre of the brainstem that can stimulate the vomiting reflex. The fixed-dose combination of netupitant and palonosetron has entered Phase III for the prevention of acute and delayed nausea and vomiting following both highly and moderately emetogenic chemotherapy.

## **About Palonosetron (Aloxi®, Onicit®, Paloxi®)**

Aloxi® (palonosetron hydrochloride) is a second generation 5-HT<sub>3</sub> receptor antagonist, developed for the prevention of chemotherapy-induced nausea and vomiting in cancer patients. Aloxi® has a long half-life of 40 hours and at least 30 times higher receptor binding affinity than currently available compounds. In clinical trials and clinical practice, Aloxi® demonstrates unique long-lasting action in the prevention of CINV. A single intravenous dose of Aloxi® provides better protection from CINV than first-generation 5-HT<sub>3</sub> receptor antagonists.

Aloxi® is contraindicated in patients known to have hypersensitivity to the drug or any of its components. The most commonly reported adverse reactions



(incidence  $\geq$  2 percent) in trials with Aloxi® were headache (9 percent) and constipation (5 percent), and they were similar to the comparators. Palonosetron has been developed by the Helsinn Group in Switzerland and today it is marketed as Aloxi®, Onicit®, and Paloxi® in more than 50 countries world-wide. Aloxi® is the leading brand in the USA and in Japan within the CINV Day of Chemo segment, and it is steadily growing in the European markets. For more information about palonosetron, please visit the website: [www.aloxi.com](http://www.aloxi.com)

### **About Helsinn Group**

Helsinn is a privately owned pharmaceutical group with headquarters in Lugano, Switzerland, and operating subsidiaries in Ireland and USA. Helsinn's business model is focused on the licensing of pharmaceuticals and medical devices in therapeutic niche areas. The Group in-licenses early to late stage new chemical entities, completes their development from the performance of pre-clinical/clinical studies and Chemistry, Manufacturing and Control (CMC), development to the filing for and attainment of their market approval worldwide. Helsinn's products are out-licensed to its network of local marketing and commercial partners, selected for their deep in-market knowledge and know-how, and assisted and supported with a full range of product and scientific management services, including commercial, regulatory, financial, legal and medical marketing advice. The active pharmaceutical ingredients and the finished dosage forms are manufactured at Helsinn's cGMP facilities in Switzerland and Ireland, and supplied worldwide to its customers. For more information about Helsinn Group, please visit the website: [www.helsinn.com](http://www.helsinn.com)

### **About Specialised Therapeutics Australia, Pty Ltd**

Specialised Therapeutics Australia Pty Ltd (STA) was established to identify, develop and commercialise innovative anti-cancer and other specialised therapies for the Australasian market. Currently STA markets two world leading cancer and cancer supportive care therapies, ABRAXANE® (nab paclitaxel) and ALOXI® (palonosetron) respectively, and has recently licensed GLIOLAN® (5-aminolevulinic acid, 5-ALA) for intraoperative visualisation of malignant glioma. Based in Melbourne, Australia, the privately held company is currently developing several more important therapeutic agents for release in Australia and New Zealand.

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# World Leading Breast Cancer Drug Now Available in New Zealand

**Melbourne, Australia and Auckland, New Zealand - 21 February 2011** - New Zealand women now have access to a leading breast cancer drug ABRAXANE® (nanoparticle albumin-bound paclitaxel) for the treatment of metastatic breast cancer after failure of anthracycline therapy.

The drug, ABRAXANE, uses novel nanoparticle technology to deliver the chemotherapeutic agent to the tumour site and has been shown to prolong patient survival times with overall fewer side effects compared with traditional solvent-based chemotherapy treatments.<sup>1-3</sup>

Some of the side effects of traditional solvent-based chemotherapy treatments include serious solvent-related anaphylactic events, which can be fatal in some patients<sup>4</sup>.

ABRAXANE is now available to patients in New Zealand via Specialised Therapeutics and will be distributed by Healthcare Logistics, based in Auckland.

Currently ABRAXANE is not subsidised in New Zealand, however a reimbursement application has been submitted to Pharmac, the Pharmaceutical Management Agency of New Zealand, for review. A decision is expected later this year.

ABRAXANE is fully reimbursed for Metastatic breast cancer after failure of prior therapy in Australia under the Pharmaceutical Benefits Scheme (PBS).

