Gene silencing technology for unmet global medical conditions – progress and potential

Clinical Investigator Group Update
17-18 November 2011
This presentation contains forward looking statements that involve risks and uncertainties. Although we believe that the expectations reflected in the forward looking statements are reasonable at this time, Benitec can give no assurance that these expectations will prove to be correct. Actual results could differ materially from those anticipated. Reasons may include risks associated with drug development and manufacture, risks inherent in the regulatory processes, delays in clinical trials, risks associated with patent protection, future capital needs or other general risks or factors.
Welcome

Dr Peter French, CEO
Benitec Biopharma Ltd
Agenda

• Overview
• Cancer-associated pain update
• Hepatitis B update
• OPMD program commencement
• Lung cancer update
• HIV/AIDS trial update
• IP Summary
• Questions
Benitec Background

- DNA-directed RNA interference (ddRNAi) is a novel technology platform capable of achieving long-term targeted gene silencing.

- Benitec is developing a range of products that utilize the ddRNAi technology to treat and cure life threatening severe conditions in infectious disease, cancer, and CNS.

- Over the last six months, functional gene silencing constructs for all of these programs have been created and characterised. Preclinical in vivo studies proving the safety and efficacy of the products are in progress.

- Benitec’s technology platform is applicable to a large number of other therapeutic areas in which up-regulation of a gene is associated with a disease or disorder, including genetic diseases. Benitec has commenced a muscular dystrophy program.
Benitec’s Novel
RNA Interference Technology

Benitec’s ddRNAi technology platform utilizes a vector to express shRNA molecules which silence a targeted gene of interest.

- The ddRNAi-based product contains a novel gene construct that codes for and expresses a short hairpin RNA (shRNA) molecule intended to silence the selected gene of interest.

- The expressed shRNA integrates into the host’s native RNAi process where it is separated into single strands and binds to the target mRNA.
  - This results in cleavage of the target RNA and silencing of the gene of interest.

## ddRNAi Product Development Timeline

Benitec has a pipeline of ddRNAi-based therapeutics targeted for indications where their technology provides a significant competitive advantage.

<table>
<thead>
<tr>
<th>Indication</th>
<th>Discovery</th>
<th>Pre-clinical</th>
<th>Human Clinical</th>
<th>Collaborator/Licensee</th>
<th>Collaboration Status</th>
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<tr>
<td><strong>Cancer-associated pain</strong></td>
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<td>University of Queensland (Australia)</td>
<td>Benitec has contracted with TetraQ to generate data to be able to conduct a clinical trial on terminally ill cancer patients suffering from pain.</td>
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<td><strong>Drug resistant lung cancer</strong></td>
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<td>University of New South Wales (Australia)</td>
<td>Researchers are collaborating with Benitec to develop a ddRNAi-based therapy to overcome chemotherapy resistance in non-small cell lung cancer.</td>
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<td><strong>Hepatitis B</strong></td>
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<td>Biomics (China)</td>
<td>Benitec is working with Biomics to develop ddRNAi candidates for hepatitis B silencing for preclinical testing and a China-based clinical trial.</td>
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<td><strong>Oculo-pharyngeal muscular dystrophy</strong></td>
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<td>Royal Holloway, University of London</td>
<td>Benitec is working with researchers at RHUL and at the Institut de Myologie in Paris to treat and cure OPMD sufferers using ddRNAi-modified muscle stem cells</td>
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Benitec funded programs | Partnered program | Licensed program
ddRNAi Technology for Cancer Pain Treatment –

A New Class of Pain Therapeutics
The Problem With Cancer-Associated Pain

Approximately 65% of all cancer patients experience pain.

Opioids are the first line of therapy for cancer pain, but their efficacy is often insufficient and their side effects can be severe.

“It could be argued that we shorten lives of many patients by giving them increasing doses of strong opioids.” - Palliative Care Specialist

Neuropathic pain was universally regarded by KOLs as the most difficult type of pain to treat with currently available therapies.

“A NEW APPROACH TO PAIN IS NEEDED”
(5th Annual Pain Summit, San Francisco, September 2011).

Global Incidence of Cancer-Associated Pain Among Cancer Patients

- 65% Experience Cancer-Related Pain
ddRNAi can uniquely fill that need

- Single treatment
- Localised administration
- Specific single target
- Well understood mechanism of action
- Long term pain relief
Key Success Factors

- The best target
- The best silencing sequence
- Medical acceptance
- Proven safety
- Proven efficacy
- FDA approval
- Big pharma partnering
- Speed to market

Overall Opinion of the ddRNAi Product

Average: 6.4

Question: On a scale of 1 to 7 (1=Very low, 7=Very high) how would you rate this product based on the product profile?
Choosing the best target

In the last six months, Benitec has been examining two possible targets in a rat model of neuropathic pain – DAO and PKCγ.

