

2025

National Project Funding of Gene- and Cell-Based Therapies



SPARK-BIH

Charité BIH Innovation SPARK-BIH
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SPARK-BIH

National GCT

Overview

SPARK-BIH: Bridging Biomedical Research and Clinical Application

At the core of biomedical research lies the challenge and necessity of translating scientific discoveries into clinical applications. This process, known as "**Medical Transfer**", is crucial for transforming innovative research into meaningful benefits for patients, society, and the economy. However, only a small fraction of biomedical discoveries is developed into new products, often due to a lack of funding, expertise, or a transfer-oriented mindset among academic researchers.

The Mission of SPARK-BIH

At SPARK-BIH, our mission is to accelerate the translation of academic research into clinically relevant therapies, diagnostics, and medical devices, addressing unmet medical needs.

In order to achieve this, we support researchers and clinicians with milestone-based funding, mentoring and education, fostering a collaborative and supportive environment. Our aim is to turn innovative ideas into impactful solutions that benefit patients and society.



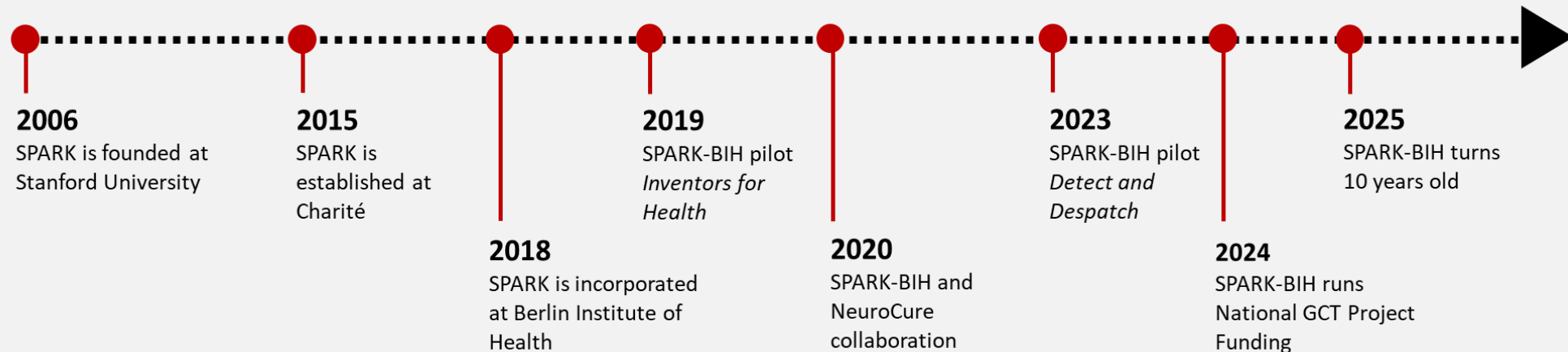
Our Journey

Founded in 2006 at **Stanford University**, SPARK has evolved into a global network comprising over 60 participating institutions worldwide.

SPARK-BIH was established in Berlin in 2015 by Prof. Dr. Craig Garner and Prof. Dr. Ulrich Dirnagl, with the support of **Stiftung Charité**. In 2018, the program became an integral part of the **Berlin Institute of Health (BIH)**, which is focused on medical translation. In 2021, the BIH was integrated into the **Charité - Universitätsmedizin Berlin**, the joint medical faculty of Freie Universität Berlin and Humboldt-Universität zu Berlin and one of Europe's largest university hospitals.

Today, SPARK-BIH is part of **Charité BIH Innovation (CBI)**, the joint technology transfer of BIH and Charité.

Furthermore, SPARK-BIH has established a long-term collaboration with NeuroCure, has developed two programs to promote early innovation and, as part of BIH, runs the project funding within the National Strategy for Gene- and Cell-Based Therapies (GCT), which is the focus of this booklet.



SPARK concept rolls out in Germany in the context of Gene- and Cell-Based Therapy

In March 2023, the Federal Ministry for Research, Technology, and Space (BMFTR, formerly BMBF) commissioned the Berlin Institute of Health (BIH) to coordinate the National Strategy for Gene- and Cell-Based Therapies (GCT).

Designed in collaboration with multiple stakeholders, it aims to develop safe and effective therapies and diagnostics for severe, currently incurable diseases, enhance collaboration across Germany's strong research landscape, and accelerate the translation of research findings into clinical application. At the same time, it seeks to strengthen Germany's international competitiveness in the field of gene- and cell-based therapies (GCT).

One aspect of the National GCT Strategy is Project Funding.

BIH decided to use the SPARK concept — so far established locally for Charité-centric projects — and expand it to a nationwide program in the field of gene- and cell-based therapies as well as associated diagnostics. This decision was driven by SPARK's ability to provide not only financial support but also a wide range of non-monetary services essential for strengthening the gene- and cell-based therapy ecosystem in Germany.

Currently, 36 projects from across Germany are participating in the program, benefiting from financial support as well as mentorship, and educational opportunities from GCT experts.

For more details on this program click [here](#) or visit this [website](#).



GCT

National Strategy
Gene- and Cell-Based Therapies
Project Funding

The national SPARK-BIH GCT Selection Process

The national SPARK-BIH GCT team invites applications for funding from public and private (state-recognized) universities in Germany for individual and coordinated projects with designated project leaders, coordinators and/or partners. Non-university research institutes and companies may apply only as partners within a consortium.

