Dr. Mojca Benčina

NATIONAL INSTITUTE OF CHEMISTRY

CTGCT

CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

The bridge between biomedical research on advanced treatments and its transfer to patients

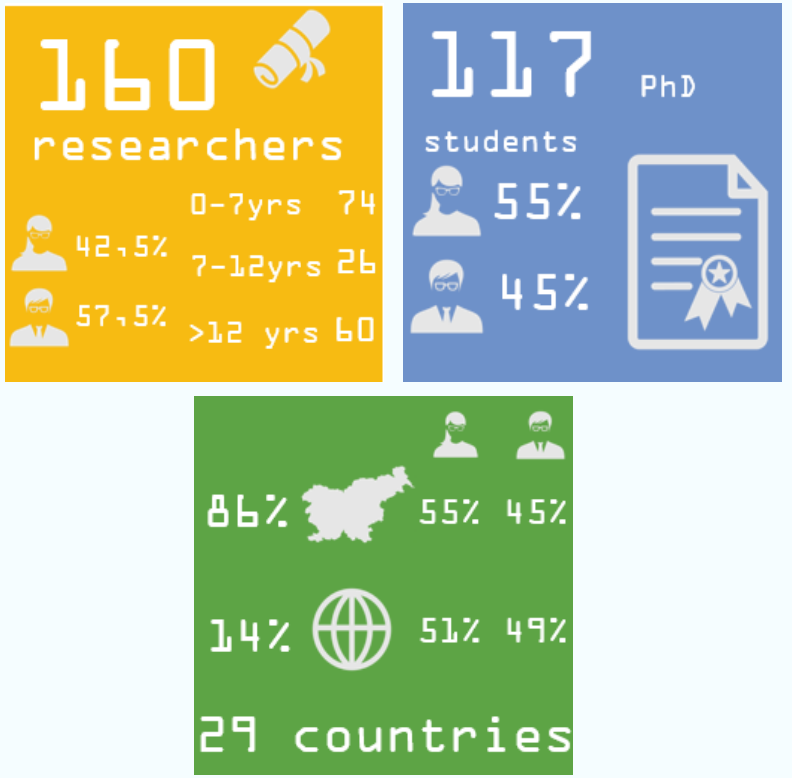
EU Digital Health Tech & Innovation Conference

