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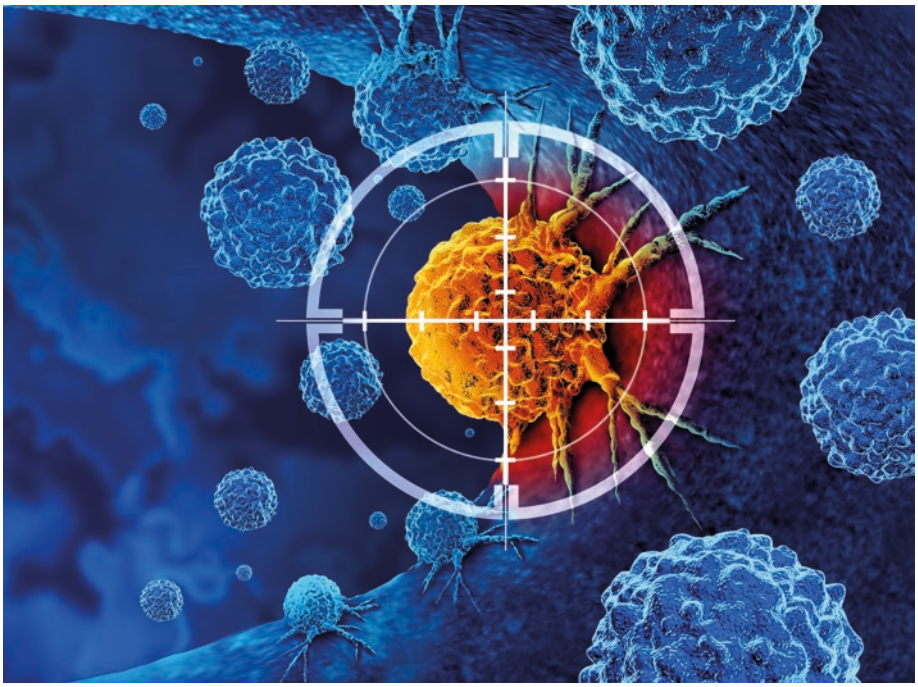
Cell and Gene Therapies in Leipzig: Stronger through Networking

Leipzig's Fraunhofer Institute for Cell Therapy and Immunology IZI is advancing innovative therapies

Medicine has made rapid advances in recent years – especially in the fields of immunotherapies as well as innovative cell and gene therapies. These promising therapeutic approaches are capable of more effectively addressing previously hard-to-treat diseases, sometimes even with the potential of curing them. But there is a significant roadblock between prospects and reality: High costs and complex approval processes slow the widespread use of these therapies. However, better collaboration and the seamless integration of different development phases could pave the way to more efficient, customized and, above all, economically viable solutions. Leipzig is proving itself to be a pioneer in this respect. **By Urs Moesenfechtel**

Many academic research institutions and pharmaceutical companies are currently pushing ahead with the development of cell and gene therapies (CGT). Because these are not only seen as a future "magic bullet" against cancer, but could also be a game changer for numerous hereditary diseases. Recently developed gene therapeutic drugs use adeno-associated viruses (AAV), for example, which do not cause diseases themselves, but replace defective human genes with functional genes. These types of CGT alone may be able to be used to treat about 7,000 inherited diseases.



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Challenges when developing cell and gene therapies

However, as with most modern therapies, the technical and human resources needed for the development, approval and introduction of CGT are enormous.

> *The manufacturing costs per patient-specific cell therapy product are between 200,000 and 300,000 euros.*

The manufacturing costs per patient-specific cell therapy product are between 200,000 and 300,000 euros. Gene therapeutics for very rare diseases are even more expensive, at up to 2 million euros. Granted that treatment methods like chemotherapy or stem cell transplantation with permanent immunosuppression are similarly time-, personnel- and cost-intensive, especially in view of societal follow-up costs such as lost working hours and patient care. However, these methods are already firmly established in the healthcare system. As a result, large segments of the population do not yet have access to CGT.

CGT would be more economically competitive if the time and associated costs of basic research, early drug development, GLP studies, preclinical work, manufacturing of test samples, clinical trials, and commercial applications were optimized. This could be achieved by better interlinking and holistically integrating these

elements in one location or through one player. So far, however, this kind of integration has been the exception rather than the rule, especially in Europe. This is because only a few pharmaceutical companies have all the necessary components for developing and manufacturing CGT, including clinical testing and regulatory approval.