Applications are welcomed for innovative projects in the fields of gene- and cell-based therapies, as well as associated diagnostics. Proposals will be evaluated based on the level of innovation, the significance of the unmet medical need, competitive advantages over existing solutions, data quality, and the likelihood of translational success, including the regulatory plan. Currently there is no open call.

The program funding in two tracks:

- Track 1** supports **early-stage** projects with up to €50,000 for one year.
- Track 2** funds more **advanced** projects with amounts exceeding €50,000, available for up to two years.

All funding is **milestone-based**, with close monitoring and tailored support from the SPARK team to ensure progress and effective resource use, accelerating the translation of biomedical research into clinical applications.



SPARK-BIH National GCT Team



Dr. Tanja Rosenmund
Director of SPARK-BIH



Dr. Sharesta Khoenkhoen
Project Manager



Dr. Marialucia Massaro
Project Manager



Dr. Stefan Köster
Project Manager



Dr. Josephine Kemna
Project Manager



Dr. César Cordero Gómez
Project Manager



Rosa Montserrat Xifré
Team Assistant of SPARK-BIH

SPARK-BIH National GCT Program in Numbers

Applications received from all over Germany

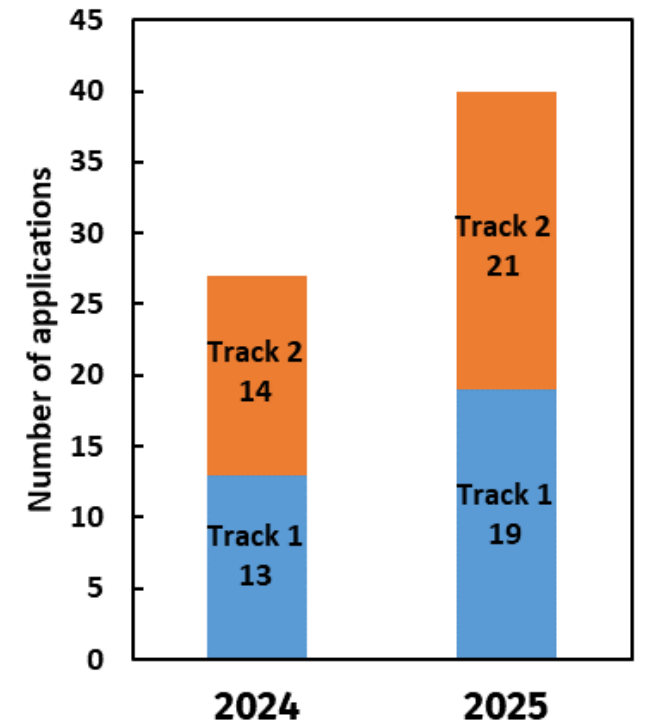
In two calls for applications, an increased number of submissions from across Germany was received, with Track 1 and Track 2 applications almost evenly distributed.



 **67** | Project proposals received

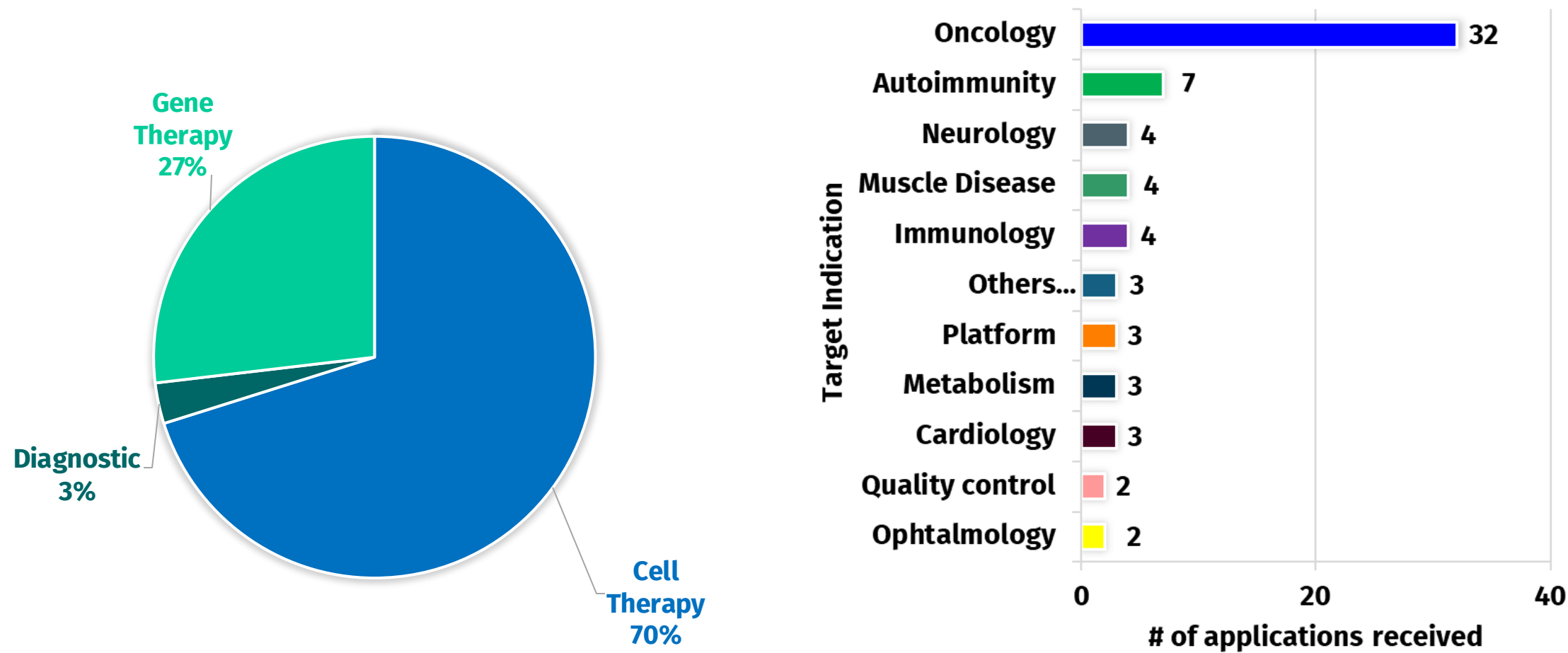
 **17** | Currently funded teams (Call 2024)
23 | Teams joining the program soon (Call 2025)

