Successful Market Access for Gene Therapies

Strategic challenges and best practice

This is an Executive Summary of our White Paper „Successful Market Access for Gene Therapies“.
The full White Paper is available upon request.

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Gene therapies are confronted with various obstacles

The breakthrough of gene therapies and the associated challenges

Amongst several rare and some very serious, i.e. life-threatening diseases, there is an extremely high unmet need for innovative and effective treatment options. Within medical supply, standard therapies often focus on the reduction of disease-related symptoms through continuous medication. In contrast, gene therapies represent **potentially curative therapeutic options** that are applied only once. Particularly patients with hereditary diseases will increasingly benefit from these innovative methods in the future. The number of gene therapy products in pre-clinical and clinical phases has doubled in recent years. Individual therapeutic areas, such as oncology (cancer therapy) or the treatment of **rare (monogenetic) diseases**, which are currently preferably evaluated in (pre)clinical trials, have emerged. The wheel of innovation is constantly spinning, but there are currently no homogenous international regulations determining the use and reimbursement of gene therapy products.

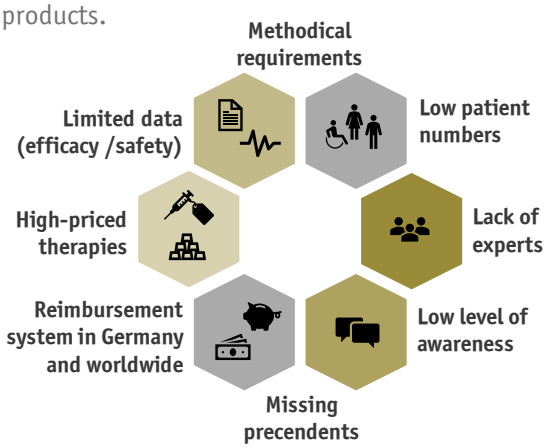

Figure 1: Challenges in the approval and benefit assessment process for gene therapies

The biggest **challenges for market access** are the available evidence and an **adequate reimbursement system** of these innovative one-time treatments. The often limited level of evidence, low patient numbers in the respective target population and the lack of long-term data on efficacy and safety, compromise the process of assessing the therapeutic benefit and the associated value of the therapy. In addition to these specific and other core obstacles that are summarized in Fig. 1, the **affordability** and the still vague **reimbursement of gene therapeutic applications** are controversially discussed at the moment.

Regulatory environment of gene therapies

Regulatory aspects (FDA, EMA, G-BA) with regard to highly innovative gene therapies

Various authorities that are in charge of the approval and market access process, such as the **FDA** in the USA and the **EMA** in Europe, as well as the **Joint Federal Committee** (G-BA) in Germany, have progressively started discussions addressing potentially new requirements in terms od the clinical study design of gene therapies in order to identify possible gaps and to adjust already existing guidelines.

FDA U.S. FOOD & DRUG ADMINISTRATION

The FDA has already published specific **guidelines for industry and stakeholders** involved in the production, planning, application and long-term follow-up of gene therapies in (pre-) clinical trials, for example. In Europe, similar actions have taken place.

EUROPEAN MEDICINES AGENCY
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In order to meet the new requirements of the approval process for gene therapies, the EMA also provides **guidelines** which pharmaceutical companies can follow in order to minimize the prevailing risks of gene therapy applications. The manufacturing, handling, application and clinical follow-up of the study participants also play an important role and are addressed within the risk management of the individual therapy.

Both, the FDA and EMA, are in **contact with relevant stakeholders** of the health care system with the aim of addressing existing challenges as early as possible.

Gemeinsamer Bundesausschuss

In **Germany**, pharmaceutical companies are obliged to undergo a **benefit assessment of the respective gene therapy** in the course of the AMNOG procedure, which is followed by price negotiations with the umbrella organization of statutory health insurance funds (GKV-SV).

Approved gene therapies allow a look into the future and potential opportunities and risks can be derived

Gene therapies on the market

The gene therapies approved so far show that pharmaceutical companies are currently testing the **opportunities of gene therapies** on the market. Between 2012 and 2018, a total of five single-use gene therapies were approved by the **FDA** or **EMA** for the treatment of rare genetic diseases. In addition to **Glybera®, Kymriah®, Yescarta™, Strimvelis®** and **Luxturna™,** further gene therapies are expected to be available on the international markets in the coming years.

The previously valid **therapy costs** of the respective one-time gene therapy application are illustrated in Fig. 2. Overall, it can be said that the first **key learnings** can already be derived from the precedents, but the system of gene therapies is still in its infancy, both with regard to **regulatory aspects** and with regard to **adequate reimbursement**. The example of Glybera® shows that the initial approval is only the first step but various other aspects are also essential along the road to a regular **reimbursement.**

