

Pheburane® receives EU Marketing Authorization for the treatment of Urea Cycle Disorders

On July 31st, 2013, the European Commission granted Lucane Pharma a marketing authorization for the medicinal product Pheburane for the treatment of Urea Cycle Disorders (UCD), involving deficiencies of carbamylphosphate synthetase, ornithine transcarbamylase and argininosuccinate synthetase.

Pheburane, a taste-masked formulation of sodium phenylbutyrate (NaPB), is the first development product from Lucane Pharma.

Sodium phenylbutyrate is well established as a nitrogen scavenger in the treatment of UCD. However, this active substance is well-known for its extremely unpleasant bitter taste, which may compromise compliance to treatment, especially in children.

Being a taste masked, coated formulation, Pheburane is well accepted by patients, including children. Pheburane is bioequivalent to the original formulation.

Lucane Pharma has developed a specific dosing spoon (CE marked) for accurate measurement of the dose prescribed. Patients can easily measure out Pheburane with the same spoon, whatever the required dose is.

“Since its introduction in the mid 1990’s, patients, parents and healthcare personal have been complaining of the taste of sodium phenylbutyrate. This issue was already raised in the very first scientific publications describing its therapeutic activity. This issue is now past history” says Pierre Mambrini, CEO of Lucane Pharma.

About Urea Cycle Disorders (UCD)

Urea Cycle Disorders are very rare inherited inborn errors of metabolism caused by the lack of specific enzymes which are responsible for the removal of waste nitrogen from protein metabolism. The excess nitrogen is converted to ammonia which is highly toxic to the brain. If untreated these disorders can lead to severe neurological impairment and in severe cases can be fatal. Sodium phenylbutyrate is rapidly metabolized to sodium phenylacetate which combines with nitrogen facilitating its removal from the body in the urine. Treatment with sodium phenylbutyrate is always combined with a protein-restricted diet. Treatment is life-long.