FDA grants Orphan Drug Designation to OptPA, the optimized human tissue-type plasminogen activator developed by Op2Lysis, for treatment of intracerebral hemorrhage

Op2Lysis SAS is pleased to announce that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation to OptPA for treatment of intracerebral hemorrhage.

The Orphan Drug Designation of OptPA provides access to valuable incentives in the development of O2L-001 for the treatment of patients with acute hemorrhagic stroke, the most severe form of stroke and an unmet medical need.

OptPA is a proprietary optimized human tissue-type plasminogen activator, which is nanoprecipitaed and formulated in a poloxamer gel to produce O2L-001, the first drug product developed by Op2Lysis.This nanoformulation is a disruptive technology which provides an increased efficacy of OptPA.

The purpose of this locally injected treatment is to liquefy the intracerebral hematoma that has formed following this form of stroke, so that the blood can easily be removed through a minimally invasive surgery technic to reduce hematoma blood volume. This type of intervention has been previously assessed in the MISTIE clinical trial with a commercially available thrombolytic agent, allowing to demonstrate the safety of the procedure and providing a clinical proof of concept for this technology, with a strong association between blood volume reduction and clinical benefit as a decrease in death and disability. The results of the MISTIE trial raised a very high hope for hemorrhagic stroke patients. However, the product tested in this prior study was insufficiently effective to allow statistical demonstration of the clinical benefit. The data available with O2L-001 indicate its potential for a much higher efficacy in this therapeutic indication.

"*The orphan drug designation by the FDA offers several strategic advantages and is a valuable step forward in our development program, which enhances our O2L-001 project and promotes its acceleration and success. We are now looking for Series A investors with the ambition to achieve preclinical regulatory stage and perform a combined Phase 1 and 2 study in hemorrhagic stroke patients. There is a potential for high upfront value after a 2-year only clinical program. This new step confirms the strong commitment of Op2Lysis to offer unique solutions for the treatment of serious acute vascular conditions with a significant risk of mortality, or which are unmet medical needs.*" said Christophe Gaudin, CEO of Op2Lysis.

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Registration number: 819 697 707 RCS Nanterre **Capital:** 174 794 Euros

About Orphan Drug Designation

The Orphan Drug Designation Program provides orphan status to drugs and biologics, which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect less than 200,000 people in the US. Orphan Drug Designation qualifies sponsors for incentives, including tax credits for qualified clinical trials, exemption from user fees, and potential seven years of market exclusivity after approval. More information about rare diseases and the Orphan Drug Designation Program is available on https://www.fda.gov/.

About Op2Lysis – www.op2lysis.com

Op2Lysis is a French and Belgian preclinical stage biotech company developing products using a disruptive technology to ensure best delivery of thrombolytic agents and address acute life-threatening vascular diseases. With its first drug product, O2L-001, Op2Lysis is dedicated to the development of the first medical treatment of hemorrhagic stroke, the most disabling form of stroke. Op2Lysis aspires to become a Leader in developing new treatments for patients suffering from vascular unmet medical needs or life-threatening conditions.

The company is located both in France, with its headquarter in Boulogne-Billancourt, near Paris, and its Research site in Caen, in Normandy, and in Belgium with its affiliate Op2Lysis Development SA near Liège in Wallonia, which is in charge of Development activities of the company.

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