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The Effects of Designating Collategene (HGF gene therapy product) as Breakthrough Therapy

In the release issued on September 18, 2024, AnGes announced that “Collategene,” a gene therapy product we are developing in the United States, has been designated as a Breakthrough Therapy by the U.S. Food and Drug Administration (FDA). We would like to inform you of the effects and outlook for how this designation as a breakthrough therapy will affect the future development of Collategene.

Breakthrough Therapy designation is a process designed by the FDA to expedite the development and review of drugs that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on clinically significant endpoint(s).

Of the total 577 applications for Breakthrough Therapy designation filed in the United States between October 1, 2019 and June 30, 2024, 222 were approved for designation, or about 38% of the total. Of these, only 3 out of 13, or about 23% of the total, were designated in the last 9 months from October 1, 2023 to June 30, 2024 for biologically-derived products, including Collategene, indicating that the breakthrough therapy designation itself is very difficult to obtain.[*1]

An examination of the products that have received Breakthrough Therapy designation to date shows that they are expected to shorten review times and improve the certainty of approval.

Specifically, the FDA's median review times for 2021 and 2022 are 12.1 months and 14.5 months, respectively, for standard review in 2021 and 2022, while the review times for those with Breakthrough Therapy designation are 8.0 months and 11.5 months, respectively, 3 to 4 months The approval period is shorter and approval is granted in a shorter period of time.[*2]

Of the 587 cases that received Breakthrough Therapy designation during the 12-year period from July 9, 2012, when the Breakthrough Therapy designation system began, to June 30, 2024, an examination of the current status of 483 cases extracted from the public information of each company that received the designation shows that 328 cases (approximately 68%) have received approval. Of these, only 37 (about 8%) have been discontinued (including those with no update for more than 6 years), except for those still in clinical trials or in the process of submission. This approval probability is extremely high compared to the general approval probability of approximately 46% (average from 2019 to 2023[*3]) after the completion of Phase II clinical trials as with Collategene.[*1]

Thus, it can be inferred that the Breakthrough Therapy designation for Collategene will shorten

the review period in the future, and that the probability of approval of the application, if filed, will be higher than that of a typical product.

There is no change in the consolidated earnings forecast for the current fiscal year as a result of this Breakthrough Therapy designation.

*1 From the FDA website
*2 From the Japan Pharmaceutical Manufacturers Association Newsletter, March 2024, No. 220.
*3 Calculated based on "Global Trends in R&D 2024: Activity, productivity, and enablers (Copyright©2024 IQVIA)