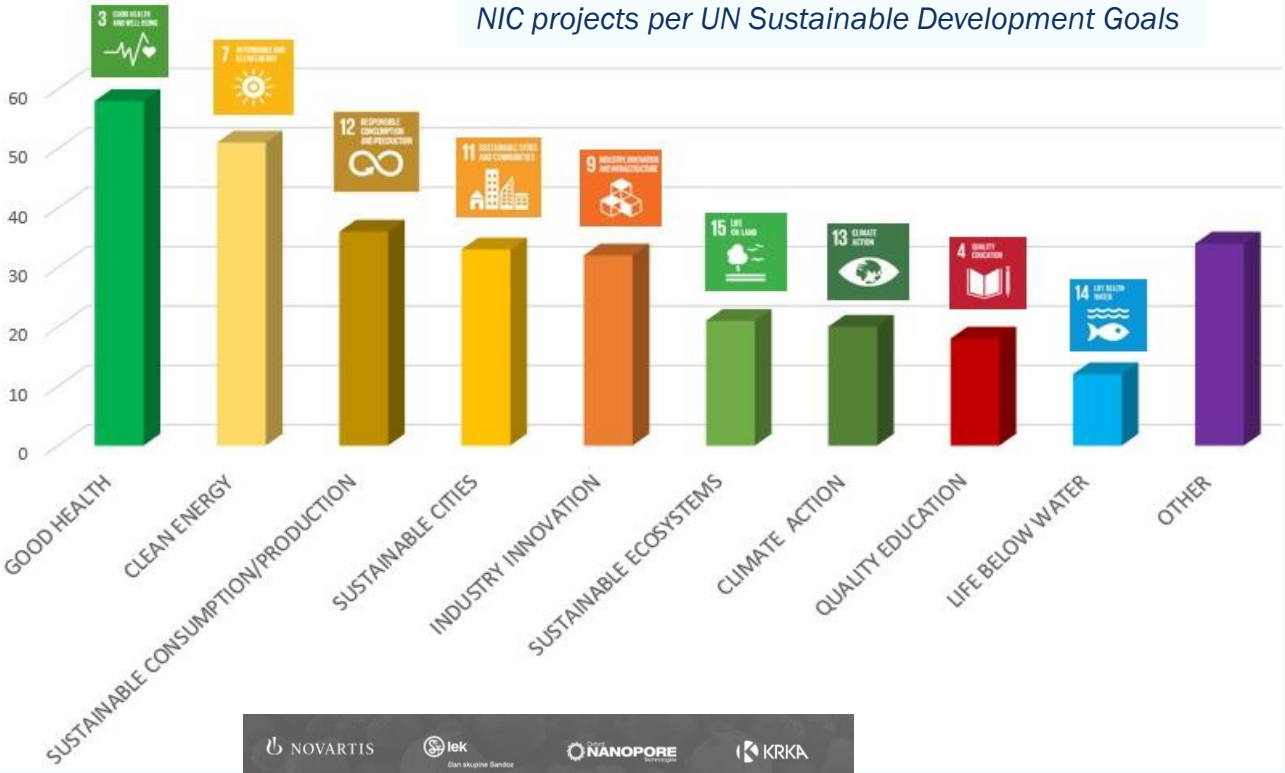
Brussels, 27th September 2022

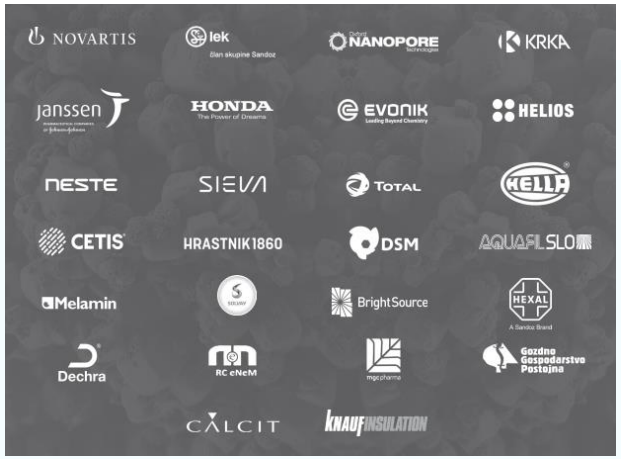
NATIONAL INSTITUTE OF CHEMISTRY

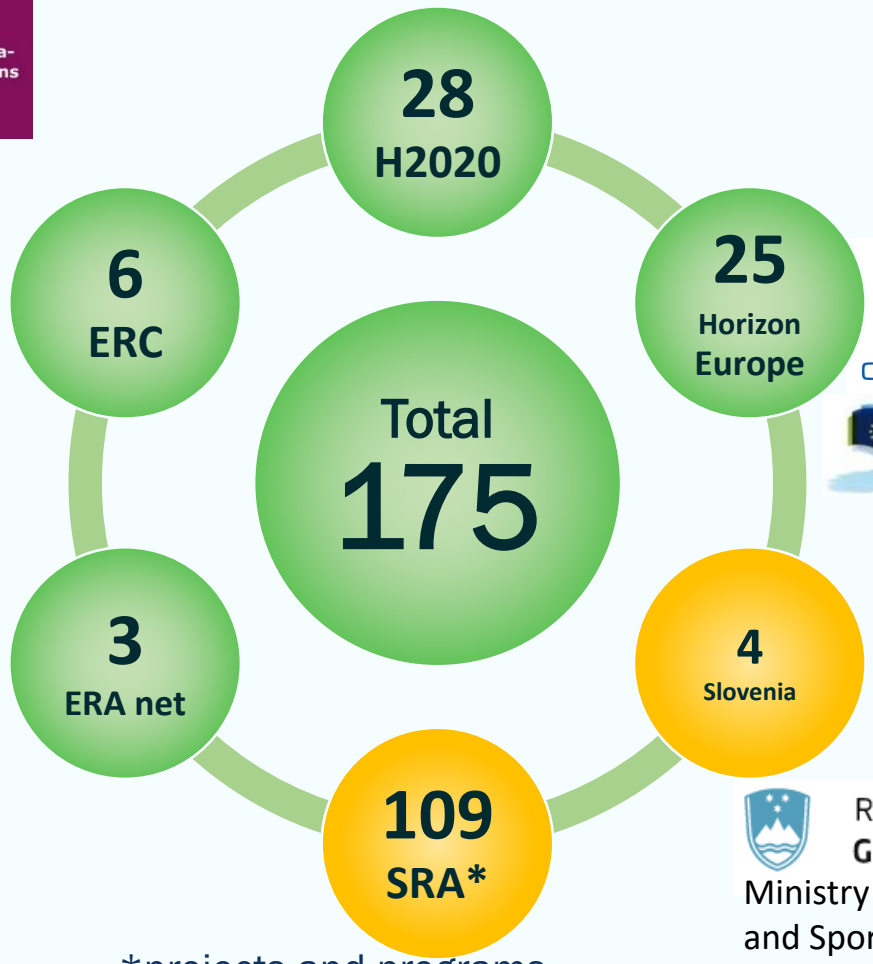
Dr. Mojca Benčina

NATIONAL INSTITUTE OF CHEMISTRY









*projects and programs

Javna agencija za raziskovalno dejavnost Republike Slovenije

Ministry of Education, Science and Sport

barbara.tisler@ki.si, project.office@ki.si

THE HEALTHCARE LANDSCAPE

CTGCT
CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

Difficult to treat cancer

1 in 3 people in their lifetime

Rare genetic disorders

affect an estimated 3-5% of the European population

FOR MOST RARE DISEASES AND CANCERS THERE ARE NO EFFECTIVE CURES!

There are more than 2000 gene and cell therapies in development worldwide.
The global gene therapy market is expected to grow:
over €5 billion in 2022 - nearly €20 billion by 2027.

Establishment of CTGCT

CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

Modern technologies make it possible to treat the direct cause of an increasing number of genetic diseases.

Mission

CTGCT Centre of Excellence will **develop** gene and cell therapy **technologies**, and work to **prepare innovative drugs** for clinical trials for diseases for which we do not yet have effective treatments.

Aim

To provide Slovenian patients and clinicians with access to modern effective treatments and to increase their availability (high cost).

Relevant at EU scale

Improve survival possibilities and quality of life for patients.

TEAMING FOR EXCELLENCE

Centre of Excellence for the Technologies of Gene and Cell Therapy

CTGCT

SLOVENIA AND THE HOST INSTITUTION OF CTGCT

NATIONAL INSTITUTE OF CHEMISTRY

Success would secure **EU funding** for the Centre's operations and **cohesion funding** for the construction of premises and equipment.

CTGCT would become fully operational within 5 years.

