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Faster from the laboratory to the patient: Initiative of Fraunhofer, Helmholtz and Hochschulmedizin promotes pilot project in the health sector

In the development of new medications and medical engineering, there is a gap between the discovery of new potential active ingredients and products and their further development into medicinal products and medical devices by the industry. The Helmholtz Association and the Fraunhofer-Gesellschaft, together with the Deutsche Hochschulmedizin, have now jointly brought the Proof-of-Concept initiative into being. It promotes the translation of innovative, promising research projects.

During the last decade, medical research has made decisive progress: The new understanding of the molecular and cellular basis of diseases allows new, innovative approaches in diagnostics, therapy and prevention. The interlinking of medical knowledge with engineering know-how makes novel medicinal products possible. However, one of the biggest obstacles on the necessary path to new drugs and medicinal products, is still the translation – the further development of new, potentially healing substances and procedures into candidates for usable therapeutic and medicinal products by the industry. "This translation, meaning the fast and targeted transfer of pre-clinical research results into useful medical applications and therapies that benefit people, is a tremendous challenge for health research. Here we see a need to catch up for Germany – the Proof-of-Concept initiative is therefore absolutely pioneering in nature", explains Prof. Reimund Neugebauer, President of the Fraunhofer-Gesellschaft.

In the next three years, the Fraunhofer-Gesellschaft, the Helmholtz Association and the Deutsche Hochschulmedizin will be providing up to twelve million euros for the Proof-of-Concept initiative. At the beginning of February, a high-ranking, specialist jury from science, industry and regulatory authorities determined the most promising research projects for funding. "We chose four innovative and outstanding research projects from a total of 82 interesting applications. Over the next few years we will optimally support the further development of these potentially pioneering active ingredients and therapeutic substances, so that they can quickly find their way into the medical sector and the market", says Prof. Otmar D. Wiestler, President of the Helmholtz Association.

The high number of submissions is proof of the substantial need for the funding of medical development projects, particularly in the field of Proof-of-Concept (PoC) in

Editorial Notes

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Germany. "The number of high-quality applications in reply to our invitation to tender was impressive. The submitted projects also show how well the facilities of the Hochschulmedizin already cooperate with their non-university partners", comments Prof. Heyo Kroemer, President of the Medizinischer Fakultätentag.

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Translation program for increased research needs

The resulting initiative must now be developed further. Prof. Otmar D. Wiestler points out: "We recognize here not only the purely scientific side, but also the need to take political action: The funding of appropriate medical and diagnostic developments must be improved." Prof. Reimund Neugebauer adds: "In order to cover the increased need for research in this vital medical sector, an in-house translation program should be set up, financed by the Federal government, the Länder and industrial partners through a common fund." The three project partners specifically propose to establish a fund with a budget of about 60 million euros, financed in two equal parts from public and industrial funds over a period of ten years for the development of potential active ingredients. The medical research should also be supervised by an experienced project management team and should benefit from the early incorporation of the regulatory and industrial aspect into the development. "University hospitals are places of translation at the interface between research and patient care. With the PoC initiative we are hoping to make good ideas for patient care come to fruition faster and more comprehensively than before, together with our non-University partners", says Professor Michael Albrecht, 1st chairman of the Association of University Clinics of Germany (VUD).

The selected projects of the PoC initiative:

1. With "Sleeping Beauty" against cancer

The US American Food and Drug Administration FDA approved for the first time in 2017 a gene therapy with so-called CAR-T cell therapy. Clinical studies of cancer diseases show that impressive success has already been achieved in the treatment of cancer using this form of therapy. This revolutionary therapy is also the focus of a research project that the PoC initiative will be supporting with about 2.8 million euros. The chimeric antigen receptors (CAR) developed the University Hospital of Würzburg detect a certain molecule (ROR1), which is barely present in healthy cells, but which occurs all the more on cancerous cells from leukemia, breast or lung cancer. In the case of the research project now being funded by the PoC initiative, the non-viral gene transfer takes place by means of the so-called "Sleeping Beauty" transposon system (SB100X). The funding is to be used to complete pre-clinical studies on the safety and efficacy of the ROR1 CAR-T cells and to achieve the clinical translation into a Phase I study (First-in-Man).

Project participants:

- University Hospital of Würzburg, Medical Clinic and Polyclinic II
- Max Delbrück Center for Molecular Medicine in the Helmholtz Association (MDC)
- Fraunhofer Institute for Cell Therapy and Immunology IZI

2. Relieving side effects in cancer patients

A new cancer drug was launched more than 25 years ago, which is now indispensable in cancer therapy: Paclitaxel. Today, Paclitaxel is used for the treatment of the most various types of cancer such as breast cancer, prostate or ovarian cancer. However, its active ingredient can have unpleasant side effects: for example so-called neuropathies such as tingling sensations or numbness and pain in the fingers and toes. So far there is no drug capable of reducing these side effects. However, a possible candidate for doing just that is the active ingredient TMP-002. The PoC initiative will now finance a new clinical study on TMP-002 with a total of 650,000 euros. This phase II study will investigate the efficacy and safety of the active ingredient in the treatment of patients with ovarian or breast cancer.

Project participants:

- Frankfurt Fraunhofer Project Group for Translational Medicine and Pharmacology TMP, Fraunhofer Institute for Molecular Biology and Applied Ecology IME
- Goethe University Frankfurt am Main
- German Cancer Consortium (DKTK) at the University Hospital of Frankfurt – a partner location of the German Cancer Research Center (DKFZ)

3. Nano particles against pulmonary hypertension

Tired, short of breath and generally little resistant to physical stress: these are the symptoms of patients with pulmonary hypertension. A new, highly promising therapeutic approach to pulmonary hypertension is currently being sponsored by the PoC initiative with around 3.6 million euros. Here, a new drug consisting of a highly effective active ingredient in bio-compatible nano particles is to be developed, administered to the patients by means of inhaling. The active ingredient directly attacks the diseased pulmonary blood vessels to heal their tumor-related changes. The nano particles serve as a miniature transport ferry, administering the active ingredient in a targeted manner with a low incidence of side effects.

Project participants:

- Justus Liebig University Giessen
- Helmholtz Zentrum München – German Research Center for Environmental Health
- Fraunhofer Institute for Toxicology and Experimental Medicine ITEM

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4. A boost for the treatment of hepatitis B

In about 260 million people, the hepatitis B virus can be detected in the blood for life. In many cases, hepatitis B leads to liver cirrhosis or liver cancer, killing up to 880,000 people every year. The PoC initiative will support a research project which for the first time could enable the healing of the chronic hepatitis B virus with just under 2.6 million euros. Different components are to be used for the innovative therapeutic vaccination for Prime and Boost. The new therapeutic vaccination against hepatitis B could already be successfully tested in pre-clinical studies. The funding enables finalizing preclinical studies, immunotoxicological investigations and a clinical phase I study.

Project participants:

- Helmholtz Zentrum München – German Research Center for Environmental Health
- Fraunhofer Institute for Cell Therapy and Immunology IZI
- University Hospital rechts der Isar of the Technical University of Munich (TUM)
- University Medical Center Hamburg-Eppendorf (UKE)

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