



PRESS RELEASE

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Preclinical Studies Test Novel Gene Therapy for Treating IgA Nephropathy

Highlights

- In animal models of IgA nephropathy, scientists have found that a viral vector gene therapy can effectively target complement activation, reduce signs of kidney disease, and result in prolonged gene expression in kidney tissues.
- Results from the study will be presented at ASN Kidney Week 2024 October 23–27.

San Diego, CA (October 26, 2024) — IgA nephropathy is an autoimmune kidney disease, and complement, a component of the innate immune system, plays a role in the condition's pathogenesis. Investigators have developed and tested a novel gene therapy that enters kidney cells and enables them to block complement activation. The research will be presented at ASN Kidney Week 2024 October 23–27.

The gene therapy, called PS-002, uses a modified virus to treat kidney cells called podocytes. Administration of PS-002 in a mouse model of IgA nephropathy 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.

“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,” said corresponding author Ambra Cappelletto, PhD, of Purespring Therapeutics, in London. “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.”

Study: “Podocyte gene therapy enables glomerular complement modulation for IgA Nephropathy (IgAN) Treatment”

Join ASN and approximately 12,000 other kidney professionals from across the globe at Kidney Week 2024 in San Diego, CA. The world's premier nephrology meeting, Kidney Week, provides

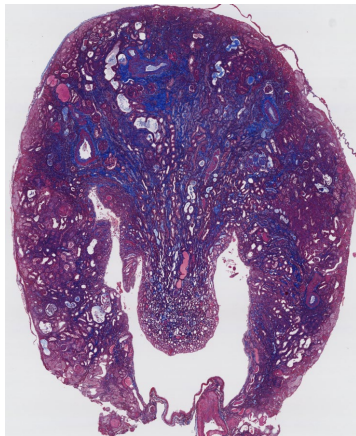
participants with exciting and challenging opportunities to exchange knowledge, learn the latest scientific and medical advances, and listen to engaging and provocative discussions with leading experts in the field. Early programs begin on October 23, followed by the Annual Meeting from October 24-27. Follow the conversation at #KidneyWk.

About ASN

Since 1966, ASN has been leading the fight to prevent, treat, and cure kidney diseases throughout the world by educating health professionals and scientists, advancing research and innovation, communicating new knowledge and advocating for the highest quality care for patients. ASN has nearly 21,000 members representing 140 countries. For more information, visit www.asn-online.org and follow us on [Facebook](#), [X](#), [LinkedIn](#), and [Instagram](#).

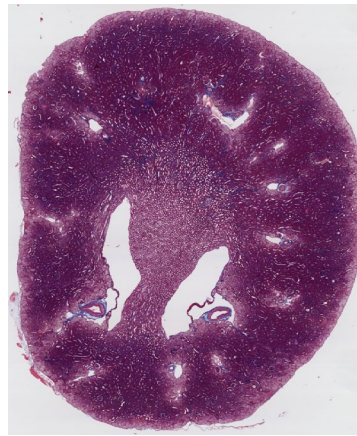
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Untreated Control



Severe kidney
glomerulosclerosis and fibrosis

PS-AAV Gene Therapy



Very low levels of
glomerulosclerosis and fibrosis

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