US FDA approves PTC Therapeutics' metabolic disorder drug

The drug, branded as Sephience, is an oral therapy for both children and adults with phenylketonuria (PKU), a genetic condition caused by a change in the gene that helps create the enzyme responsible for breaking down phenylalanine - an amino acid found in most protein-containing foods.

Bengaluru: The U.S. Food and Drug Administration has approved PTC Therapeutics' oral drug to treat a rare inherited metabolic disorder that prevents the body from properly breaking down certain amino acids, the company said on Monday.

The approval allows PTC to expand its revenue base as its top-selling Duchenne muscular dystrophy therapies, Translarna and Emflaza, face revenue declines due to regulatory scrutiny and loss of patent protection.

The drug, branded as Sephience, is an oral therapy for both children and adults with phenylketonuria (PKU), a genetic condition caused by a change in the gene that helps create the enzyme responsible for breaking down phenylalanine - an amino acid found in most protein-containing foods.

All newborn babies in the United States are screened for phenylketonuria shortly after birth to catch the condition early and prevent serious brain damage.

Sephience works by boosting the activity and stability of the enzyme phenylalanine hydroxylase (PAH), helping patients better manage the disorder.

Without treatment, phenylalanine can build up to toxic levels, leading to irreversible brain damage, intellectual disability and other neurological problems.

The overall incidence of PKU in the U.S. is about 1 in 15,000 live births, according to the National Institutes of Health.

Treatment options for PKU include a lifelong low-phenylalanine diet, special medical formulas and drugs such as BioMarin Pharmaceutical's Kuvan and Palynziq, which are approved for patients who respond to them.

Jefferies analyst Kelly Shi expects peak annual sales of $741 million from Sephience by 2030.

PTC's application was based on a late-stage trial in which the drug significantly reduced blood phenylalanine levels by an average of 63%, with most patients reaching guideline-recommended levels and some able to ease their strict diets while maintaining control.

Sephience was approved in Europe last month. The drug is under review in several other countries, including Japan and Brazil.

(Reporting by Kamal Choudhury and Christy Santhosh in Bengaluru; Editing by Alan Barona)

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