Following Positive Phase 2 Results And Orphan Drug Designation, Biodexa's FAP Drug Receives FDA Fast Track Status

Biodexa Pharmaceuticals PLC. (NASDAQ: BDRX), a clinical-stage biopharmaceutical company developing a pipeline of innovative products for the treatment of diseases with unmet medical needs, received Fast Track status from the U.S. Food and Drug Administration for eRapa, a proprietary encapsulated form of rapamycin being developed for the treatment of familial adenomatous polyposis (FAP). The news follows Biodexa reporting positive 12-month data from a phase 2 clinical trial in July 2024, as previously covered by Benzinga.

The FDA's Fast Track designation is designed to facilitate the development and expedite the review of drugs to treat serious conditions where there is an unmet medical need. FAP, an inherited condition that puts people at a much greater risk of developing colon cancer, falls into that category. The condition is typically diagnosed in the early teenage years and results in a nearly 100% lifetime risk of colorectal cancer.

To be considered a "serious" disease by the FDA, a condition must have a substantial impact on survival and daily functioning or the likelihood that it will worsen over time if left untreated. An unmet medical need is defined as the absence of an existing treatment or the availability of a potentially more effective treatment. Companies granted Fast Track status are able to have more frequent meetings and written communications with the FDA to discuss the drug's developmental plan, the design of proposed clinical trials and the use of biomarkers. This designation may also lead to faster drug approval.

Meeting An Unmet Need

With FAP, hundreds or thousands of precancerous polyps grow throughout the gastrointestinal tract. There is no approved therapeutic option for treating FAP patients, for whom active surveillance and surgical resection of the colon and/or rectum remain the standard of care. People with FAP, which usually appears in the patient's mid-teens, end up eventually having their entire colon removed.

There's a significant hereditary component to FAP, with a reported incidence of one in 5,000 to 10,000 in the U.S. and one in 11,300 to 37,600 in Europe. Biodexa, which has already received U.S. FDA Orphan Drug designation for eRapa in FAP, plans to seek a similar designation in Europe.

eRapa is a proprietary oral tablet formulation of rapamycin, also known as sirolimus, which slows down the mammalian Target Of Rapamycin (mTOR) protein. Too much mTOR has been

linked to cancer and has been shown to be over-expressed in FAP polyps – thereby underscoring the rationale for using an mTOR inhibitor like eRapa to treat FAP. eRapa has a number of patents that the company says extend through 2035.

Promising Clinical Trials

It doesn't seem surprising that Biodexa received Fast Track designation for eRapa for FAP. Data from the company's phase 2 study of eRapa in FAP showed eRapa demonstrated a 17% median decrease in overall polyp burden and an overall non-progression rate of 75%. Meanwhile Biodexa said patients in cohort 2 experienced an 89% non-progression rate and 29% median reduction in polyp burden at 12 months compared with baseline. The dosing given to cohort 2, which was daily every other week, is the preferred dosage regimen for the upcoming registrational phase 3 study. The phase 3 study will be a double-blind placebo-controlled design recruiting approximately 168 high-risk patients diagnosed with germline or phenotypic FAP. That could be gaming-changing for FAP patients if it means fewer and/or delayed surgeries. As it stands, multiple screenings and surgeries are a way of life for sufferers of this hereditary disease.

"The promising phase 2 results, if confirmed in a registrational phase 3 study, may delay or potentially obviate the need for resection of the colon and/or rectum in FAP patients," said Stephen Stamp, CEO of Biodexa. "The six-month and 12-month data together with its apparent tolerability suggest longer-term use of eRapa may be possible, with the potential to forestall resection and substantially increase the quality of life of patients with this devastating precancerous condition impacting up to 40,000 patients in the U.S. and up to 60,000 in Europe."

People suffering from FAP deserve better options than screening and surgeries. Biodexa wants to deliver that with eRapa, and with the FDA Fast Track designation, it is getting closer to giving the thousands of people suffering from this precancerous disease a better alternative.

To learn more about Biodexa and eRapa, click here.

Featured photo by farland9 on stock.adobe.com.

This post contains sponsored content. This content is for informational purposes only and is not intended to be investing advice.