



## **OBI-1 Receives Orphan Drug Designation in Europe**

### **Product Candidate to Treat Hemophilia patients who have developed Inhibitors to Human Factor VIII**

**Paris (France), October 19, 2010** – Ipsen (Euronext: IPN; ADR: IPSEY) announced today that the European Commission has granted orphan drug status for OBI-1 for the treatment of hemophilia. Expected to enter pivotal clinical trials before the end of this year, OBI-1 is designed to treat individuals with hemophilia who have developed inhibitory antibodies (inhibitors) against human Factor VIII (hFVIII). The orphan drug status would trigger a 10-year market exclusivity for OBI-1 in the European Union after its marketing approval. The U.S. Food & Drug Administration (FDA) issued an Orphan Drug Designation for OBI-1 in March 2004.

**Stéphane Thiroloix, Ipsen's Executive Vice-President, Corporate Development, commented :** *"The European Commission's decision to grant OBI-1 orphan drug status for the treatment of hemophilia reinforces Ipsen's commitment to focus its resources and know-how on its four targeted disease areas (oncology, endocrinology, neurology and hematology), where severe medical conditions remain without appropriate therapeutic response. We are proud that the European authorities have recognized the medical value of treating hemophilia in individuals who have developed inhibitors against human Factor VIII. Our partnership with Inspiration Biopharmaceuticals is a key lever in building a world-leading hemophilia franchise covering a wide range of bleeding disorders."*

According to the partnership agreement signed in January 2010, Ipsen has granted a licence to Inspiration Biopharmaceuticals (Inspiration) for OBI-1. Inspiration is responsible for the clinical trial development and commercialization of OBI-1.

#### **About Hemophilia**

Hemophilia is a group of bleeding disorders caused by low levels or absence of proteins called a coagulation factors, essential for blood clotting. The two most common forms of hemophilia are types A and B. Hemophilia A is caused by a factor VIII deficiency and occurs in ~1 out of every 5,000 male births. Hemophilia B is caused by factor IX deficiency and occurs in ~1 out of every 30,000 male births. Approximately 60% of persons with hemophilia have a severe condition, which results in frequent spontaneous bleeding episodes in addition to serious bleeding after injuries. The annual market for hemophilia treatments is \$7.5 billion worldwide.

#### **About OBI-1**

Approximately one-third of individuals with hemophilia A develop an immune reaction (inhibitors) to hFVIII and can no longer respond to treatment with the coagulation factor. Current therapies, specifically FVIIa and FEIBA, work by bypassing the natural hemostatic pathway and driving coagulation by raising FVIIa and other activated coagulation factors to higher levels than normal. In contrast, OBI-1, a recombinant B-domain deleted FVIII bioengineered based on the porcine amino acid sequence that typically possesses low cross reactivity to anti-hFVIII antibodies, is a physiologic replacement therapy, activating the natural hemostatic pathway. This would allow clinicians to correlate activity and efficacy with a biomarker and therefore guide dosing and better predict treatment

outcomes. OBI-1 presents a unique and alternative approach to address the needs of individuals who have developed inhibitors to FVIII and is highly desired by the medical and patient communities.

OBI-1 has been evaluated in a Phase 2 study in patients with congenital hemophilia A who have developed inhibitors to FVIII and who presented with a non life/non limb threatening bleed. The Phase 2 study demonstrated OBI-1 was well-tolerated, stopped the bleeding in all subjects, and can be given over a short infusion time. Inspiration expects to initiate a pivotal trial of OBI-1 in individuals with acquired haemophilia in the fourth quarter of 2010 and a separate pivotal trial in individuals with congenital hemophilia who have developed inhibitors against hFVIII in the first half of 2011.

## **About Ipsen**

Ipsen is a global biopharmaceutical group with total sales in excess of 1 billion euros in 2009, and total worldwide staff of more than 4,400. Its strategy is based on fast growing specialty care drugs in oncology, endocrinology, neurology and hematology, and primary care drugs, which significantly contribute to research financing. This strategy is also supported by an active policy of partnerships. Ipsen's specific Research & Development (R&D) centers and peptide & protein engineering platform give the Group a competitive edge. Nearly 900 people are dedicated to the discovery and development of innovative drugs for patient care. In 2009, R&D spend reached close to €200 million, representing more than 19% of total Group sales. Ipsen's shares are traded on Segment A of Euronext Paris (stock code: IPN, ISIN code: FR0010259150). Ipsen's shares are eligible to the "Service de Règlement Différé" ("SRD") and the Group is part of the SBF 120 index. Ipsen has implemented a Sponsored Level I American Depositary Receipt (ADR) program, which trade on the over-the-counter market in the United States under the symbol IPSEY. For more information on Ipsen, visit our website at [www.ipсен.com](http://www.ipсен.com).

## **Ipsen's Forward Looking Statement**

The forward-looking statements, objectives and targets contained herein are based on the Group's management strategy, current views and assumptions. Such statements involve known and unknown risks and uncertainties that may cause actual results, performance or events to differ materially from those anticipated herein. Moreover, the targets described in this document were prepared without taking into account external growth assumptions and potential future acquisitions, which may alter these parameters. These objectives are based on data and assumptions regarded as reasonable by the Group. These targets depend on conditions or facts likely to happen in the future, and not exclusively on historical data. Notably, future currency fluctuations may negatively impact the profitability of the Group and its ability to reach its objectives. Actual results may depart significantly from these targets given the occurrence of certain risks and uncertainties. The Group does not commit nor gives any guarantee that it will meet the targets mentioned above. Furthermore, the Research and Development process involves several stages each of which involve the substantial risk that the Group may fail to achieve its objectives and be forced to abandon its efforts with regards to a product in which it has invested significant sums. Therefore, the Group cannot be certain that favourable results obtained during pre-clinical trials will be confirmed subsequently during clinical trials, or that the results of clinical trials will be sufficient to demonstrate the safe and effective nature of the product concerned. The Group also depends on third parties to develop and market some of its products which could potentially generate substantial royalties; these partners could behave in such ways which could cause damage to the Group's activities and financial results. The Group expressly disclaims any obligation or undertaking to update or revise any forward looking statements, targets or estimates contained in this press release to reflect any change in events, conditions, assumptions or circumstances on which any such statements are based, unless so required by applicable law. The Group's business is subject to the risk factors outlined in its registration documents filed with the French Autorité des Marchés Financiers.

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