

Editorial

Will personalized medicine be a driver for widespread price controls?

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PERSONALIZED MEDICINE—PRESCRIPTION OF drugs most likely to benefit and least likely to harm individual or groups of patients—promises welcome positive changes to healthcare. It may, however, also have negative sequelae originating from incompatibilities with the current healthcare delivery system and the need for regulatory and policy changes to accommodate personalized medicine.

Personalized medicine is the delivery of medical treatments to individuals or groups based on their susceptibility to disease or response to a treatment. Through the use of genomic and other biomarker technologies, personalized medicine holds the potential to identify which subsets of patients are most likely to benefit from a treatment and also which patients may be susceptible to certain side-effects. A majority of drugs are effective only in a small proportion of people who take them. Unfortunately, it is difficult to determine in advance which patients will respond positively, so many patients are simply prescribed potentially-effective drugs in sequence until a suitable drug emerges. This means that resources are wasted in prescribing ineffective drugs, while patients may see their disease progress unchecked and may also experience unnecessary side-effects from the ineffective drugs. Personalized medicine has the potential to reduce this waste and to speed appropriate drugs to patients, while reducing the prevalence of unnecessary side-effects.

A potential downside of the increased use of personalized medicine is that the regulatory system and healthcare policies may not be properly calibrated to accommodate it. For example, the lack of advance knowledge of which drugs may be most effective in a patient creates competition among branded drugs, in advance of generic entry. Consider the cases of top-selling biologics Enbrel, Humira, and Remicade. The three drugs, each of which has worldwide sales in excess of \$6 billion, cover overlapping indications. Physicians, payers, and patients, and other prescription-decision influencers may consider price in deciding which of these drugs to first prescribe for a given indication, increasing price elasticity and keeping prices in check. Because personalized medicine holds the potential to improve knowledge of which drugs may be most-effective and least detrimental for a

subset of patients, it holds the potential to create mini-monopolies, decreasing price elasticity, and indirectly facilitating higher drug prices.

Would patients, payers, and society in general gladly pay higher prices for a more streamlined prescription system with increased drug effectiveness and advanced knowledge to avoid some side-effects? Potentially, but this is where the conflict with current regulatory and other policies comes into play.

Well before modern advanced biomarkers and targeted therapies such as Herceptin and Gleevec were developed there was another class of personalized medicines—the treatments for orphan diseases. These diseases are defined by the FDA those affecting fewer than 200,000 people in the U.S., or which affect more than 200,000 persons but not are not expected to recover the costs of developing and marketing a treatment drug. The FDA provides developers of orphan drugs with seven years of market exclusivity—independent of patents—and tax credits.

Drugs for orphan diseases are essentially personalized medicines: they target a small group of patients for whom other drugs are ineffective. Despite the small populations served, orphan drugs can be very profitable. Companies like Genzyme have built their enterprises on these drugs. Genzyme has earned billions of dollars selling orphan drugs, which prices as high as \$300,000 per patient per year. They justify their high prices in three ways. Firstly, the high prices are necessary to allow them to recoup R&D investments with a relatively low sales volume (due to the small populations served). Second, the small populations means that the high prices have a relatively small impact on health payer budgets. Finally, Genzyme provides the drug for free to those without insurance or whose payers are unwilling to pay.

The orphan drug program is a valuable one, as it promotes the development for diseases that might otherwise not merit interest by biopharmaceutical developers. Genzyme's pricing system is also rational, rewarding the company for its risky R&D investments while ensuring that needy patients are not deprived access to medicines for lack of financial resources.

A conflict arises when personalized medicine enables relatively prevalent diseases to be divided into subsets of individual orphan diseases, or when personalized medicine provides sufficiently reliable predictions of drug efficacy in subsets of patients that it creates a niche-monopolies.

In the first case, where a relatively prevalent disease is divided into individual orphan diseases, the potential exists for the seven-year marketing exclusivity and tax credits to be granted for drugs that do not technically meet the orphan criteria. This unintended use of orphan drug designations could lead to higher prices for these drugs without merit. The second case, creation of a niche-monopoly by removing uncertainty regarding which of a group of similar medicines is most likely to work in a patient subpopulation, could also see drug prices rise as price elasticity decreases.

Drug pricing is a growing concern among patients, payers and policy makers (it is worth noting that drug expenditures are only a small portion of healthcare expenditures, and that drugs frequently save money by preventing/postponing the need for more expensive interventions). While personalized medicine offers many benefits to patients and other stakeholders, it could also drive the implementation of widespread price controls, a policy change not welcomed by many. As more personalized medicines are developed, the potential exists for an expansion in the number of high-priced drugs. Regardless of whether these high-priced drugs actually have a significant impact on payer budgets or simply serve as fodder for special interests, they could fuel a backlash and strengthen calls for U.S. price controls. The impact would almost certainly extend beyond personalized medicines, impacting the industry as a whole. So, it is worth examining if the current regulatory and policy structure merits amendment to accommodate personalized medicine.

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