MedicalXpress

Researchers urge new payment model for gene therapy

September 9 2014

Hoping to encourage sufficient investments by pharmaceutical companies in expensive gene therapies, which often consist of a single treatment, a Penn researcher and the chief medical officer of CVS Health outline an alternative payment model in this month's issue of *Nature Biotechnology*. They suggest annuity payments over a defined period of time and contingent on evidence that the treatment remains effective. The approach would replace the current practice of single, usually large, at-point-of-service payments.

"Unlike most rare disease treatments that can continue for decades, gene therapy is frequently administered only once, providing many years, even a lifetime, of benefit," says James M. Wilson, MD, PhD, professor of Pathology and Laboratory Medicine, Perelman School of Medicine at the University of Pennsylvania. "Under current reimbursement policies, private insurers and the government typically pay for this therapy once: when it is administered. But these individual payments could reach several million dollars each under current market conditions. We're proposing a different approach that spreads payments out and only keep coming if the patient continues to do well."

Wilson and co-author Troyen A. Brennan, MD, JD, MPH, chief medical officer of CVS Health, note that while large single payments for gene therapy may be the simplest approach, they carry substantial encumbrances. For example, approval of gene therapy treatments is unavoidably based on data derived from trials carried out over several years at most—considerably shorter than the expected duration of the

therapy. Payers may therefore be unwilling to pay large up-front sums for treatments whose long-term benefit has not been established. Additionally, large payments for medications, such as the $84,000-a-patient cost of the hepatitis C treatment Sovaldi, have been criticized in the prevailing climate of curbing health care costs. This, despite the fact that effective gene therapy may reduce the overall financial burden to the health care system.

Wilson and Brennan further note that while a liver transplant, for example, can cost up to $300,000, physicians and hospitals that "transplant livers know they will be compensated at market rates through existing contracts—gene developers lack that assurance." Annuity payments, they say, could help address these problems.

An example of an annuity-type disbursement could be a hypothetical payment of $150,000 per year for a certain number of years for gene-therapy-based protein replacement for patients with hemophilia B—so long as the therapy continues to work. According to the authors, the cumulative amount should be less than the cost of a one-time payment of $4-6 million, which would be the expected rate for a gene-based therapy to be comparatively priced to existing, conventional therapies for hemophilia B. "One would presume," they write, "that gene therapy will have to represent a discount in order for insurers to approve its use."

"The annuity model that we're proposing would eliminate the misguided incentive to invest in drugs and treatments with ongoing revenue streams but which require continuing, perhaps lifetime daily administration, with all the attendant inconveniences and burdens to patients and their families, as well as direct and indirect costs to the nation's health system," says Wilson.

The authors point out that gene therapy differs substantially from the case of "orphan" drugs. Development of the latter, which target rare

diseases affecting small patient populations, is supported by the Orphan Drug Act of 1983, which provides pharmaceutical manufacturers with grants, tax credits, and an extended period of market exclusivity for their medications. What's more, in virtually all of these cases, the business costs of developing the drugs are further attenuated by ongoing administration of—and payment for—the medication over the lifetime of the patient. "The contrast with gene therapy, especially that which produces a durable cure with one administration," the authors write, "is clear."

Adding further details to their proposal, the authors write that "The original annuity payment could … be set with certain types of 're-opener' clauses, such as with patent expiration [death], or if a less expensive new therapy came on line—thus subjecting the gene therapy annuity to the same vagaries of market competition that standard pharmaceuticals face."

A crucial issue would be the calculation of the annual annuity payment. One option would be for the government to set the price through the Medicare program, since many of the patients with rare diseases are disabled and thus qualify for Medicare. The Medicare rate could in turn become a benchmark for the commercial market.

Another key test in developing an annuity model is determining the correct linkage between payments and the therapy's continued effectiveness and safety. In most diseases, this would entail identifying a biomarker reasonably correlated with efficacy, for example, plasma measures of clotting in hemophilia patients treated with gene therapy.

Further provisos could be added. For example, a gene-based treatment for high triglycerides that showed a reduction in germane biomarkers but no reduction in the frequency or cost of hospitalization tied to the condition might not be satisfactory for payers.

"Gene therapy offers enormous potential for helping very sick patients," says Wilson. "But we have to keep in mind that the economic factors associated with these interventions are significant on both sides of the equation. Annuity payments represent a way of ensuring that non-medical factors do not impede access to assistance for those desperately in need of these treatments."

Provided by University of Pennsylvania School of Medicine