 **7.7 Mio €** | Funds provided (Call 2024)
9.4 Mio € | Funds will be provided (Call 2025)



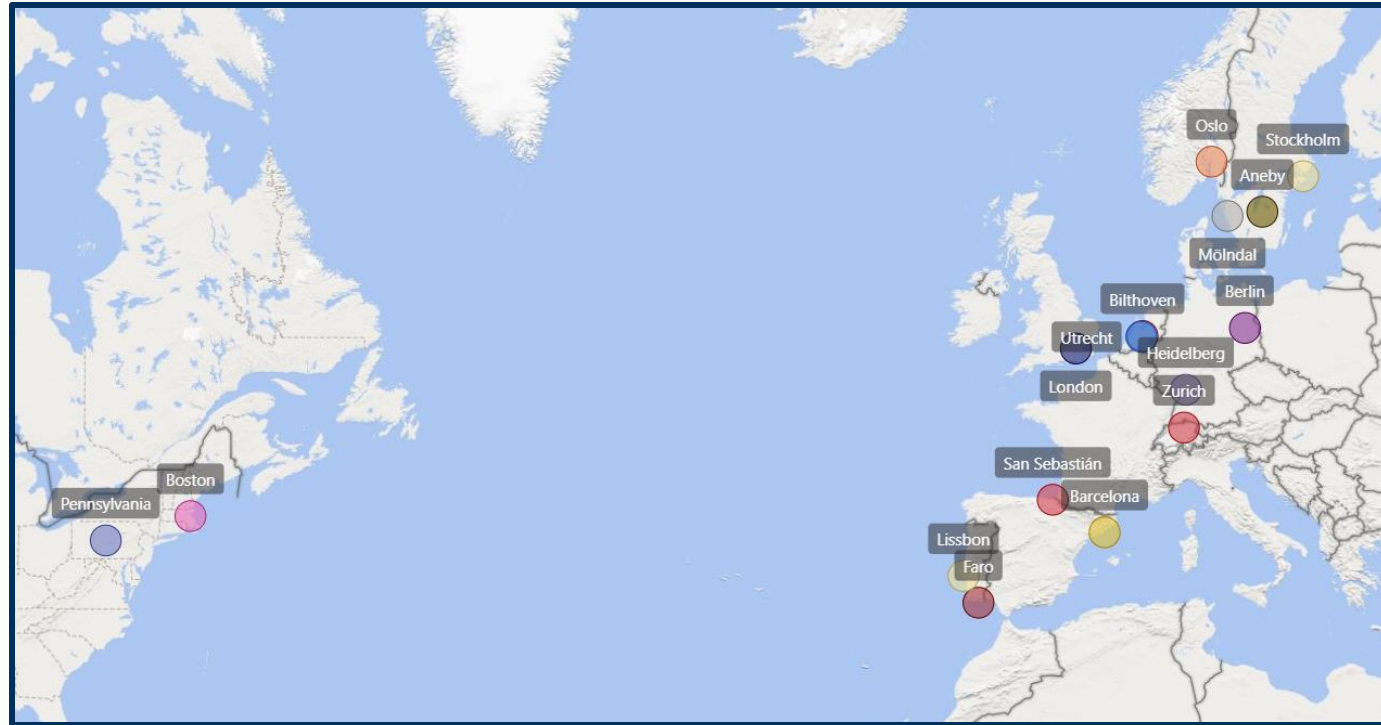
Cell Therapy is the lead modality among the submitted applications from both calls 2024/2025

Most projects are centered on somatic cell therapies, with a focus on oncological indications



SPARK-BIH GCT connects teams with global expertise

29 international experts in the field of gene and cell therapies have joined regular meetings as advisors.



Our global jury and advisor network brings together experienced experts who guide and support funded teams. The advisor network provides interdisciplinary insights, expert feedback, and experience-driven support, helping teams improve, overcome challenges, and accelerate progress for greater impact.

*If you are interested to join the program as an advisor, please contact us!

