Skin cancer treatment: Biggest breakthrough in 30 years

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Being Human
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Two new drugs for metastatic melanoma - the deadliest form of skin cancer - are being hailed as the biggest breakthrough therapies for cancer in the last 30 years. The drugs reduce tumour size, significantly increasing survival rates.

Although melanoma can be cured if caught early enough, individuals in the late stages of the disease are only expected to survive for an average of six months. One of the two drugs - vemurafenib - works by inhibiting the effects of a mutated form of the BRAF gene, which is thought to accompany around half of the cases of malignant skin tumours.

Early trials of the drug had shown that around half of patients with the mutation responded to the treatment. Now, Paul Chapman at the Memorial Sloan-Kettering Cancer Center in New York and colleagues have shown that vemurafenib outperforms dacarbazine - the most commonly prescribed chemotherapy drug for metastatic melanoma.

In a study presented this week at the American Society of Clinical Oncology annual meeting in Chicago, and published in the New England Journal of Medicine, Chapman's team compared both drugs on 672 patients with late stage, inoperable melanoma and a mutation in the BRAF gene.

The group found that 48 per cent of those receiving vemurafenib responded to the treatment, while only 5 per cent of patients responded to dacarbazine. At 6 months, survival was 84 per cent in the group taking vemurafenib compared to 64 per cent in those taking dacarbazine.

The results were so impressive that the team stopped the trial early in order to switch all the participants to vemurafenib. Hal Barron, head of global development at Roche, who funded the study, said in a statement: "We are greatly encouraged by the results, which showed that vemurafenib not only extended life and reduced the risk of disease worsening, but also led to significant tumour shrinkage, an important result for this devastating cancer."

Yet more encouraging results were presented at the meeting by a group hoping to boost the effects of dacarbazine by combining it with another drug - ipilimumab - developed by pharmaceutical company Bristol-Myers Squibb.

Ipilimumab is an antibody that works by boosting the immune system's response to a tumour. Caroline Robert at the Gustave Roussy Cancer Institute in Villejuif, France, and her colleagues compared the effects of the drug in combination with dacarbazine to a placebo in people with late stage, inoperable melanoma.

Robert's team found that 28.5 per cent of the 250 patients who received combination therapy survived for two years - a marked improvement on the 17.9 per cent of those taking dacarbazine alone (New England Journal of Medicine, DOI: 10.1056/NEJMoA1104621).
The US Food and Drug Administration approved the drug for late stage melanoma back in March after an early viewing of the study results. Now, the European Medicines Agency has recommended the drug for approval in Europe - a decision that the European Commission will make before August.

The success of the trials is being met by overwhelming enthusiasm from the cancer research community. Peter Johnson, chief clinician at Cancer Research UK told the BBC: "For the first time, we have effective treatments becoming available for melanoma. Both [studies] show how the research we have been doing is feeding through into help for patients".

Lynn Schuchter at the University of Pennsylvania's Abramson Cancer Center told the Associated Press: "This is really an unprecedented time of celebration for our patients."

She says that the new drugs are not by themselves cures, but "the future is going to be to build upon the success" by testing combinations of these newer drugs.

And that's just what the two pharma giants have promised to do: "We have made significant progress in treating metastatic melanoma and hope to further improve outcomes by combining two agents that target this deadly disease in different ways," said Roche's Barron. "We look forward to working with [Bristol-Myers Squibb] in this ground-breaking collaboration to explore new options for patients."