Berlin Institute of Health at Charité
Funding programme

National funding of translational projects in the field of gene and cell therapy and associated diagnostics – second call

Translation of selected parts of the full German version of the funding programme that was published December 17, 2024. Please note that the German version is the legally binding one: Nationale Förderung von Translationsprojekten zur Therapie mit gen- und zellbasierten Produkten und assoziierter Diagnostik – zweite Förderbekanntmachung | DLR Projektträger

1 Aim of the call

Gene- and cell-based therapies (GCT) are an important and urgent topic for the future of biomedical research and personalised clinical care. There are numerous possible applications. These include blocking gene functions with oligonucleotides and using viral or synthetic vectors to deliver therapeutic nucleic acids in vivo or ex vivo, the use of cell products or cell replacement therapies, virotherapy (with or without transfer of therapeutic genes) and tissue engineering.

In order to improve access to GCT for patients and strengthen Germany as an international hub for research and innovation in this field, the Berlin Institute of Health at Charité (BIH) has coordinated the development of a national strategy for gene- and cell-based therapies on behalf of BMBF (Federal Ministry of Education and Research) using a multi-stakeholder approach. In addition to a comprehensive review of the current status of the GCT field in Germany, the strategy comprises various measures to accelerate the translation of results from Germany's excellent basic research into clinical practice while making GCT safe, efficient, affordable and widely accessible.

The aim of this funding programme is to support the translation of research results in the field of GCT, thereby contributing to the implementation of the National Strategy. The funding is intended to improve and accelerate the translation of innovative gene and/or cell-based therapies and associated diagnostics into marketable and clinically applicable products or processes. In addition to financial support, this funding line also provides comprehensive project-specific advice based on the SPARK concept. Here, projects receive guidance by SPARK project managers, as well as targeted advice and support from external experts. Another component is the educational programme consisting of workshops and webinars on translational topics in the field of GCT, held by gene and cell therapy experts. In order to strengthen national networking, joint meetings are planned among funded project teams.

The aim of this funding programme is met if the projects make significant progress in translating GCT with regard to the development of new products and processes or their clinical application. Concretely, this means that the results of the funded projects contribute to filing a patent application, successful out-licensing of an existing patent, founding of a company or securing additional third-party funding for further development of the therapeutic or diagnostic approach in academia. In addition, the funding programme aims to foster new interdisciplinary collaborations between researchers from academia with industry research partners, both in regional and cross-regional consortia.

The purpose of the programme is to fund necessary and defined steps of already initiated, translational product or process developments in the field of GCT. The results should help to improve and accelerate the translation of basic research findings into marketable and clinically applicable products and processes.

This funding guideline serves to implement the National Strategy for Gene and Cell Therapy, Field of Action VI: Research and Development

(https://www.bihealth.org/fileadmin/GZT/NationaleStrategie_GCT_DE.pdf)

and is based on the SPARK-BIH programme.

The results of the funded projects may only be used in the Federal Republic of Germany or the EEA and Switzerland during the funding period.

[1.2 not translated…]

2 Funding objectives

Funding can be provided for projects of single applicants (tracks 1 and 2) or collaborative projects/consortia (track 2, only). The projects must address necessary and defined work packages for further translation of research results. Projects must build on and supplement recently completed or ongoing translational product and/or process development in the field of GCT. Projects should improve the quality or accelerate the development of the product and/or process.

Projects in Track 1 and Track 2 must

- have translational character. Basic research is not funded;
- serve an "unmet medical need";
- be innovative and novel, and be based on existing solid data that demonstrates the general feasibility of the approach (proof-of-principle). Depending on the stage of development of the product or process, this can be provided in different ways (*in vitro, in vivo* data, etc.);
- aim for a solution/product that has a clear competitive advantage over the existing gold standard.

Translational research in the following areas can be funded:

- Therapeutic approaches with advanced therapy medicinal products (ATMPs):
    - o Somatic cell therapeutics (for example stem cell applications, immunotherapies or use of mesenchymal stromal cells,);
    - o Gene therapeutics in the form of gene substitution, addition or suppression therapies using viral and non-viral vectors or genome editing;
    - o Tissue engineering products such as the production of replacement tissue for surgical use, including the use of novel biomaterials.
- Therapeutic approaches with novel biological products, such as mRNA and other nucleic acid-based methods, extracellular vesicles or exosomes, which are used in the context of a gene- and cell-based therapy;
- Diagnostic approaches with a clear link to GCT, especially in the field of companion diagnostics but not limited to it.

