

## BENITEC CEO PRESENTS AT BIO-INVESTOR FORUM – SAN FRANCISCO

**Sydney, Australia, 8 October 2014:** Benitec Biopharma Limited (ASX: BLT, OTC: BTEBY ) Chief Executive Officer, Dr Peter French, presented an update on the Company at the 13th Annual BIO Investor Forum in San Francisco on October 7 at 9.30am local time.

The BIO Investor Forum is an international biotech investor conference focused on early and established private companies as well as emerging public companies. The event features plenary sessions, business roundtables and therapeutic workshops, company presentations, and one-on-one meetings between companies and investors and industry business development, licensing and therapeutic franchise heads.

Dr French has more than 10 such meetings scheduled. He is accompanied at the BIO Investor Forum by Dr David Suhy, Benitec's Senior Vice President of Research and Development.

The presentation highlighted the value proposition that Benitec offers as a relatively undervalued company in the area of gene silencing through RNA interference with an extensive pipeline of programs in significant life threatening diseases, any one of which, if successful, would be a company-maker in its own right. The presentation gives an update on the HCV clinical trial, and describes the current situation with sub-licensee Calimmune's HIV/AIDS clinical trial. Benitec's ability to transact on its significant pre-clinical and clinical technology is exemplified through its international and experienced Board and management.

The presentation was well received by the audience of investors and industry representatives. Dr French is conducting a range of other meetings in the US outside of the conference.

For more information, please contact the persons below or visit the Company's website at [www.benitec.com](http://www.benitec.com).

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### **About Benitec Biopharma Limited:**

Benitec Biopharma Limited is an ASX-listed biotechnology company (ASX: BLT, OTC: BTEBY) based in Sydney, Australia. The company has a pipeline of in-house and partnered therapeutic programs based on its patented gene-silencing technology, ddRNAi. Benitec is developing treatments for chronic and life-threatening human conditions such as Hepatitis C, Hepatitis B, wet age-related macular degeneration, cancer-associated pain, drug resistant lung cancer and oculopharyngeal muscular dystrophy based on this technology. In addition, Benitec has licensed ddRNAi technology to other biopharmaceutical companies who are progressing their programs towards the clinic for applications including HIV/AIDS, retinitis pigmentosa and Huntington's disease. For more information on Benitec refer to the Company's website at [www.benitec.com](http://www.benitec.com).



Dr Peter French,  
Chief Executive Officer

San Francisco  
October 7, 2014

# GENE SILENCING: A quiet revolution in healthcare

# Forward Looking Statements



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 Biopharma 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.

This document does not constitute an offer, solicitation or recommendation in relation to the subscription, purchase or sale of securities in any jurisdiction. Neither this presentation nor anything in it will form any part of any contract for the acquisition of securities.

# Company Financial Snapshot



Key financial details	ASX:BLT OTC: BTEBY
Share Price as at close of trade 16 <sup>th</sup> September 2014:	AUD \$1.05
Market Capitalisation as at 16 <sup>th</sup> September 2014:	AUD \$121M
Issued Securities as at 16 <sup>th</sup> September 2014:	
Ordinary shares	115,218,993
Options	22,695,098
Cash balance at 30 <sup>th</sup> June 2014:	AUD \$31.3 M

# Access to the US OTC Market



## ADR Benefits to U.S. Investors:

- ADRs give direct access to our listed equity capital base allowing participation in cross-border market liquidity
- Company disclosure via OTC website
- ADRs are cost-effective
- ADRs are convenient to transact and own
- Quoted in U.S. dollars
- Settle via standard U.S securities settlement process
- Program is administered by a market leading global depositary

## Benitec Biopharma ADR trading

Symbol	BTEBY
CUSIP	082053208
Ratio	1 ADR : 5 ORDs
Country	Australia
Effective date	May 30, 2014
Underlying SEDOL	6710507
Underlying ISIN	AU000000BLT8
Depositary	BNY Mellon

