Purespring Therapeutics presents new preclinical data on novel gene therapy for IgA Nephropathy at ASN Kidney Week 2024

Results from the study underpin the potential of Purespring's lead AAV gene therapy programme, PS-002, as an important novel modality to treat IgAN

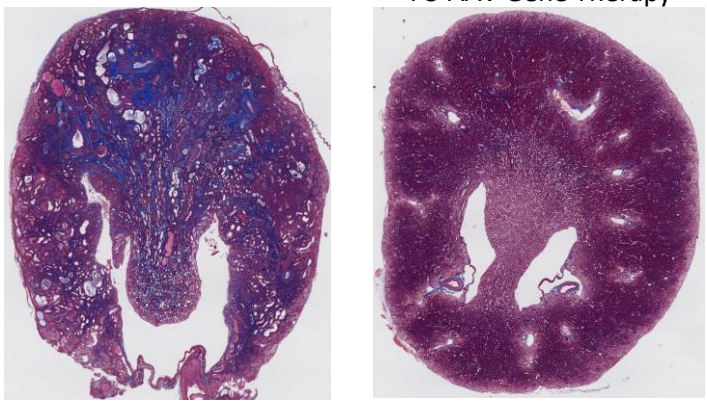
Data support progression towards a Phase I/II clinical trial

London – 28 October 2024 - Purespring Therapeutics, a pioneering gene therapy company focused on transforming the treatment of kidney diseases, presented preclinical data at the American Society of Nephrology (ASN) Kidney Week 2024 showing that transgenes can be effectively targeted to podocytes to modulate complement activation and reduce signs of kidney disease in animal models of IgA nephropathy (IgAN), an autoimmune kidney disease.

The presentation, "*Podocyte gene therapy enables glomerular complement modulation for IgA Nephropathy (IgAN) treatment*," was presented on 26 October at the American Society of Nephrology (ASN) Kidney Week 2024, taking place in San Diego, USA from October 24–27.

PS-002, uses a modified virus to treat podocytes, with the research demonstrating the administration of PS-002 in a mouse model of IgAN reduced signs of kidney dysfunction, lowered complement deposition and ameliorated kidney scarring and other structural characteristics of kidney disease. In pigs, treatment with PS-002 resulted in elevated and prolonged gene expression in kidney tissues, with no safety issues.

Ambra Cappelletto, Principal Scientist at Purespring, commented: "Our data demonstrate that targeting podocytes to modulate complement activation is an effective therapeutic strategy, and PS-002 paves the way to become the first gene therapy in development for the treatment of IgA nephropathy. Purespring's gene therapy platform, exemplified by PS-002, demonstrates therapeutic genetic material can be delivered with high efficiency to podocytes, opening up a new and highly differentiated modality with the potential to treat a broad range of kidney diseases."



Cross-sectional images of IgA nephropathy murine kidneys. Purespring gene therapy significantly improves kidney morphology and reduces the areas of scarring and fibrosis stained in blue.

Julian Hanak, Purespring's Chief Executive Officer, added: "Chronic kidney disease is an area of huge unmet need, affecting around 840 million people globally, and we were thrilled to demonstrate the potential of our novel platform technology with the international nephrology community. The data presented at ASN Kidney Week 2024 demonstrate the potential of our gene therapy platform technology to deliver working copies of genes with high efficiency and specificity directly to the podocyte, and as we further progress the development of PS-002, we hope that these promising results will translate into clinically meaningful benefits for patients."

Purespring recently announced it had raised £80 million ($105 million) in a Series B financing to support the initiation of a Phase I/II clinical trial for IgAN. There is currently no cure for IgAN, and about one third of patients will go on to lose their kidney function within five years and require a kidney transplant or dialysis.

Purespring is the first company to successfully treat kidney disease models by directly targeting the podocyte, a specialised cell implicated in approximately 60% of renal diseases, through its proprietary adeno-associated virus (AAV) gene therapy platform. The data presented at ASN follows earlier critical preclinical data presented by Purespring at the 61st European Renal Association (ERA) Congress in May establishing the potential of AAV gene therapy to deliver transgenes to the podocyte to replace defective genes or to modulate protein production.

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Notes to Editors

About Purespring

Purespring is developing gene therapies to halt or prevent kidney disease, one of humankind's most poorly treated disease areas.

Founded on the work of Professor Moin Saleem, Professor of Paediatric Renal Medicine at the University of Bristol, Purespring is the first company to successfully treat kidney disease by targeting the podocyte, a specialised cell that is implicated in the majority of renal disease. Purespring's platform approach enables streamlined gene therapy development for both monogenic and non-monogenic renal diseases, offering the potential to halt, reverse and even cure both rare and common kidney diseases.

The Company currently has a pipeline of programmes in development including the lead asset for treatment of IgA Nephropathy (IgAN) and other complement mediated kidney disease. The Company also has programmes for diseases caused by mutations in the gene NPHS2, as well as other monogenic glomerular kidney diseases.

Based in London, the Purespring team combines world-leading expertise in podocyte biology and kidney disease with a wealth of experience in gene therapies, anchored in a culture of diversity, creativity and delivery.

Purespring is backed by leading biotech investors, including Syncona Limited, Sofinnova Partners, Gilde Healthcare, Forbion, and British Patient Capital and has raised £115M to date.

For more information please visit: purespringtx.com and follow us on LinkedIn.