Whilst encouraging results have been achieved with DAO,

data from an independent group have persuaded us to move to PKCγ as the lead target.
Validation of PKC_γ as the Best Neuropathic Pain Target

- PKC_γ is *increased in the spinal cord in cancer pain*

- Researchers have *proven the concept* in a rat model of neuropathic pain – the rats with ddRNAi silencing of PKC_γ showed a significant *increase in pain threshold*.

- The increase in pain threshold was dose-dependent and *lasted for the duration of monitoring*.

- *No toxicity* was observed in any animals for six weeks following injection of vectors.

- Switching off PKC_γ also *overcomes morphine tolerance*

*Source: Results of nine KOL interviews conducted by Campbell Alliance in July and August 2011*
Validation of PKCγ as the Best Neuropathic Pain Target

Leading clinical pain specialists strongly endorsed the mechanism of action because of the strong connection between PKCγ and neuropathic pain.

“Noting the mechanism of neuropathic pain and the cascade of evidence associated with the activation of kinases, this product seems to be good, especially in neuropathic pain but also in opioid tolerance.”
—Palliative Care Specialist

“The type of cancer pain with nerve compression is the hardest to treat…PKCγ would be better…PKCγ is involved in the development of allodynia, so blocking it makes sense”
—KOL in Pain Research

Source: Results of nine KOL interviews conducted by Campbell Alliance in July and August 2011.
Potential Lead Indication for the ddRNAi Pain Product

KOLs saw terminal cancer patients as an ideal lead indication for the ddRNAi product due to the level of novelty of the product and the length of trials required for an indication in this patient segment.

- KOLs see strong potential for the ddRNAi cancer pain product to be used in terminal patients suffering from all types of cancer.

"Virtually all cancer patients at some time in their disease experience neuropathic pain. It’s more common in terminal patients…performance and pain are intertwined…"
—Palliative Care Specialist

"Terminal cancer patients are a good group to target…it’s a group that the FDA will allow you to go after relatively easily."
—KOL in Pain Research

Source: Results of nine KOL interviews conducted by Campbell Alliance in July and August 2011.
Progress and Plans

Feb – June 2011  Target Selection (I)
• Confirmation that DAO inhibition blocks pain
• DAO constructs selected
• Tested in neuropathic pain model
• Confirmed gene silencing achieved in vivo
• Needs dose optimisation

July – November 2011  Target Selection (II)
• Publication of ddRNAi silencing of PKCγ POC
• Development of testing model
• Design and testing of range of sequences to determine most effective across all species
Progress and Plans

May-August 2011  Market Assessment
•  Product specification defined
•  Interviews with key pain specialists
•  Target market identified

September 2011- May 2012  IND Package Development
•  Confirmation of target
•  Design of FDA compliant experiments
•  Regulatory requirements incorporated into experimental design
•  Preliminary clinical trial design

November 2011 - Partnering Outreach Program
•  Big pharma companies advised of pain program opportunity
•  Feedback being gathered
Progress and Plans

Overview of in vivo and laboratory testing

- BEN-001 DAO 1st gen (mission clones)
- BEN-002 qRT PCR testing DAO mRNA
- BEN-003 Monitor in vivo delivery
- BEN-004 Assay glial cells
- BEN-005 Banks from C6 (mouse) & U87 (human)
- BEN-006 Primary screen k/oDAO in C6 & U87
- BEN-007 Secondary screen DAO k/o in C6 & U87
- BEN-008 Na benzoate
- PWT testing validated DAO
- BEN-009 PWT Testing PKCg lentis (x2) * Ordered July 8; 5-8 weeks
- BEN-010 Test 7 PKCg shRNAs in vitro
- BEN-011 Secondary PKCg shRNA screening
- BEN-012 PWT testing
- Pre-IND Regulatory Process

Proof of Concept

X
**ddRNAi Cancer Pain Product Original Development Plan and Timeline**

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<td>Phase I/II Clinical</td>
<td>IND Preparation and Submission</td>
<td>Phase I/II Trial (cancer patients with intractable pain)</td>
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<td>Studies</td>
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**Additional Planned Studies:**
- Toxicology and biodistribution studies
- Large Phase II and III studies in cancer patients
- Other indications*

* Other indications include: diabetic neuropathy, postherpetic neuralgia, and HIV/AIDS
**ddRNAi Cancer Pain Product**

**Updated Development Plan and Timeline**

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**A New Class of Pain Therapeutics**

**Additional Planned Studies:**
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- Other indications*

* Other indications include: diabetic neuropathy, postherpetic neuralgia, and HIV/AIDS
Current ddRNAi Programs: Hepatitis B

The ddRNAi technology is also being developed for HBV under collaboration with Biomics Biotechnologies in China.