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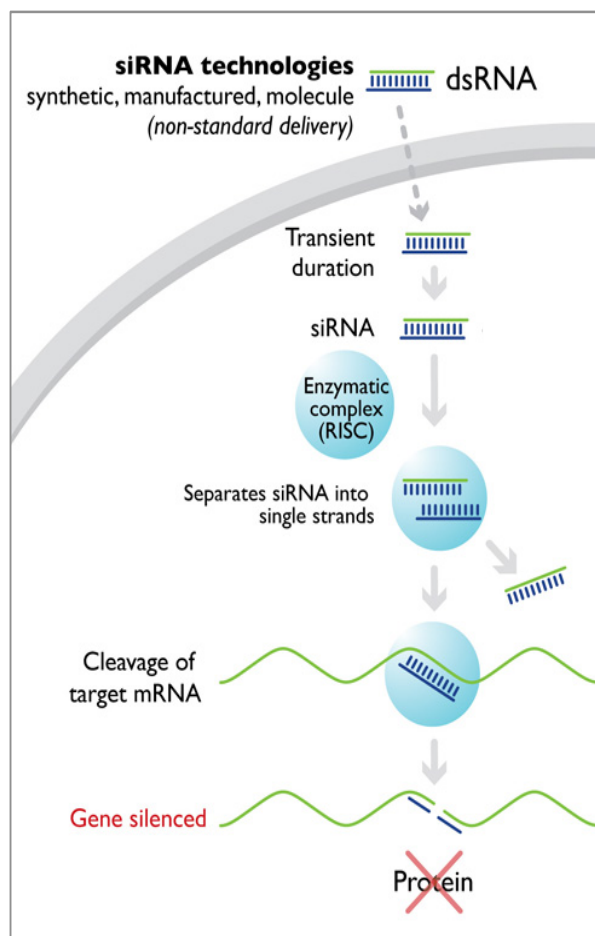
# Benitec Investment Case



- Developing a novel gene silencing technology – ddRNAi – a treatment and “single shot cure” for a range of diseases.
- Recently secured \$31.5m in funding from quality US institutions, which will see Hepatitis C (TT-034) trial funded to Phase IIb and progress of other programs in pipeline, and platform development.
- Commenced patient dosing of TT-034 Phase I/IIa clinical trial in Hepatitis C.
- Successful trial results will validate the specific disease approach and the broad technology for human health.
- Pipeline of interesting and “company making” programs – each designed to respond to unmet clinical needs.
- RNAi is a hot space as positive clinical trial data starts to emerge for Benitec’s peers. Partnering activity has been renewed and accelerated as a result.

