Purespring Therapeutics granted European Medicines Agency (EMA) orphan drug designation for PS-002 for the treatment of patients with primary IgA nephropathy (IgAN)

- EMA designation provides orphan status to medicines being developed for rare conditions that are intended for the diagnosis, prevention or treatment of diseases affecting fewer than 5 in 10,000 people in the European Union
- Purespring has previously demonstrated the potential of PS-002, the Company's lead programme, as an important novel targeted treatment for primary IgAN
- PS-002 is progressing towards a Phase I/II clinical trial, supported by a strong preclinical data package

London – 28 April 2025 - Purespring Therapeutics, a pioneering company focused on transforming the treatment of kidney diseases, today announces that the European Medicines Agency (EMA) has granted orphan drug designation to PS-002, Purespring's lead programme, in IgA nephropathy (IgAN).

Orphan drug designation in the European Union (EU) is granted by the European Commission based on a positive opinion adopted by the EMA Committee for Orphan Medicinal Products. Orphan designation provides incentives to developers of medicines for limited patient populations, including market exclusivity, protocol assistance (guidance on study design and scientific evaluation) and regulatory fee reductions.

IgAN is a common, chronic autoimmune kidney disease that primarily affects young adults. In patients with the disease, a protein called immunoglobulin A (IgA) becomes trapped in the filters of the kidney, known as glomeruli, causing inflammation, damage and scarring to the whole kidney. There are currently no approved therapeutic options to stop disease progression, nor a cure, and about one third of high risk IgAN patients will go on to lose kidney function within five years and require a kidney transplant or dialysis.

Haseeb Ahmad, Purespring's Chief Executive Officer, commented: "Orphan drug designation will significantly support Purespring's goal of progressing PS-002 through clinical development and, more broadly, with bringing solutions for kidney disease indications to patients. We're delighted that the EMA has recognised the promise of this potential breakthrough medicine and we look forward to working with them on the development pathway.

"By developing a novel approach to treat IgAN, we believe we can offer hope to patients across Europe and globally who currently have limited therapeutic options, furthering our mission to transform the lives of people suffering from kidney diseases."

Supported by an oversubscribed £80 million / $105 million Series B financing, which completed in October 2024, Purespring is progressing PS-002 towards a Phase I/II clinical trial for IgAN, with additional gene therapy programmes in development for the treatment of both rare and common kidney diseases.

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 disease, through its proprietary adeno-associated viral (AAV) gene therapy platform. As detailed in a presentation at the American Society of Nephrology (ASN) Kidney Week 2024, Purespring has previously demonstrated the potential of PS-002 as an important novel modality to treat IgAN.

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Notes to Editors

About Purespring

Purespring is developing 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 acquired and genetic 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 disease 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 ($149m) to date.

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