**Rationale:**
- Successful ddRNAi delivery to the liver has been demonstrated.
- The targeted enzyme is key to HBV replication.
- The strategy provides treatment of the existing infection and long-lasting protection from re-infection.

**Market Size:**
- There are about 400 million people worldwide with chronic HBV infection.
- Carriers of HBV are up to 300 times more likely to develop liver cancer than non-carriers, and HBV causes 60-80% of the world's primary liver cancers.

**Collaborator:**
- Benitec is working with Biomics to evaluate RNAi candidates for hepatitis B silencing for preclinical testing and a China-based clinical trial.

**Status:**
- Benitec is working with Biomics to evaluate RNAi candidates for hepatitis B silencing for preclinical testing and a China-based clinical trial.

**Of 5000 potential sequences, 14 RNA sequences have been identified by Biomics that provide ≥70% knockdown of HBV gene mRNA.**
Summary of the Project

• Phase I (Biomics)
  - EsT libraries
  - *In vitro screening*

• Phase II (collaboration between Biomics & Benitec)
  - Convert siRNAs to shRNAs
  - Validate shRNAs *in vitro*
  - Design construct(s) expressing 3 active shRNAs
  - Validate *in vivo* using AAV delivery
HBV program:
target identification – from virus to gene to effective sequences

5000 potential target sequences

14 RNA sequences identified by Biomics that provide ≥70% knock down of HBV gene mRNA
**Overview of Phase II**

**In vitro**
- siRNA candidates validation
- Convert siRNAs to shRNAs
- Validate shRNAs *in vitro*
- Optimize the promoters
- Definite triple cassette components (*specify minimal expression and full potency against HBV*)

**In vivo**
- Peak expression time and dose optimization of AAV8
- siRNA validation *in vivo*
Reduction of HBV DNA Levels by Continuous Transfection of shRNAs

Continuous siRNA transfection inhibited HBV DNA levels in a time-dependent manner.
Calibration of the Activity of shRNAs

The relative activity of shRNA is:

shRNA-8 > shRNA-12 > shRNA-3 > shRNA-17 > shRNA-11.
Optimization of delivery vector

20 days after dsAAV8-GFP treatment

- **Vehicle**: Few GFP positive
- **2$\times$10$^{11}$ v.p/mouse**: ~20% GFP positive
- **4$\times$10$^{11}$ v.p/mouse**: ~60% GFP positive
- **8$\times$10$^{11}$ v.p/mouse**: Close to 100% GFP positive
Conclusions

Achieved to date
• Several shRNA constructs have been identified
• Expression levels being optimised
• Proof of concept of delivery to 100% liver cells achieved

Next steps
• Build a triple cassette construct with maximum silencing activity and minimal shRNA expression
• Package into AAV8 for *in vivo* testing
• *In vivo* validation of safety and biodistribution
• Preparation for clinical trial
## Original Development Plan and Timeline for HBV Program

### HBV Program Clinical Development Timeline

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<td>Preclinical testing (in vivo models)</td>
<td>GLP Manufacturing</td>
<td>Toxicology studies</td>
<td>IND Preparation and Submission</td>
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<td>Design vector-expressed constructs</td>
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**HBV Program Clinical Development Timeline**

**Program**
- Hepatitis B

**Year**
- 2011
- 2012
- 2013
- 2014
- 2015

**Activities**
- Target Sequence Efficacy
- Design vector-expressed constructs
- Preclinical testing (in vivo models)
- GLP Manufacturing
- Toxicology studies
- IND Preparation and Submission
- Phase I/II Trial
# Updated Development Plan and Timeline for HBV Program

## HBV Program Clinical Development Timeline

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Oculopharyngeal Muscular Dystrophy

Background

Oculopharyngeal muscular dystrophy is a rare dominant inherited genetic condition for which no effective treatment exists.

