

## Phylogica's technology is effective in human cells

Phylogica (ASX: PYC) ('PYC') is a drug development company creating a pipeline of therapies for blinding eye diseases. PYC provides the following update in relation to the first ever assessment of its Cell Penetrating Peptide (CPP) delivery technology in a pilot study in human cells.

### Highlights

- **PYC's CPP technology has successfully delivered** an Anti-Sense Oligonucleotide (ASO) drug cargo into human Retinal Pigment Epithelial (RPE) cells
- This **critical read-out** has been **achieved in our flagship program** directed towards the delivery of an ASO cargo into a target cell type in the back of the eye
- The result compared exon skipping (a measure of efficacy) achieved by an ASO both 'with' and 'without' PYC's CPP – **the ASO alone achieved 8% exon-skipping and the CPP-ASO achieved 100% exon-skipping** in the human cells (see Figure 1)
- This result represents the second major pre-clinical milestone on **PYC's pathway to developing CPP-ASO treatments for a range of blinding eye diseases** (see Figure 2) - the company has now addressed the two fundamental pre-clinical issues of:
  - demonstrated effect in animal models; and
  - demonstrated effect in human cells.

**6 August 2019:** PYC has completed the first assessment of our Cell Penetrating Peptide (CPP) delivery technology in human cells. Following on from successful animal models (see ASX announcements of 27 June and 23 July), PYC has now demonstrated the ability of our CPPs to deliver an Anti-Sense Oligonucleotide (ASO) into human cells. These two pieces of data represent the critical inputs in a pre-clinical data pack.

These experiments are part of our flagship development program - testing our CPPs in the cell type of interest to create treatments for blinding eye diseases. The cells are known as Retinal Pigment Epithelial or RPE cells and represent a high value target cell in drug development.

*Technical details*

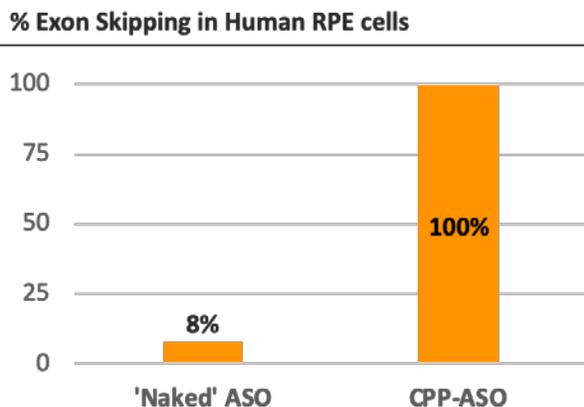


Figure 1. Performance of a 'naked' SMN1 (Survival of Motor Neuron-1) ASO compared to performance of PYC's CPP-ASO conjugate in human Retinal Pigment Epithelial (RPE) cells – both at 5 micromolar, n=1. The result demonstrates the efficacy of the CPP in facilitating delivery of the ASO inside the cell to enable it to engage with its target mRNA. Higher levels of exon skipping indicate more effective delivery of the ASO by the CPP.

*Relevance of results to our drug development program*

Figure 2



There are two critical pieces of data that inform the prospect of successful translation of pre-clinical results into clinical (human) outcomes (and a potential marketed drug). They are animal model results and human cellular results respectively. This data represents successful achievement of the second technical milestone described above and complements our earlier animal model experiments with the same CPP (see ASX announcements of 27 June and 23 July).

An additional indicator of our prospects of success in the clinic is available for retinal diseases in the form of 3-Dimensional organoid models (also known as 'retina in a dish'). The 3D organoid models combine the structural complexity of a living organism represented in the animal models with the unique elements of human cellular material. The benefits of successful studies in 3D organoids are an increased probability of success in the subsequent clinical (human) evaluation of the drug molecule.

PYC will now focus on delivery of the final pre-clinical technical milestone (3D retinal organoids) in the final quarter of this year before initiating Investigational New Drug (IND) enabling studies to facilitate clinical evaluation of our technology.

PYC is grateful for the support of our two key collaborators in Murdoch University/the Centre for Molecular Medicine and Innovative Therapeutics and the Lions Eye Institute/the Ocular Tissue Engineering Laboratory in designing and conducting these latest experiments.

**ENDS**

*Delivering drug cargoes across cell membranes is the major challenge in the development of a revolutionary new class of drugs. Cell Penetrating Peptides (CPPs) can overcome this challenge and provide access to the 'undruggable genome' – the highest value drug targets that exist inside cells. Phylogica (ASX:PYC) owns the world's most structurally diverse peptide library and is using these libraries to identify a new generation of highly efficient CPPs.*

For further information, please contact:



### **About Phylogica**

Phylogica Limited (ASX: PYC) is a biotech company focused on commercialising its intracellular drug delivery platform and screening its peptide libraries to identify drug cargoes for development against a wide range of disease targets. Phylogica controls access to the world's most structurally diverse source of peptides that have the ability to act as effective drug delivery agents and drug cargoes, penetrating cell membranes to reach previously 'undruggable' targets across a range of disease types. Phylogica's platform of proprietary cell penetrating peptides has been validated across multiple animal models for the ability to deliver a diverse range of drug cargoes into cells. The company has collaborations with several pharmaceutical companies including Roche, Medimmune, Pfizer, Janssen and Genentech.

### **Forward looking statements**

Any forward-looking statements in this ASX announcement have been prepared on the basis of a number of assumptions which may prove incorrect and the current intentions, plans, expectations and beliefs about future events are subject to risks, uncertainties and other factors, many of which are outside Phylogica's control. Important factors that could cause actual results to differ materially from assumptions or expectations expressed or implied in this ASX announcement include known and unknown risks. Because actual results could differ materially to assumptions made and Phylogica's current intentions, plans, expectations and beliefs about the future, you are urged to view all forward-looking statements contained in this ASX announcement with caution. Phylogica undertakes no obligation to publicly update any forward-looking statement whether as a result of new information, future events or otherwise.

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