Taking nedosiran once a month appears to safely treat people with primary hyperoxaluria type 1 based on data collected over 2.5 years

Primary hyperoxaluria 1, also called PH1, is a rare genetic disease that causes a build-up of a naturally occurring chemical called oxalate, which leads to massively elevated urinary excretion of oxalate. People living with PH1 are therefore at risk of developing kidney stones repeatedly, and over time this can cause permanent damage to the kidneys and other organs.

Nedosiran is a new drug being developed to treat PH1 and aims to decrease the amount of oxalate made by the body. It is taken once a month as an injection under the skin. To test how well nedosiran works in people living with PH1, a long-term study called PHYOX3 (Clinicaltrials.gov: NCT04042402) is being conducted. This study includes 13 people living with PH1 who had previously taken part in a short-term study called PHYOX1. PHYOX3 is due to last up to 6 years. Here we report results from the 13 people living with PH1 based on data collected over 2.5 years in PHYOX3.

After 2.5 years, nedosiran reduced the average amount of oxalate in the urine by more than half (at least 60%) at every monthly visit after the 2nd month. All patients experienced a side effect, most of which were mild or moderate, such that no patient stopped the study. The most common side effect related to nedosiran was reactions at the injection site, all of which resolved more or less promptly after injection.

Overall, these results show that after 2.5 years in PHYOX3, nedosiran reduces the average amount of oxalate in the urine in a persistent manner. No major health concerns were shown during this period. PHYOX3 is an ongoing study.