

Retrophin's Fort crumbles



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Retrophin has done nothing to confound some investors' low expectations for fosmetpantotenate in the ultra-rare disease pantothenate kinase-associated neurodegeneration (PKAN), announcing the failure of its pivotal [Fort trial](#). The miss was comprehensive, and the group's chief executive, Eric Dube, eschewed the go-to measure of data mining, confirming that all development of the project would stop. The limited prospects around fosmetpantotenate had led to heavy shorting; this contributed to today's 28% share price fall. The theories as to why fosmetpantotenate missed in the 84-patient Fort trial included the different disease profiles of the patient population and the failure of the molecule to cross the blood-brain barrier. Positives for Retrophin include its substantial \$425m cash cushion, which should help it get to the next stock catalyst: data from the Duplex study of its lead project, sparsentan, are due in 2021, though this readout was recently delayed. The group also recently settled with its former chief executive, Martin Shkreli, who had sued it for \$30m from his prison cell, alleging that he had been ousted illegally. Retrophin's financial security might also allow it to buy in more rare or ultra-rare disease assets, so the company is down but perhaps not entirely out.