- Oculopharyngeal muscular dystrophy (OPMD) is an inherited, slow progressing, late onset degenerative muscle disorder that usually starts in the fifth or sixth decade of life.

- The disease is characterised by drooping of the eyelids, swallowing difficulties and limb weakness.

- The condition often leads to death following swallowing difficulties and choking

- OPMD is an orphan disease. It is a rare condition (1 in 100 000 in Europe) with a worldwide distribution.

- No cure, even no medical treatment is presently available for oculopharyngeal muscular dystrophy.

Oculopharyngeal Muscular Dystrophy Incidence and Prevalence

Source: http://healthmedcare.com/Health/muscular-dystrophy/

Healthy muscle tissue

Affected muscle tissue
The ddRNAi OPMD Treatment Target

A mutation in the Poly(A) Binding Protein Nuclear 1 gene (PABPN1) is known to be responsible for the onset of oculopharyngeal muscular dystrophy.

- OPMD is particularly adapted to *gene therapy based on RNA interference* since targeted cells are limited, the genetic mutation is small, known and located on a relatively small gene.

- We are working with Professor George Dickson and Dr Capucine Trollet at the Royal Holloway, University of London and the Institut de Myologie in Paris, where there are a variety of different *validated models and materials* are available for use in preclinical studies.

- A focused but inexpensive study will commence in January 2012 to develop the first therapy for this previously untreatable disease.

Source: *Institut de Myologie OPMD research data*
**ddRNAi OPMD Treatment Development Plan and Timeline**

<table>
<thead>
<tr>
<th>OPMD</th>
<th>2012</th>
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<td>Materials Development</td>
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<td>Lentivector studies</td>
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<td>AVV Local Delivery Studies</td>
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Current ddRNAi Programs: Drug Resistant NSCLC

Benitec is collaborating with University of New South Wales researchers to develop a ddRNAi-based therapeutic targeting a gene associated with chemotherapy resistance.

**Rationale:**
- βIII-tubulin has been shown to be associated with drug resistance in non-small cell lung cancer (NSCLC).
- Silencing βIII-tubulin using ddRNAi increases the killing of NSCLC cells by chemotherapy agents.

**Market Size:**
- Lung cancer is the leading form of cancer worldwide in terms of incidence and mortality.
- NSCLC account for >80% of all lung cancers and has a high mortality rate due to rapid development of resistance to chemotherapy drugs.

**Collaborator:**

**Status:**
- Researchers are using Benitec’s technology to develop a ddRNAi-based therapy to overcome chemotherapy resistance in NSCLC cells.

*In vitro results show highly effective silencing of βIII-tubulin and effective targeting of NSCLC cells with the ddRNAi construct.*
Professor Maria Kavallaris

Head, Pharmacoproteomics Program
NHMRC Senior Research Fellow
Director, Australian Centre for Nanomedicine, UNSW

Proof of concept study:

Silencing of drug resistance gene βIII-tubulin in NSCLC cells using ddRNAi constructs in vitro and in vivo
Non-small Cell Lung Cancer (NSCLC)

- Lung cancer is the most common cause of cancer death in the world
- Majority of cases diagnosed at advanced disease stage
- Treatment of NSCLC typically involves the use of surgery, radiation and chemotherapy
- 2 common classes of chemotherapy agents used in NSCLC:
  - Tubulin binding agents (TBAs) eg. Taxol
  - DNA damaging agents eg. Cisplatin

Despite advances in the treatment of NSCLC, 5 year survival rates for advanced disease are still dismal
Increased βIII-tubulin in NSCLC associated with clinical:

- drug resistance and aggressive disease


*Seve et al. Mol Cancer Ther 2005
Validating βIII-tubulin as a therapeutic target in NSCLC Cells

βIII-tubulin shRNA vector

GENE TARGETING with RNAi

Delivery into cancer cells lines

Vector incorporates into cancer cells and expresses βIII-tubulin shRNA

Suppression of βIII-tubulin expression
Validating βIII-tubulin as a therapeutic target in NSCLC Cells

βIII-tubulin expressing NSCLC

Low dose chemotherapy

Most cells alive

Suppression of βIII-tubulin expression

Low dose chemotherapy

Most cells dead
Gene and protein silencing of βIII-tubulin using ddRNAi

% Knockdown of βIII tubulin RNA

- H460-Luc
- RS mix
- pS5
- pS6

55 KDa βIII-tubulin

32 KDa GAPDH loading control

βIII tubulin PROTEIN
Chemosensitisation of NSCLC

βIII-tubulin triple cassette vector

Suppression of cancer gene

Chemotherapy
Orthotopic NSCLC mouse model

Human lung cancer cells orthotopically xenografted in mouse lung

Josh McCarroll, Rafael Erlich, Tanya Dwarte

2X10^6 cells

14 days post-injection

βIII-tubulin

Tumour

X40

Lung

H460-luc cells
βIII-tubulin gene expression of tumour tissue (preliminary data)

