

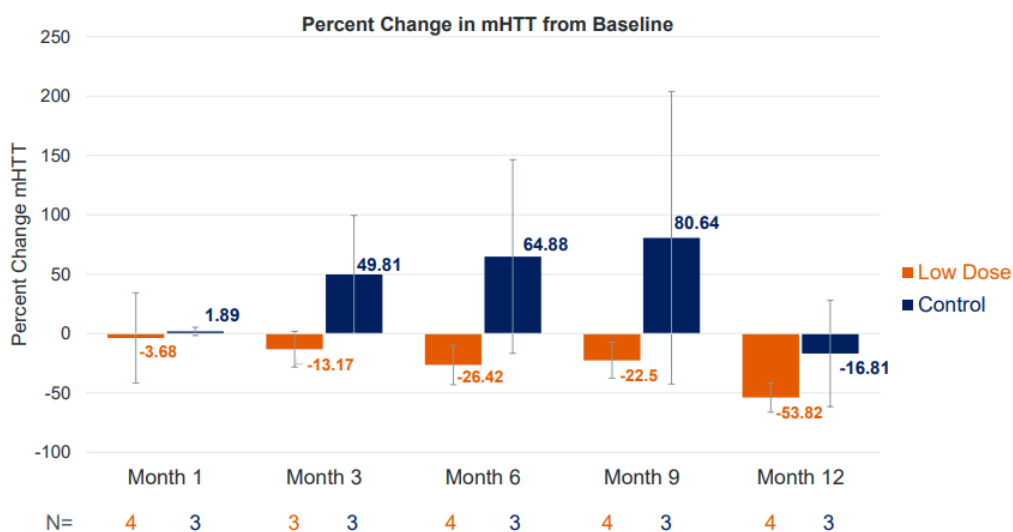
## A strange trend for Uniqure



Elizabeth Cairns

Uniqure's reluctance to disclose specific data on its Huntington's gene therapy AMT-130 has [stung it in the past](#), so today's reveal has been welcomed by investors. In four evaluable patients in the low-dose cohort of the [ongoing phase 1/2 trial](#), mean levels of mutant huntingtin protein (mHTT) in the cerebrospinal fluid fell by between 58.3% at 12 months, versus 16.8% among three evaluable patients in the sham control group. But the odd trend over time – sham patients were doing poorly until the one-year point, when they staged a startling reversal – has raised questions. On a call today management attributed this to the size of the trial, saying that [one of the sham patients was an outlier](#) and distorted the overall picture. Nonetheless Uniqure is up 13% in early trade, likely because the reduction in mHTT was at the higher end of what Uniqure had hoped for. But this is just biomarker data; results on functional endpoints, including from a 16-patient high dose cohort, will come in 2023, and the company said that in order to get approval – even accelerated approval – it will need to show clinical benefit. The group has no plans to seek a partnership.

### uniQure AMT-130: change from baseline of mHTT in CSF in evaluable, treated patients shows trend toward decline



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