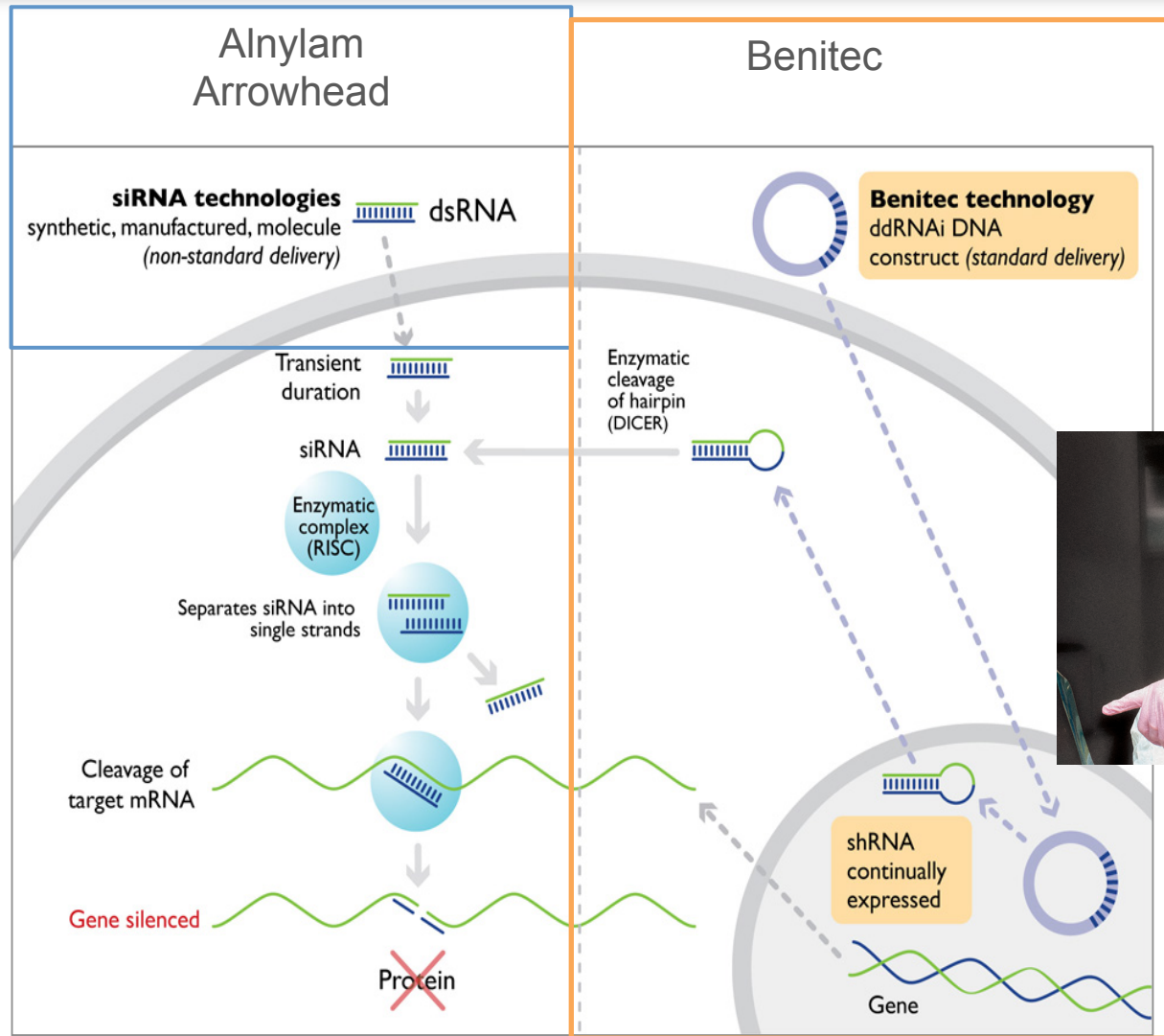
# RNAi

## The Next Revolution in Medicine



# RNAi

## The Next Revolution in Medicine



# ddRNAi Technology The Next Revolution in Gene Silencing

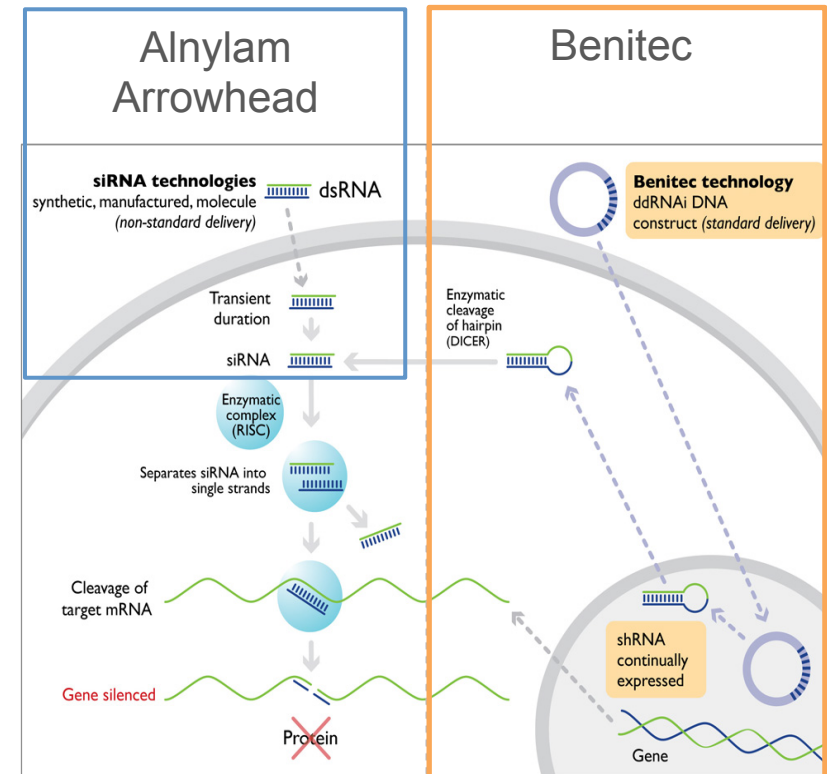


A specific and long lasting method for turning off disease-associated genes.