NB: One mouse in each group (1) Saline control and (2) βIII-Triple cassette shRNA vector. Received three injections of vector.
Chemosensitisation of NSCLC

βIII-tubulin triple cassette vector

Suppression of cancer gene

Chemotherapy
## Development Plan and Timeline for NSCLC Program

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<td>Animal model (lung)</td>
<td>In vivo Toxicology</td>
<td>IND Preparation and Submission</td>
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### NSCLC ddRNAi Program Updated Clinical Development Timeline

- **Animal model (lung)**:
  - 2011

- **In vivo Toxicology**:
  - 2012

- **IND Preparation and Submission**:
  - 2013

- **Phase I Clinical Trial**:
  - 2014
Gene silencing for a potential HIV/AIDS cure
Why Do We Need New Therapies for HIV?

Toxicities associated with the current drug therapies. Medications have to be taken daily (often multiple times per day) for the rest of the patient’s life.

There are strains of HIV with resistance to current anti-viral drugs and there are no alternatives for these patients. The current drugs simply keep the virus in check but do not cure the patient.

Gene Therapy can be a way of making cells immune to HIV infection eventually resulting in a cure.
Combining Small RNAs as Anti-HIV Therapeutic Agents
First in man-Lentivirus-shRNA Stem Cell Transplant for HIV
HIV/AIDS Phase I results

- ddRNAi therapy is **safe and feasible**
- **Long lasting** (at least 2 years) from a single treatment
- Able to rebuild a **resistant immune system**

A. Gene Marking of Peripheral Blood Monocytes

Expressed shRNA persists **for at least 2 years**

B. Gene Marking in Immune Cells

Differentiated immune cells (T and B cells, monocytes and granulocytes) carry the expressed shRNA.
Clinical follow up at 36 months

- shRNA expression still present in patient 306 CD34+ stem cells at 36 months
- Number of peripheral blood cells carrying the shRNA expanded 6 fold
- Professor Rossi described these results as “spectacular”
Summary and Significance

• First in human trial of ddRNAi based gene therapy.
• Patients are in complete remission from lymphoma, all have suppressed HIV loads.
• ddRNAi expression now out to 3 years from a single treatment.
• The safety and feasibility of this study has opened the door for this treatment approach to possibly cure HIV by supplying gene modified stem cells that totally repopulate the patients’ blood cells with HIV resistance.
• Long term expression of shRNA from a single treatment has important implications for the feasibility of Benitec’s other programs targeting long term gene silencing for treatment or cure of the disease.
Benitec Biopharma Corporate Overview

Business Overview

Based in Sydney, Australia, Benitec is a biopharmaceutical company developing a novel DNA-directed RNA interference (ddRNAi) platform for therapeutic use. The company is listed on the Australian stock exchange (ASX: BLT).

Business Strategy

Benitec is pursuing licensing, partnering and co-development activities for its transformational, proprietary ddRNAi platform technology for human therapeutics and research.

Product Strategy

Benitec is currently utilising ddRNAi technology internally across multiple therapeutic areas where there is a significant unmet need to develop ddRNAi-based therapeutic products for a range of conditions including lung cancer, neuropathic pain, infectious disease (hepatitis B and hepatitis C) and genetic disease (muscular dystrophy).

Intellectual Property

Benitec has a robust patent portfolio protecting platform technology across the major pharmaceutical markets with patent coverage extending through 2027.

Management Team

Benitec has a strong management team with deep scientific and clinical resources and extensive experience with the commercialization of biological intellectual property.
Investment Opportunity Summary

**ddRNAi Product Asset Summary**

- **Large Market Opportunities**
  - Non-Small Cell Lung Cancer, Pain, Hepatitis
  - and a plethora of other opportunities

- **Favorable KOL Responses**

- **Transformational Approach**
  - New class of therapeutics for treatment and cure

- **Favourable preclinical data**
  - From Extensive in vitro and in vivo Studies

- **Unmet Medical Need**
  - ddRNAi can treat undruggable targets

- **Extensive IP Estate**