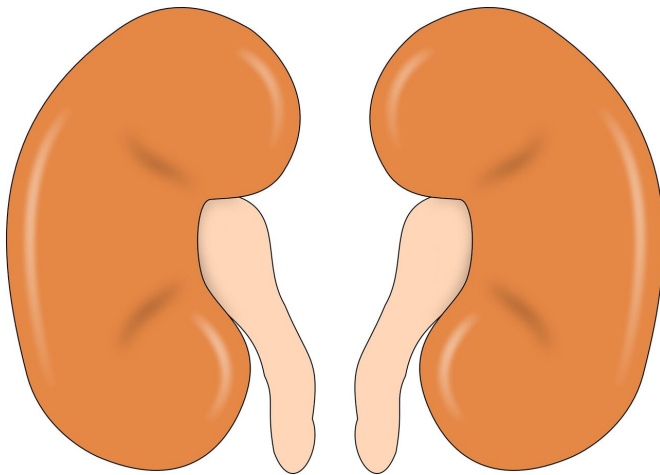


CER-001 helps restore kidney function and vision for patient with rare genetic kidney disease

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CER-001, an HDL mimetic, may restore kidney function and vision to patients suffering from the rare genetic kidney disease, LCAT (lecithin-cholesterol acyltransferase) deficiency. A case report is published in *Annals of Internal Medicine*.

LCAT deficiency is a rare recessively inherited disease characterized by circulating free cholesterol-rich lipoprotein X (LpX) and very low levels of HDL, leading to the progressive development of corneal ring opacities, anemia, splenomegaly, and glomerulopathy. Currently, there is no approved treatment, but in an animal model of familial LCAT deficiency, CER-001 improved dyslipidemia, and prevented inflammation and renal damage.

Researchers from the INSERM U1297 (Institut National de la Science et de la Recherche Médicale) and the University Hospital of Toulouse sought to determine whether CER-001 could improve [kidney](#) function and vision in a human

patient who tested positive for familial LCAT deficiency and was experiencing rapidly declining kidney function and blurred vision. Because treatment with antihypertensive medications and atorvastatin did not stop further kidney function decline, treatment with CER-001 was started. The researchers found that [kidney function](#) improved and was stable up to 11 months of follow-up. In addition, the patient reported full reversal of her [vision](#) blurring that persisted over time. The treatment was well tolerated and no adverse events were observed during treatment or the follow-up period.

According to the authors, this case suggests that further studies are warranted. Whether an earlier introduction of CER-01 with iterative treatment periods may fully prevent kidney progression should be tested in further studies.

More information: *Annals of Internal Medicine* (2021).
<https://www.acpjournals.org/doi/10.7326/L20-1300>

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