A4I Analysis for Innovators

Supporting the development of a novel product to ensure the safety of cell therapy treatments

Cell therapies, where living cells are transplanted in to a patient, are seen as the future of treatment in a number of therapeutic areas including diabetes and cardiovascular disease.

> These therapies can be derived from stem cells, tissues, or organs grown in laboratories and are expected to revolutionise medical care for a range of currently untreatable diseases.

Cell therapies are increasingly using pluripotent stem cells (PSCs) to generate derived cell therapy products. PSCs, by their very nature, can potentially be used to create any cell or tissue the body might need. However, residual PSCs in the final cell therapy product also have the potential to form tumours and so concerns have been raised around the safety of PSC-derived cell products.

There is therefore a need to accurately measure the level of residual pluripotent stem cells, i.e. those that could form tumours, in a derived cell therapy product. It is expected that the likely regulatory guidelines will permit residual PSCs at levels of less than 10 PSC in 1 million derived cells. Accurate, specific and sensitive quality controls will be required to allow cell therapies to be developed in to safer clinical products more quickly.

Sistemic has developed a prototype product based around microRNA biomarkers to detect these residual PSCs in a simple, highly sensitive and rapid test format. Through the Analysis for Innovators (A4I) partnership, Sistemic had access to innovative and advanced measurement and analytical technologies at the National Measurement Laboratory (NML), hosted at LGC, to further develop their prototype product and make it ready for the rapidly developing market in PSC-derived cell therapy products.

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The NML worked with Sistemic Ltd to enhance the sensitivity and specificity of their novel prototype microRNA-assay to the levels required for market (<10 PSCs per million derived cells).

Using the NML's expertise within digital PCR, a microRNA-based droplet digital PCR assay was developed and optimised for detection of residual PSCs. The final assay, capable of consistently detecting both 10 and 5 residual PSCs in 1 million derived cells, was developed in a commercially viable format alongside a data dossier detailing the full technical specifications of the assay.

> The success of this project has led to the granting and filing of a number of patent applications and the technical evidence has enabled Sistemic to demonstrate their capability to meet the requirements for new customers in providing validation data for regulatory product approval.

This has supported the signing of a new Global Service and License Agreement between Sistemic and Cynata Therapeutics Ltd and the creation of two new jobs to further develop their novel product (Sistem*PSC*Check™). The details of the License and Service Agreement were not disclosed, but it is thought that the valuation of Sistemic has increased by several million pounds due to the development of Sistem*PSC*Check™ resulting from the work done under A4I.

Sistemic's assay will help ensure producers of stem cell products can now accurately assess levels of residual PSCs in derived cell therapy products. This will in turn result in patients getting quicker access to these novel therapies during clinical trial development phases, and ultimately, to a successfully launched clinical product and therefore improvements in quality of life.

"We are delighted to have worked with LGC, utilising their expertise as the National Measurement Laboratory for chemical and bio-measurement. The successful outcome of this project has the potential to facilitate the clinical progress of pluripotent stem cell-derived cell therapy products, allowing them to be developed to safer clinical products more quickly."

David Mallinson
Vice President of Scientific Operations, Sistemic

Sistemic

A4I

A4I is a programme that gives UK businesses, of any size, access to cutting-edge R&D expertise and facilities to help solve problems that they have been unable to tackle using standard techniques. The focus is on solving issues affecting product cost, reliability or lifetime and production problems.

NPL nel flow measurement services NML

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