Personalized Medicine

What is the price of a human life? An economic view at personalized medicine

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Until the pandemic, most people had never heard of mRNA vaccines. Today, they are widely expected to hold the key to curing life-threatening diseases such as cancer - unfortunately at a potentially prohibitive price.

Pfizer-BioNTech's and Moderna's Covid-19 vaccines have saved millions of lives and impressively demonstrated that mRNA vaccines work, are well tolerated, and can be produced quickly and flexibly. And the potential of this new technology is far from exhausted. Its real strength lies in the fact that the vaccine, i.e., the sequence of the mRNA, can be individually customized.

Cancer cells, for example, are different in every patient. The mRNA therapy is intended to tailor each vaccine to the characteristics of the specific tumor. To do this, tumor cells are taken from the patient and analyzed using gene sequencing. Once the cancer cells have been decoded, a patient-specific vaccine is produced that will enable the immune system to recognize and fight the tumor. In this process, the "foreign" structure that the immune system is supposed to recognize is no longer produced in the laboratory, but by the patient's own body using the inoculated mRNA as blueprint.

The potentials inherent in mRNA therapies go far beyond cancer treatment

The strategy of using mRNA to tell the body what to do holds even greater potential. From a scientific point of view, it can be assumed that mRNA therapies can be used in the medium and long term not only for most types of cancer, but also for HIV, diabetes and many other life-threatening diseases. Dr. Ugur Sahin, CEO of BionTech, predicts that in 15 years, one in three newly approved drugs will be based on mRNA technology.[1)]

We conducted our own study to estimate the expected development of personalized medicine for mRNA and pDNA drugs over the next years. Based on current clinical trials[2)], the statistical probabilities of moving from one clinical phase to the next and eventually to approval[3)], the entry of new drugs into phase 1[4)], and the average duration of clinical phases[3)], we consider the following new approvals of personalized mRNA and pDNA drugs in the field of oncology to be achievable:

Year	2025	2026	2027	Total
Expected new approvals	1	3	6	10

Currently, drugs for 25 different cancer types are in clinical trials[2], each of these indications affects ø 640,000 new patients worldwide annually[5]. If we assume that the 10 new drugs, we predict shall be approved by 2027 will affect 5 different indications, the expected patient populations can be calculated as follows:

Year	2025	2026	2027	Total
Expected new patients	320.000	960.000	1.920.000	3.200.000

So, millions of patients could be helped. But there is still one major hurdle to overcome: the significant cost of personalized therapies.

How much may a therapy cost?

While no personalized mRNA drug has yet been approved, the costs of other personalized treatments are well known. In 2019 and 2020, about 300 patients were treated with new CAR-T cell therapies in Germany within 12 months - at a cost of about 100 million euros.[6] A November 2020 study extrapolates the total financial burden on the healthcare systems of Germany, France, the United Kingdom, Italy, Spain, and the Netherlands for CAR-T cell therapies to 32.9 billion euros by 2029 - for the treatment of just over 100,000 patients.[7] It seems plausible that future mRNA therapies will cost about as much as current CAR-T cell therapies, especially since person-specific diagnostics will have to also be made before any treatment.

The U.K.'s National Institute for Health and Care Excellence (NICE) currently uses a cost-effectiveness threshold in the range of £20,000 to £30,000 per quality-adjusted life year (QALY) to evaluate new drugs for the National Health Service (NHS). Thus, the new personalized drugs would have to increase a patient's life expectancy by 9 to 14 years to be reimbursable at the assumed price. Whether this is achievable can be doubted. For example, a study by the Kite company on the effect of the approved CAR-T drug Yescarta® in patients with refractory large B-cell lymphoma showed a median survival rate of just over 2 years.[8] Every therapy with Yescarta® costs € 327,000. What healthcare system can - and will - afford this?

If costs cannot be reduced, the new therapies will only be accessible to the few

The Covid 19 pandemic has already shown that sufficient healthcare is only available to a privileged part of humanity. Unless costs can be reduced quite substantially, personalized medicine will at best be deployed in countries with per capita incomes above US$40,000[9] - these are countries in North America, Western and Central Europe,

Australia, New Zealand, and the wealthy nations of Southeast Asia and the Middle East. The table shows the number of patients treated in these countries:

Year	2025	2026	2027	Total
Expected eligible new patients	45.000	135.000	270.000	450.000

In this scenario less than 15% of all global patients will be treated and personalized medicine will not be available to the vast majority of humanity in China, India, Africa and Latin America.

But even wealthy countries will quickly reach the limits of what their healthcare systems are capable of affording. Let's take Germany as an example. Around 520,000 people are currently diagnosed with cancer each year[10]. If in the future only a quarter of all patients will have access to personalized mRNA therapies, this will lead to annual expenditures of €43 billion at today's costs. This is roughly equivalent to the amount spent by public health insurers on *all* drugs in 2020.

Do these numbers mean the end for the new therapeutic approaches?

We think the following steps are necessary to reduce the expected costs through economies of scale effects and shortening of time-to-market:

- Establishment of a platform approach for mRNA and pDNA therapies
- Combination of patient-specific therapeutic approaches with those suitable for larger patient groups with identical tumor markers
- Optimized supply chain strategies based on accurate make-or-buy analyses
- Integration of specialized CDMOs as centers of excellence for process development and manufacturing
- Standardization of production technology for API manufacturing as well as fill/finish

In addition, we could certainly also ask ourselves whether all treatments are useful, that might extend life by a few months, often only at the price of significant side effects.

Sources

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