Holistic approach is needed

Drug pipelines are largely filled by academic research and smaller start-ups, GLP studies and preclinical work are often outsourced to specialized companies or application-oriented research institutions. In turn, cost-intensive clinical testing is performed by financially viable pharmaceutical companies, and approved drugs are made by contract manufacturers with the appropriate production capacities. CGTs seeking approval as so-called Advanced Therapy Medicinal Products (ATMPs) are far more complicated and costly to develop than conventional drugs. This is mainly due to the personalized therapy approaches as well as the biological starting material. Living cells, whether from patients themselves or from healthy donors, can only be standardized to a limited extent and require biological expertise in handling and processing. In addition to an appropriate infrastructure, both preclinical testing and process transfer to GMP-compliant manufacturing processes in accordance with pharmaceutical standards also need expertise in all development steps, as well as close links to clinical studies and application centers.

Leipzig: Building bridges between research and clinical application

In Europe, this much-needed bridge between research and clinical application is currently being built primarily in Leipzig. The city's Fraunhofer Institute for Cell Therapy and Immunology IZI offers more than 17 years of expertise in manufacturing CGTs and supporting studies and approval processes. By means of GLP testing, process development and the GMP-compliant manufacture of clinical trial samples, the Institute ensures the transfer of experimental drug development stages into clinical application. The Fraunhofer IZI was already involved in the production of the clinical investigational medicinal products for the European registration trial of the world's first CAR-T cell therapy called Kymriah (manufacturer Novartis). Since 2006, more than 3,500 cell therapy test samples for clinical trials have been manufactured at Fraunhofer IZI, including more than 500 CAR-T cell products. Europe currently has no comparable institute.

Photo: © Fraunhofer IZI

More than 500 CAR-T cell products for treating certain forms of leukemia have already been manufactured in the clean rooms of the Fraunhofer IZI. One of the three cleanroom facilities in Leipzig is currently being converted and modernized for the GMP-compliant production of AAV-based drugs.

The Fraunhofer IZI is also addressing the increasing demand for CGT by developing, together with clinical and industrial partners, new technologies for the (viral and non-viral) modification of immune cells. New effector cells for cell-based therapies, such as natural killer (NK) cells and macrophages, are also being investigated and tested. A central focus of the Fraunhofer IZI's research is the development of CAR-NK cells to treat solid tumors which to date cannot be effectively addressed by CAR-T cell therapies.

SaxoCell as a node of innovation

The close cooperation with certified and experienced application centers located in the immediate vicinity promotes a climate of innovation and enables the rapid transfer of knowledge and applications. These centers include the university hospitals in Leipzig and Dresden, as well as Chemnitz Hospital. Their close ties to the academic world guarantee a seamless transition from research to clinical practice. The Fraunhofer IZI is also a key member of the SaxoCell innovation cluster. This consortium of Saxon research institutes, hospitals and industrial partners is the only future cluster funded by the German Federal Ministry of Education and Research that merges CGT with automation technologies, digitalization and artificial intelligence.

Prospects for Leipzig in the life sciences

Thanks to this interconnected life science ecosystem, Leipzig has the potential to remain a leading European hub for regulatory preclinical work, process development, and CGT manufacturing. In addition, the city and region are also making a name for themselves in future industries such as diagnostics, digital health, robotics and artificial intelligence. The prospects for future growth are good, because the municipality of Leipzig and the state of Saxony have not only recognized the strategic importance of these technologies: All the players involved are also aware that, in addition to providing appropriate funding instruments and financial resources, further networking and cooperation with each other will be crucial for future success. ■

Leipzig for Life Changers

Successful translation – made in Leipzig

Getting cell and gene therapies from bench to market can be challenging. That's why you need a strong partner at your side. Leipzig's life science cluster in the heart of Europe offers a unique range of support: State-of-the-art R&D. Efficient transfer services. Swift approval pathways. Extensive networks. A thriving business landscape. Whether you are still a start-up or a mature company set to expand - look to Leipzig and become a LifeChanger with us!

Leipzig-for-lifechangers.com

Leipzig Immune Oncology Conference (LION), November 8th - 9th 2023

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