The complexity and rewards of gene treatments for rare diseases
Gene therapies can transform the approach to rare diseases and improve the quality of life for patients. But developing and accessing them is hampered by regulatory burdens, cost and reimbursement schemes.

In this special report, Euractiv takes a look at gene treatment approaches across countries, and examines the EU's approach to such therapies for rare diseases.

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Gene therapies have the potential to treat rare diseases from their genetic root. [SHUTTERSTOCK/vchal]

EU needs to step up to unleash gene therapies potential, stakeholders say

By Marta Iraola | Euractiv.com

Gene therapies are innovative treatments that try to address the genetic cause of a disease by replacing the malfunctioning gene, inactivating it, or introducing a new or modified gene into the body to help fight the disease.

They are referred to as Advanced Therapy Medicinal Products (ATMPs). These treatments are mostly used to treat rare diseases which do not come as a surprise as around 80% of rare diseases are of genetic origin.

There are currently 6,000 different rare diseases in Europe affecting 36 million people.

Gene therapies have been investigated by the pharmaceutical sector since the 1990s. However, it was only in 2012 that the first gene therapy – an in vivo treatment targeting an ultra-rare disease – was approved for the EU market.

Since then, there have been 18 gene therapies authorised by the European Medicines Agency. However, not all of them are still available. In May 2023, there were only 15, after three withdrawals of marketing authorisation as the companies did not find the commercialisation of their product profitable.

The small number of therapies that reach marketing authorisation is worrying for the industry as the development of the treatments is exceptionally expensive, but also for the patients, who see their opportunities highly reduced.

Need for a common approach

To guarantee access for all patients to these kinds of therapies, Simone Boselli from Euronids, an NGO working for rare diseases in Europe, stressed the need for cross-border care and harmonisation of the framework across the EU.

“Collating the evidence generated from one country to another leads to potential better decision making, also in pricing and reimbursement,” he said.

Due to the complexity of the diseases and the low prevalence, expertise and capacities are often missing within a region or country of the European Union.

François Houÿez from Eurordis recognised that it is clear that not all countries need to have specialised centres able to administer these therapies for a small number of patients, but patients, wherever they live, should be able to travel to the specialised centre and receive treatment there.

That is why stakeholders criticised the incentive scheme set by the European Commission in the new proposal for pharmaceutical legislation, aimed to encourage investment in Europe and address the current industry challenges.

The Commission’s proposal includes extra regulatory protection for those companies that place their products in the 27 member states. This tool, aiming to ensure accessibility across Europe, is being questioned by stakeholders, in the case of rare diseases, for being unattainable.

“These incentives are not attractive enough for gene therapies”, explained Boselli. Eurordis maintains that there is no need for a product to be placed in the market in all member states but it should be available only for a few centres to serve the entire population.

“A common European pathway would help in this situation to get to resolve this conundrum that we have, that we have more scientific innovation, but less patient access to those therapies,” he added.

Long way ahead

One of the tools the EU could use to harmonise the gene therapies environment across member states is the new regulatory framework for Health Technology Assessments (HTA).

The legislation, adopted in 2021, will enter into force in 2025 when all cell and gene therapies, among others, will undergo a single EU assessment instead of the current 27 for each country.

The HTA regulation will help EU countries determine the effectiveness and value of new technologies and decide on pricing and reimbursement by health insurers or health systems.

Novartis, one of the few companies with a gene therapy authorised in Europe, also told Euractiv that the fragmentation of the framework was one of the main challenges they encountered when commercialising their product.

“We were talking to HTAs in Europe and we were looking at nearly 30 different systems that all required a tailored approach,” a spokesperson explained.

The spokesperson added that their future hope is for a greater alignment of regulatory and HTA evidence requirements, adding that without payers and HTA bodies creating pathways for ATMPs, the true impact of innovation may not reach patients.

Houÿez told Euractiv that they have “good hopes” as it seems the EU has taken the initiative to address this situation.

However, he recognised that small fixes will not solve the current situation.

“We will need a huge change in the organisation of the pharmaceutical market.”
In France, gene therapy suffers from weak economic model

By Bartosz Sieniawski | Euractiv.pl

France is one of the most advanced countries for gene therapies, both in Europe and worldwide. However, the lack of funding and the economic fragility of the sector are holding back the development of these innovative treatments.

