

# Melanoma drug trials give terminal patients a lifeline

4:00AM Wednesday Jun 03, 2009

By [Martin Johnston](#)

Scientists have developed the first targeted drug effective against advanced melanoma, the deadliest form of skin cancer.

Repeated sunburn increases the risk of developing malignant melanoma and New Zealand has one of the highest rates of it.

Each year, it is diagnosed in about 1900 people and it kills about 250.



Repeated sunburn increases the risk of developing malignant melanoma and New Zealand has one of the highest rates of the cancer. Photo / Glenn Jeffrey

Starting as a blemish or change to a mole on the skin, melanoma is readily treated by surgery in its early stages, but once it has spread to other organs such as the lungs and liver, it is usually incurable and therapy can only help to control symptoms such as pain.

Patients usually die within months.

In the first human trial of the new drug, known as PLX4032, those treated lived on average for six months without their disease getting worse.

Asked what difference the drug made, one doctor said that for the first time he had "time to get to know my patients".

The drug is the first of half a dozen in development to target melanomas with a genetic make-up containing the so-called "BRAF" mutation.

They are the harbingers of a revolution in cancer medicine which is developing drugs tailored to the genetic make-up of individual cancers, so treatment can be targeted for each type of tumour, rather than being determined only by the cancer's location or stage.

Sixty per cent of advanced melanomas and 8 per cent of solid tumours are BRAF positive.

The new drug works by targeting and destroying tumour cells that carry the BRAF gene. It has no effect against melanomas that lack the mutation.

It is also being developed for use in other cancers, including bowel cancer.

Tumours shrank by more than 30 per cent in nine of the patients treated in a trial and survival without disease progression was extended.

# The New Zealand Herald

The results were presented at the American Society for Clinical Oncology meeting in Orlando, Florida, on Monday.

Trial leader Assistant Professor Keith Flaherty, of the University of Pennsylvania, said: "Seven years after BRAF mutations were first identified, we have validation that this mutation is a cancer driver and therapeutic target. In addition to a new and important chapter in the story of targeted therapy development in cancer, we are especially excited for our melanoma patients for whom there are few treatment options."

Auckland cancer specialist Dr Mike McCrystal said it seemed to be one of the few promising drugs for melanoma.

But it was still early days. Many experimental melanoma drugs that had shown promise early on had failed in later trials.

"It's also likely to have a few side effects I suspect."

One of his patients was enrolled in a study of PLX4032 in Melbourne and brought the pills home to take in New Zealand.

"He's experiencing quite a lot of side effects - a lot of aches and pains and skin rashes."

In Britain, the number of people diagnosed with melanoma has risen fourfold since the 1970s and has exceeded 10,000 annually for the first time.

Roche developed the drug with its partner Plexxikon, a biotech company. It is being introduced with a diagnostic test to screen patients for the BRAF mutation.

Professor Caroline Springer, the head of the gene targeting team at the Institute of Cancer Research in London, which is developing its own BRAF inhibitor, said the gene mutation was an "excellent target" for drug development.

"Our agent is very different to [PLX4032]. We intend to trial it at the Royal Marsden Hospital in London. We are optimistic there will be a trial.

"I think Roche are in the lead - they are certainly getting there quite quickly but there is a long way to go. It is obviously very exciting to have a drug whose potential could be very good."

She said half a dozen BRAF inhibitors were being prepared for clinical trials but the teams developing them were yet to prove they were safe and effective.

- INDEPENDENT