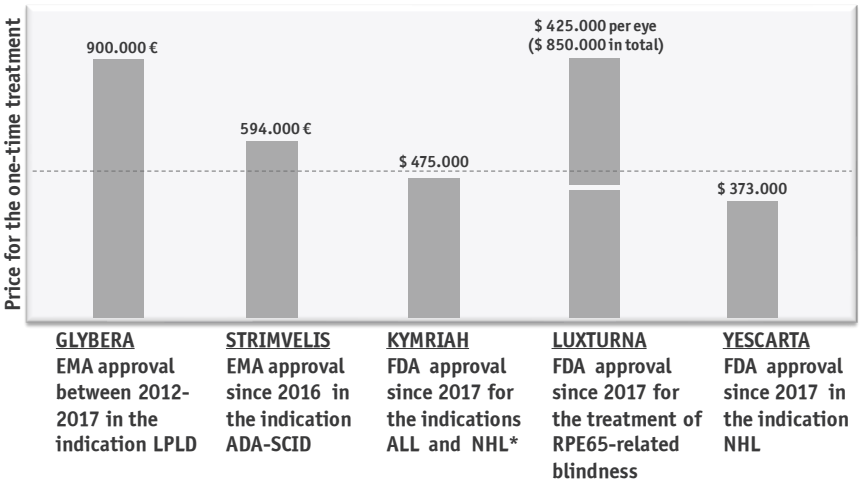

Figure 2: Overview of previously approved gene therapies with regard to their approval backlog and current reimbursement amounts.
* second indication (unpriced)

Potential reimbursement models can be mapped according to the extent of risk sharing and the type or level of evidence

Reimbursement dilemma in the field of gene therapies

One of the **main challenges** in the field of gene therapy is the question of an **adequate reimbursement**. Existing systems are increasingly reaching their limits and do not do justice to the reimbursement dilemma inherent in gene therapy. For this reason, **potential solutions** are presented and discussed. The strategic options for **innovative reimbursement models** for gene therapies are immense.
Hereinafter, **five archetypes** are shown, on the basis of which the potential possibilities can be represented in differentiated ways. The **status quo** is defined by the **upfront payment**. Pricing is based on the evidence available for approval; further evidence does not have to be collected, which means that the extent of risk sharing is considered low, as long-term efficacy and safety can only be assessed hypothetically.

Installment payment plans, which are not necessarily based on further evidence, can be similarly estimated. In case of **post-launch evidence**, however, the extent of risk sharing between the payers and the pharmaceutical companies increases, which can be seen in **individual and collective outcome-based approaches**. Despite a low level of post-launch evidence, a high degree of risk sharing on the social level can only be achieved by **collectivizing the effort** involved.

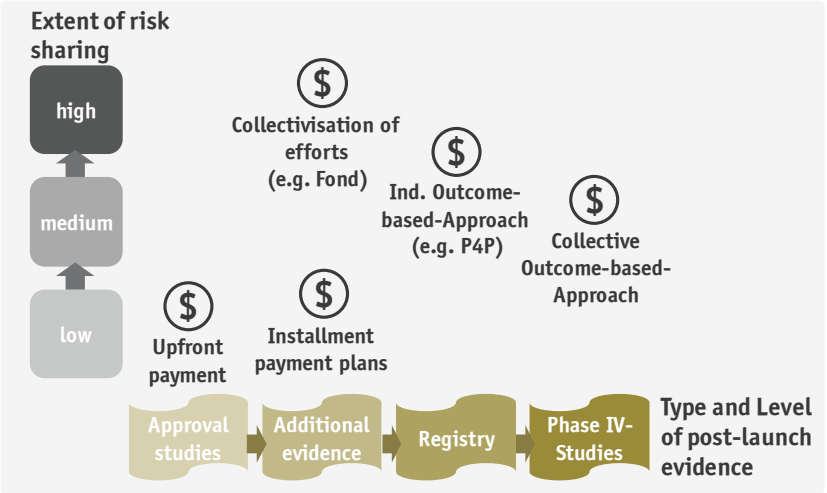

Figure 3: Overview of potential reimbursement models

SKC – Key Learnings

The VUCA of the gene therapy field requires a sound and comprehensive strategy for successful market access

Based on the analyses, specific aspects have emerged which are of decisive importance for successfully mastering the approval and reimbursement process:

- The role of the drug in the **reality of medical care** should be evaluated;
- **Follow-up** data should be collected to address the uncertainty aspect;
- The **target population** should be determined taking into account patients who are suitable in principle and in fact;
- A **differentiated benefit assessment**, taking into account the payers' view and on the basis of the target population, should take place;
- The **manufacturing** of gene therapy options can have a decisive influence on the market uptake;
- Application-specific aspects and thus the development of **training courses** should be integrated into the process at an early stage;
- With an **international overall strategy**, country-specific peculiarities can be addressed and used to one's own advantage;
- **Mobilizing the market** is a key factor in the introduction of new gene therapies;
- The overall integration of the **value story** is a decisive success factor.

Overall, a **collaborative approach** by all parties involved is of central importance to provide all patients with access to innovative gene therapies.

VUCA = volatility, uncertainty, complexity, ambiguity

SKC – Partners of trust

Vision and Mission

We at SKC think that every activity in the health care industry should focus on the improvement of the patient's situation.

We are convinced that the best solution for a health care problem can only be found in a competitive landscape, shaped by innovation and empowered by trust.

We believe a better, more effective, efficient and fair health care system is possible due to digital technologies, interdisciplinary networks and highest professional standards.

Pharmaceutical Industry
- Stakeholder and Public Affairs Management
- Market Access, AMNOG and Reimbursement Strategies
- Strategic Pricing

MedTech & Biotechnology
- Market Access for Innovative Medical Devices (National / International)
- Portfolio Analysis & Management

Statutory Health Insurance
- Digital Agenda Setting
- Health Care Management
- Strategic Organization Development

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