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Regenerative (Stem Cell) Companies Continue to Fail – This Time it's BrainStorm (Not Rated)

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Athersys (ATHX) Not Rated
BrainStorm (BCLI) Not Rated
Mesoblast (MESO) Not Rated
BioCardia (BCDA) Neutral Rated

BrainStorm (BCLI-not rated) - FDA Panel Reject NurOwn

We previously followed most of the regenerative medicine companies including BrainStorm (BCLI-not Rated), Mesoblast (MESO-Not Rated), Athersys (ATHX-Not Rated), and BioCardia (BCDA-Neutral Rated). A recent string of clinical/regulatory failures does not bode well for the promise of these therapies. Why do they keep failing? We believe a combination of factors such as a limited therapeutic effect combined with under-capitalized companies have led to poorly designed (underpowered) trials. In addition, inconsistent potency of the product, improper dosing and dosing schedules, a poor understanding of endpoints, and the disease variability being treated have contributed to failures.

Investment Highlights

What happened to Brainstorm's ALS therapy – NurOwn? An FDA advisory panel voted on the question: Has substantial evidence of effectiveness meeting the approval standard been demonstrated by the evidence presented? 1 Yes, 17 No, 1 Abstain. From the FDA review documents: BrainStorm argues that the "ALSFRS-R [which measures 12 aspects of physical function] cannot measure further decline once items reach 0, making a treatment effect difficult to measure in participants with lower ratings", and "a floor effect could appear as an improvement or slowing of decline and thereby be misclassified as a clinical response". BrainStorm put forth that the lack of efficacy in the overall population was due to the subgroup impacted by the floor effect. The FDA documents say that the FDA did not observe a "floor effect" in the subgroup and says that "...the lack of efficacy of MSC-NTF over placebo cannot be explained by a floor effect".

Mesoblast (MESO - Not rated): The FDA rejected remestemcel-L last month. This is the second time regulators rejected the therapy. In both rejections, the FDA pointed to the same issues: the potency assay and an adult-population study. The FDA flagged the lack of a suitable potency assay for the candidate used during the last pivotal trial in pediatric patients. According to the regulator, this has been a crucial roadblock in considering the trial an "adequate study" that could demonstrate remestemcel-L's effectiveness.

BioCardia (BCDA-Neutral Rated): The company recently reported that the phase 3 trial was likely to fail when the independent data safety monitoring board had recommended pausing enrollment pending the analysis of patients that were then completing one-year follow-up. BioCardia has now seen results from the 102 randomized patients who have completed their one-year review—and the trial failed its multiple endpoints.

Risks to our thesis include the following: (1) clinical; (2) regulatory; (3) commercial; (4) manufacturing; (5) financial; (6) liability; and (7) intellectual property.

Companies mentioned in this report:

Athersys (ATHX), BioCardia (BCDA), BrainStorm(BCLI) and Mesoblast (MESO).

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