Highlight: Scientific breakdown of cancer reveals promising results

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The chemical structure of the melanoma-shrinking drug PLX4032 is revealed in a new *Nature* paper that describes the drug's discovery, development and functioning.

PLX4032 recently showed promising results in an early clinical trial of melanoma patients carrying a mutated version of the BRAF gene. Gideon Bollag and colleagues now explain the science behind the success story.

In particular, they note that sufficiently high levels of the drug, which works by blocking the activity of the oncogenic B-RAF protein, are needed to yield clinical effects, because the ERK signalling pathway, downstream of B-RAF, needs to be almost completely blocked.

The study demonstrates how the design of early clinical trials based on the biological mechanisms underlying tumour formation can speed the translation of anti-cancer drug from laboratory to clinic. And whilst the long-term effects of PLX4032 remain uncertain, it's hoped that the small molecule inhibitor prove useful in combination with other targeted agents, immunotherapies or chemotherapies.

More information: Paper: DOI: 10.1038/nature09454

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