

Drug Pipeline Insights Report

Spring 2024 Summary



Fidanacogene elaparvovec: Brand name TBD **Expected FDA decision: 2Q 2024**

Intended to treat adults with moderate to severe hemophilia B. It could reduce or eliminate the need clotting factor replacement therapy, which has an average lifetime cost up to \$23 million.

In trials, patients experienced a 71% reduction in bleeding vs. the pre-treatment period. However, the long-term effectiveness and safety of fidanacogene elaparvovec is still unknown.

Fidanacogene elaparvovec will compete with Hemgenix® (etranacogene dezaparvovec), the first gene therapy for hemophilia B. For reference, the Wholesale Acquisition Cost (WAC) for Hemgenix is \$3.5 million for a one-time dose.



mRNA-1345: Brand name TBD **Expected FDA decision: April 2024**

mRNA-1345 will be used to prevent respiratory syncytial virus (RSV)-associated disease in adults aged 60 years or older.

Each year RSV infection causes between 6,000 to 10,000 deaths, and between 60,000 and 120,000 hospitalizations among the elderly. mRNA-1345 would be the third vaccine to join this class since mid-2023.

In trials, efficacy for mRNA-1345 appears to be similar to existing RSV vaccines. However, longer-term data is still lacking.

For reference, existing RSV vaccines cost \$280 and \$295 for a single dose.



Ensifentrine: Brand name TBD

Expected FDA decision: June 26, 2024

Ensifentrine will be used as maintenance treatment for patients with chronic obstructive pulmonary disease (COPD).

Up to 50% of COPD patients continue to have symptoms despite their current treatment. Ensifentrine would be the first novel maintenance mechanism for COPD in more than 10 years.

In trials, demonstrated significant improvements in lung function. However, studies did not include all combinations of medications, or patients currently receiving dual or triple therapies.

For reference, WAC prices for potential competitors include Breztri Aerosphere (\$7,600/year) and Dupixent (over \$40,000/year).

[Please refer here for additional technical background and supplemental sources.](#)