Specialised Therapeutics CEO Mr Carlo Montagner said ABRAXANE had rapidly become a standard of care in Australia and the US for the treatment of metastatic breast cancer.

“We are pleased to provide this new treatment option for women in New Zealand with metastatic breast cancer,” he said.

“We are hopeful that reimbursement approval will provide all women in New Zealand with metastatic breast cancer the option of a safer and more efficacious taxane therapy”.<sup>2,3</sup>

International Phase III registration trials of Abraxane for metastatic pancreatic and melanoma cancers are currently enrolling patients, with results expected in the next two to three years.

With the approval in New Zealand, ABRAXANE is now approved in 41 countries.

### About Specialised Therapeutics

Specialised Therapeutics is a bio-pharmaceutical company primarily established to identify, develop and commercialise innovative anti-cancer and other specialised therapies for the Australasian market. Currently Specialised Therapeutics markets two world leading cancer and cancer supportive care therapies, ABRAXANE and ALOXI® (palonosetron) respectively. Based in Melbourne, Australia, the privately held company is currently developing several more important therapeutic agents for release in Australia and New Zealand.

## **About ABRAXANE**

ABRAXANE is a solvent-free, nanoparticle chemotherapy treatment option for metastatic breast cancer.<sup>1</sup>

In Australia, ABRAXANE is currently listed on the PBS for the treatment of metastatic breast cancer after failure of prior therapy.

Developed using Celgene’s proprietary nanoparticle albumin-bound (nab)(TM) technology platform, ABRAXANE is a nanoparticle protein-bound chemotherapy agent. ABRAXANE combines paclitaxel with albumin, a naturally-occurring human protein, to deliver the drug, eliminating 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.<sup>1,2</sup>

## About nab-Driven Chemotherapy

nab technology leverages albumin nanoparticles for the active and targeted delivery of chemotherapeutics to the tumour. This nab-driven chemotherapy provides a new paradigm for penetrating the blood-stroma barrier to reach the tumour cell. The proposed mechanism of delivery of this nab-driven chemotherapy is thought to be by targeting a previously unrecognised tumour-activated, albumin-specific biologic pathway with a nanoshell of the human blood protein albumin. This nano-shuttle system is believed to activate an albumin-specific (Gp60) receptor-mediated transcytosis path through the cell wall of proliferating tumor cells, using caveolin-1 activated caveolar transport. Once in the stromal micro-environment, the albumin-bound drug may be preferentially localised by a second albumin-specific binding protein, SPARC, a protein secreted into the stroma by tumour cells. The resulting collapse of stroma surrounding the tumour cell may thus enhance the delivery of the nab-chemotherapeutic to the intracellular core of the tumour cell itself.

ABRAXANE is approved for metastatic breast cancer in 41 countries including the U.S., Canada, European Union, Japan and China, and more than 100,000 cancer patients have received ABRAXANE therapy in the past five years.

In a randomised Phase III study of metastatic breast cancer patients, ABRAXANE demonstrated a significant improvement in response rate and progression free survival compared to solvent-based paclitaxel,<sup>1,2</sup> while anthracycline pre-treated patients lived significantly longer.<sup>5</sup>

The tolerability with ABRAXANE and solvent-based paclitaxel was comparable, despite the 49% greater dose of paclitaxel administered as ABRAXANE.<sup>1,2</sup> Neutropaenia 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.<sup>1,2</sup>

In Australia, ABRAXANE has also been granted orphan drug designation by the Therapeutic Goods Administration (TGA) 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. Additionally, ABRAXANE is currently under Phase III investigation for the treatment of the following cancers: non-small cell lung, malignant melanoma, and metastatic pancreatic.

Contraindications and side effects:

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.

For further information please refer to [www.specialisedtherapeutics.com.au](http://www.specialisedtherapeutics.com.au) for the New Zealand ABRAXANE Product Information.

References:

1. Abraxane Product Information
2. Gradishar WJ et al. J Clinical Oncology 2005;23:7794-7803
3. Gradishar WJ et al. J Clinical Oncology 2009; 27(22): 3611-19
4. Irizarry LD et al. Community Oncology 2009;6(3):132-134
5. Vukelja SJ et al. ASCO 2008 Abstract 1082