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A New Drug Class With Important Cost Implications

The U.S. Food and Drug Administration (FDA) has approved two new drugs to treat patients with high cholesterol. These new therapies belong to a class of drugs known as PCSK9 inhibitors and have the potential to reduce the risk of heart attack and stroke for millions of Americans. However, the prices of these drugs—which are administered by injection every two to four weeks—have been set at $14,600 and $14,100 annually per patient.[1]

Current treatment for high cholesterol

High cholesterol—which affects approximately 73.5 million Americans[2]—causes plaque to develop in arteries and results in the narrowing of a person's blood vessels. The plaque buildup and narrowed blood vessels can stop blood flow and cause a heart attack or stroke.

Statins are traditionally prescribed to treat patients with high cholesterol.[3] These drugs work by slowing the production of cholesterol in the liver and have been shown to prevent heart attack and stroke in patients with[4] and without a history of cardiovascular disease.[5] More than 30 million Americans[6] take a statin pill daily, costing as little as $4 per month.[7]

Issues associated with statin therapy

Research indicates that some patients do not benefit from therapy with statins and that many fail to take their medication as prescribed. Some patients can also experience adverse effects such as pain, muscle aches, and weakness. In fact, one study showed that 57,292 of 107,835 patients (53.1 percent) in a routine care setting discontinued statin therapy, if only temporarily, for at least 12 months.[8]

Furthermore, it is estimated that more than 600,000 people in the United States[9] have a genetic condition known as familial hypercholesterolemia (FH), which puts them at significantly higher risk for premature death from a cardiovascular event.[10] Not all patients with FH respond well to statins.

New therapies on the market

The new PCSK9 inhibitors have the potential to benefit both of these patient populations. PCSK9 inhibitors have been shown to lower high cholesterol in FH patients and in those who cannot tolerate or do not benefit from statin therapy.[11]

Researchers continue to investigate the long-term effects of PCSK9 inhibitors on specific patient populations, because their effect may vary across patient groups. Given concerns over balancing the benefits with any side effects, these drugs have been approved only for select patient groups that are most likely to benefit from them, such as people with FH. However, because of their set price—$14,600 and $14,100 per patient per year—payers are likely to closely monitor, if not constrain, both on- and off-label uses (i.e., prescribing the drug for FDA-approved uses or nonapproved uses).

The challenge for payers

With these new therapies on the market, health care payers, patients, and providers must answer a critical question: How much should we be willing to pay for drugs that have the potential to prevent a heart attack or stroke?

Answering this question requires an examination of the PCSK9 inhibitors' value—that is, how effective these treatments are in preventing heart attack and stroke relative to their associated costs.

The long-term effects of PCSK9 inhibitor drugs remain under investigation, but preliminary data indicate that treatment with one of them can decrease a patient's risk of a cardiovascular event, such as heart attack or stroke, from 2.18 percent to 0.95, a 1.23-percentage-point decrease over a year.[12] The number needed to treat (a metric of the number of patients who must be treated to prevent one adverse event) is 81. Assuming an annual price of $10,000 per patient, the total cost of medications to avoid such an event with the drug would be $810,000 for one year.

The effects of these drugs on health outcomes in clinical practice over time, including on- and off-label indications, have yet to be determined. Given the information currently available, it is unclear how stakeholders will value PCSK9 inhibitors, but health care payers will soon be required to develop coverage and payment policies for these new therapies.

The long-term effects of PCSK9 inhibitor drugs are still under investigation.

Endnotes

1 Sanofi, "Sanofi and Regeneron Announce FDA Approval of Praluent (Alirocumab) Injection, the First PCSK9 Inhibitor in the U.S., for the Treatment of High LDL Cholesterol in Adult Patients," news release, accessed July 24, 2015, http://www.news.sanofi.us/2015-07-24-Sanofi-and-Regeneron-Announce-FDA-Approval-of-Praluent-alirocumab-Injection-the-First-PCSK9-Inhibitor-in-the-U-S-for-the-Treatment-of-High-LDL-Cholesterol-in-Adult-Patients; Amgen, "FDA Approves Amgen's New Cholesterol-Lowering Medication Repatha," news release, accessed Aug. 27, 2015, http://www.amgen.com/media/media_pr_detail.jsp?releaseID=2082837.

2 Centers for Disease Control and Prevention, "High Cholesterol Facts," last modified March 17, 2015, http://www.cdc.gov/cholesterol/facts.htm.

3 Neil J. Stone, et al., "Treatment of Blood Cholesterol to Reduce Atherosclerotic Cardiovascular Disease Risk in Adults: Synopsis of the 2013 American College of Cardiology/American Heart Association Cholesterol Guideline," *Annals of Internal Medicine* 160, no. 5 (2014): 339–43, http://annals.org/article.aspx?articleid=1818923.

4 Li Wei et al., "Statin Use in the Secondary Prevention of Coronary Heart Disease in Primary Care: Cohort Study and Comparison of Inclusion and Outcome With Patients in Randomised Trials," *The BMJ* 330 (2005), doi: http://dx.doi.org/10.1136/bmj.38398.408032.8F.

5 Fiona Taylor et al., "Statins for the Primary Prevention of Cardiovascular Disease," *Cochrane Database of Systematic Reviews*, Issue 1, Article CD004816, http://www.ncbi.nlm.nih.gov/pubmed/23440795.

6 Fiona Taylor et al., "Statin Therapy for Primary Prevention of Cardiovascular Disease," *Journal of the American Medical Association* 310, no. 22 (2013), http://jama.jamanetwork.com/article.aspx?articleid=1785551.

7 *Consumer Reports*, "Are You Taking the Right Treatment for Your Cholesterol?" (2014), http://www.consumerreports.org/cro/2014/03/treating-high-cholesterol-with-statins/index.htm.

8 Huabing Zhang et al., "Discontinuation of Statins in Routine Care Settings: A Cohort Study," *Annals of Internal Medicine* 158, no. 7 (2013): 526–534, http://www.ncbi.nlm.nih.gov/pubmed/23546564.

9 Estimate obtained from multiplying the assumed U.S. population of 300 million by the FH prevalence obtained from Børge G. Nordestgaard et al., "Familial Hypercholesterolaemia Is Underdiagnosed and Undertreated in the General Population: Guidance for Clinicians to Prevent Coronary Heart Disease," *European Heart Journal* 34 (2013): 3478–3490, http://eurheartj.oxfordjournals.org/content/34/45/3478.

10 The Familial Hypercholesterolemia Foundation, "What Are the Risks With FH?" (2015), http://thefhfoundation.org/about-fh/what-is-fh.

11 Jennifer G. Robinson et al., "Efficacy and Safety of Alirocumab in Reducing Lipids and Cardiovascular Events," *New England Journal of Medicine* 372, no. 16 (2015): 1489–99, http://www.nejm.org/doi/full/10.1056/NEJMoa1501031; Patrick M. Moriarty., et al., "Efficacy and Safety of Alirocumab, a Monoclonal Antibody to PCSK9, in Statin-Intolerant Patients: Design and Rationale of ODYSSEY ALTERNATIVE, a Randomized Phase 3 Trial," *Journal of Clinical Lipidology* 8(6): 554-61 (2014), http://www.lipidjournal.com/article/S1933-2874(14)00331-6/abstract; Marc S. Sabatine et al., "Efficacy and Safety of Evolocumab in Reducing Lipids and Cardiovascular Events," *New England Journal of Medicine* 372, no. 16 (2015): 1500–1509, http://www.nejm.org/doi/full/10.1056/NEJMoa1500858.

12 Sabatine et al., "Efficacy and Safety of Evolocumab."

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