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Red blood cell-encapsulated enzymes: An innovative therapeutic approach to overcome challenges of enzyme replacement therapies for rare diseases

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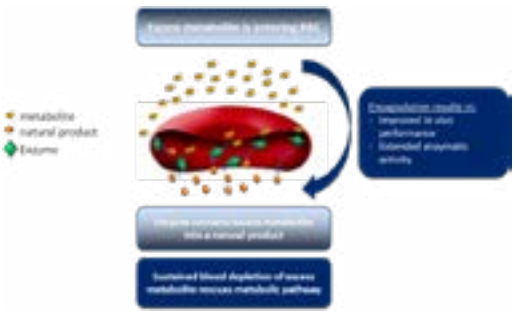
Many inborn errors of metabolism (IEM) disorders are due to defects in single genes encoding key metabolic enzymes. In most cases, clinical manifestations of these disorders are driven by the over-abundance of a metabolite or the scarcity of an essential metabolite. Though rare, IEM disorders can have devastating consequences for patients and their families. While some Enzyme Replacement Therapies are commercially available for a few IEM disorders, the clinical benefits of these approaches are often outweighed by the emergence of hypersensitivity and the rapid clearance of enzymes. Therefore, there is a high need for better tolerated and longer-acting replacement enzymatic activity to alleviate the burden of IEM disorders. RBCs are the most abundant cell type in the human body and their biology is characterized by a long lifespan and access to all tissues and organs. Thanks to their biocompatibility and shielding properties, they can serve as a circulating bioreactor when loaded with enzymes. ERYTECH is a leader in RBC therapeutics. Its ERYCAPS® platform enables the encapsulation, at industrial scale, of active drug substances inside RBCs using hypotonic loading, which has been shown to maintain all the RBC functionalities. ERYTECH has demonstrated that RBC-encapsulated enzymes exhibit substantially improved *in vivo* performance vs. non-encapsulated enzymes, including extended enzymatic activity. Results from two early programs using enzyme-loaded RBCs in *in vivo* models for Arginase-1 Deficiency and Classical Homocystinuria will be presented. These promising results combined with ERYTECH's extensive clinical experience with RBC therapeutics, support the possibility that RBC-loaded enzymes may provide superior safety and efficacy as compared with traditional ERT approaches for the treatment of IEM disorders.



Recent Publications

1. Gay F, et al. (2017) Methionine tumor starvation by erythrocyte-encapsulated methionine gamma-lyase activity controlled with per os vitamin B6. Cancer Med. 6(6):1437-1452.
2. Bourgeaux V, et al. (2016) Drug-loaded erythrocytes: on the road toward marketing approval. Drug Des Devel Ther. 10:665-76.
3. Thomas X and Le Jeune C (2016) Erythrocyte encapsulated l-asparaginase (GRASPA) in acute leukemia. Int J Hematol Oncol. 5(1):11-25.

4. Yew N, *et al.,* (2013) Erythrocytes encapsulated with phenylalanine hydroxylase exhibit improved pharmacokinetics and lowered plasma phenylalanine levels in normal mice. Mol Genet Metabol. 109(4):339-344.
5. Bourgeaux V, *et al.,* (2012) Efficacy of homologous inositol hexaphosphate-loaded red blood cells in sickle transgenic mice. Br J Haematol. 157(3):357-369.

Biography

Emmanuelle Cecile Dufour has obtained her PhD in Biochemistry and has been working with Erytech Pharma for 10 years. She is involved in the preclinical development of enzyme loaded-red blood cells as therapeutics for inborn errors of metabolism.

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