OUR PARTNERS of EXCELLENCE

UCL

UMC Utrecht

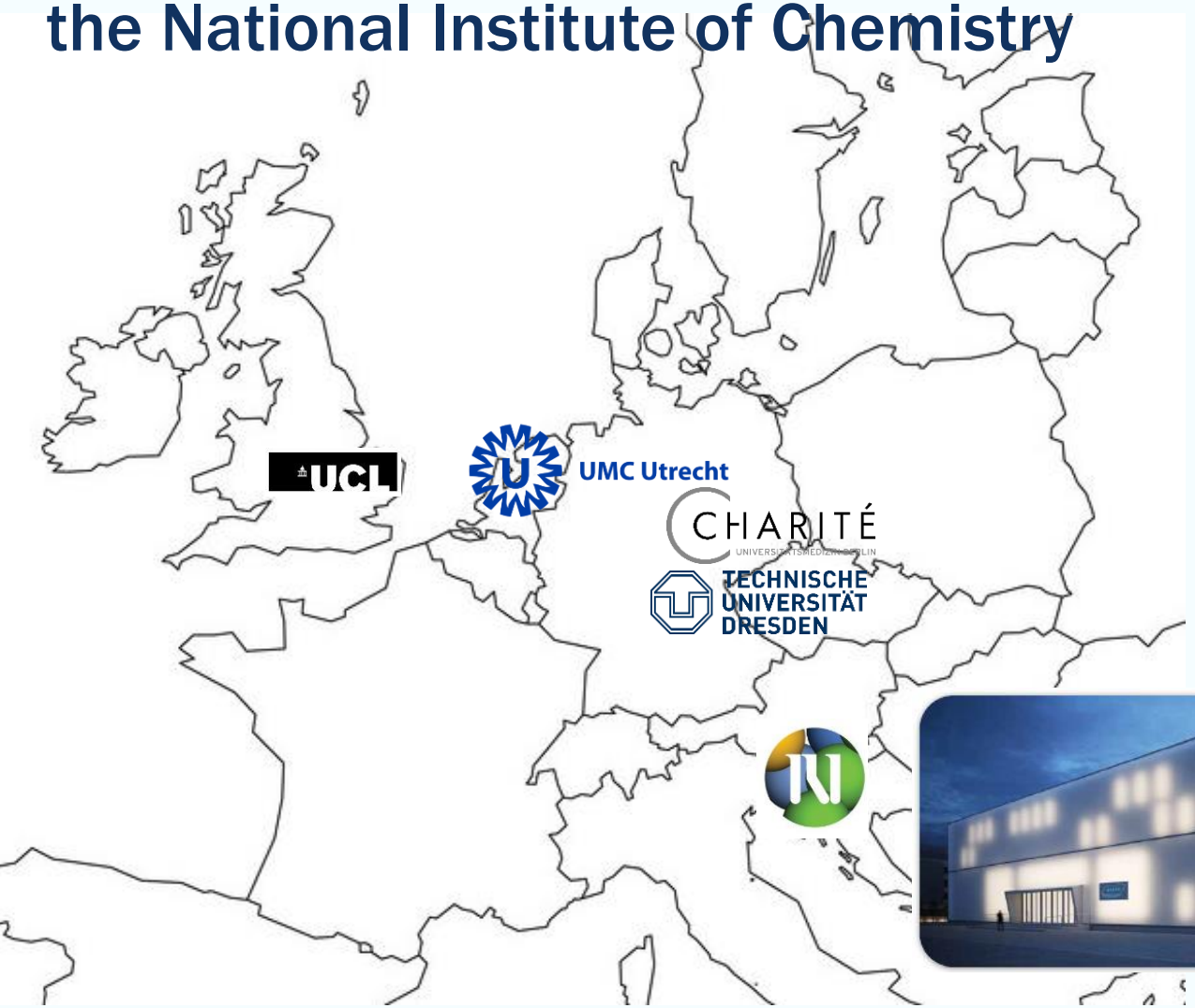
CHARITÉ UNIVERSITÄTSMEDIZIN BERLIN

TECHNISCHE UNIVERSITÄT DRESDEN

Bridging gaps in translation

1400 square meters of a new research infrastructure linked to the research environment of the National Institute of Chemistry

CTGCT
CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY



Support from **national and regional** authorities

- National Medical Council of Slovenia
- Ministry of Education, Science and Sport (MIZS), Slovenia
- Ministry of Health, Slovenia
- Municipality of Ljubljana, Slovenia
- National Organisation for Rare Diseases, Serbia

Research: gene and cell therapy technologies

rare genetic diseases (neurological disorders)
cancer immunotherapy

Key distinguishing technologies
GMP facility
Preclinical trials facility
Genome editing with CRISPR
CAR technology for cancer
RNA targeting

Unique CTGCT outputs
R&I activities and services
Advanced technological solutions

Core research themes
Gene and cell therapy
Rare genetic diseases
Cancer immunotherapy

Translation
Clinical trial support
Emerging biotech industry
Strengthening community

1 2 3 4

CTGCT
CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

Stakeholders engagement

NATIONAL INSTITUTE OF CHEMISTRY · UCL · UMC Utrecht · CHARITÉ · TECHNISCHE UNIVERSITÄT DRESDEN

Public · Clinics · Universities · Institutes · SME · Industry · Government bodies

THE HEALTHCARE LANDSCAPE

Synthetic biology

CTGCT
CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

mRNA dCas

Technology Strand 1: Use of dCasRx to correct splicing defects in amyotrophic lateral sclerosis and frontotemporal dementia, a devastating neurodegenerative disease, serve as **a roadmap for new therapies in the nervous system**.