Creating a new educational series tailored specifically to gene- and cell-based therapy topics

SPARK-BIH
National Gene and Cell Therapies

Webinar Lecture Series 2025
Therapy Development: From Target Identification to Clinical Studies
Thursday 6th February 3:30-4:30 CET

Madhusudan Natarajan, PhD
Madhusudan Natarajan is the Founder and CEO of Crosswalk Therapeutic. Madhu has over 25 years of experience in human pharmaceutical development (Pfizer, Shire/Takeda), startups (Physiome Sciences), and academic research (Asst. Professor, University of Texas Southwestern Medical Center in Dallas).

SPARK-BIH
National Gene and Cell Therapies

Webinar Lecture Series 2025
How to Develop a Target Product Profile (TPP) in Gene and Cell Therapy with Catapult UK
Thursday 27th February 3:30-4:30 CET

Dr Rehma Chandaria
Rehema joined the Cell and Gene Therapy Catapult in November 2023 as Regulatory Affairs Lead. Prior to this, Rehema worked in regulatory consulting, supporting companies with the full scope of US, EU and UK regulatory affairs. During this time, Rehema provided technical and strategic support to small and medium sized companies developing novel medical products including cell and gene therapies. Rehema previously worked with C&B cells as a scientist at Autolus and has a PhD in Tissue Engineering.

SPARK-BIH
National Gene and Cell Therapies

Webinar Lecture Series 2025
Intellectual Property Matters in Gene and Cell Therapies
Thursday 15th May 3:30-4:30 CET

Dr Stefanie Grunwald
Dr. Stefanie Grunwald is an expert in intellectual property with a focus on advanced therapeutic medicinal products (ATMPs). With a background in biotechnology and a PhD and postdoctoral training focused on small and medium sized companies developing novel medical products, she has contributed to translational and experimental research. She combines scientific expertise with a strong foundation in patent law, having qualified through the German Patent Lawyer examination and currently training as a European patent attorney. Since 2003, she works at the Berlin Institute of Health (iG) | Charité for the evaluation of breakthrough therapies and ATMP related inventions. Her work aims to develop robust IP protection and to facilitate the translation of research in commercially viable products. In this session we start with some key players and emerging trends in the field. The following topics will be covered:

- IP Protection in Gene and Cell Therapy:** Basics and scope of industrial IP rights, what is considered confidential, and patients and potential subject matter. You will learn about what can and cannot be patented, understanding key concepts such as state of the art, novelty, and inventive step.
- Global Patent Offices and Regulatory Considerations:** Important jurisdictional differences and legal landscapes.
- Case Studies and Litigation Trends:** cGMP vs. cGMP examples.

SPARK-BIH
National Gene and Cell Therapies

Webinar Lecture Series 2025
Understanding Regulatory Requirements for Gene and Cell Therapies
Thursday 19th June 3:30-4:30 CET

Dr. Barbara Volz
Bringing 25 years in translational research on ATMPs and Biologicals, Barbara has led cGMP compliant manufacturing of cell-based tumor vaccines and recombinant proteins, she also guided non-clinical development of immuno-oncology therapies. In April 2024 she joined the Regulatory Support Unit (RSU) at the Berlin Institute of Health (iG) as a Regulatory Affairs Specialist for Biologicals.

SPARK-BIH
National Gene and Cell Therapies

Webinar Lecture Series 2025
Road to the clinic: how to translate your research into a manufacturing process
Thursday 26th June 3:30-4:30 CET

Dr. Michael Moles
Michael holds a PhD in Cancer Studies and focused on CAR-NK cell therapies in his postdoc. In April 2024, he joined the BIH-RSU as a Regulatory Affairs Specialist, supporting academic ATMP developers and contributing to the EU SafetyATMP consortium and RESC Task Force on regulatory convergence.

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Thursday 26th June 3:30-4:30 CET

Dr. Melissa van Pel
Melissa van Pel obtained her PhD degree at the University of Utrecht in 2005, after an academic career focusing on molecular, cellular, and functional aspects of hematopoiesis and stromal cell populations. Melissa's work shifted towards Cell Therapy product development, process development, product characterisation and clinical manufacturing of Cell Therapies according to cGMP and other international standards. In 2020, she joined NeocGen as Head of Cell Therapy where she contributed to the design, engineering and set up of NeocGen. At NeocGen, Melissa is responsible for process development and clinical manufacture of novel Cell and Gene products. This session will provide an in-depth analysis of process development. Process development aim is to translate a research-grade process into a cGMP compliant manufacturing method, which is robust and reproducible despite the diverse raw materials. During process development, key decisions should be made on the production process, testing strategy, target product profile and activities can be undertaken to challenge the cost of goods.

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6
276

Lectures so far

Participants



4.5/5 rating*

The educational series is open to anyone interested in developing gene and cell therapy products. Three more lectures are scheduled until the end of the year, covering the following topics: Business and Innovation, Competitive Landscape Analysis Tools, and Clinical Trial Design.

* based on surveys

SPARK-BIH National GCT Projects

A novel self-inactivating alpharetroviral vector-based gene therapy strategy for IL7RA deficient severe combined immunodeficiency



PRINCIPAL INVESTIGATOR and PROJECT PARTNERS:

**Dr. Teng Cheong Ha, Prof. Axel Schambach,
Prof. Michael Morgan, Dr. Melanie Galla & Dr. Michael Rothe**
Institute of Experimental Hematology
Hannover Medical School



SUMMARY

Children suffering from IL7RA-deficient severe combined immunodeficiency (SCID) have almost no functional T-cells to fight infections and, without treatment, often do not survive beyond their first year of life.