In 2016, a 13-year-old boy suffering from sickle cell anaemia, an inherited genetic disorder affecting red blood cells, was successfully treated with gene therapy by doctors at Necker Hospital in France. It was a world first.

"France is a major country for the invention of gene therapies, and many of them were born in France, notably at the Necker hospital," said Eric Baseilhac of the professional organisation of pharmaceutical companies operating in Leem, France, in an interview with Euractiv.

Gene therapies, which form part of innovative therapies, involve introducing genetic material into cells to treat a rare disease. This treatment is used in particular to treat cancers and paediatric diseases.

Worldwide, around 2,000 clinical trials have been conducted or are underway since 1989, 65% of which involve cancer. In France, around 30 gene therapy trials have taken place or are underway, according to the French National Institute for Health and Medical Research (INSERM).

However, despite this success, it is difficult to make the development of gene therapies economically viable for pharmaceutical laboratories.

"France is finding it very difficult to raise the funds needed to support start-ups developing gene therapies all the way," Baseilhac said.

If gene therapies are not very profitable for the companies that market them, it is mainly because of their high price, which is explained by the complexity of their manufacturing.

"Production costs for gene therapies can currently amount to several hundred thousand euros per patient. The challenge is to bring them down," Frédéric Revah, a specialist in the research and development of gene therapies, told Le Monde newspaper.

According to Baseilhac, the high cost of gene therapies must be understood "in terms of cure", i.e. lifelong efficacy.

Health Innovation Plan 2030

In June 2021, French President Emmanuel Macron announced the launch of the Health Innovation Plan 2030, which aims to make France the leading European nation in terms of innovation and sovereignty in healthcare.

"We are currently experiencing a real revolution in the fields of health and life sciences," Macron said.

With a budget of €7 billion, the plan was intended to finance "major transformations" in healthcare, such as gene therapies and the use of artificial intelligence (AI).

However, according to Baseilhac, the plan alone will not be enough to accelerate France’s development and commercialisation of gene therapies.

"The plan does not solve the problem of the economic model for gene therapies and their funding," he highlighted.

In France, the majority of companies working in such treatments are start-ups – and face a complicated business model.

According to the Leem expert, marketing a treatment that cures a rare genetic disease means "resolving to have nothing left to sell after a few years".

The only way for these small companies to continue to be economically viable would be to create new gene therapies every five years or so, but this is a real challenge.
Europe lags behind the US

Access to gene therapies for patients is restricted not only in France but throughout Europe, not least for economic reasons.

“In Europe, we are still looking for a sound economic model that will enable us to offer access to these therapies on our territory,” said Baseilhac.

Christian Anastasy, chairman of strategic healthcare consultancy firm Persan Conseil, said in a press release: “While the unit cost of the treatment is significant, let’s not forget that this investment is sustainable and that it will generate savings on the various forms of care that will no longer be necessary.”

Gene therapies are mainly used to treat rare diseases. “Up to 36 million people in the EU live with a rare disease. There are more than 6000 distinct rare diseases in the EU. So, whilst one rare disease may affect only a handful of patients, another may touch as many as 245 000. Around 80% of rare diseases are of genetic origin and, of those, 70% already start in childhood,” according to the Commission data.

“For the time being, there is no great pressure from European patients because these therapies affect so few people. This is unfair compared with patients based in the United States,” added Baseilhac.

In fact, there are dozens of gene therapies in the United States, compared with just a handful in Europe and one in France. “The EU is out of step with the United States. This is not acceptable”, he concluded.

Picture on the right: quote from French president Emmanuel Macron on the Health Innovation Plan 2030. [Shutterstock / Frederic Legrand - COMEO]
A European Special Report | The complexity and rewards of gene treatments for rare diseases | Euractiv

By Simone Cantarini | Euractiv.com

Italy’s advanced therapy journey

Advanced Medicinal Therapies (ATMPs), which include gene and cell therapies, are opening treatment options for patients suffering from rare or ultra-rare diseases, [SHUTTERSTOCK/Gorodenkoff]

Advanced Medicinal Therapies (ATMPs), including gene and cell therapies, offer treatment options for patients suffering from rare or ultra-rare diseases. Italy is learning to see expenditure on advanced therapy drugs as an investment but the EU’s pharmaceutical revision is causing concerns among Italian stakeholders.

"We now have 18 advanced therapies in Europe, including 14 gene therapies, two cell therapies, and two tissue therapies," Celeste Scotti, director of research and development at the Telethon Foundation, told Euractiv.