European Research Council

nature International journal of science

Science AAAS

Technology Strand 2: Nonviral genome editing and targeted genomic integration of therapeutic genes (CRIPSR-mediated insertion) can deliver therapeutic code into the precise position. The increased polynucleotide cargo size of nonviral delivery, not limited by viral constraints, will enable the introduction of innovative synthetic biology devices developed for enhanced safety and control of gene therapies. **The foundational technology for CRISPR-based CCexo and efficient large cargo genome insertion without dsDNA breaks was developed at NIC.**

CRISPR

nature biotechnology

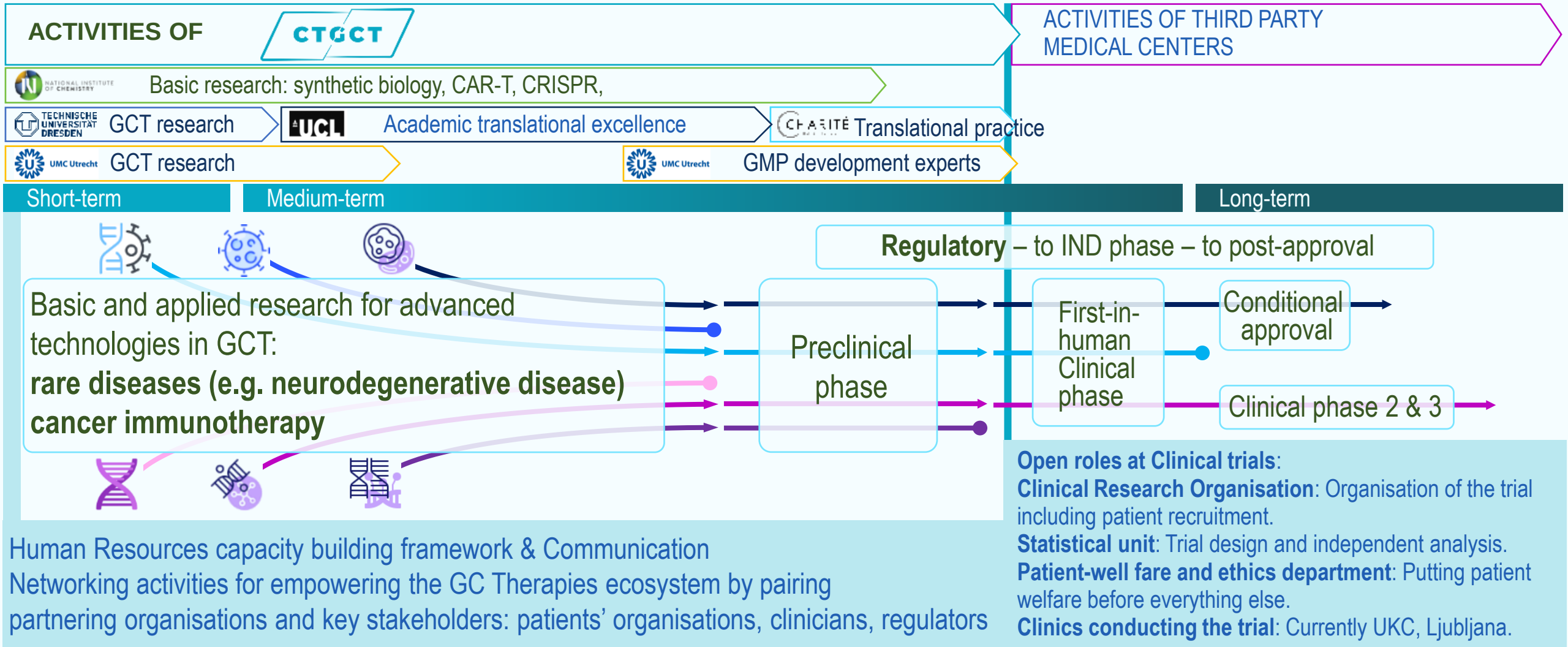
CAR T-cell

Technology Strand 3: Improved safety and efficacy of cancer immunotherapy through improved range of target cell recognition and safety of CAR technology that can be tuned by clinicians (e.g., using approved pharmaceuticals as **the regulators of humanised protein effectors** based on the NIC technology ***INSPIRE***) will broaden the range of therapeutic applications.

nature COMMUNICATIONS

iGEM

THE HEALTHCARE LANDSCAPE



Human Resources capacity building framework & Communication
Networking activities for empowering the GC Therapies ecosystem by pairing partnering organisations and key stakeholders: patients' organisations, clinicians, regulators

Open roles at Clinical trials:
Clinical Research Organisation: Organisation of the trial including patient recruitment.
Statistical unit: Trial design and independent analysis.
Patient-well fare and ethics department: Putting patient welfare before everything else.
Clinics conducting the trial: Currently UKC, Ljubljana.