The current standard treatment is a bone marrow transplant (BMT), which depends on identifying suitable donors and carries significant risks and long-term health issues. The goal of ARISE is to develop a safe and effective gene therapy for IL7RA-deficient SCID patients.

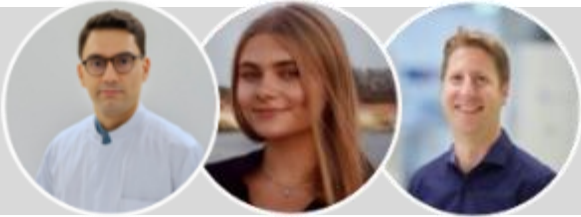
PROJECT GOALS

- Develop a safe and effective gene therapy to repair the defective IL7RA gene in SCID patients

LONG TERM GOALS:

- Fulfil regulatory requirements
- Perform phase I/II clinical trials with further funding
- License to Biotech/Pharma or clinical co-development
- Deliver a safer alternative therapy for patients

Gene therapy targeting neuroinflammation in Alzheimer's Disease



PRINCIPAL INVESTIGATOR and PROJECT PARTNERS:

**Dr. rer. hum. biol. Dr. med. Sergio Castro-Gomez, Ida Kulinska,
Prof. Dr. Florian I. Schmidt**

Center for Neurology / Institute of Physiology II
University Hospital Bonn



SUMMARY

Alzheimer's Disease (AD) is a devastating and incurable disorder. Currently, research is shifting from addressing classical neuropathological hallmarks to neuroinflammation. The common adaptor inflammasome protein ASC (Apoptosis-associated speck-like protein containing a CARD) has been implicated as a target in AD. Our project aims to develop AAV-based gene therapy targeting ASC to modulate neuroinflammation and protein aggregation in AD, with the goal of creating a breakthrough treatment.

PROJECT GOALS

- Optimize AAV vectors for efficient and brain tissue-specific transduction.
- Develop an AAV-based gene therapy targeting the ASC inflammasome protein to specifically to modulate neuroinflammation in early AD.
- Validate ASC *in vivo* as a viable therapeutic target in AD therapy

LONG-TERM GOALS

- Develop a systemic gene therapy for Alzheimer's disease

Safety of genome editing as therapy for autosomal dominant osteopetrosis



PRINCIPAL INVESTIGATOR:
Prof. Dr. rer. nat. Uwe Kornak
Institute of Human Genetics
Georg-August-University Göttingen



SUMMARY

Autosomal dominant osteopetrosis (ADO), a hereditary skeletal disorders caused by mutated CLCN7 gene impairing osteoclast function, leads to fractures and bone pain, with no effective or safe causative therapy currently available. We aim to develop a genome editing therapy for ADO and have already demonstrated proof-of-concept for restoring osteoclast function *in vitro*. In this project, the safety of this therapeutic approach will be investigated, by carefully assessing off-target effects.

PROJECT GOALS

- Optimisation of DISCOVER-seq for use in cells of the hematopoietic lineage
- Apply optimized DISCOVER-seq to identify localization and frequencies of off-target editing sites in the genome
- Independent validation of identified off-target candidates

LONG-TERM GOALS

- Clinical application of ADO gene therapy

Reference Material for Mesenchymal Stromal Cell Critical Quality Attribute Assays



PRINCIPAL INVESTIGATOR:

Prof. (apl.) Dr. rer. nat. Karen Bieback

Cell and Immune Therapy, Institute of Transfusion Medicine and Immunology, FlowCore
Mannheim, Medical Faculty Mannheim, Heidelberg University



SUMMARY

Strict quality standards apply to medicinal products like mesenchymal stromal cells (MSCs) to ensure safety. However, reference materials for testing the identity, purity and efficacy of MSCs are not available on large scale yet. This project aims to develop, produce, and test large-scale reference materials to verify the identity, purity, and efficacy of MSCs for use in cell therapies. These standards will support consistent quality and help overcome regulatory barriers to MSC-based therapies.

PROJECT GOALS

- Generate reference material for use in critical quality assays of MSCs

LONG-TERM GOALS

- Support development and commercialization of MSC therapies

SIMPLE-seq – Advancing Safety in Genome Editing



PRINCIPAL INVESTIGATOR and PROJECT PARTNER:
Dr. Carla Fuster García, Prof. Dr. Toni Cathomen
Institute for Transfusion Medicine & Gene Therapy
Medical Center – University of Freiburg



SUMMARY

The SIMPLE-seq project aims to develop an innovative, user-friendly in vitro method for evaluating the off-target activity of base editors (BEs). While CRISPR-Cas9 induces double-strand breaks, BEs create single-strand breaks. SIMPLE-seq converts these into detectable signals, enabling accurate, high-throughput mapping of unintended DNA alterations. The aim is to comprehensively assess the safety and specificity of BEs, making their clinical applications safer and more predictable.

