# CY2024 CBER Breakthrough Therapy Approvals

Data as of June 30, 2024

| Application Number | Submission Type and Number | Proprietary Name | Established Name | Applicant | Approval Date | Use |
|---|---|---|---|---|---|---|
| BLA 125786 | ORIGINAL-1 | BEQVEZ | fidanacogene elaparvovec-dzkt | Pfizer, Inc. | 25-APR-2024 | Treatment of adults with moderate to severe hemophilia B (congenital factor IX deficiency) who are receiving routine prophylaxis and without pre-existing neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid detected by an FDA-approved test. |
| BLA 125746 | SUPPLEMENT-74 | CARVYKTI | ciltacabtagene autoleucel | Janssen Biotech, Inc | 05-APR-2024 | Treatment of adult patients with relapsed or refractory multiple myeloma, who previously received a proteasome inhibitor (PI), an immunomodulatory agent (IMiD) and an anti-CD38 antibody |

* Breakthrough Therapy designation was enacted in the Food and Drug Administration Safety and Innovation Act on July 9, 2012.