

PIPELINE INSIGHTS REPORT

DRUGS TO WATCH

See the full Q4 2020 Drug Pipeline Insights report for in-depth analysis and “Market Trend to Watch,” on how FDA approvals are faring during COVID-19.



Treating anemia associated with chronic kidney disease

Working name: roxadustat

Roxadustat leverages a cluster of genes that helps high-altitude populations adapt to chronic low oxygen levels (hypoxia). In trials roxadustat significantly increased red blood cells and hemoglobin levels for both dialysis-dependent and non-dialysis patients compared to standard therapy (injected erythropoiesis-stimulating agents (ESAs)). Multiple biosimilar ESAs are now available, however roxadustat, with superior efficacy and oral administration may be competitive. Prices not available.



Treating advanced prostate cancer in men

Working name: relugolix

Standard treatments for advanced prostate cancers work by reducing the amount of testosterone in the body. In trials, relugolix was found superior to existing drugs in rapid, sustained suppression of testosterone, plus a 54% lower risk of major adverse cardiovascular events and no increased side effects. Relugolix would be the first oral drug in this class however, it enters a crowded marketplace with generic alternatives available. Prices not available.



Treating elevated LDL-C in patients with atherosclerotic cardiovascular disease (ASCVD) and familial hypercholesterolemia (FH)

Working name: inclisiran

Inclisiran would join a relatively new drug class called PCSK9 inhibitors (Repatha[®] and Praluent[®]), which are used for those who do not respond adequately to statins to lower LDL-C. Inclisiran may not compete strongly with statins or the existing PCSK9 drugs. There are many generic statins; and both statins and existing PCSK9s have well-documented long-term cardiovascular (CV) outcomes benefit data, which inclisiran currently lacks.



Preventing hereditary angioedema (HAE) attacks

Working name: berotralstat

If approved, berotralstat would be the first oral, once daily treatment for HAE; a genetic defect that causes swelling in the hands, feet, face and airway. Berotralstat would compete in a crowded market against well-established biologic therapies. While oral administration is an advantage, berotralstat seems to be less effective than the existing injectable alternatives. While pricing for berotralstat has not been announced, the non-biologic berotralstat could potentially be priced less than the current drugs (up to \$635,000 per year).



Treating patients with diffuse large B-cell lymphoma (DLBCL)

Working name: lisocabtagene maraleucel (“liso-cel”)

Liso-cel is a one-time infusion that would be a new entry in the class of chimeric antigen receptor (CAR) T cell therapies to attack DLBCL, an aggressive type of non-Hodgkin’s lymphoma. In trials, liso-cel shows promising efficacy. All CAR T’s come with a risk of severe side effects, although liso-cel may have a comparatively lower rate of these. While no price is available for liso-cel, prices for existing CAR Ts range from \$373,000 to \$475,000.