Good manufacturing practice

academic GMP multipurpose (GCT) facility

4 Units at

CTGCT
CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

1. Research unit

Internal and Collaborative research projects between partners addressing:
- **Innovative technologies**
- **Biological mechanisms**
- **Disease biology**

2. GMP unit

Process development
- **viral vector production, proteins, nucleic acids, LNP**

Production of reagents (GMP quality) for patients
- **clinical trials, hospital exemption**

3. Translational research unit

- Application of advanced technologies to specific therapies
- Coupling therapies to delivery modalities
- Preclinical testing (design, perform, report)
- Communication with clinicians & patient organization
- Documentation: ethical and regulatory authorizations

4. Technology transfer office

Affirms that innovations generated by CTGCT create benefits for society and bring revenue back to CTGCT through IP management and promotion.
- Training
- Consulting service
- Assisting SMEs in public funding
- Partnering for public calls
- Bringing technologies from the laboratory to market.

The platform

a national and Central East Europe regional point for the dissemination of GCT

Collaboration

Researchers
Clinicians
Patient organisations
Regulators (EMA, JAZMP)
Social scientists
Entrepreneurs
Policy makers
Public

In Slovenia

The future CTGCT collaborators at NIC have a fruitful cooperation with **clinicians** and **patient organisations** and are already working intensively on the development of therapies.

CTGCT
CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

Commercialisation

Start-ups
'Big-pharma'
Open-innovation

In 2021, >1300 developers were working on gene therapy and related technologies, raising more than €22 billion.

THE SOCIETY BEHIND THE PROJECT

CTGCT
CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

KNOWLEDGE TRIANGLE AND MORE

Academic institutions

Academic institutions
University of Ljubljana, Slovenia
University of Ljubljana, Faculty of Medicine, Slovenia
University of Belgrade, Faculty of Pharmacy, Serbia
University of Belgrade, Institute of Molecular Genetic Engineering (IMGGE), Serbia
University of Niš, School of Medicine, Serbia
Goce Delcev University, Rep.Macedonia
Ruder Boškovič Institute, Croatia
Semmelweis University, Budapest, Department of Genetics, Cell- and Immunobiology, Hungary
International Centre for Genetic Engineering and Biotechnology (ICGEB), Italy
Central European Institute of Technology CEITEC, Czech Republic
Paracelsus Medical Private University Salzburg, Germany
TicBiomed, Spain
Foundation for researching and training of health professionals in Extremadura (FUNDESALUDA), Spain
University of North Carolina, Department of Pharmacology, USA
Chinese University of Hong Kong, Nexus of Rare Neurodegenerative and Neuromuscular Diseases (NRND), China

THE SOCIETY BEHIND THE PROJECT

CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

KNOWLEDGE TRIANGLE AND MORE

Academic institutions

Industry and entrepreneurial stakeholders

Industrial partners

Krka, d.d., Slovenia
Lek d.d. (a Sandoz company/Novartis division), Slovenia
Educell d.o.o., Slovenia
Niba LABS d.o.o., Slovenia
Jafral d.o.o., Slovenia
Technology Park Ljubljana, d.o.o., Slovenia
Slovenian Innovation Hub, European Economic Interest Grouping, Slovenia
CGT Catapult, UK
Mreža znanja d.o.o., Croatia

Teaming centre

International Institute of Molecular and Cell Biology, Poland

THE SOCIETY BEHIND THE PROJECT

CTGCT
CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

KNOWLEDGE TRIANGLE AND MORE

Academic institutions
Industry and entrepreneurial stakeholders
Patient rights organisations
NGO and others

