Cell-& Gene Therapy

Is payers' fear of the budgetary implications justified?

This newsletter deals with the question of whether the fear of health insurance companies of the cumulative budget effect of cell and gene therapies (CGTs) is justified or not.

Background

The payer is more concerned with the cumulative budget effect than with the often very high costs of a single CGT, which are manageable [1]. As a result, payers view affordability as key issue [2], [3], [4]. On the other hand, CGTs sales in 2026 are estimated at $ 59 B [5], which is around 4% of the expected global prescription market of $ 1'408 B[i]. The key question, therefore, is whether CGTs sales will remain in the low single-digit market share of pharmaceutical sales or cause the pharmaceutical market to explode. The number of American patients receiving CGT is expected to more than double from 2026 to 2030, indicating significant growth [6]. More than 2,500 clinical trials are currently underway, of which 250 are in Phase III [7]. The FDA expects 10-20 CGTs annually to receive market approval by 2025.[ii]

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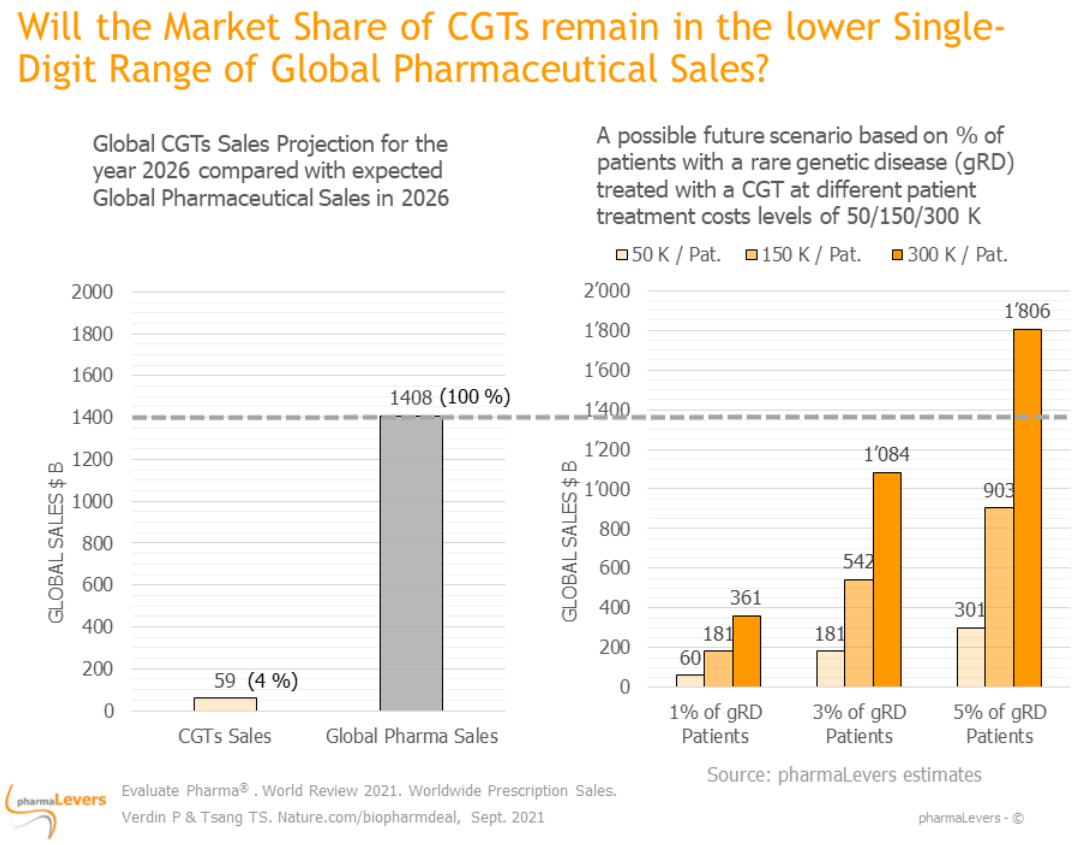
CGTs Market Estimates



For 2026, Evaluate Pharma® estimates the global market for pharmaceutical prescriptions at $1,408 billion and CGTs at $59 billion (about 4%). To remain in the single-digit market share range (≤ $ 140 B), the number of patients with genetic rare diseases (gRD) treated with CGTs should not exceed 1% in the future. At 3%, CGTs will account for a significant portion of pharmaceutical sales; at 5%, future CGT sales could be higher than the entire pharmaceutical market in 2026.

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Discussion & Consequences

We don't know if CGTs will shift the pharmaceutical market, as the transition from synthetic chemistry to biotechnology has done. Payers' concerns about the potential cost consequences are justified based on the calculations and estimates made. The number and speed of CGTs available to patients determines possible health interventions. At present, careful and cautious economic assessment is appropriate, even if the cost-effectiveness ratios may be favorable. For the pricing of new CGTs, this means that the benefits for patients must be demonstrated even more strongly and the medical and economic risks for payers must be further reduced through specific managed entry agreements.

> ***"At present, careful and cautious economic assessment is appropriate, even if the cost-effectiveness ratios may be favorable"***

How the calculation was performed

There are 36 million people with a rare disease in Europe, with 80% having a genetic cause behind it[iii]. The number of people with a genetic rare disease (gRD) is thus estimated at 28.8 million. pharmaLevers intuitively estimates that a CGT will be available for 1-5% of this population in the future. To determine the costs, the number of these patients (1%, 3% and 5%) was multiplied by three different treatment cost levels per patient ($ 50K, $ 150K and $ 300K). The price levels used are at reduced levels of the two price categories "Decent" and "Challenging" (see price category below). To carry out a comparison with the global sales data, the above European market estimates were extrapolated to the global level based on the global market share of the European pharmaceutical industry[iv]. The growth of the pharmaceutical market beyond 2026 was not considered.

pharmaLevers recognizes that CGTs, even at high prices, can provide a solid investment for healthcare. However, favorable cost-effectiveness (below ICER thresholds) leads to continuously rising costs and affordability problems in the long term. The high upfront costs of CGTs reinforce this trend. For this reason, it is appropriate to consider only the cost of the product to answer the key question of this newsletter.

Product Price Category cf. [8], [9]

1. **Decent**: $ 25K - < $ 100K, e.g., Imlygic®, Alofisel®
2. **Challenging**: $ 300K – $ 500K, e.g., CAR-T as Kymriah®, Yescarta®
3. **Sky rocketing**: > $ 2 Mio e.g., Zolgensma®, Zynteglo®

Limitation

The aim of this newsletter is to roughly estimate whether the cost consequences of cell and gene therapies are really becoming a central problem. There are many factors that can influence the estimation on both sides. The main message that CGTs can trigger a considerable cost consequence in the future remains highly likely. To be able to conclusively assess this question, these approaches would have to be reviewed in a scientific paper. Completeness and correctness are not claimed; Additions, corrections and comments are welcome.

A Video on **YouTube** is available:

https://www.youtube.com/watch?v=Sb8HpJkeruM

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[i] https://info.evaluate.com/rs/607-YGS-364/images/WorldPreviewReport_Final_2021.pdf

[ii] https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-and-peter-marks-md-phd-director-center-biologics

[iii] https://health.ec.europa.eu/non-communicable-diseases/steering-group/rare-diseases_en

[iv] https://www.efpia.eu/media/602709/the-pharmaceutical-industry-in-figures-2021.pdf