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**Centre for research and treatment of rare diseases to be established at Masaryk University**

**A unique international project has been launched at Masaryk University’s Faculty of Medicine, which is dedicated to the research, development and production of advanced therapy medicinal products for patients for whom no medical solution currently exists.**

Over half a million people in the Czech Republic suffer from one of the approximately 7,000 described rare diseases. At the same time, many patients are still waiting for their diagnosis. The number of newly diagnosed rare diseases is constantly growing, which is why a cutting-edge scientific centre is being established at the Faculty of Medicine of Masaryk University to research and produce drugs for modern therapies for selected rare diseases. “In my original profession as a doctor, I have many times encountered situations when it was no longer possible to help a patient, and believe me, these are strong moments for the treating doctor as well. But now, thanks to the Faculty of Medicine and its project CREATIC, we have given at least some of them an imaginary spark of hope. And as the Rector of Masaryk University, I am proud that our institution can be part of such a unique project that aims to help and offer hope for life,” says Rector Martin Bareš.

The new centre will be called CREATIC – Central European Advanced Therapy and Immunotherapy Centre. It will operate within the structure of the Faculty of Medicine of MU at the Bohunice University Campus, where the reconstruction of Building C03, which will become its headquarters, will start in February next year. “Our mission is to contribute to a quality and healthy life through our scientific activities, student education and social action. Universities should also deal with societal problems, and in the case of rare diseases, I believe that research, development and production of drugs on campus will in the future be a necessary complement to the activities of the pharmaceutical industry, for which it is neither capacity- nor economically feasible to develop all drugs for the rapidly growing number of very rare diseases,” adds Martin Repko, Dean of the MU Faculty of Medicine.

The CREATIC project is led by Regina Demlová, Head of the Department of Pharmacology at the Masaryk University Faculty of Medicine. In recent years, her team of laboratory colleagues has devoted great efforts to the development of somatic cell drugs for a pair of diagnoses for which commonly available chemical drugs do not work. These are epidermolysis bullosa, also known as butterfly wing disease, and rare cancers affecting mainly pediatric patients. Pharmacologists have already developed two drugs from the advanced therapies group for these patients. The first is an anti-cancer vaccine used for pediatric cancer patients. “This vaccine works on the principle of activating the patient’s own immune system. It recognises tumour cells in the body and destroys them in a targeted manner,” explains Associate Professor Demlová. More than 40 patients have been treated with this type of therapy in the clinical trial so far. Many of them are in long-term remission of the disease. The second drug helps wound healing in patients with butterfly wing disease. “Both types of products enable very precise and targeted treatment of rare diseases and can be produced on a small scale for a limited number of patients,” Demlová adds.

One of the pediatric patients who has already been helped by an anti-cancer vaccine produced in the laboratories of the MU Faculty of Medicine is Daniel from Ostrava, whose disease first started to manifest itself at 14 months. "At first it looked like a virus, but my son’s condition deteriorated very quickly. Daniel was diagnosed with cancer. After two blocks of chemotherapy it turned out that there was no solution for his type of brain tumour; all available treatment options had been exhausted," explains Lydia, Daniel’s mother. So his parents were offered one last option: to take part in a clinical trial in which he was treated with a dendritic cell-based cancer vaccine. The boy’s body responded very positively to the treatment. Today he is 9 years old, in long-term remission, without any signs of disease, and doing very well.

The limited treatment options for patients with rare diseases on the one hand, and the rising costs of existing therapies on the other, are also intensely perceived in a wider regional context by the European Commission. The work of the team from the Department of Pharmacology, Faculty of Medicine, MU has not gone unnoticed. For its project, it has been awarded a prestigious European grant from the Teaming Horizon Europe research and innovation programme worth 15 million EUR for the next six years, and at the same time received support of over 500 million CZK from the national Johannes Amos Comenius Programme. The purpose of the CREATIC project is, among other things, to connect geographically close academic and clinical sites. The scientists from Brno will be supported by experienced colleagues from the German Fraunhofer Institute for Cell Therapy and Immunology IZI and the University of Leipzig, who already have many years of experience in the development and production of gene and cell therapies. The partners from the University of Copenhagen will offer their expertise in legislation and data handling. "The experience with the development and non-commercial production of gene therapies at Fraunhofer IZI is quite unique at the national level and thanks to the partnership and know-how sharing, we will be able to offer these medicines developed at the university to Czech patients," says Demlová.

In this way, an excellent scientific workplace will be created in Brno, where medicines will be developed not only for patients with butterfly wings disease but also with other diseases caused by a specific gene mutation. One of CREATIC’s priorities is to open of a social discussion on the availability of care within the public healthcare system and the issue of "fair medicine", i.e. that such drugs are produced without profit maximization and their price is transparent for payers. This debate should pave the way for treatment for patients for whom there is currently no solution or it is legislatively and financially difficult to access. "In connection with ever-improving diagnostics and treatment, the number of patients has increased by a third over the past three years, and since the costs of therapies for rare diseases are in the order of tens of millions of Czech crowns, securing resources for the development of this care is a societal challenge. The development and production of such medicinal products on a non-commercial basis is one of the ways to ensure the availability of care and the stability of public budgets," concludes Regina Demlová.

