

## **Pivotal study is being conducted with Oxabact™ for the treatment of Primary Hyperoxaluria**

OxThera today announced that all 42 patients have been enrolled in their pivotal phase II/III study using Oxabact™ for the treatment of Primary Hyperoxaluria. Results from this multicenter study will be presented during Q4 of 2008 and be used to file for licensure in EU, US and the rest of the world.

Primary Hyperoxaluria is a rare genetic disease in which excessive oxalate is produced by the liver and excreted in the urine by the kidneys. High levels of urinary oxalate cause kidney stones and/or calcification of the kidney which could lead to kidney failure and in many cases premature death. OxThera estimates that there are about 2000 patients with Primary Hyperoxaluria in EU and US combined.

Oxabact™ consists of a unique intestinal bacterium, *Oxalobacter formigenes*, naturally colonizing the intestinal tract of most humans with the purpose to degrade oxalate. Previous studies with Oxabact™ have already shown a significant effect in lowering urinary oxalate which in turn leads to a decreased risk of kidney damage. Oxabact™ has been designated orphan drug status in both EU and the US.

The 28 week pivotal study is a randomized, double-blind, placebo-controlled, multi-center study being conducted at eight Primary Hyperoxaluria referral sites in the Netherlands, France, UK, Germany and US.

"Primary Hyperoxaluria is a very serious disease often leading to early kidney failure and in particular systemic oxalate deposition with all its complications including death with no effective medical therapy currently available. For majority of patients the only real option today is a combined liver-kidney transplantation which is available to a very limited number of patients worldwide. Therefore, the Primary Hyperoxaluria community has great hopes that Oxabact™ will offer a new treatment opportunity. A confirmation of earlier study results with Oxabact™ will reflect scientific breakthrough and a new chapter in the treatment of this rare and severe disease", says Prof. Bernd Hoppe, University Hospital in Cologne, Germany.

Jon Heimer, CEO and President of OxThera comments: "After several years of intensive research on *Oxalobacter formigenes* and Oxabact™, a significant milestone is met with the inclusion of all patients in this pivotal study. A successful outcome will put us in a position to file for licensure and making the product available to treating physicians and patients during 2009 which is very exciting".

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# OxThera

## Short facts about OxThera

OxThera is a biotechnology company active in the development of products for the treatment of metabolic disorders resulting from excess levels of oxalate from endogenous and exogenous sources. Currently, OxThera has two products in its pipeline, Oxabact™ for the treatment of primary hyperoxaluria, and Oxazyme™, for the prevention of recurring calcium-oxalate kidney stones due to secondary hyperoxaluria.

Oxalate is a metabolic end product in humans. It is endogenously produced by the liver and also derived by absorption from the diet. The majority of oxalate is eliminated from the body through the kidneys and a small percentage is eliminated through the GI-tract. Oxalate forms a calcium-oxalate salt which is insoluble at physiological pH and its accumulation can result in serious renal conditions. Consistent high levels of urinary oxalate are known as “hyperoxaluria”, which can result in recurrent kidney stones and renal complications. Hyperoxaluria is currently classified as:

- ❑ Primary hyperoxaluria types I and II are rare genetic diseases resulting from overproduction of oxalate in the liver (PH I) or in all body cells (PH II); urinary oxalate excretion is usually greater than 100 mg/day (normal level <45 mg/day).
- ❑ Secondary hyperoxaluria due to excessive absorption of dietary oxalate. This is common in patients with excessive absorption of dietary oxalate and in patients with fat malabsorption due to underlying enteric diseases such as IBD, or cystic fibrosis. Further, it is often seen in patients following jejunioileal bypass surgery or bariatric surgery, and in patients with absorptive hyperoxaluria.

Primary hyperoxaluria is a rare, serious disease with very limited treatment options available. The urinary oxalate excretion rate in affected patients is typically three to six times normal with severe clinical consequences. Kidney stones and/or calcification of the kidney occur in childhood or adolescence. Renal injury due to oxalate and consequences of the stones often leads to renal failure. Loss of renal function, if not addressed promptly by transplantation, leads to markedly increased plasma concentrations of oxalate with deposition of calcium-oxalate in body tissues. Renal failure occurs in 50% of the patients by the age of 15 years and has reached 80% by the age of 30 years. Renal replacement therapy is not able to eliminate sufficient amounts of oxalate, hence systemic oxalate deposition occurs.

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