Human trials have begun on a potentially groundbreaking cancer drug

September 18 2012, by Linda Mcsweeny

(Medical Xpress)—In medical science, remarkable things sometimes happen that make years of toiling in the lab worthwhile.

Most rewarding is a discovery that could save lives, or at least makes an illness easier to bear.

"The odds are very small but you have that chance," says Professor Philip Hogg, who is at the helm of the first human trials of a potential new therapy designed to shrink cancerous tumours.

Only a handful of Australian researchers get the chance to be involved in, yet alone lead, a human trial of a drug that could transform clinical practice.

Professor Hogg, who is director of the Lowy Cancer Research Centre and was the 2009 NSW Cancer Researcher of the Year, and his colleague Dr Pierre Dilda developed the compound that could provide an alternative to chemotherapy.

The compound, named PENAO, inhibits how tumours metabolise sugar and is the latest invention for the scientists, who began working together on new cancer drug strategies more than a decade ago.

PENAO works by making inactive a key protein in energy-producing structures in tumour cells known as mitochondria, Hogg says.

PENAO is a second-generation compound developed out of the success of trials in the UK of the first-generation compound, named GSAO.

Human trials of PENAO began in July at the Peter MacCallum Cancer Centre and at Royal Melbourne Hospital and will include patients with solid tumours, such as those of the breast, prostate, colon and brain.

"Tumours metabolise sugar differently than normal tissue," Hogg says. "The compounds we have made target this difference."

While there are hurdles to overcome, Hogg is hopeful the compounds will form the basis of new cancer therapies.

"The first-generation molecule we made – the GSAO – at best, would stop tumours from growing, so the hope for that compound was to turn cancer into a manageable disease.

"With this second-generation compound, we're hoping to be able to take the process a step further and actually shrink the tumours."

Hogg says the "early signs are good – PENAO is well tolerated like the first-generation drug and is much more effective in pre-clinical testing".

The trial involves about 20 patients and is expected to last 18 months.

Medical oncologist Dr Jayesh Desai is the principal investigator who oversaw treatment of the first patient, a woman with late-stage cervical cancer, at Royal Melbourne Hospital.

"We are very excited to have treated the first patient. PENAO offers a very positive step forward," he says.

The drug is pumped directly into the bloodstream, which is considered

the best delivery pathway and easiest for the patient. Participants can take the pump home with them and need only weekly hospital visits for check-ups. The drug is delivered in two cycles over a total of 42 days.

Dilda says being able to take cancer drugs from the bench to the bedside has been "a great achievement".

"As research scientists, we are fortunate to be involved in projects that have an impact in 'real life'," he says.

Hogg admits he was excited when, about 12 years ago, he and his team discovered the compounds they made were knocking out the power supply of cancer cells.

But he adds there are "degrees of excitement", with many stages to complete before the discovery can translate into cancer treatments.

However it is the hope of delivering a breakthrough – similar to the story of the discovery of insulin that inspired him as a student – that keeps him going.

"You have the chance of doing something fantastic. We all understand it's very infrequent and the odds of us doing something really worthwhile are pretty small – very small – but there's that chance and often that's enough."

Provided by University of New South Wales