

## **Growing support for gene and cell therapies in Germany**

*The commitment of the German Stem Cell Network to translational research and the development of cell therapies attracts considerable support. Through the National Strategy for Gene and Cell Therapies, the German Federal Ministry for Education and Research (BMBF) is devoting increasing attention to the development of gene and cell therapies. In addition, the newly founded National Network Office for Gene and Cell Therapies aims to network, support, inform and serve as a point of contact for both researchers and patients. Daniel Besser, Managing Director of the GSCN, and Claudia Waskow, President of the GSCN, met with Elke Luger, Head of the Network Office at the Berlin Institute of Health (BIH), to discuss their goals, visions and potential means of achieving them. All three expressed a key, shared goal: simplified regulation on the road to new cell therapies – in the interests of researchers and patients.*

**GSCN:** The BIH has overseen the development of a National Strategy for Gene and Cell Therapies in Germany and simultaneously established the National Network Office for Gene and Cell Therapies. What is the thinking behind this?

**Elke Luger:** The Federal Ministry of Education and Research (BMBF) commissioned the BIH to develop the National Strategy for Gene and Cell Therapies. The BIH was tasked with coordinating and moderating this process, bringing together experts from all over Germany to jointly define the topics of the eight fields of action and draw up recommendations for action. The resulting strategy paper is intended to define solutions and milestones to ensure that Germany remains internationally competitive and fully exploits the value chain in the field of gene and cell therapies (GCT). The National Network Office for Gene and Cell Therapies (Network Office for GCT) has the task of bringing stakeholders together, retaining the experts from individual working groups as valuable long-term contacts, and then identifying and meeting their needs.

**Daniel Besser:** There is also the German Society for Gene Therapies (DG-GT), which was heavily involved in the process of developing the strategy and will engage closely with the Network Office for GCT in the future. The strategy ultimately arose from the joint Charité-Universitätsmedizin-Bayer initiative in Berlin, which is to be put on a national footing. A long preliminary phase with several roundtables and various events has now produced the strategy paper.

**GSCN:** What are the interfaces between the German Stem Cell Network and these BIH gene and cell therapy projects?

**Claudia Waskow:** It is particularly important that the strategy development process and the projects pursued by the Network Office for GCT include close cooperation with the German Stem Cell Network (GSCN). After all, stem cells are the cells used in gene and cell therapy. Hematopoietic cells are particularly important in this context because they have been extensively studied and are used in clinical settings. In many preclinical and clinical studies, researchers are examining treatments that involve genetic corrections of mainly monogenetic diseases. Close collaboration between our two networks and entities therefore make a lot of sense given the linked subject matter.

SUBHEADING: COMING TOGETHER

**Daniel Besser:** There is considerable crossover in the scientists who work in the two networks. However, as the GSCN, we have also already done a lot of groundwork in terms of engaging with

society – through materials for schools, exhibitions and providing information online and at events. We also have European partners, such as EuroGCT and the European Society for Gene and Cell Therapy, with whom links could be forged. I can also imagine joint white papers and a host of other excellent projects that could be added in the future.

**Elke Luger:** Referring to existing structures is crucial; this is also fundamental to the work of the Network Office for GCT. We will exploit synergies and avoid creating redundancies. As the network office for GCT, we want to build a GCT database for the community, a knowledge concentration of all existing structures, networks and training opportunities. Essentially, a central location to represent clinics, research institutions and contacts - for patients, students and researchers. In other words, a record of all the structures that exist and facilitation of effective national – and, in the long term, international – collaboration, as Daniel mentioned.

**GSCN:** [What vision do the Network Office for GCT and the GSCN have for the future of the GCT research and therapy landscape in Germany?](#)

**Elke Luger:** One of the visions for a potential future is the ability to fall back on joint protocols or joint GMP manufacturing sites to maximize the use of resources. We could close gaps where supply chains prove problematic. We could create a network, here in Germany, that extends from the developers to the users to the manufacturers. My aim is to keep this information up to date through regular exchanges with the community and to make it centrally accessible to everyone.

SUBHEADING: VISION FOR THE FUTURE OF THERAPIES

**Daniel Besser:** I think it's too narrow to think of cell therapies in the German context alone. There is a lot of work underway in Europe. There will have to be a give and take in development activities between different countries. I am also firmly convinced that we have to get to grips with the costs. At present, we have a structure that is ultimately geared towards the pharmaceutical industry with low-molecular substances in the regulatory process. This process is incredibly expensive. If I end up with a small molecule, i.e. a drug that I can produce centrally, then I can bear these costs of one to two billion euro for the entire clinical process. But if we don't bring these costs down in the future, I don't see how we'll be able to offer such therapies to many patients with serious diseases, such as Parkinson's, diabetes and a variety of rare diseases, who are waiting for new gene and cell therapies.

