PCSK9 Inhibitors entering U.S. drug market soon

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PCSK9 inhibitors, named for their mechanism of action, are being introduced to the U.S. market. These are a new category of biologic agents for the treatment of hypercholesterolemia. Currently under various stages of FDA review and development, the PCSK9 inhibitors are being evaluated concurrently with a transition in the approach to the treatment of hypercholesterolemia. The American College of Cardiology (ACC) and the American Heart Association (AHA) released the revised Guideline on the Treatment of Blood Cholesterol to Reduce Atherosclerotic Cardiovascular Risk in Adults in 2013. A significant modification was the elimination of the former, specific LDL-C numeric treatment goals recognized as the objective of treatment of hypercholesterolemia. Without having a specific target level, the revised guidelines emphasize treatment on cardiovascular risk reduction in high-risk patient populations by managing hypercholesterolemia through the effective use of HMG CoA reductase inhibitors, or statins.

The drug pricing information is not yet firm, but the projected annual costs of these drug agents range from $7,000 to $12,000. The data available on the agents in development have shown their effectiveness at decreasing cholesterol, but the proposed scope of their use is being debated. The American College of Cardiology and the American Heart Association recently released new cholesterol guidelines that are being considered in the use of these new inhibitors. The guidelines stress the use of statins due to the documented results from their ability to reduce risk for heart attack, stroke or other cardiovascular events.
CRKX101

The US Food and Drug Administration (FDA) has granted Orphan Drug designation to Cerulean Pharma Inc.’s investigational ovarian cancer drug.

The company announced that the agency granted the designation to its nanoparticle-drug conjugate (NDC), CRLX101, for the treatment of patients with ovarian cancer. The designation provides certain incentives, such as federal grants, tax credits, and a seven-year period of marketing exclusivity if the drug is approved. CRLX101 is a NDC designed to concentrate in tumors and slowly release its anti-cancer payload, camptothecin, inside tumor cells. CRLX101 inhibits topoisomerase 1 (topo 1), which is involved in cellular replication, and also inhibits hypoxia-inducible factor 1a (HIF-1a), which research suggests is a master regulator of cancer cell survival mechanisms. The drug is in Phase II clinical development and has been dosed in more than 250 patients.

Cerulean is testing two combination treatments involving CRLX101 for relapsed ovarian cancer. Currently, the company is enrolling patients in a Phase II trial evaluating the drug in combination with Avastin. The company plans on initiating enrollment in a Phase Ib trial evaluating the drug plus weekly paclitaxel in collaboration with GOG Foundation in the second quarter of 2015.

Rapamune

US Food and Drug Administration (FDA) approved its Rapamune (sirolimus) for the treatment of lymphangioleiomyomatosis (LAM), a rare, progressive disease that affects the lungs, kidneys and the lymphatic system. The disease impacts mainly women of childbearing age and has an incidence rate of just two to five out of every million women globally. The disease can result in abnormal growth of smooth muscle cells in the lung. Over time, the muscle growth can cause airway obstructions and limit the delivery of oxygen to the body. There are currently 800 patients in the US diagnosed with LAM, a disease that is often fatal.

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