ddRNAi technology utilises the power and specificity of RNAi while avoiding many of its problems:

- Specific delivery to target cells
- Fewer side effects
- Lasting benefits –dsRNA generated continuously for the life of the cell
- Multiple therapy in a single molecule - can be engineered to silence a specific gene, multiple sites on a gene or multiple genes

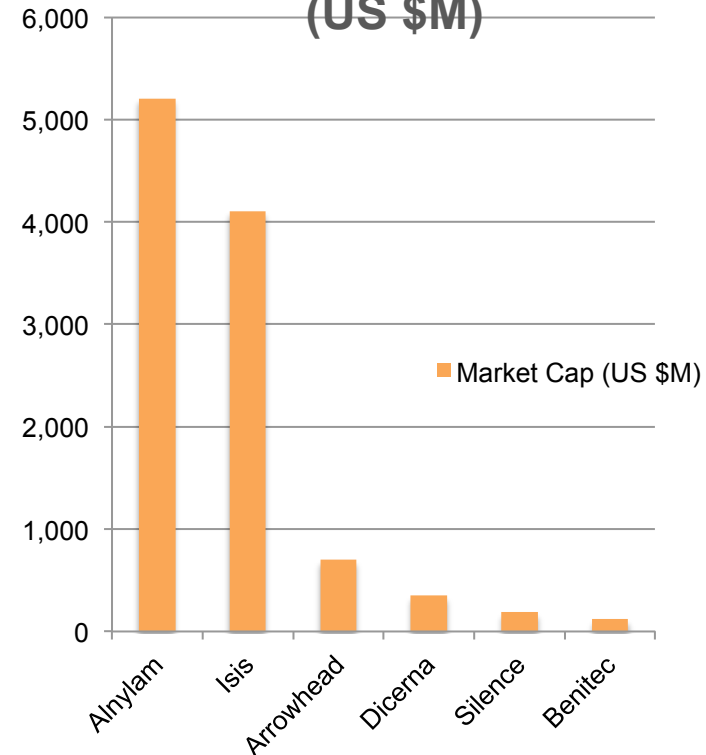
Protected by a dominant, global patent estate - over 100 patents covering ddRNAi and specific disease targets.



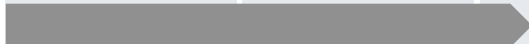
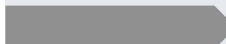
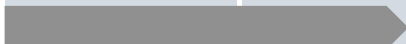
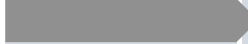

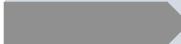
# Value proposition

Company	Technology	Stage	Market Cap (US \$M)
Alnylam	siRNA	Phase II	5,200
Isis	Antisense	Phase II/III	4,100
Arrowhead	siRNA	Phase I	700
Dicerna	siRNA	Pre-clinical	350
Silence	siRNA	Pre-clinical	187
<b>Benitec</b>	<b>ddRNAi</b>	<b>Phase I/IIa</b>	<b>120</b>

Market Cap of Comparator  
Gene Silencing Companies  
(US \$M)



# In-house Programs

Focus	Indication	Partners / Collaborators	Discovery	Pre-clinical	Clinical
Infectious Disease	Hepatitis C				
	Hepatitis B	Biomics Biotechnology (JV)			
Cancer	Non Small Cell* Lung Cancer	University of New South Wales (RC)			
	Cancer Associated Pain	Stanford University (RC)			
Ocular Disease	AMD**				
Genetic Disease	OPMD***	Royal Holloway London University (RC)			

# Hepatitis C clinical trial progress



TT-034 is an RNAi therapeutic that is intended as a “one-shot-cure”.