Of these advanced therapy medicinal products available on the market, 10 have been approved for reimbursement by the Italian Medicines Agency (AIFA), paving the way for wider access for patients. Rare diseases affect at least 2,000,000 people in Italy. Of those, one in five is under the age of 18.

"In Italy, the cost of the drug is covered by the national health system," said Scotti. His non-profit organisation runs some of the centres of excellence in the field of gene therapies: the San Raffaele–Telethon Institute for Gene Therapy (SR-Tiget), based in Milan, and the Telethon Institute of Genetics and Medicine (TIGEM), based in Pozzuoli.

For example, cutting-edge gene therapy for the treatment of ADA-SCID, a rare immunodeficiency of genetic origin, which was developed by researchers at the SR-Tiget, is fully reimbursed by the national health system.

Regardless of being a pioneer and one of the leaders in ATMPs – out of the 18 advanced therapies authorised in the European Union, four are the result of Italian academic research – Italy, like other countries, encounters obstacles from the pre-clinical and clinical phases to access and sustainability.

The complexity of ensuring access to treatment

"Talking about accessibility and equitable distribution of drug treatments means dealing with a very wide world, which brings with it a number of facets that each need specific in-depth studies," the president of UNIAMO – Italian Federation of Rare Diseases Annalisa Scopinaro told Euractiv.

But out of this "very wide world" Scopinaro highlighted economic sustainability, "which must be combined with universal access to advanced and innovative therapies so as not to leave anyone behind".

In this regard, Scopinaro said that in 2023, UNIAMO, Italy’s association for rare diseases, carried out an institutional training hosted by the Parliamentary Intergroup for Sustainable Innovation in Healthcare to raise awareness in the Italian Parliament of the need for adequate regulatory and legal framework for innovative therapies.

One of the results of the training course is a document, currently being finalised by the participants, that accepts the proposal of Senators Franco Zaffini and Daniela Manca to introduce in the new Italian budget law an amendment for a fund for gene therapies of approximately 150 million euros.

The discussion of the 2024 budget law is underway in Parliament, which will have to approve and possibly modify it with amendments by 31 December.

A philanthropic business model

In the meantime, Scotti has another proposal: "We need a new model for the development of therapies for rare and ultra-rare genetic diseases and a new model for market access."

He believes the non-profit sector is a way forward "where there is no business case for a for-profit company because of such high costs and the rarity of the disease". Indeed, with high costs and few patients, "it is very difficult for such a therapy to be profitable in a for-profit context."

Telethon is an example of this. Research and development costs are covered by donations by Italian citizens and companies, "who expect a benefit for society from us as a return on their investment, not a financial gain as when investing in a for-profit company," Scotti said calling the model "philanthropic".

"In this sense, we hope to be trailblazers. So, what we can say at this time, which is peculiar to Italy, is we hope that others will take their cue from Telethon," Scotti added.

As an example, Scotti referred to the case of the Strimvelis therapy which is used for the treatment of ADA-SCID, developed in the laboratories of SR-Tiget.

In 2022 the Anglo-American pharmaceutical company Orchard Therapeutics PLC, owner of the Strimvelis therapy, announced its intention to disinvest in the field of primary immunodeficiencies.

To prevent the therapy from no longer being available, Telethon took over the marketing task from Orchard, following the positive opinion of the European Medicines Agency (EMA) and the authorisation by the European Commission.

The Strimvelis therapy drug was authorised in 2016 by the AIFA and has already been administered at the San Raffaele in Milan, the only authorised centre: over the years, 45 patients from 20 countries have been treated.

"The one thing we could not do was to let an effective therapy, developed by Telethon researchers with Telethon funds, be put in a drawer," Scotti pointed out.

Concern about a potential crisis for advanced therapies in Europe

While Italy is navigating its way in the field, industry players are concerned about the possible exit of pharmaceutical companies from the EU’s market. On April 26, the Commission proposed a new directive and regulation to review and replace the current general pharmaceutical legislation with one of the aims being to make the EU’s market more attractive.

"[The revision] could introduce important changes for access, on the one hand, but on the other hand partly discourage investments in rare diseases and the observation of worrying phenomena," Scopinaro said.
The president of UNIAMO stated that “there are already two pharmaceutical companies that have abandoned the European market or even stopped producing the therapy”.

