**Innovation Portfolio**
**16VNM001**

# Urine biomarker and reprofiling drug for cystinuria treatment and/or relief

**INVENTORS**: López de Heredia M (CIBER), Nunes V (IDIBELL - UB)

## HIGHLIGHTS

- ✓ **Novel urine biomarker for cystine lithiasis**
- ✓ **Natural compound with no described toxicity to retard cystine lithiasis onset**

## TECH STATUS

- ✓ **Biomarker: Experimental proof of concept**
- ✓ **Compound: Technology validated in an *in vivo* animal model**

## Problem to be solved

One in 7000 people suffer from Cystinuria, and 94% of the patients will develop cystine stones, 40% of them during the pediatric age. Renal colic produced by cystine kidney stones is very painful.

Currently, cystine lithiasis onset can only be determined by X-rays or TAC. A methodology to easily determine the onset of a lithiasic episode before the colic would facilitate patient management. Moreover, current treatments for cystine lithiasis show important side effects, forcing the **discontinuation and the need of renal interventions to remove the cystine stones.**

## Background

Cystinuria is a rare disease with an estimated prevalence of 1/7000 newborns. It is responsible of about 2% of all renal lithiasis and about 8% in the pediatric age. Approximately 94% of cystinuric patients develop cystine stones and the recurrence of lithiasic episodes is high, ending in most of the cases with renal failure.

Current adherence to available treatments for cystine lithiasis is low due to the secondary effects of the treatments used. Frequently, cystine stones have to be removed by different urologic interventions; these continuous interventions diminish patients' life-quality.

## Technology

1. A novel urine biomarker for cystine lithiasis. This diagnostic tool can determine the presence of cystine stones, diagnosing potential cystinuria development, by means of the quantification of specific compounds (related to the disease) in Cystinuria patient's urine.

2. A non-toxic natural compound, that when orally administered, prevent and lower cystine stones formation, with no-side effects compared to current treatments (D-penicillamine (Cuprimine®) or tiopronin (Thiola®)).

**Contact Information**:
**Business Development & Innovation Area**
innovacio@idibell.cat
Innovation Portfolio Unit: Gisela Lorente glorente@idibell.cat (+34) 93 2607649
www.idibell.cat

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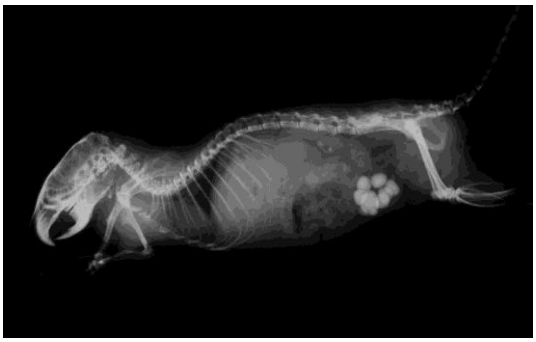

## Applications

1. When determined in routinary urine analysis, the biomarker could be used to monitor the onset of cystine lithiasis before its detection by other means or before renal colic episode.

2. The principal goal of the therapy with the compound in Cystinuria treatment is to avoid the formation of cystine stones, or at least, to delay its onset. This compound could be used, either since Cystinuria diagnosis or after stone removal by renal interventions, to prevent stone formation. Due to its lack of toxicity, it could be chronically administered.

## Technology status

1. The proposed biomarker has been shown to discriminate between lithiasic and non-lithiasic animals in a relevant murine model for the disease. Confirmation of these results has to be assessed.

2. In the Cystinuria murine model (*SLc7a9*$^{-/-}$), oral administration of the compound before cystine lithiasis onset (week 3) was able to significantly reduce the number of lithiasic animals and cystine stone size at the end of the study (week 24).

## Market Opportunity

There are approximately 12,000 patients affected with Cystinuria in the US, about 20,000 in Europe, and a worldwide prevalence of 1 in 7,000 people. Given this market size, it could be considered as an **"orphan" indication**.

Historically, the primary therapy focused on Cystinuria was Penicillamine, marketed by Valeant. However, nearly a third of people are allergic to it. Consequently, this opened the door to Tiopronin, which is marketed by Retrophin Inc, and showed fewer side effects. According to RTRX, it has identified 3,000 patients with Cystinuria (currently treating 425). At pricing of ~US$80,000 per patient per year, 3,000 patients would represent a market opportunity of US$240 million per year.

## Pipeline competitors

PK10 (PharmaKrysto Ltd.) has been granted last Jun-2018 **Orphan Designation** by both the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

Bucillamine [INN] (Revive Therapeutics Ltd) Currently in CT phase II, has been granted I 2015, **Orphan Designation** by the US Food and Drug Administration (FDA)

## Business Opportunity

Co-development or license agreement.

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