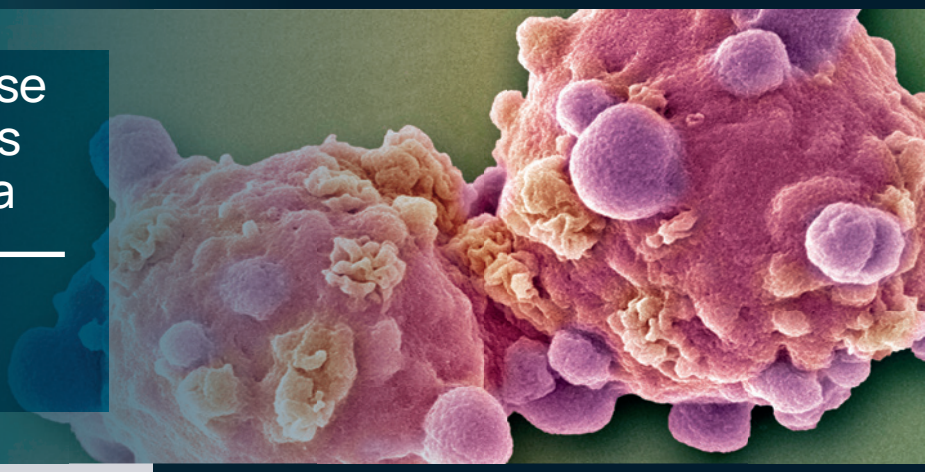
Case study

SIMBEC-ORION

Efficient enrolment and dose escalation process ensures rapid access to critical data

Non-hodgkin's Lymphoma and
Chronic Lymphocytic Leukaemia



An open-label, multi-centre, non-randomised phase I dose-escalation study to investigate the safety and tolerability of the study drug given as monotherapy in patients with relapsed/refractory non-hodgkin's lymphoma (NHL) and relapsed/refractory chronic lymphocytic leukaemia (CLL).

Background

Our Sponsor needed to ensure first-inpatient study success in Europe, in a highly competitive oncology field amongst sites and patient availability. A minimum enrolment of 42 patients was required to complete the study.

Objectives

- To investigate the safety and tolerability of escalating oral doses of (study drug) given as monotherapy in patients with relapsed/ refractory NHL and relapsed/ refractory CLL.
- To examine peripheral blood lymphocytosis and recovery with increasing doses of (study drug).
- To obtain preliminary data on the antitumour efficacy of (study drug) given as monotherapy in patients with relapsed/ refractory NHL and relapsed/refractory CLL.
- To investigate pharmacogenomic markers and their association with clinical response and toxicity.

Dose escalation study in a highly competitive oncology indication

Built strong site relationships in a competitive field for patient recruitment

Study design optimised to boost investigator and patient participation

Agile cohort expansion after enrolment more than doubled

Forward-thinking IMP management to ensure drug availability during dose escalation

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Challenges

Due to the highly competitive field, it was important sites fully committed to the study and did not have any competing projects planned.

It was crucial that the study design fulfilled all scientific/regulatory objectives without being excessively complex to avoid discouraging investigators and patients from taking part.

Favourable IMP results led to the potential for 'competition' among centres for slots in the dose escalation cohorts. This placed critical importance on the timely and efficient provision of data to the safety committee to ensure dose escalation decisions could proceed rapidly without bias.

Study enrolment increased from 42 patients to 90 through changes in the study and decisions by the Sponsor to expand various cohorts.

Timely supply of the drug was necessary to ensure it was available at the time any new cohort was going to be enrolled, to prevent any delay in patient enrolment.

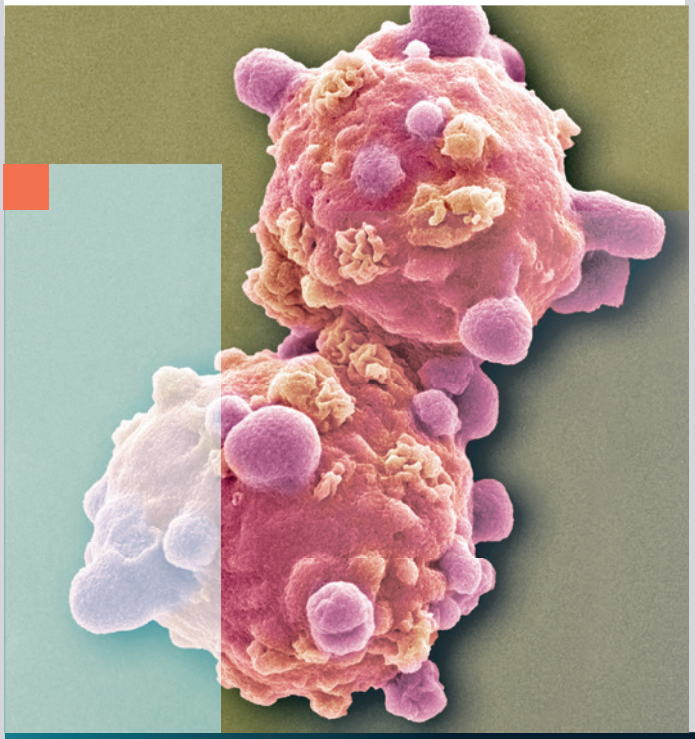
Objectives

- Working closely with the sponsor, we ensured that the fi nal protocol was as 'patient friendly' as possible.
- Each potential site was visited by experienced Simbec-Orion medically qualifi ed personnel and/or an oncology Project Manager to ensure they were fully committ ed and engaged with the project, this was key to keep momentum and enrolment of patients.
- Careful monitoring of patients enrolled by each site within each cohort to ensure that where multiple patients were available in any one site, then priority was given to sites that had not enrolled in that cohort.
- Thanks to close communication with the Sponsor, we could optimise IMP management eff iciency ensuring cost eff ectiveness for the IMP.
- When a decision was made to escalate the dose, an order for the next level was prepared rapidly, ensuring the study drug was available when required without storing excessive quantities at the sites.

Outcome

The therapeutic principle was rapidly established, thanks to swift and efficient conduct of the study.

The sponsor was able to communicate the results to the scientific and business communities earlier than anticipated with a larger data set of 90 patients rather than 42 patients originally planned. This led to a highly favourable outcome for the sponsor in terms of the commercial development of the product.



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