PRESS RELEASE

PharmaKrysto Ltd announces licensing agreement with Rutgers University for new treatment for people with cystinuria

New treatment now also granted Orphan Designation in the US and EU

UK, 12 July 2018. PharmaKrysto Ltd, a biopharmaceutical company based in Scotland, UK, announces today that it has completed an exclusive global licensing deal with Rutgers University, New Jersey, USA, for PK10, a potentially revolutionary compound to treat people with cystinuria – an inherited disease that causes large, painful and debilitating crystals of amino acid to form in the kidneys.

PharmaKrysto also announces that based on preclinical data demonstrating that this treatment has the potential to be effective in patients with cystinuria and, in a clear recognition of the seriousness of this medical condition as well as its rarity in the USA and Europe, PK10 has been granted Orphan Designation by both the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

Cystinuria is a genetic condition, affecting children and adults, which causes large amino acid crystals to form in the kidneys. These crystals are responsible for repeated episodes of severe pain and ultimately irreparable kidney damage. PharmaKrysto's new compound, PK10, acts as a molecular 'imposter' to prevent amino acid crystals forming in the kidneys. PharmaKrysto will continue its preclinical development of PK10 with the goal of entering clinical studies and treating people with cystinuria as soon as possible.

The company is currently initiating an early stage fundraising round to prepare for patient trials and, in an early indication of the potential for this technology, has already received indications of support and interest from private and regional public sources.

Julian Howell, CEO of PharmaKrysto, said: "These three events represent the achievement of major milestones for PharmaKrysto as we seek to develop this exciting new treatment for people with cystinuria. This is a painful condition with a significant impact on the quality of life for people with cystinuria. There are currently no effective treatments for many patients so we are determined to develop this solution for those people who continue to experience severe, frequent pain and complicated kidney disease."

About the licensing agreement, Distinguished Macmillan Professor Jay Tischfield, of the Department of Genetics at Rutgers University, said: "The teams of geneticists and medicinal chemists at Rutgers have collaborated on designing important molecules that have shown promising early results in treating cystinuria. We are very pleased to continue this work with the experienced team from PharmaKrysto with the goal of starting clinical studies as soon as possible."

Prof David Goldfarb, Chief of Nephrology, NYU Langone Health, NY, US, commented: "I know from my work with people with cystinuria that treatments available to them are not always effective and the side effects can be a significant problem. It's very encouraging that we have new ways of treating patients being developed."

Prof John Sayer, Clinical Professor of Renal Medicine at the Institute of Genetic Medicine, Newcastle University, UK also said: "There have been no new approaches to preventing the build-up of cystine within the kidney for many years; this approach of disrupting crystal growth has been effective in the laboratory, and our department looks forward to collaborating with PharmaKrysto on clinical studies in cystinuria patients."

Ends 12 July, 2018

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Notes to editors

PharmaKrysto Ltd is an early stage biotech company based near Edinburgh in Scotland. Its therapeutic focus is on developing novel medicines to treat people with rare conditions. Its lead programme, PK10, has the goal of freeing people with cystinuria from pain and the risk of kidney failure.

Cystinuria is a rare genetic condition causing build-up and crystalisation of an amino acid, cystine, in the kidneys of people affected. The cystine crystals can grow to many centimetres in size, causing pain, blockage of urine flow and permanent kidney damage. Children and adults can be affected; repeated build up of crystals results in frequent hospital visits and surgery to remove them. There is a significant impact on family, education and work life. Current treatment options are inadequate, and can have significant side effects.