

Centre for the Technologies of Gene and Cell Therapy

THE BRIDGE BETWEEN BIOMEDICAL RESEARCH ON
ADVANCED TREATMENTS AND ITS TRANSFER TO PATIENTS

PROJECT LEAD



PARTNER OF EXCELLENCE



IN COOPERATION WITH AN EXPERT PARTNER



Known Hundreds of types of cancer
1 in 2000 Slovenians
has a rare disease,
half of them, children.



For most rare diseases and cancers, there are no effective cures yet. Remarkable advances in the field of life sciences promise to cure the cause of many diseases in the years to come. **Advanced treatment approaches such as gene and cell therapies are highly effective because they are targeted, tailored to individual patients or groups of patients,** and can even lead to a lasting cure.

The approach to each patient is central to the development of new therapies. Access to the latest treatment methods is particularly important at this time.

Genome editing technologies can be used to insert a desired genetic signature into cells or to correct a part of the genome that causes a disease.

Today, we know the genetic cause of thousands of diseases. Modern technologies that allow us to precisely repair DNA or RNA, or insert a replacement gene, are rapidly evolving so that we can use the most appropriate technologies for different genetic diseases.

In cellular gene therapy, cells with a therapeutic effect are introduced into the body that presents a medicine that can perform its tasks in the body for decades.



Therapeutic cells for cancer immunotherapy such as CAR-T cells recognise and destroy cancer cells.



Researchers at the National Institute of Chemistry have made outstanding advances in synthetic biology, neurobiology, genetics and immunology, contributing significantly to our understanding of the mechanisms of disease, and the development of gene and cell therapy technologies.

This is also demonstrated by two ERC projects for established researchers and an ERC PoC project for technology validation and commercialisation.

The acquired knowledge, technological expertise and excellent staff represent a comparative advantage on which we will build a successful Centre, where we aim to facilitate the transfer of this knowledge to patients.

At the CTGCT Centre of Excellence, we will develop gene and cell therapy technologies, and work to bring advanced medicines to clinical trials for diseases for which we do not yet have effective treatments. This is to improve survival possibilities and quality of life for patients.

The Centre will connect biomedical researchers with clinicians, patients and society to develop and transfer technologies into clinical research.

It will encourage the development of biotechnology companies with high added value, and link up with the pharmaceutical industry.



More than **1400 m²** of advanced research infrastructure linked to the research environment of the National Institute of Chemistry



The investment aims to ensure access to the most up-to-date treatment methods and advanced personalised medicines from treatment planning to clinical testing, by establishing appropriate facilities and equipment.

The investment will ensure adequate organisation, working space and sophisticated equipment. The Centre will enable cutting-edge research, the preparation of advanced gene and cell therapy products suitable for treatment, and their transfer into clinical research.

This will contribute to a higher quality of therapies and treatment services in Slovenia and internationally. At the same time, the Centre will promote the creation of high-tech start-ups, which will have a positive impact on the development of the Slovenian economy. The Centre's excellence and global competitiveness will also attract international pharmaceutical companies to cooperate with the Centre.

Partners in the project



PROJECT LEAD

National Institute of Chemistry

The National Institute of Chemistry will contribute to the Centre's cutting-edge expertise in the field of the development of therapeutics for cancer and genetic diseases in combination with synthetic biology.



PARTNER OF EXCELLENCE

University College of London

UCL will represent a key international strategic alliance through several of its units (IoN, Biochem Eng, IIT, TRO). They will advise on the organisation of the Centre, support the transfer of therapies from laboratories to patients and the market, train Centre staff and connect with regulators, companies and clinics. Through their experience and contacts, they will support the translation of Slovenian knowledge to the clinic. The Centre will also host joint research projects.



ASSOCIATED EXPERT PARTNER

Cell and Gene Therapy Catapult

The Centre will collaborate with the Cell and Gene Therapy Catapult on important services such as assessing the health economics of new therapies, participating in regulatory processes, conducting pre-clinical safety studies, and industrialising manufacturing processes for the production of gene and cell therapies.

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Research and transfer into the clinic

The Centre will enable the development of advanced therapies, particularly in the field of gene therapy for diseases including rare neurodevelopmental disorders, cancer immunotherapy, treatment of neurodegenerative diseases, and other advanced biomedical medicines.



