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DIRECTION DE LA COMMUNICATION

## - NEWS -

### **FDA grants Orphan Drug Designation to LFB's « Complement Factor H »**

In December 2009, the Food and Drug Administration (FDA) granted LFB (Laboratoire français du Fractionnement et des Biotechnologies) with the Orphan Designation for its Complement Factor H, a human plasma-derived product, currently in pre-clinical development.

The designated orphan indication is for the "Treatment of atypical Hemolytic Uremic Syndrome (aHUS) associated with an inherited abnormality of the complement system".

Scientific knowledge has progressed in the last decade; however the therapeutic options have remained limited for this pathology.

**Complement Factor H**, the main regulator of the alternative complement pathway, is a plasma glycoprotein mostly synthesized in the liver. Even though Factor H has been known for some time, its involvement in aHUS was only discovered in the late 1990s.

**aHUS (Hemolytic and Uremic Syndrome)** is an extremely rare kidney disease characterized as a thrombotic microangiopathy. aHUS mainly affects children less than 3 years old and often results in an acute renal failure. aHUS is listed as a rare disease by the Office of Rare Diseases at the National Institutes of Health, which defines the rare disease as an illness or condition affecting fewer than 200,000 American people. aHUS might currently affect less than 1000 people in the USA.

According to the available databases and registries on aHUS in Europe, the estimated prevalence of FH-dependant aHUS is currently estimated at 1 to 2 cases per million of inhabitants. aHUS might affect around 450 to 800 patients in Europe.

In January 2007, LFB's Complement Factor H was granted the same indication by the European Commission.

## **About LFB**

LFB is a biopharmaceutical group that develops, manufactures and markets medicinal products for the treatment of serious and often rare diseases in several major therapeutic fields, namely Hemostasis, Immunology and Intensive Care.

LFB Group is the leading manufacturer of plasma-derived medicinal products in France and 6<sup>th</sup> worldwide and is also among the leading European companies for the development of monoclonal antibodies and new-generation proteins based on biotechnologies.

With its strong focus on research, the LFB Group is pursuing a growth strategy that seeks to extend its activities at international level and develop innovative therapies.

LFB's 2008 turnover reaches €353 million, of which €67 million were dedicated to the R&D effort.

LFB markets its products in 20 countries and is present in Germany and the UK since 2007 and in Brazil since 2004.

Christian Béchon is president and CEO of the LFB Group (1531 employees).

*For more information, please visit [www.lfb.fr](http://www.lfb.fr)*

## **Press Contacts :**

### **LFB**

Sandrine Charrières, Corporate Communications Director

01.69.82.72.80

[charrieres@lfb.fr](mailto:charrieres@lfb.fr)

### **Financial Dynamics**

Florence de Montmarin / Nathalie Baudon

01.47.03.68.10

[florence.demontmarin@fd.com](mailto:florence.demontmarin@fd.com) / [nathalie.baudon@fd.com](mailto:nathalie.baudon@fd.com)