PMAS/21/07 - Centre for Precision Cell Therapy for the Liver.

We aim to bring new products for the treatment of liver diseases and Type I diabetes to patients and boost our ability to perform liver related clinical trials in Scotland.

Since 1970, deaths due to liver disease have quadrupled and it is the biggest cause of death in people aged 35 to 49. It is a disease of inequalities, unfairly affecting the poorest and the most vulnerable in society. People who live in more deprived areas are up to six times more likely to die of liver disease than those who live in wealthier areas. Chronic liver disease often results in liver cirrhosis, but there are currently no specific medical treatments for liver cirrhosis. Liver transplantation is a cure but only for a small number of patients. It is limited by the supply of suitable organs and a global shortage of donor organs means that many patients die waiting for a transplant.

Liver transplants cannot meet the numbers of Scottish patients with liver failure or end-stage liver disease, therefore alternative treatments are urgently needed. As many patients with chronic liver disease are diagnosed at an advanced stage, there is a need to develop better therapies for patients with advanced liver disease. Because of the different causes of liver damage, each condition has different disease processes, so a different 'precision medicine' approach is required for each specific liver disease. Cell and gene therapies (known as Advanced Therapy Medicinal Products, ATMPs) offer the ability to precisely fix biological defects in damaged organs and are now providing cures in previously untreatable diseases (e.g. CAR-T cell therapy for cancer).

We will use funding from CSO to build on our outstanding existing skills and facilities to create a 'Centre for Precision Medicine for the Liver'. We will use precise advanced therapies to target the liver to treat severe liver disease, reduce liver mortality and morbidity and the need for liver transplantation. This will involve different forms of cell therapy that we have shown are effective in model systems and in one type of cell therapy safe in patients with cirrhosis. This funding will allow investment in critical staff and expertise in key "bottleneck areas" within Scottish National Blood Transfusion Service, pharmacy and advanced therapy trial management. This will serve the Scottish population and be a platform for the delivery of these novel therapies.