Therefore, it is “necessary to understand the underlying reasons and to try to remedy them, where possible, in order to ensure treatments for those who have no other therapeutic alternatives”.

As a UNIAMO report highlighted, the EMA has a centralised authorisation that refers to the individual states for a definition of price & refund.

This leads to differences in approval times and therefore actual availability for patients in various countries. As underlined by UNIAMO’s president, Italy is suffering some delays in the inclusion of rare diseases in the regional handbooks and is also facing global uncertainty due to the wait for the new EU’s pharmaceutical legislation.

In the interview, Scotti also raised the alarm regarding the future of advanced therapies in Europe, particularly after Bluebird Bio announced in October 2021 that it would withdraw its Beta Thalassaemia gene therapy from Europe shortly after its approval, due to difficulties in finding a financial settlement.

“Other companies are also at risk of withdrawing their marketing authorisation for commercial reasons,” Scotti noted.

He pointed out that right now the US Food and Drug Administration is approving gene therapies, including others from Bluebird Bio, and “so we are looking at a future where patients in the US will have access to drugs, to effective therapies, that will not be readily available to patients in Europe”.

Glossary

**Gene therapy** is a medical technique to correct genetic defects or treat diseases by using genetic material to improve the body’s ability to prevent, diagnose, or treat disease.

**Rare disease** is a term used to describe any disease that affects a small proportion of the population.

**EMA** is the European Medicines Agency, responsible for regulating medicines and medical devices in the European Economic Area.

**UNIAMO** is the Union of Italian Medicines and Medical Devices, representing stakeholders in the Italian pharmaceutical and medical devices sector.

**POCare** is a Greek Patient investment programme that focuses on Personalised Cellular & Gene Therapies.

**Stakeholder** is an individual or group that has an interest or concern in an issue or activity.

**Concordance** is the level of agreement between the expected value of a variable and the actual value observed.
the patient’s situation and available coverage by the public health system or private insurance.

He added that in Greece some of these therapies “are not always guaranteed covered by the National Health System”.

The Greek National Health System (NHS) provides medical care to patients with rare diseases and they can receive medical treatment through the system. However, in some cases, this treatment needs to be reimbursed by private insurance as the NHS cannot fully cover it.

Some patients resort to charities and foundations which provide financial support or enforce the development of gene therapies in Greece. One typical example is Marianna Vardinogianni Foundation, which established the First Cellular and Gene Therapy Center for children and adolescents in Greece.

Indeed, according to reports in Greek media, a new plan by the Ministries of Health and Finance, for a separate fund is being worked on, to which some wealthy Greeks could contribute with sponsorships, with special provisions to prevent the costs of gene therapies.

Yet, free access to gene therapies is still difficult to ensure for Greek patients.

According to the Chair of Rare Diseases Greece Athanasiou, there are only special early access programmes in which “the state can come to an agreement with the pharmaceutical company in order to give access in gene therapies to a small number of patients and for a limited period”.

However, the “details and prerequisites” for patients’ eligibility “may vary depending on patient’s disease and the treatment process the patient can undergo”, Athanasiou told Euractiv.

**Restrictions in therapy availability**

Another important issue regarding gene therapies is their limited availability.

“95% of rare diseases have no cure since, due to the complexity of the diseases and the limited number of patients, no specific treatments have been efficiently developed,” Athanasiou told Euractiv, referring to recently published data.

This can lead to further difficulties in accessing gene therapies, since patients may not have access to effective treatments and pharmaceutical products which save their lives.

In Greece, the problem is bigger due to the lack of appropriate infrastructure.

Athanasiou explained that the “small number of specialised laboratories and infrastructures for carrying out gene therapies” put additional constraints on gene therapy development as well as on patients’ access to them.

He added that while there has been an increased interest by the scientific community in Greece regarding these therapies in the last years, “there is still a lot of work to be done to ensure safe and universal access”.

Another issue Greek patients face when accessing gene therapies is delayed or incorrect diagnosis. Due to their scarce nature, rare conditions are difficult to diagnose.

Almost 50% of patients with rare diseases are undiagnosed or remain with incorrect diagnoses from five to 30 years, if not their entire lives, according to data derived from the Rare Disease Greece Association.

Therefore, access to gene therapies and treatments becomes more complicated for patients and any delays in treatment could lead to further deterioration of their condition and quality of life.

