World-first blood cancer drug trial reveals life-changing results

November 11 2015

Shown is a close-up of an intravenous (IV) bottle. Credit: Linda Bartlett/public domain

Researchers from the University of Leicester and Leicester's Hospitals

have announced a breakthrough advance in the results of the world-first clinical trial with actual patients of a new drug to treat particular blood cancers.

Results of an international clinical trial led by Dr Harriet Walter and Professor Martin Dyer from the Ernest and Helen Scott Haematological Research Institute at the University of Leicester and from the Leicester Royal Infirmary have just been published in the journal *Blood*.

This clinical trial, a first-in-man study, looked at the efficacy of a new inhibitor, ONO/GS-4059, in the treatment of Chronic Lymphocytic Leukaemia and Non-Hodgkin Lymphoma patients refractory or resistant to current chemotherapies.

ONO/GS-4059 targets BTK, a protein essential for the survival and proliferation of the tumour cells.

This study opened in January 2012 and 90 patients were enrolled in different centres in the UK and in France, with 28 coming from Leicester. Patients with Chronic Lymphocytic Leukaemia showed the best response and most of them are still on the study after 3 years, and remarkably without notable toxicities.

The success story of this drug, has paved the way for its future development in combination studies, which will be opening to recruitment shortly in Leicester.

Dr Harriet Walter, from the Department of Cancer Studies at the University of Leicester, said: "These patients were confronted with a cruel reality: they had failed multiple chemotherapy lines and there were no other treatment options available for them. This drug has changed their lives; from desperate and tired they are now leading a normal and really active life. This is hugely rewarding and encouraging".

Professor Martin Dyer is Professor of Haemato-Oncology at the University of Leicester and Honorary Consultant Physician in the Department of Haematology at Leicester Royal Infirmary. He said:

"I am so delighted that we have been able to run this study in Leicester. The establishment of the Hope Against Cancer Clinical Trials Facility, under the directorship of Professor Anne Thomas, allows us to lead these kinds of studies that really do change the life of our patients.

"We are dedicated to offer the best treatment options to our patients and the development of targeted therapies that increase the chance of therapeutic success and at the same time avoid toxicities generally observed in chemotherapies, is the most exciting progress in cancer research."

Asking one of the patients on the trial what was their feelings about it, they said: "After just 48 hours of taking this tablet is was like turning the lights on".

Nigel Rose, Chief Executive of local cancer research charity, Hope Against Cancer said: "We are delighted that these results are due to the establishment of our Hope Clinical Trials Facility. Improved, life-saving treatment for the people of Leicestershire is why our charity was set up here locally and is exactly what we are aiming to achieve".

The next step is now to see how best we can improve on these outstanding results. A further study using this drug in combination with additional targeted agents is shortly to open in Leicester with the aim of achieving cure. In parallel with the clinical development of the drug, our team of scientists at the Haematological Research Institute are studying how this drug is working and how to overcome potential resistance.

More information: H. S. Walter et al. A phase I clinical trial of the

selective BTK inhibitor ONO/GS-4059 in relapsed and refractory mature B-cell malignancies, *Blood* (2015). DOI: 10.1182/blood-2015-08-664086