

Analysis for Innovators (A4I): Supporting industry

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

Background

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 PSCs at no more than 10 PSC in 1 million cells. Accurate, specific and sensitive quality controls will be required to allow cell therapies to be developed into safer clinical products more quickly.

Sistemic had developed a prototype product based around miRNA 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.

Impact

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

Using our expertise within digital PCR, a microRNA based droplet digital PCR assay was developed and optimised for assessment of residual PSC contamination. 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 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 worth several million pounds between Sistemic and Cynata Therapeutics Ltd and the creation of two new jobs to further develop their novel product (SistemPSCCheck™). 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 SistemPSCCheck™ 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

Analysis for Innovators (A4I)

The A4I programme is run by the UK National Measurement Laboratories, the Science and Technology Facilities Council and Innovate UK. It provides companies with access to state-of-the-art measurement and analytical technologies to solve existing analysis and measurement problems that are a barrier to competitiveness or productivity.



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