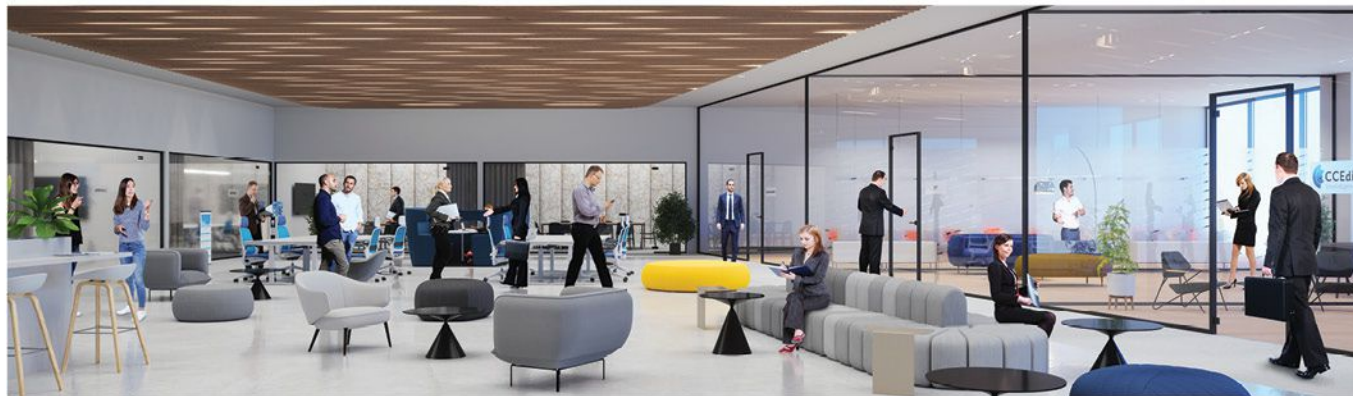
The sustainability and the competitiveness of the Centre will be based on the cutting-edge science of Slovenian researchers in the fields of synthetic biology, immunology and genetics. The training of researchers will take place at the Centre as well as in UCL programmes, in fields that are crucial for the development and transfer of advanced therapies to patients.

The bottleneck in the translation of biomedical research to patients is the preparation of medications or reagents for clinical studies, which must meet high purity and safety requirements according to Good Manufacturing Practice (GMP) criteria. This involves the production of products for the preparation of therapeutic cells in so-called 'clean rooms', under strict quality control. The Centre will include a multi-purpose academic GMP manufacturing laboratory.



Within six years of operation, we will have established a platform to prepare reagents for clinical studies of cancer therapies and gene therapies. Knowledge of the production of gene and cell therapies will open up the possibility of developing therapies in different fields of medicine. **The Centre will act as a national focal point for the dissemination of a range of therapies developed in Slovenia, with a wider regional and global impact.**

The implementation of good business practices and openness to innovation will be key points of the Centre. We will pursue the objectives of involving all stakeholders in the support and adoption of new therapeutic technologies and the professional development of experts. We will work closely with doctors to develop medicines and clinical research. The Centre will act as a messenger for patients and their loved ones and will provide them with access to information on the latest therapeutic approaches.



The Centre will facilitate the establishment of biotechnology companies with high added value. The high quality of the work, from development, pre-clinical and clinical research, will also make the technologies and therapies developed attractive to the pharmaceutical industry. This will bring Slovenia into the latest development and market trends.

As an EU13 Member State with a less developed R&D infrastructure compared to more research-intensive Member States, Slovenia is below the European average in the R&D segment.

In response to this challenge, we are establishing the Centre for the Technologies of Gene and Cell Therapy, which will enable both professional advances and better knowledge transfer to the Slovenian healthcare industry. The biotechnology companies established on the basis of the Centre's knowledge will increase the number of new jobs in a highly promising field.

Gene and cell therapies are highly effective, but the cost of treatment for each patient is high:

- CAR T-cell immunotherapy for cancer (Yescarta), approx. EUR 327.000,
- Treatment of spinal muscular atrophy, single dose (Zolgensma), approx. EUR 2 million,
- Treatment of retinal dystrophy (Luxturna), approx. EUR 345,000 to EUR 680,000.

The high cost of treatment is due to the high cost of manufacturing the medicine, which needs to be tailored to the individual patient, which could be addressed through an in-house production at the CTGCT Centre of Excellence, making therapies more affordable and accessible for the domestic healthcare system.

In more research-intensive countries, the opportunities to strengthen national healthcare systems through the in-house development and production of advanced therapies are already being effectively addressed through the establishment of high-tech biomedical organisations.



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