Optum Rx®

Pipeline insights report

Drugs to watch.

Updated: December 7, 2022

Oral

Sparsentan: Brand name to be determined. Expected FDA decision: November 17, 2022

Sparsentan is under review for the treatment of immunoglobulin A (IgA) nephropathy (also known as Berger's disease). Over time, IgA nephropathy can lead to end-stage kidney disease (ESKD) and the need for dialysis. Approximately 30,000 to 50,000 cases are addressable with sparsentan.

There is an unmet need for effective IgA nephropathy treatments. In the pivotal trial, sparsentan demonstrated promising improvements in proteinuria vs. a standard of care treatment option.

However, sparsentan could be reserved as a second-line therapy after inadequate response to existing drugs.

Price TBD.

Gene Therapy

Etranacogene dezaparvovec: Brand name Hemgenix®.

Hemgenix (etranacogene dezaparvovec) has now been approved for sale as new gene therapy to treat adult patients with moderately-severe-to-severe hemophilia B. Approximately 6,000 people are affected in the U.S.

People with hemophilia B are treated clotting factor IX (FIX). FIX replacement therapy is arduous and has a lifetime cost up to $23 million.

Etranacogene dezaparvovec uses a highly enhanced version of the gene that governs FIX production. Once delivered to the liver cells, the enhanced genes produce more FIX.

In trials, 98% of treated patients could discontinue use of prophylactic FIX replacement.

Hemgenix is the first gene therapy approved for hemophilia B, as well as the most expensive drug, at $3.5 million per treatment.

Injectible

Pegcetacoplan: Brand name to be determined. Expected FDA decision: November 26, 2022.

Pegcetacoplan is intended to treat geographic atrophy (GA), an aggressive form of dry age-related macular degeneration (AMD). GA is a leading cause of blindness affecting more than 1 million people in the U.S.

Pegcetacoplan is administered via injection into the eye. Once there, it slows down part of the body's immune system that otherwise can cause destruction of healthy cells in the eye, leading to GA.

While currently there are no FDA-approved treatments for GA, pegcetacoplan is entering a hot market. Multiple drug companies have drugs in late-stage development, some of which may perform better than pegcetacoplan.

Price TBD.

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