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Sarepta finds faith in big batch gene therapy data

Amy Brown

The failure of a placebo-controlled trial of Sarepta's Roche-partnered Duchenne muscular dystrophy gene therapy SRP-9001 might have erased 50% of the company's market cap, but it has done nothing to dent executives' confidence. Presenting new data from patients treated with commercial process material, the group's chief executive, Doug Ingram, bullishly claimed to have addressed all the issues raised when Study 102 disappointed. That setback was blamed on an imbalance in baseline characteristics in older children and lower than expected gene expression in some subjects; today's consistent results put those concerns to bed, investors were told today. True, 52% of normal micro-dystrophin expression is encouraging, and data largely replicate earlier findings, from smaller batches of material for clinical trials. However, biomarker results were never the concern here – Study 102's failure to show improvements on functional endpoints caused confidence to collapse. The 13% climb in Sarepta's share price this morning suggests that only those already keeping the faith will find comfort in these new data. Others will need to see results from a placebo-controlled phase 3 trial, which the company hopes to start towards the end of the year, after meeting the FDA in the coming weeks.

Study 102 Part 2 Placebo Crossover Patients (Clinical Process Material) and Study 103 Patients (Commercially Representative Process Material) Show Consistent Results

Micro-dystrophin Clinical Process Material

Study 102 Part 2 Placebo Crossover Patients, Mean (n=11)

Vector Genome Copies per Nucleus	% of Normal Expression	% Dystrophin Positive Fibers	% Intensity
2.62	51.7%	79.2%	100.6%

Micro-dystrophin Commercially Representative Process Material

Study 103 Patients, Mean (n=11)

Vector Genome Copies per Nucleus	% of Normal Expression	% Dystrophin Positive Fibers	% Intensity
3.87	55.4%	70.5%	116.9%

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

Evaluate Americas
+1-617-573-9450

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