

Breakthrough announced in treatment of patient with rare type of leukemia

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L-R: This shows Ph.D. student Jesvin Samuel, Dr Salvador Macip and Professor Martin Dyer. Credit: University of Leicester

A team of scientists from the University of Leicester has demonstrated a novel treatment for Hairy Cell Leukaemia (HCL), a rare type of blood cancer, using a drug administered to combat skin cancer.

The research, which is published today (Thursday 16 January) in the

New England Journal of Medicine, indicates Vemurafenib, a BRAF inhibitor that has been approved as a treatment for advanced melanomas, is also successful in treating leukaemia. The study shows the treatment, which can be taken orally, cleared the malignant cells from the patient's blood and led to a complete clinical recovery in a number of days.

The study was led by the University of Leicester and involved treatment of a patient at the Leicester Royal Infirmary.

Dr Salvador Macip, from the University of Leicester's Department of Biochemistry, explained: "A genetic study of the patient's blood cells allowed us to identify a mutation in the BRAF gene that is commonly found in skin cancers. This knowledge enabled us to combat the [cancer cells](#) with Vemurafenib, which has had proven success as a BRAF inhibitor in melanomas, and showed similar success for this patient who had exhausted all other treatment options, which is fantastic.

"What was most surprising was that the drug did not work in the way we expected it to. Whilst it successfully blocked BRAF and killed the cancerous cells, there was no ability to block the downstream cascade of signals. Therefore more research is required to better understand how this drug works to ensure we are able to use it in the best possible way.

"This is one of the first clinical examples of this treatment for HCL and we are the first researchers to do a biochemical study of the samples and discover that the drug does not do what it's supposed to be doing."

This approach to targeting cancer is an example of precision medicine with clinicians and research scientists working side-by-side to ensure the best treatment, tailored to the individual patient, was provided.

Professor of Haemato-Oncology at the University of Leicester, Professor Martin Dyer, who is Honorary Consultant Physician,

Department of Haematology at Leicester's Hospitals, said: "Precision medicine in which clinicians and basic scientists collaborate to deliver novel and rapid personalised therapies to cancer patients like this is essential.

"We drew blood from the patient on a daily basis which was analysed back in the lab to monitor the effects of the drug. The more understanding we have of how treatments such as Vemurafenib kill cancer cells, the more effective and targeted treatments can be."

Professor Dyer is based in the University of Leicester's Department of Cancer Studies and Molecular Medicine and the [treatment](#) of the patient took place at the Leicester Royal Infirmary.

This research shows that drugs currently used to target certain cancers could be applied in other malignancies that share similar genetic backgrounds.

University of Leicester Pro-Vice-Chancellor and Head of the College of Medicine, Biological Sciences and Psychology, Professor David Wynford-Thomas, said: "The importance of the close working partnership between the University of Leicester and Leicester's Hospitals is highlighted in advances such as this. World-class research at the University brings direct benefits to patients in Leicester's hospitals in diverse areas including cardiovascular health, kidney research, lung health, diabetes, [cancer](#) research and many other areas.

"I am delighted that our research has had such a direct benefit locally - it is another first for the University of Leicester and Leicester Royal Infirmary."

More information: Jesvin, S; Macip, S and Dyer, M.J.S. Efficacy of Vemurafenib in Hairy-Cell Leukemia, *New England Journal of*

Medicine, [DOI: 10.1056/NEJMc1310849](https://doi.org/10.1056/NEJMc1310849)

Provided by University of Leicester

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