However, the following areas are excluded:

- Approaches developed for applications other than gene- and cell-based therapies (for example, mRNA vaccination against infectious diseases);
- Approaches based exclusively on small molecules and/or recombinant proteins (including therapeutic antibodies).

Depending on the respective development stage of the project and the scope of the planned work, funding can be applied for in two tracks.

Track 1 funds projects at an early stage that can present preliminary data demonstrating the general feasibility and potential of the therapeutic approach for the treatment of the targeted disease, but do not yet have to be patented (see also No. 4 Funding requirements). In this track, funding of up to 50,000 euros including overhead is possible for an individual project of a single applicant for a maximum duration of one year. Track 1 is aimed exclusively at research groups from public and private (state-recognized) universities.

Track 2 is intended for more advanced projects that fulfil certain requirements with regard to patenting or market access strategy (see no. 4 Funding requirements). Here, funding of more than 50,000 euros is possible for individual or collaborative projects for a maximum duration of two years. Non-university research institutions and industrial partners can also be involved in collaborative projects/consortia but the consortia have to be coordinated by a university (see 3).

The work packages applied for can include the following aspects (the list provides examples of alternative options; several options can be combined for a target product or process development):

- Design and conduct of relevant experiments as well as development of methods and protocols necessary for translational purposes, e.g. pharmacological-toxicological studies;
- Project-specific, external advice, e.g. intensive preparation for meetings with regulatory authorities, market analyses;
- Preclinical assessment of special product-specific properties that address expected or already required regulatory requirements (e.g. safety and efficacy studies);

- Transfer to and optimisation of GMP production;
- Development and implementation of quality criteria and standards;
- Development of study protocols and informed consent forms for early phase I-II clinical trials;
- Development of a business plan that analyses critical variables in product definition e.g. to reduce manufacturing and marketing costs;
- Health Technology Assessments;
- Involvement of patients and other stakeholders.
- **Only Track 2**: Conducting an investigator-initiated, interventional, clinical study (Phase I to IIb for new therapeutic approaches. For further funding requirements for clinical studies see No. 4.
- **Not eligible for funding:**
- Founding of a company (“Start-up” or “Spin-off”);
- Phase III clinical studies

3 Eligibility criteria - applicants

Public and private (state-recognized) universities are eligible to apply for individual projects/as single applicant or as coordinators or partners of a consortium. Non-university research institutions (Außeruniversitäre Forschungseinrichtungen) and companies are eligible to apply as partners in a collaborative project/consortium. For applicants who are affiliated with non-university research institutions and employees who are organisationally assigned to it, the budget they apply for must not exceed 50% of the total project budget. At the same time, the budget of non-university research institutes cannot be higher than that of other applicants

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Research institutions with federal or state basic funding can apply for additional project funding if they clearly outline in their application how the project relates to or differs from their basic funded activities.

For information on the conditions under which state aid is/is not present and the extent to which funding can be granted free of aid, see the R&D&I Framework[1].

For this funding programme, small and medium-sized enterprises or "SMEs" are companies that fulfil the requirements of the EU definition of SMEs. The applicant must declare its categorisation in accordance with the GBER (General Block Exemption Regulation) to the funding authority as part of the formal application.

[1] Mitteilung der EU-Kommission (2022/C 414/01) vom 28. Oktober 2022 (ABl. C 414 vom 28.Oktober 2022, S. 1).

4 Special funding requirements

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Funding

In accordance with Section 7 (3) of the administrative agreement on the framework conditions for further developing the Berlin Institute of Health (BIH), project funding for institutions outside Berlin requires the state where the institution is based to cover 10% of the funding. Coverage of the financing share must be confirmed as part of the application (for details see chapter 7 in the German call text). For organisations based within the state of Berlin, the funding share is deemed to have already been provided by the state of Berlin's funding of BIH.