Goal is to achieve complete and sustained elimination of virus with a single infusion.

## US-based open-label dose-escalation Phase I/IIa trial underway

- Protocol reviewed and approved by NIH RAC with unanimous panel support
- FDA released IND January 12, 2014
- Patient dosing commenced May 29, 2014
- **DSMB recommends study continue with no modification**
- Next patient dosing delayed due to patients falling outside of clinical criteria but screening is in progress

## Trial sites

- Duke Clinical Research Unit, North Carolina - currently dosing & continuing to screen additional patients
- University of California, San Diego – currently screening patients
- Additional trial sites being brought on to accelerate patient recruitment

# Phase I/IIa Dose Cohorts

Cohort	Dose (vg/kg)	Dose escalation step (log 10)	Total No subjects	Dosing scheme for subjects	Observation period per subject and between cohorts before dose escalation
1	$4.00 \times 10^{10}$	Starting dose	2	Sequential (1+1)	6 week
2	$1.25 \times 10^{11}$	0.5	3	Sequential and parallel (1+2)	6 week
3	$4.00 \times 10^{11}$	0.5	3	Sequential and parallel (1+2)	6 week
4	$1.25 \times 10^{12}$	0.5	3	Sequential and parallel (1+2)	10 weeks
5	$4.00 \times 10^{12}$	0.5	3	Sequential and parallel (1+2)	10 weeks

# TT-034 Clinical



- Clinical demonstration of a “game changer” for treatment of HCV - As a “single shot cure,” TT-034 would compete with small molecule cocktails:
  - Compliance, side effect profile, efficacy, cost effectiveness
- Provides a validation of Benitec’s other pipeline programs – for safety, efficacy
- Will provide clinical data and leverage for partnering discussions

# Hepatitis B Update



- A replica of TT-034 approach. 350 million people infected worldwide, major unmet medical need – substantial interest from big pharma
- Strategy to leverage success of RNAi therapies utilising long term benefit of ddRNAi
- Expanded collaboration with Biomics
  - Sequence validation – homology search completed
  - Optimisation of construct in progress
  - Animal model of HBV identified

# Drug Resistant Lung Cancer: Tribetarna™ Update

- **Tribetarna Proof of Principle established:**
  - A single injection of Tribetarna™ effectively silences the  $\beta$ III tubulin gene
  - Tribetarna™ significantly enhances survival in a preclinical model of lung cancer in combination with chemotherapy
- **A Phase I/IIa clinical trial of Tribetarna™ in conjunction with cisplatin is being prepared**
  - Undertaken prep-preIND meeting with US FDA
  - Manufacture of GLP Product for reg/tox studies underway
  - European CRO appointed – CTG CRO



With clinical success in lung cancer, this approach can be developed to target other cancers that express high  $\beta$ III tubulin (breast, ovarian & gastric)

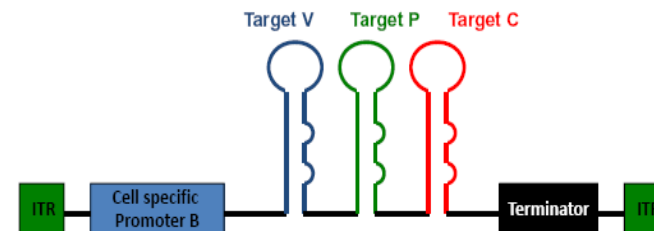
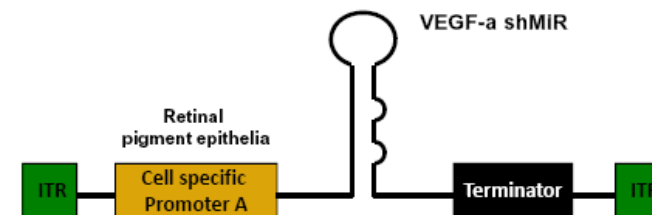
# AMD Update

**Age-related macular degeneration (AMD)** – A single injection of a ddRNAi construct to replace current standard of care (monthly injections into the eyeball).

