Evaluate Vantage

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No calm after Brainstorm ALS failure

Lisa Urquhart

Brainstorm's decision to try to press on with a filing of its autologous cell therapy NurOwn in ALS, despite the unequivocal failure of a pivotal trial, says a lot about the lack of options in this intractable disease. Both primary and secondary endpoints of the 189-patient study were missed; the company blamed this on the proportion of more severe patients in the study and a higher-than-expected placebo effect. In a pre-defined subgroup of patients with early disease, a “clinically meaningful” – although not even nominally statistically significant – response was seen, executives said on a call today. They also highlighted recent dealings with the FDA, which was described as “eager” to discuss the results, leading executives to “believe the agency is open to discuss pathways to approval”. It is true that with no approved disease-modifying treatments for ALS, the bar for approval might well be lower. A request for another trial seems the most likely outcome. Investors, meanwhile, appear to be assuming that the project is over, with Brainstorm shares slumping 66%. With just $25m in the bank Brainstorm will need the FDA on its side, as any decision really will be make or break.

NurOwn ALS study (NCT03280056)					
% reporting 1.25 point per month improvement on ALSFRS-R slope (primary endpoint)			**Average change in ALSFRS-R total score (secondary endpoint)**		
NurOwn	**Placebo**	**P value**	**NurOwn**	**Placebo**	**P value**
Full study population					
34.7%	27.7%	0.45	-5.52	-5.88	0.69
Early disease subgroup*					
34.6%	15.6%	0.29	-0.177	-3.78	0.19
based on ALSFRS-R baseline score 35 or higher. Source: company press release.					

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Evaluate HQ
44-(0)20-7377-0800

Evaluate Americas
+1-617-573-9450

Evaluate APAC
+81-(0)80-1164-4754