

Cell-penetrating Phylomers improve delivery of oligonucleotide drugs inside cells

Oligonucleotide drugs are new-generation therapies to treat genetic diseases such as muscular dystrophy. A key challenge with these drugs is to optimise their delivery into cells to make treatments much more effective.

- *Independent pilot studies show that a Phylogica cell-penetrating Phylomer (CPP) can improve the delivery of an antisense oligonucleotide drug inside human cells*
- *The cell-penetrating Phylomer was also shown to improve the efficiency of oligonucleotide delivery in a mouse model using two different delivery routes*
- *Study confirms Phylogica's cell penetrating Phylomers can deliver a broad range of cargoes inside cells*
- *Collaboration will be expanded to focus on improving the effectiveness of oligonucleotide therapies for genetic diseases such as Duchenne muscular dystrophy with results expected in the second half of 2016.*

Perth, Australia, 15th June 2016: In February, 2016, Phylogica (ASX: PYC) initiated a collaboration with Professors Sue Fletcher and Stephen Wilton from the Centre for Comparative Genomics at Murdoch University to assess whether a cell penetrating Phylomer (CPP) could improve the delivery of an antisense oligonucleotide drug inside cells. This effort is aligned to our strategy of engaging in external collaborations to provide independent validation that our technology can expand the druggable landscape.

Phylogica is pleased to announce a successful outcome to these studies, which confirmed the CPP significantly enhanced intracellular delivery of the drug relative to a control oligonucleotide that was not linked to the CPP. Similar outcomes were achieved when the drug was delivered into cultured human cells and also using a mouse animal model.

Antisense oligonucleotides work by binding to the genetic message (mRNA) and editing mutated regions that cause disease. Such drugs are showing great promise as therapies to reduce the severity of genetic diseases.

Professor Sue Fletcher said, "We're really encouraged by these pilot studies and will expand our collaboration to focus on developing more effective treatments for Duchene muscular dystrophy (DMD)."

Phylogica's Chief Scientific Officer, Dr Paul Watt, commented, "We are delighted to be working with the team at Murdoch University, which has pioneered the use of antisense oligonucleotide technologies to treat mutations that cause Duchenne muscular dystrophy. These efforts by the team at Murdoch University have led to clinical trials that are currently ongoing."

Phylogica's CEO, Dr Richard Hopkins said, "This study further validates our cell-penetrating Phylomers for their ability to deliver different therapeutic cargoes inside cells more safely and with greater efficiencies. We've now shown we can deliver a whole new class of drug that has the potential to open up commercial opportunities in the oligonucleotide field, which has seen a significant resurgence over recent years."

Based on the successful outcome to the pilot studies, this collaboration is being expanded to focus on development of more effective treatments for DMD. These studies will target the genetic mutations underlying DMD in cell and animal models of disease.

A further study aim is to determine whether Phylogica's delivery technology can improve drug activity without compromising drug safety – a challenge the previous generation of cell-penetrating peptides failed to overcome.

Phylogica looks forward to reporting on the outcome of these studies in the second half of 2016.

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About The Molecular Therapy Laboratory and The Centre for Comparative Genomics

(<https://ccg.murdoch.edu.au/about/>)

The Molecular Therapy Laboratory (MTL) is embedded within the Centre for Comparative Genomics at Murdoch University in Perth. The MTL undertakes design and development of antisense compounds to modify gene expression. The MTL has a focus on rare inherited diseases, with a particular interest in neuromuscular disorders. The MTL researchers have expertise in manipulating alternative splicing, antisense oligomer design and evaluation, synthesis of research grade antisense oligonucleotides, designing therapeutic strategies for a range of genetic disorders and assessing genetic modifications in vitro and in vivo.

About Phylogica

Phylogica Limited (ASX: PYC) is an oncology-focussed biotech company discovering and developing a new generation of biologics-based therapies against intracellular cancer targets. The company was originally spun out from the Telethon Kids Institute (Perth, Australia) and the Fox Chase Cancer Centre (Philadelphia, USA). Phylogica controls access to the world's most structurally diverse source of peptides - called Phylomers. The company specialises in Phylomer-based solutions to discover and deliver novel biologics drugs against intractable intracellular cancer targets with unprecedented potencies. Phylogica is advancing its proprietary oncology programmes developing first-in-class therapies against transcription factors such as MYC and STAT5. These targets play a critical role in many common cancers such as breast, lung, prostate and pancreatic, but have proven undruggable with conventional small molecule therapies. Within the last six years, the company has entered into discovery collaborations with Roche, Genentech, MedImmune, Pfizer, Janssen and Cubist Pharmaceuticals.