PROJECT GOALS

- Validate SIMPLE-seq in benchmark studies using established BEs

LONG-TERM GOALS

- Position SIMPLE-seq as a reliable, cost-effective, and scalable method with broad applications in both research and industry
- Establish SIMPLE-seq as the new gold standard for safety assessment of BEs

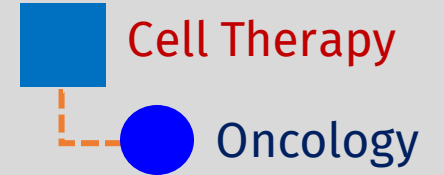
Gene-edited CD38/CD45-CAR-NK cells for leukemia treatment and non-toxic conditioning



PRINCIPAL INVESTIGATOR:

Prof. Dr. Boris Fehse

Research Dept. Cell & Gene Therapy, Dept. of Stem Cell Transplantation
University Medical Center Hamburg-Eppendorf



Cell Therapy

Oncology

SUMMARY

CAR-based immunotherapies have revolutionized the treatment of some blood cancers but have not been successful in treating acute myeloid leukemia (AML) so far due to the lack of suitable target antigens and the rapid progression of the disease. To address this, we are developing off-the-shelf CAR-NK cells that target both CD45 and CD38, antigens commonly found on AML cells. We previously demonstrated that CD38 and CD45 double-knock-out prevents fratricide and results in highly effective elimination of cancer cells.

PROJECT GOALS

- Develop a method for the efficient production of CAR-NK cells with double-knock-out of CD38 and CD45 and dual CARS targeting CD38 and CD45

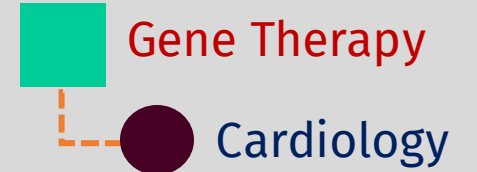
LONG-TERM GOALS

- Further develop the technology for future clinical use

Development of a nanobased mRNA therapy for heart diseases



PRINCIPAL INVESTIGATOR and PROJECT PARTNER:
Univ.-Prof. Dr. med. Georg Daniel Dürr, Dr. Tim Stüdemann
Department of Cardiovascular Surgery
University Medical Center Mainz (Johannes Gutenberg-University Mainz)



SUMMARY

Engineered mRNA, a novel and highly adaptable class of therapeutics, offer a new solution for treating cardiac diseases. In this project, we aim to evaluate nanoparticles that may target human cardiomyocytes derived from explanted myocardium ex vivo. These nanoparticles will be used to deliver mRNAs encoding factors demonstrated to elicit cardiomyocyte proliferation and cardiac regeneration in transgenic mice.

PROJECT GOALS

- Evaluate nanoparticles for targeted mRNA delivery to cardiomyocytes

LONG-TERM GOALS

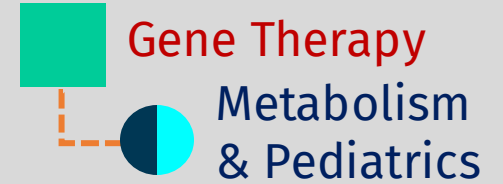
- Facilitate treatment of cardiac diseases and improve patient outcomes

RNA-Based Delivery of Prime Editors targeting MYO5B Deficiency



PRINCIPAL INVESTIGATOR:
Prof. Dr. med. Tobias Cantz

Dept. of Gastroenterology, Hepatology, Infectious Diseases and Endocrinology: RG
Translational Hepatology and Stem Cell Biology, Hannover Medical School



SUMMARY

Proper hepatocyte polarization is essential for functional hepatobiliary transport, relying on motor protein such as Myosin-5b (MYO5B). Myosin-5b and Rab11a are crucial for the bile canaliculus formation, and defects in either can alter the polarization, leading to major pathological consequences.

Thus, RAPIDMYO aims to define a robust Prime Editing approach targeting MYO5B-mediated progressive familial intrahepatic cholestasis (PFIC) as unmet clinical need.

PROJECT GOALS

- Develop defined mRNA/ pegRNA combination for Prime Editing

LONG-TERM GOALS

- Efficient and safe restoration of liver function

Bridging deep gaps in translation and tissue: a blueprint for TEP development from a corneal sealer



PRINCIPAL INVESTIGATOR and PROJECT PARTNER:
Dr. Anna Resch, Prof. Dr. Günther Schlunck
University of Freiburg & University of Freiburg Medical Center



SUMMARY

Around 1.6 million people worldwide are estimated to be blind due to eye injuries and around 19 million have impaired unilateral vision. Severe corneal injury, one of the main causes, is typically treated with sutures, which can cause scarring, inflammation and corneal opacity. We aim to develop a safe, elastic corneal adhesive containing living keratocytes in a protein-based hydrogel. The adhesive can seal wet wounds; it is elastic and biocompatible. It should be degraded by keratocytes and replaced by natural extracellular material.

