July 19, 2021

ISTH 2021 – Bayer's haemophilia A gene therapy raises questions

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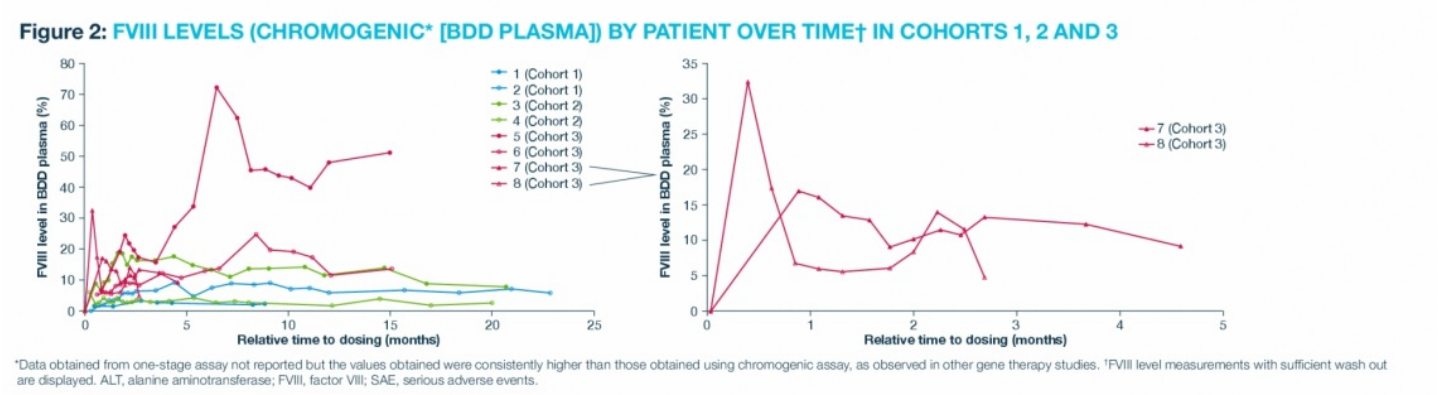
Bayer/Ultragenyx's haemophilia A gene therapy BAY 2599023 is supposed to be more durable than other contenders, but based on data presented on Saturday the jury is still out. True, the lower doses tested in the phase 1/2 trial did lead to consistent factor VIII levels, but these are unlikely to represent therapeutic doses. And the highest tested so far, $2x10^{13}$vg/kg, has spurred liver enzyme elevations. Bayer tried to combat this by using prophylactic steroids, and it was data from two patients treated with this regimen, presented for the first time at the ISTH meeting, that could cause alarm bells. One of these patients experienced severe liver enzyme elevations; it appears that the subject was given famotidine to treat nausea related to steroid treatment, and then had a rise in liver enzymes. The enzymes returned to normal after stopping famotidine, the investigators said. FVIII levels from the two new patients were also not convincing, with one subject's levels declining rapidly. Bayer has already has the go-ahead to start the highest-dose cohort in the trial, testing $4x10^{13}$vg/kg. Perhaps this will answer the question of whether BAY 2599023, already late to the party, could be a real contender.



Source: Dr Steven Pipe and ISTH.