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Enzyme Disorder Set to be Conquered as Phenylketonuria is Due New Treatments

LONDON, UK (GlobalData), 13 June 2012 - New treatments for Phenylketonuria (PKU) are set to revolutionize the lives of many patients, as pharmaceutical development swiftly takes control of the orphan disease, according to new research released by healthcare analysts GlobalData.

The new report* shows that restricted diets may soon be a thing of the past, as medication looks to conquer this rare enzyme mutation.

PKU is a metabolic genetic disorder which renders the enzyme phenylalanine hydroxylase (PAH) unable to metabolize the amino acid phenylalanine (Phe). Abnormally high levels of Phe accumulate in the blood and can affect brain development, leading to progressive mental retardation, brain damage and seizures.

PKU cannot be cured, but early diagnosis and strict treatment plans can sometimes offer patients an average life span and improve mental development. Before 2007, a phenylalanine-restricted diet was the only available method of treatment, but the approval and subsequent launch of Kuvan (sapropterin dihydrochloride) in 2007 in the US has meant that medical advances now surpass dietary regimes. Kuvan is also expected to be approved for patients under four years of age by 2015.

In terms of safety and efficacy, Kuvan exceeded the expectations of physicians and patients. However, a significant unmet need remains, as the majority of the patient pool is unresponsive to the drug due to underlying genetic mutations.

PEGylated recombinant phenylalanine ammonia lyase (PEG-PAL) is due to be launched in this period, targeting 80-90% of PKU patients who do not currently respond to Kuvan. However, the phenylketonuria market remains largely untapped, and provides significant potential for companies who can develop new molecules with enhanced bioavailability, similar safety and

efficacy profiles, and better responses from the wider patient pool.

For companies willing to get involved, GlobalData estimates that the phenylketonuria therapeutics market in the US, the UK, Germany, Italy, France, Spain and Japan will collectively reach a value of $272.70m in 2019.