PROJECT GOALS

- Build an early basis for a quality-by-design manufacturing strategy
- Study preliminary toxicity in rabbits

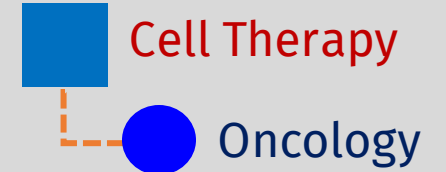
LONG-TERM GOALS

- Providing a regulatory strategy as blueprint for similar products

Enhancing Cellular Therapy for B-Cell Neoplasms



PRINCIPAL INVESTIGATOR:
PD Dr. Antonia Busse
Charité University Medicine Berlin



SUMMARY

Immunotherapy with CAR-T cells has shown success in the treatment of malignant B-cell tumors, but cure rates are only 40-60%. Major limitations are loss of target expression in tumor cells and the limited lifespan of CAR-T cells. TCR-T cells offer an alternative therapy option, as they recognize peptides from proteins if they are presented by MHC molecules. We have developed a TCR, T3225, which specifically targets the CD22 antigen on B cells and has shown better efficacy than CD22 CAR-T cells.

PROJECT GOALS

- Develop a bispecific T cell product based on T3225

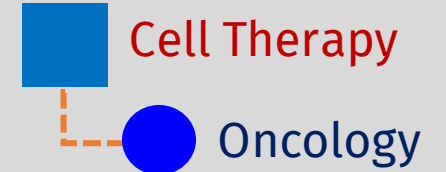
LONG-TERM GOALS

- Commencing a clinical phase I trial

Preparation of a clinical trial of CAR-NK cells in patients with Her2- expressing tumors



PRINCIPAL INVESTIGATOR:
Prof. Dr. Torsten Tonn
Goethe University Frankfurt/M



SUMMARY

Her2 is a tumor antigen expressed by approx. one-third of breast, ovarian, colon cancer, osteosarcomas and other tumor indications, which often respond particularly poorly to standard therapies. We plan to treat patients with Her2-expressing tumors using NK cells genetically engineered to carry a CAR that allows the specific targeting and killing of Her2-expressing cells. This CAR-Her2-specific cell line, NK-92/5.28.z, is currently used in clinical trials in patients with recurrent glioblastoma.

PROJECT GOALS

- Validating GMP manufacturing of a CAR-Her2-specific NK cell line
- Preparing a study protocol and investigational medicinal product dossier (IMPD)

LONG-TERM GOALS

- Commencing a clinical phase I trial

All-in-One Genome Editing Therapy for the Treatment of Duchenne Muscular Dystrophy



PRINCIPAL INVESTIGATOR and PROJECT PARTNERS:

Prof. Dr. Wolfram-Hubertus Zimmermann, Prof. Dr. Hildegard Büning,

Prof. Dr. Rabea Hinkel, Prof. Dr. Bernd Wollnik

University Medical Center Göttingen, Hannover Medical School & German Primate Center



Gene Therapy

Muscle Disease
& Pediatrics

SUMMARY

Duchenne Muscular Dystrophy (DMD) is a genetic disease with current treatments providing palliative care only, which do not prevent loss of ambulation or premature death. Genome editing is emerging as a promising option to convert DMD into a milder or even asymptomatic condition. In this collaborative project, we use optimized AAVs for All-in-One delivery of small CRISPR/Cas9 to skeletal and heart muscle. Preliminary results show that this approach can improve contractile function.

PROJECT GOALS

- Complete nonclinical testing of a novel DMD genome editing therapy candidate

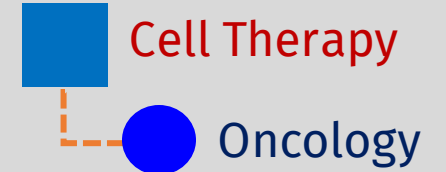
LONG-TERM GOALS

- Converting patient-tailored approaches to an off-the-shelf exon-tailored therapy adaptable to most DMD-causing deletions/ mutations

Innovation in AML Treatment



PRINCIPAL INVESTIGATOR and PROJECT PARTNER:
Prof. Dr. Toni Cathomen, Prof. Dr. Evelyn Ullrich
University of Freiburg & Goethe University Frankfurt



SUMMARY

The neClectAML consortium is developing a novel immunotherapy for acute myeloid leukemia (AML), an aggressive and challenging form of blood cancer. At the core of the project are genetically engineered natural killer (NK) equipped with a chimeric antigen receptor (CAR) derived from a llama nanobody targeting CLEC12A, a molecule found on AML cells. To further enhance NK cell activity, an NK cell-specific immune checkpoint will be selectively deactivated.

PROJECT GOALS

- Establish clinical-scale production of these gene-edited CAR NK cells and conduct preclinical testing to ensure their safety and efficacy
- Define preclinical work packages based on scientific advice from PEI

LONG-TERM GOALS

- Prepare for a Phase I/II clinical trial

Clinical-grade manufacturing of NY-ESO-1 TCR-modified stem-like T cells overexpressing the pre-miR-155 SNP, rs377265631



PRINCIPAL INVESTIGATOR and PROJECT PARTNERS:

**Dr. Dr. Roland Schelker, Prof. Dr. Wolfgang Herr, Prof. Dr. Luca Gattinoni,
Prof. Dr. Simone Thomas, Prof. Dr. Matthias Edinger**

University Hospital Regensburg & Leibniz Institute for Immunotherapy



SUMMARY

This project focuses on generating stem-like T cells (T_{SCM}) from naive $CD8^+$ T cells of patients and equipping them with a NY-ESO-1 TCR and an immunostimulatory microRNA, the miR-155 SNP rs377265631. These T cells are expected to trigger an enhanced and sustained anti-tumor response in patients with metastatic sarcoma. The project marks an important step towards clinical application and could significantly improve treatment outcomes for patients in the long term.