Medical (clinical) institutions

- Department of Haematology, University Clinical Centre of Ljubljana, Slovenia
- Institute of Oncology, Ljubljana, Slovenia
- Blood Transfusion Centre of Slovenia
- Boston Children's Hospital, Division of Genetics & Genomics, MA, USA
- Telethon Foundation, Italy

Patient organisations

- CTNNB1 Foundation, Slovenia
- the Slovenian Lymphoma and Leukemia Patient Association, Slovenia
- SATB2 Gene Foundation, Slovenia
- The 3rd floor Heroes, Slovenia
- IDefine Europe – Foundation for the Advanced Treatment of Rare Genetic Diseases, Slovenia
- ZORA Foundation, Croatia
- Dravet Sindrom, Croatia
- Rare Disease Croatia
- Coalition of Organisations for Patients with Chronic Diseases (COPAC), Romania
- SLC6A1 Connect, USA
- CureGPX4, San Jose, USA
- National Organisation for Rare Diseases of Serbia (NORBS), Serbia

CTGCT

CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

Benefits and impact

SCIENTIFIC Breakthrough scientific discoveries.

HEALTH Improvements to the health of patients affected by diseases directly addressed by CTGCT as well as others via partners applying similar technological platforms.

ECONOMIC More therapies at lower costs; increased national funding, patient organisations' support for fundraising.

ECONOMIC/TECHNOLOGICAL A new market for advanced technologies for GCTs and connections with pharmaceutical companies’ open-innovation programmes.

SOCIETAL Improved quality of life for patients; increased societal acceptance of novel gene-related technologies; establishment of a new type of institutional organisation that will support scientific and technological excellence.

THE TEAM BEHIND THE PROJECT

NATIONAL INSTITUTE OF CHEMISTRY

PROF DR ROMAN JERALA
National Institute of Chemistry
Innovative use of synthetic biology for the programming of molecules and cells for improved efficacy and safety of advanced treatments

PROF DR JERNEJ ULE
National Institute of Chemistry and UK Dementia Research Institute at King's College London
Research liaison between Slovenia and Great Britain

PROF DR MOJCA BENČINA
National Institute of Chemistry
Ultrasound applications in conjunction with synthetic biology to regulate molecular biological cell processes

DR DUŠKO LAINŠČEK
National Institute of Chemistry
Expert in the field of genome modification

BARBARA TIŠLER
National Institute of Chemistry
Project office

CHARITÉ
UNIVERSITÄTSMEDIZIN BERLIN

PROF PETRA REINKE
Berlin Center for Advanced Therapies (BeCAT) Charité
Enhance CTGCT's capabilities for refined transfer of research results to the first-in-human clinical practice and further accessibility of the ATMP as a treatment option for patients

UCL

PROF GIAMPIETRO SCHIAVO AND PROF PIETRO FRATTA
Queen Square IoN
Application of gene therapies to neurologic diseases

STEPHANIE SCHORGE
GeneTxNeuro facility at the UCL School of Pharmacy
Viral vector production

PROF QASIM RAFIQ
UCL Department of Biochemical Engineering
Production technology development for GCT bioprocessing

PROF EMMA MORRIS
Institute of Immunity & Transplantation, UCL
Development of immunotherapies

DR JANE KINGHORN AND DR PAMELA TRANTER
Translational Research Office, UCL

UMC Utrecht

Utrecht University

PROF JURGEN KUBALL
Department of Hematology, Cancer Center at UMC Utrecht and OncodePACT
Therapeutic T-cells and the valorisation of CAR T-cell development

ASSOC PROF ZSOLT SEBESTYEB
OncodePACT
Building a preclinical development infrastructure to de-risk and accelerate the drug development process and leads DARE-NL platform for cancer specific ATMP research

TECHNISCHE UNIVERSITÄT DRESDEN

PROF EZIO BONIFACIO
Center for Regenerative Therapies Dresden, TU Dresden
SaxoCell association
Expertise, technology and equipment for gene editing and regeneration towards new therapies such as neurodegenerative and haematological diseases

www.ctgct.com
info@ctgct.si

CTGCT

CENTRE FOR THE TECHNOLOGIES OF GENE AND CELL THERAPY

The bridge between biomedical research on advanced treatments and its transfer to patients

EU Digital Health Tech & Innovation Conference

Brussels, 27th September 2022