This means that we will have to think about cost structures and reimbursement structures in the future. There are over 10,000 diseases waiting for a cure. We have a lot of work ahead of us: beyond networking stakeholders and manufacturers, we must also make progress on national and European regulation, cost structures, and discussions with health insurance companies and the Federal Joint Committee (G-BA), working at both national and European levels.

**Claudia Waskow:** We must ensure that teams working in Germany are represented in all major international scientific initiatives in the future. This will require a coordinated flow of information, which the National Strategy can provide via the Network Office. There are large international consortia on gene and cell therapies that involve surprisingly few groups from Germany. Major centers in London, Paris and Milan are more strongly represented, so this requires urgent adjustment. Strategic collaboration should reduce the complexity of regulatory requirements in Germany, specifically regarding regulations on genetic engineering.

**GSCN:** Looking at the situation internationally, what are the specific bureaucratic and regulatory disadvantages facing Germany?

**Claudia Waskow:** The implementation of European guidelines differs slightly in every country, which means that their interpretation also differs. In Germany, however, there are even differences between individual federal states, with regulations interpreted slightly differently. This soon leads to confusion.

SUBHEADING: SIMPLE REGULATION

**Elke Luger:** A straightforward regulatory framework is also crucial for international investors, who want to provide funding swiftly for these very current, technical procedures. If this process is so cumbersome in Germany, with such differences between the legislation of different federal states, it is easier to invest venture capital in France or Italy – where there is only one law in force nationwide.

**GSCN:** How can we change these regulations and bureaucratic processes in Germany?

**Elke Luger:** We obviously cannot abolish federalism. However, one aim of the National Strategy for Gene and Cell Therapies is to draw up guidelines upon which the individual German states can base their legislation.

**Claudia Waskow:** One approach would also be to cooperate with the relevant groups in the German Research Foundation (DFG).

**Daniel Besser:** The problem is more far-reaching: in Germany, companies must obtain authorization to manufacture cell and gene products from the state authorities and not just the Paul Ehrlich Institute (PEI) or the European Medicines Agency (EMA). The result is regulatory demands from two sides – the state authorities and the EMA – and these demands often do not coincide. We should strive to find common ideas and regulations.

**GSCN:** How will researchers benefit from the National Strategy for Cell and Gene Therapies? How can they get involved?

**Elke Luger:** They were invited to contribute to the development and drafting process, where the aim was to highlight deficits and propose solutions to move the whole topic forward. However, we want to continue to uphold and leverage this spirit of cooperation in the Network Office for GCT. We see ourselves as a platform for GCT-related communication, information, cooperation matching and events. We also want to provide information on how to secure funding and on future funding lines, both in Germany and across Europe. This is crucial for researchers; we see it as our duty. We also provide support in the scientific advice process. At present, we are setting up a regulatory support unit that will offer help with initial interactions with the Paul Ehrlich Institute.

**Daniel Besser:** For one thing, scientists are helping shape the strategy process, which is important. As soon as a financial structure for the strategy has been established, researchers who are already well informed will have a good chance of getting their projects funded, subject to a successful review. The distribution of funds must be – and will be – transparent.

In terms of regulation, I think it is very important that start-ups and developers are supported and offered assistance with less directly connected topics, such as human resources and intellectual property (IP). Where can I find the right people to facilitate certain things, such as GMP (Good

Medical Practice) facilities? How should I protect my intellectual property? What support do I need when it comes to sponsors and legal matters? We have to offer support on these issues – nationwide!

**Elke Luger:** I agree. Researchers also need to broaden their horizons. To date, publications have been the currency of scientific impact.

SUBHEADING: HOW DO I LAUNCH A SPIN-OFF?

**Elke Luger:** Yet, if we see the goal of our research as providing treatments for patients, then the way we achieve that is by developing therapies. This idea of publishing less and dealing instead with the mechanisms of patent development and license development, is not yet anchored in any university curriculum. It is for precisely this reason that we need to develop a Germany-wide technology transfer program in the field of gene and cell therapies with partners in Munich and Mainz, where we are dealing with precisely these topics. How do I patent something? How do I secure a license? How do I launch a spin-off? We're trying to show that there are also intermediate forms between academic research careers and spin-offs. The program is called GeneNovate: Empowering Innovators and Entrepreneurs in Gene and Cell Therapies. In the future, we hope to work with partners to roll the program out at many other locations across Germany.

**GSCN:** [How will the GSCN collaborate with the Network Office for GCT and contribute to its initiatives?](#)

**Claudia Waskow:** I expect that the GSCN will benefit greatly from this new platform with its information and funding. The topics of gene and cell therapies are of interest to both of our members. Cooperation will yield benefits. We can do this in the GSCN Working Group on "Stem cells in regenerative therapies" and also at the GSCN Conference.

**Elke Luger:** We are delighted by the collaboration with the GSCN and see many interfaces – not only in terms of research and funding, but also in public outreach to groups of the population including patients, pupils and students.

**Daniel Besser:** We have positive experience of working together in the past; the collaboration between the GSCN and the Network Office for GCT is an enriching one.

*Interview by Stefanie Mahler*