PROJECT GOALS

- Develop large-scale manufacturing process
- Develop comprehensive quality control tests to ensure that the product meets the required clinical standard

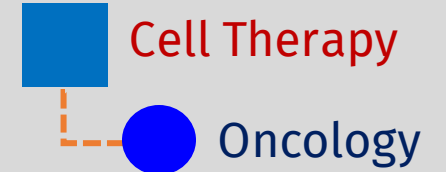
LONG-TERM GOALS

- Prepare for Phase I trial

In Vivo Generation of Chimeric Antigen Receptor T cells with T cell-retargeted Adeno-Associated Virus Vectors



PRINCIPAL INVESTIGATOR and PROJECT PARTNER:
Prof. Dr. rer. nat. Hildegard Büning, Prof. Dr. Michael Hudecek
Hannover Medical School & University Clinic Würzburg



SUMMARY

CARtrAAVic aims to revolutionize CAR-T therapy by generating CAR-T cells directly in the patient's body through an cells *in vivo* approach. This eliminates the need for complex *ex vivo* manufacturing and enables a more physiological anti-tumor response with low toxicity. The approach also addresses key clinical challenges, such as cytokine release syndrome, limited response duration, safety concerns related to off-target integrations, while significantly reducing production costs and manufacturing time.

PROJECT GOALS

- Provide preclinical proof-of-concept
- Prepare for clinical development

LONG-TERM GOALS

- CARtrAAVic aims to bring a scalable, chemotherapy-free, next-generation CAR-T therapy platform with broad therapeutic potential, rapid and broad availability and a sustainable cost structure into clinical application

PHOENIX: Promoting Healing and Overcoming ELANE Neutropenia with ex vivo CRISPR



PRINCIPAL INVESTIGATOR and PROJECT PARTNERS:

Prof. Dr. Julia Skokowa, Dr. Masoud Nasri, Dr. Cornelia Zeidler, Prof. Dr. Wolfgang Bethge, Prof. Dr. Claudia Lengerke, Prof. Dr. Karl Welte, Prof. Dr. Peter Lang, PD Dr. Dr. Markus Mezger, Prof. Dr. Stefanie Joos, Prof. Dr. Toni Cathomen, Prof. Dr. Doris Steinemann
University Hospital Tübingen, University Hospital Freiburg & Hannover Medical School



SUMMARY

In the PHOENIX project, we aim to advance the clinical development of the MILESTONE gene therapy approach for congenital neutropenia. This innovative procedure inactivates the disease-causing gene through the editing of its promoter. Here, we will use it to target the non-essential *ELANE* gene, which is mutated in half of patients with congenital neutropenia. In addition to the preclinical work, we are actively engaging patients by structured patient interviews and engaging with patient organizations.

PROJECT GOALS

- Adapt and apply GMP-compliant editing process to large-scale editing of hematopoietic stem cells
- Develop IMPD

LONG-TERM GOALS

- Conduct clinical study (IIT)

Innovative lipid nanoparticles for cell and gene therapy



PRINCIPAL INVESTIGATOR and PROJECT PARTNER:
Prof. Dr. med. Petra Reinke, Dr. Ing. Christoph Hein
BeCAT Charité & Fraunhofer IPK



SUMMARY

There is a high clinical need for improved GCT products, as current transfection methods, mainly based on viral vectors, are inefficient and costly. In the “NanoGen” project, non-viral multiplex gene editing of T cells is being developed to treat B-cell-mediated autoimmunity. Fraunhofer IPK’s proprietary Fdmix technology enhances lipid nanoparticle (LNP) delivery, improving particle stability and transfection efficiency, and creating a scalable platform for efficient gene editing in human T cells.

PROJECT GOALS

- Develop a novel process for nucleic acid-based drugs delivery into human T cells
- Provide proof-of-concept for multiplex T-cell therapy for B-cell-mediated autoimmunity

LONG-TERM GOALS

- Advance the approach to clinical trials

The journey continues:

**11 new Track 1 and 12 new
Track 2 projects from call
2025 will be added soon**

Testimonials



"I wish I had something similar to **SPARK-BIH** when I started developing ATMPs. I thoroughly enjoy helping scientists in their quest to develop new products and, in the long run, contribute to this Program in giving answers to patients in need."

**Ander Izeta, Former President of
the Spanish Society for Gene
and Cell Therapy (SETGyC)**



*As a scientific/CMC advisor to the **SPARK-BIH** program, I have witnessed firsthand the transformative impact of the program to bring innovative ideas discussion in open and interdisciplinary collaborative environment. I have seen phenomenal engagement from the world class experts and innovators for fostering new discoveries in Cell and Gene Therapy domain. The program effectively bridges the gap between academic research and clinical application, empowering researchers to navigate the complexities of translational medicine for patient focused solutions. By fostering innovation and providing strategic guidance, **SPARK-BIH** plays a pivotal role in advancing early-stage biomedical discoveries toward tangible patient solutions.*

Dharmesh Patel, Ph.D., MBA
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