**Cooperation among Greek stakeholders is imperative**

Technology has made big advances during the last decade regarding gene therapies and rare disease treatments, however, cooperation among all EU member states and stakeholders is necessary to ensure patients’ access to therapies.

Athanasiou also highlighted the need to develop national access strategies with the cooperation of all stakeholders following the technology and scientific developments and new advances in gene therapies.

He added that while reimbursement systems for gene therapies and the timing of access to them, are improving as the benefits of gene therapies are increasingly being recognised, “Greece has not yet reached the general average of the European Union”.

According to him, it is important to encourage cooperation between stakeholders such as pharmaceutical companies, research institutions, scientists and patient organisations.

“Concerted efforts” are required to improve patient accessibility to gene therapies in Greece, Athanasiou said.

There is also “the need to develop policies that will provide, inter alia, for the adoption of a National Action Plan for Rare Diseases and the creation of specialized Reference Centres for Rare Diseases”, Athanasiou told Euractiv, stressing out the need for concrete and collective action.

“Almost 50% of patients with rare diseases are undiagnosed or remain with incorrect diagnoses from five to 30 years, if not their entire lives”

According to data derived from the Rare Disease Greece Association [Shutterstock / Andrey_Popov]
Rare disease patients across Spain don’t have the same access to treatments

By Fernando Heller | EuroEFE.EURACTIV.es

Access to quick and accurate diagnosis and affordable drugs in Spain varies across the regions and depending on patients’ economic status.

Ignacio Muela, 63, has suffered from Leber Hereditary Optic Neuropathy – a rare hereditary disease that causes a sudden loss of vision – since the age of 22. Those who suffer from this pathology usually have less than 10% vision, which is officially classified as blindness.

In Spain there are only 104 officially diagnosed cases of this disease, which has a prevalence of approximately 1 in 45,000 people, Muela, president of the Asociación de Atrofia del Nervio Óptico de Leber (ASANOL), told Euractiv’s partner EFE in an interview.

“At the moment, we have enormous difficulties in gaining access to a specific drug, Raxone, a potent antioxidant approved since 2015 by the European Medicines Agency (EMA) and by the Spanish Medicines Agency (AEM), but due to its high price and, according to the Ministry of Health, its low reliability, it is not financed by the Spanish public health system,” Muela said.

Unfortunately, this is not a unique case – of the 148 orphan medicines authorised in Europe, only 52% are currently accessible in Spain, Juan Carrión, president of the Spanish Federation of Rare Diseases (FEDER), told Euractiv’s partner EFE in an interview.

He said the situation was similar in other EU countries, as well as across Spanish regions.

“All of this has an impact on the expectations and life plans of both the patient and their family,” the FEDER president stressed.

Spanish rare disease patients have to wait on average over four years for a diagnosis, and once it is finally done, access to treatments is hampered.

“Diagnosis is the gateway to treatment. Therefore, it is necessary that once a diagnosis is made, access to available medicines and non-pharmacological therapies is made available,” Carrión pointed out.

He stressed the importance of ensuring quick access to treatments, given that the authorisation of a drug in Europe does not always translate to quick and affordable access in the member states.

“Although the authorisation of an orphan medicine is granted by the European Union (EMA), decisions on pricing and reimbursement are made at the national level, resulting in different prices in the member states,” Carrión said.

Additionally, as a result of the authorisation and marketing process, in Spain, “it usually takes almost three years for medicines to reach families in Spain after their approval in the EU”.

Addressing national policies

Among the main challenges in terms of health policy for boosting patient access to gene therapies, FEDER pointed to the public funding system.

“One of the main problems we have identified is that, in recent years, many express resolutions of non-funding [resoluciones expresas de no financiación] are issued by the Ministry of Health, which is usually the point at which many of the pricing processes for medicines with a high economic impact end,” Carrión warned.

In Carrión’s view, these resolutions are a major stumbling block to achieving the goal of improving access to gene therapies.

Regarding Spain’s position in terms of access to therapies for rare diseases at the EU level, Carrión recalled the different realities – and sometimes multiple speeds – that exist not only in Europe but also in Spain’s regions.

In Spain, a large part of the competencies in health policy is transferred by the central government to the 17 Autonomous Communities, which often leads to inequalities.

“Adding to the different realities of each European country, in Spain there is the added challenge that each Autonomous Community has a different policy on access to treatment (...) and the consequence is that access to drugs and therapies is conditioned by the postcode of the families who need them,” Carrión said.

