The premise of gene therapy is simple: a single treatment to cure an otherwise lifelong serious disease. However, while this powerful concept is being validated in human clinical trials, a new challenge has now arrived at center stage: what will such therapy cost, and how much are health-care providers and insurance companies willing and able to pay?

This question comes under the spotlight following approval of Glybera—adeno-associated virus type 1 (AAV1)-based gene medicine for the treatment of lipoprotein lipase deficiency—for clinical use in the European Union in November 2012. A Dutch company, UniQure, and its Italian marketing partner, Chiesi, have filed a pricing dossier with the German Federal Joint Committee that recommends a retail price of €53,000 per vial, bringing the total cost of 21 vials needed for the treatment of one patient to €1.1 million ($US1.4 million), setting a new record for a medicine to treat a rare disease. With only 150–200 patients eligible for Glybera treatment across Europe, and similar numbers in the United States, the impact on overall health-care budgets will be small. However, the pricing of Glybera will set a precedent for subsequent gene therapies for rare diseases that are currently under development and is therefore of great importance to the field, health-care providers and regulators, and companies developing gene therapy products.

High price tags for specific treatments are not new to health-care providers. The previous record for an orphan drug was held by Soliris (eculizumab), with a price of ~$540,000. A liver transplant can cost $250,000–300,000; if complications occur, it can be much higher. Enzyme-replacement therapies (ERTs) for rare diseases can easily cost $50,000/year, and a full treatment schedule with the new hepatitis C drug Sovaldi costs ~$85,000. Clotting-factor treatment of hemophilia can cost up to $100,000/year, depending on the frequency and severity of the bleeding episodes. UniQure claims that the $1.4 million cost of a single treatment is justified because it provides a long-term sustainable cure that generates significant lifetime savings for health-care providers.

For ERTs, revenues come from long-term repeated administration of the drug, and the same is true for many new monoclonal antibody therapies. However, a price tag higher than $1 million will probably spark vigorous debate among health-care providers and the public given that in many cases the R&D costs have already been covered in large part by public research funds. It will also be too easy to ask the question “Do you want to spend over $1 million on improving the condition of one patient with Glybera versus 10–20 women with advanced breast cancer or 200 patients who require a hip replacement?” Indeed, it is likely that some government agencies will step in and demand a compromise. On the other hand, the biotech and pharmaceutical industries require significant investment toward R&D of new products and thus require an income stream sufficient to continue their operations and provide returns to their investors.

A single payment at the time of treatment would be the simplest solution for a company producing the therapy. However, alternative funding models have been suggested whereby the annual payments could be made over a defined period of time based on evidence that the treatment is effective. This approach might provide a more stable, albeit much slower, revenue stream to companies focusing on developing novel treatments for rare diseases. However, such an approach could create complications, for example, if a patient's insurance coverage or health-care provider changes following initiation of the treatment.

A rational approach to pricing would be to determine the cost-effectiveness of the therapy. Although cost is an issue, much emphasis has recently been placed on the improvement of quality of life as well, which is difficult to measure in monetary terms. Therefore, the case of Glybera’s pricing and the policy for reimbursement is of
great importance and will clearly become a watershed in the long road of bringing gene therapy to the clinics. German authorities are expected to rule on Glybera’s pricing in April 2015, and further negotiations are likely to follow. UniQure will seek approval for Glybera in the United States, but it will probably be two to three years before a decision is made. Therefore, thoughtful discussion among all stakeholders will be required to bring this issue to a fruitful outcome so as to facilitate bringing novel treatments to patients with rare diseases while maintaining the ability of researchers and biotech companies to develop new treatments and making the pricing justifiable to health-care providers and society at large.

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REFERENCES