Optum Rx®

Pipeline insights report

Drugs to watch.

Infused

Leqembi™ (lecanemab): FDA approved January 6, 2023

Lecanemab, from Eisai/Biogen, is under review for the treatment of mild cognitive impairment (MCI) due to Alzheimer's disease and mild, or early, Alzheimer's disease.

Alzheimer's disease is an irreversible brain disorder caused by changes in the brain, including abnormal accumulations of amyloid beta plaque. Alzheimer's disease affects about 6.5 million people in the U.S.

In trials, lecanemab has shown "robust plaque clearance" plus a "modest slowing" in cognitive decline compared to placebo based on a standard dementia test score.

Lecanemab has similar adverse event concerns as other amyloid-targeting drugs: brain swelling or bleeding associated with amyloid-related imaging abnormality (ARIA).

Price: The wholesale price for Leqembi has been set at $26,500 per patient per year. For reference, the Wholesale Acquisition Cost (WAC) for Aduhelm® (aducanumab) is approximately $28,000 per year.

Infused

Donanemab: Brand Name: TBD
Expected FDA decision: Delayed

Donanemab, from Eli Lilly, is under review for the treatment of MCI due to Alzheimer's disease and mild, or early, Alzheimer's disease. On January 19, 2023, the FDA denied accelerated approval for donanemab due to insufficient data. Eli Lilly says that expanded data will be available by the second quarter of 2023, when they expect to apply for full approval.

In trials, donanemab slowed the clinical decline of Alzheimer's disease by 32% relative to placebo on the Integrated Alzheimer's Disease Rating Scale, and also substantially reduced amyloid plaque.

Donanemab has similar adverse event concerns as other amyloid-targeting drugs: brain swelling or bleeding associated with ARIA.

Price: TBD. For reference, the WAC for Leqembi is $26,500 and the cost for Aduhelm is approximately $28,000 per year.

Gene Therapy

Valoctocogene roxaparvovec: Brand Name: Roctavian™ Expected FDA decision: March 31, 2023*

** Target date may be delayed if the FDA incorporates a new phase 3 analysis (GENEr8-1) into the application.13 See below.*

Valoctocogene roxaparvovec, from BioMarin, is under review for the treatment of adults with severe hemophilia A. Hemophilia A is caused by mutations in the Factor VIII (FVIII) gene.

Roctavian uses a viral vector (AAV5) to deliver functional copies of the FVIII gene to the liver, where clotting factors are produced. The added genes spread and restore effective FVIII production, and potentially reduce or eliminate the need for routine preventive treatments.

After one year of treatment, trial participants experienced substantially reduced annualized bleeding rates, reduced factor VIII utilization, and increased factor VIII activity, compared to the year prior to the study.

Valoctocogene roxaparvovec would be the first gene therapy for hemophilia A.

Price is TBD. Note that the first genetic treatment for hemophilia B has recently been approved. For reference, etranacogene dezaparvovec (marketed as Hemgenix®) has a WAC of $3.5 million for a one-time dose.

Oral

Fezolinetant: Brand Name: TBD Expected FDA decision: February 22, 2023

Fezolinetant, from Astellas Pharma, is under review for the treatment of moderate-to-severe vasomotor symptoms (VMS) associated with menopause.

For most women, VMS symptoms are manageable. However, in the U.S., about 60% to 80% of women experience symptoms that can negatively affect sleep, mood, and quality of life.

Trial results showed a statistically significant reduction in the frequency and severity of moderate to severe VMS for women receiving fezolinetant vs. a placebo.

Price TBD. A third-party analysis of short-term clinical trials suggests fezolinetant would be cost-effective if priced between $2,000 – $2,500 per year.

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