“This is particularly evident in access to medicines in special situations. Equal access to medicines is also conditioned by territory, even by hospitals and specialists.”

Patients searching for alternatives

The long waiting times for access to drugs and treatments often lead patients’ families to opt for urgent measures.

“While families are waiting, the disease progresses and worsens [...]. Families cannot wait, so many are forced to take legal action to access treatment. We see this through our direct care services,” Carrión warned.

The alternatives, he said, include accessing medicines abroad, using off-label or even pharmacy master formulas [fórmulas magistrales], “which creates inequalities between some patients and others depending on where they live.”

In Spain, a large part of the competences in health policy are transferred to the central government to the 17 Autonomous Communities, which often leads to inequalities. [SHUTTERSTOCK/Shidlovski]
German researchers welcome national, EU support for gene therapy research

By Julia Dahm | Euractiv.de

National and EU-level support goes a long way towards supporting research into gene therapies against rare diseases, according to German researchers, but Europe still lags behind North American countries in innovation.

An estimated four million people in Germany suffer from rare diseases – a term used to refer to a group of more than 6,000 different diseases, with each one affecting less than five in 10,000 people.

The symptoms and course of different rare diseases vary widely, and due to the small number of patients and often complex clinical picture, many patients find themselves without an available cure.

But as most rare diseases are genetic, gene therapies are increasingly viewed as a potential silver lining for those affected.

“For rare genetic diseases, we often have the situation that there are none or almost no therapy options available – and this is where gene therapies could become a game changer,” Sarah Hedtrich, professor at Charité’s Berlin Institute of Health, told Euractiv.

Gene therapies are innovative treatments that address the genetic cause of a disease by replacing the malfunctioning gene, inactivating it, or introducing a new or modified gene into the body to help fight the disease.

“This way, you can not only effectively treat a patient, but potentially also heal them, because by re-introducing the healthy gene sequence, the cause of the disease is eliminated,” explained Hedtrich, whose current research focuses on treatments for serious rare diseases affecting patients’ skin or lungs.

Research funding on offer

Between the public funding available from different levels, Hedtrich feels there is generally sufficient support for academic research into gene therapies.

“All in all, I think that, in Germany, we cannot complain about the available support, also because the EU, for instance, offers funding opportunities for projects in rare diseases,” she said.

The German government funds research into diagnostic and therapeutic measures for rare diseases, according to the Health Ministry.

Since 2003, the Research Ministry also supports research networks on rare diseases with a total funding of up to €141 million earmarked for 2003 to 2025. Meanwhile, research on gene therapies can also receive support from other funding pools.

The EU has also addressed rare diseases via various pieces of legislation, including pharmaceutical legislation, which is currently being revised by co-legislators based on the Commission’s proposal or a directive on non-communicable diseases.

Once it comes to finding investors and getting a newly developed therapy onto the market, things can get a bit more complicated. Typically, developing a treatment for rare diseases can be expensive, while the business case is limited due to the small number of patients.

“I do think this is a problem,” Hedtrich said. “But it has been detected, and there is already a lot of support and funding.”

According to the researcher, one example is the so-called orphan drug designation, through which the EU grants special property rights for treatments against rare diseases, also known as orphan drugs, thus making their development more profitable.

More targeted, timely support

However, according to Hedtrich, more targeted government initiatives for gene therapies would help move the issue forward.

“In Germany, one is often in this ‘wait and see’ position, waiting to see what happens first, and thus clear, targeted programmes for gene therapies are missing,” she said.

In the researcher’s view, this results from a more hesitant approach displayed by many European countries when it comes to new scientific developments such as gene editing, leading to Europe lagging behind countries like the US or Canada.

“I think Germany risks falling behind,” she warned.

So far, new developments on gene therapies against rare diseases “mostly come from America,” Holger Lerche, medical director at Tubingen University Hospital, also told BR earlier this year, adding the challenge is now to bring the new therapies “from blueprints to the patient.”

“I do think that, over the next five to ten years, we will see many developments – there is such an enthusiasm and drive at the moment,” she said. “In ten years, we will be able to treat many diseases completely differently and better than we currently do – of this, I am sure.”

However, Hedtrich is optimistic that this development will proceed swiftly.
Gene therapy genetic modification of cells to produce a therapeutic effect. [Shutterstock/ luchschenF]