Preliminary work

Applicants must demonstrate relevant research and development work on GCT topics. The project must serve to validate existing research findings and must fulfil the criteria listed under section 2. The project must build on already initiated, recently completed (no longer than six months) or ongoing projects of translational product and/or process development in the field of GCT and complement these with defined work packages necessary for further product or process development.

The following applies for Track 1: The general feasibility (proof-of-principle) of the product or process/methodology must have been demonstrated and proven beforehand, e.g. by relevant publications or data shown in the project description.

The requirements for Track 2 are met if one of the following scenarios applies in addition to the criteria mentioned under 2:

- Patent granted (*Patent erteilt*) or
- Patent filed (*Patentanmeldung eingereicht*) or
- Invention disclosure claimed, patent application planned (*Inanspruchnahme mit geplanter Patentanmeldung*)

In the case of shared rights to the relevant patents (academia/private individual/company), only projects where the majority of these patents are held by academia and thus allow for later utilization by the academic institution are eligible to apply.

Exploitation and utilisation options

The expected results must contribute specifically to the development of a product or process in the field of GCT and must significantly increase the likelihood of successful translation or accelerate translation. In the long term, projects should aim to improve prevention, diagnosis or treatment of diseases that currently cannot be adequately addressed with existing, approved drugs and methods.

Cooperation (only for collaborative projects/consortia within Track 2)

Partners in collaborative project must agree on a common coordinator. The partners of a collaborative project have to define their cooperation in a written cooperation agreement. All collaborative partners, including research institutions within the meaning of Article 2 (No. 83) of the GBER, must ensure that no indirect aid flows to companies within the framework of the collaborative project. To this end, the provisions of point 2.2 of the R&D&I Union Framework Programme must be considered. Before a funding decision is taken on a collaborative project, a basic agreement on further criteria specified by the BMBF must be demonstrated (cf. BMBF form no. 0110[2]).

Quality of the methods used

A prerequisite for funding is the high quality of the methodology of the project applied for. When planning the project, the national and international state of research must be adequately considered. The validity of methods used must be guaranteed with respect to the research question addressed. The continuous integration of the required methodological expertise into the project must be guaranteed.

Clinical Studies

A prerequisite for funding a clinical study is the availability of high-quality, relevant data with high scientific rigor and robustness, which suggest clinical efficacy of the therapeutic approach to be investigated in the respective indication area. The strengths and weaknesses in the rigor of previous research must be thoroughly presented. Ideally, a consultation with the Paul-Ehrlich-Institute as the responsible regulatory authority has already taken place.

Scientific standards

Applicants are required to comply with national and international standards for quality assurance in preclinical and clinical research. This applies particularly to biobanks, animal studies, and the development of study protocols for early clinical studies in Phases I-II.

For funding applications for clinical studies, the following international standards in their current versions must be applied: Declaration of Helsinki, ICH Guideline for Good Clinical Practice (ICH-GCP), EU Directive 2005/28/EC, EU Regulation No. 536/2014, CONSORT and STARD statements.

For funding applications for animal studies, the ARRIVE guidelines in their current version must be applied.

The relevant standards are further specified in the guidelines/template for this funding directive.

[2] https://foerderportal.bund.de/easy/easy_index.php?auswahl=easy_formulare
Bereich BMBF Allgemeine Vordrucke und Vorlagen für Berichte.

Accessibility and long-term preservation of research data and results

Access to scientific findings and data is an essential basis for research, development and innovation. The long-term preservation and provision of research data contributes to the traceability, reproducibility and quality of scientific work. The following conditions therefore apply to the funding of clinical studies:

- Research results generated within the framework of this funding guideline must be published irrespective of their outcome.
- The study results should always be published as open access publications (see also point 6).
- Original data for the publications should be made available for subsequent use (digital; in compliance with the rights of third parties, in particular data protection, copyright).
- The criteria and the access route to the study data for analysis by third parties must be specified and made transparent in the application.

Participation

Within the scope of this funding programme, the involvement of stakeholders, in particular patients, should be considered. Participation should be carried out at the highest intensity appropriate to the respective research question and should be adequately considered when planning the budget.

With regard to the involvement of patients, it is recommended that the planning of research projects is guided, for example, by the following approach to the active participation of patients in health research: https://zenodo.org/record/7908077

The specific approach chosen to involve patients and their role in the project must be stated in the application.