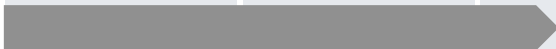


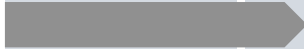
- Advanced negotiations for a suitable vector for delivery of ddRNAi therapy
- Research collaborator identified – primate model validation



## Constructs designed and tested:



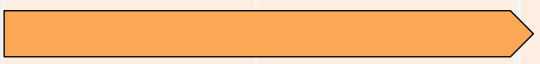


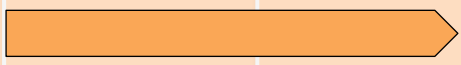




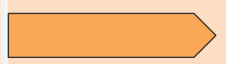

# Sub-licensed Programs

Focus	Indication	Partners/Collaborators	Discovery	Pre-clinical	Clinical
Infectious Disease	HIV/AIDS	Licensed to Calimmune			
Cancer	Cancer Vaccines	Licensed to Regen BioPharma			
Ocular Disease	Retinitis Pigmentosa	Licensed to Genable			
Genetic Disease	Huntington's Disease	Licensed to uniQure			

## **CALIMMUNE** engineering immunity

- **Developing a ddRNAi-based ex vivo treatment for HIV/AIDS**
- **Currently conducting a Phase I/II clinical trial**
- **12 HIV-positive participants infused with their own T cells and hematopoietic stem cells, which have been modified to silence the HIV receptor CCR5, and so prevent HIV fusion.**
- **The procedure is designed to prevent the virus from entering and damaging protected cells.**
- **The first patients have been dosed and on reviewing the safety data the DSMB have approved the next cohort dosing. Data from this study are expected in 2015.**

## In-house and sub-licensed Programs

Focus	Indication	Licensees/Collaborators	Discovery	Pre-clinical	Clinical
Infectious Disease	Hepatitis C				
	HIV/AIDS	Licensed to Calimmune			
	Hepatitis B	Biomics Biotechnology (JV)			
Cancer	Non Small Cell* Lung Cancer	University of New South Wales (RC)			
	Cancer Associated Pain	Stanford University (RC)			
	Cancer Vaccines	Licensed to Regen BioPharma			
Ocular Disease	AMD**				
	Retinitis Pigmentosa	Licensed to Genable			
Genetic Disease	OPMD***	Royal Holloway London University (RC)			
	Huntington's Disease	Licensed to uniQure			

RC = research collaboration  
JV = joint venture

\*and other chemotherapy-resistant cancers

\*\*Age-Related Macular Degeneration

\*\*\*Oculopharyngeal Muscular Dystrophy, an orphan disease

# Commercially-focused Management and Board



## MANAGEMENT

**MD and CEO: Peter French, MBA, PhD**  
CSIRO, St Vincent's, Cryosite founder

**CSO: Michael Graham, PhD**  
Inventor of ddRNAi technology  
CSIRO, Benitec founder

**CBO: Carl Stubbings, BSc**  
Panbio, Quest Diagnostics, Focus Diagnostics

**SVP R&D: David Suhy, PhD**  
Tacere Therapeutics, Avocel, Antara  
Biosciences, PPD Discovery

**CFO: Greg West, CA**  
Price Waterhouse, Bankers Trust, Deutsche Bank, NZI

## BOARD

### Chairman:

**Peter Francis, LLB, Grad Dip.**  
(Intellectual Property)  
Partner at Francis Abourizk Lightowlers

### Directors:

**John Chiplin, PhD**  
Polynoma, Arana, ITI Life Science Fund

**Iain Ross, BSc, CH.D.**  
Silence Therapeutics, Tissue Therapies,  
Ark Therapeutics

**Kevin Buchi**  
Cephalon, Teva, Mesoblast, Tetralogic

# Summary



- Developing Nobel Prize-winning technology – RNAi
- “Single shot cure” for a range of diseases.
- Over 100 patents and patent applications
- Extensive pipeline of “company making” programs
- Clinical stage – HCV and HIV/AIDS programs poised to validate the technology for other diseases
- Well funded - over \$30M from 10 US institutional investors in Feb 2014
- Internationally credible and experienced Board and Management

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