Introduction

In addition to agricultural, environmental and industrial application, gene editing techniques present substantial possibilities in medicine and healthcare for the diagnosis, treatment and prevention of serious illness and disease.

Refinement of these techniques and subsequent development of safe and effective products for these purposes, in many cases, has been underway for several decades. Recent examples include mRNA vaccines, as well as treatments for sickle cell disease, muscular dystrophy, HIV and other conditions.

While progress in addressing other conditions that have previously been out of reach has been made, unnecessary regulatory barriers continue to hinder the research and development needed to substantiate safe and effective medicines and accelerate processes from development to clinical trials.

Under existing regulatory conditions and provisions of the National Gene Technology Scheme and associated legislation, SDN-2 and Oligo-Directed Mutagenesis (ODM) gene editing techniques are regulated as conventional genetic modification, despite some of the resulting products being indistinguishable from those developed through conventional processes.

Some applications of SDN-2 and ODM use site-specific guides and repair templates that allow for highly specified changes and predictable outcomes in comparison to randomly induced mutations when using SDN-1 techniques. This increased specificity not only accelerates product development, but it also allows for greater predictability and increased success of the desired alteration.

Continuing to regulate these techniques and their products as genetic modification will hamper the development of critical technologies in medicine and healthcare sectors, like affordable diagnosis and treatment options to address serious health issues.

Gene technology techniques such as SDN-2 and ODM which have a history of safe use should be excluded from regulation. This can be achieved as part of the current review of the Scheme by linking to changes in definitions. This would ensure the Scheme remains relevant and would pave the way for a more proportionate regulatory system. It would eliminate the need for further, unnecessary and lengthy steps of regulatory reform and would ensure better access for researchers and developers to the full range of tools needed to support Australian innovation.

Australia needs a National Gene Technology Scheme that encourages and supports agricultural, medical, and pharmaceutical research and enables innovation. This will increase and improve Australian food security, health, wellbeing, and economic performances. This can only be achieved if biotechnology techniques are regulated in a proportionate, science-based manner.

Case Study – Individualised treatment for cancer patients

In early 2020, the Therapeutic Goods Administration (TGA) of Australia approved a new cancer treatment known as CAR T-cell therapy, via the product Kymriah® (tisagenlecleucel), for the treatment of leukaemia and lymphoma in patients where alternative treatment options have been exhausted.

CAR T-Cell therapy is a once-off, individualised treatment that uses gene editing techniques beyond the capability of SDN-1 to reprogram the patient's own T-Cells to fight cancer. It is primarily used to treat blood cancers; however clinical trials are underway to also apply CAR T-Cell therapies to solid tumours.

Prior to approval, Australians receiving immunotherapy were required to have blood collected and sent overseas to undergo the reprogramming process before the cells could be returned and used for treatment.

Domestic regulatory approval removed a major bottleneck of international processing and transport. Enabling Australia's first onshore commercial manufacture of the therapy substantially reduced patient treatment time, from blood collection to infusion. Government approval also led to subsidised access to the treatment, providing eligible patients with new hope and the prospect for improved quality of life and even a cure for their life-threatening illness.

Therapeutic products like Kymriah® can be life-changing for patients and their loved ones. In addition to the direct medical benefits, the success of these developments attracts vital funding for health and medical research for discoveries that will drive future innovation. This also contributes more broadly to Australia's health and economic future by improving public health and quality of life, maintaining a strong workforce and reducing the cost of health care through improved techniques and technology. These improvements could subsequently lead to savings in the health budget. Definitions of medical research, health practice activities and use of data for these programs according to current regulations, however, can become complex and limit prospects of important medical research.

Due to this approval, institutions such as the Peter MacCallum Cancer Centre can continue to explore the technology and expand possibilities for the treatment of other cancers. This in turn can lead to clinical trials and the potential commercialisation of products to improve health outcomes and improve the lives of patients suffering from chronic and critical illnesses.

CAR T-cell therapy is just one application of gene editing in medicine that has the potential to improve patients' lives. It is just one example of the positive impact appropriate regulation can have for patients and for the Australian community more broadly. More needs to be done to ensure Australian and global communities can access the lifechanging products of gene editing by supporting technology providers to move beyond proof of concept, to clinical trials of safe and effective novel treatment solutions.