HOPE-2 One-Year Results Show Clinically Relevant Improvements in Upper Limb and Cardiac Function in Patients with Later Stage Duchenne Muscular Dystrophy

Abs # 1117

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BACKGROUND

HOPE-2 (Halt cardiOmyopathy ProgrEssion in Duchenne 2, NCT03406780) is a randomized, double blind, placebo-controlled trial designed to evaluate whether CAP-1002, a cell therapy, improves skeletal muscle function, measured by the performance of the upper limb (PUL), and cardiac function measured by MRI in patients with later stage Duchenne Muscular Dystrophy (DMD).

CAP-1002 is a biologic consisting of allogeneic cardiosphere-derived cells (CDCs) (FIGS 1,2):

- Manufactured from donated heart muscle
- Cells do not act by stemness - the cells do not engraft into host tissue
- MOA: cells secrete exosomes
- Contain miRNAs, non-coding RNAs and proteins
- Internalized by target cells
- Stimulate diverse and lasting changes in cellular behavior
- 3 known miRNAs drive CAP-1002 potency

CAP-1002 has been investigated in multiple independent clinical trials and in approximately 200 human subjects to date

METHODS

Evaluated in 20 participants (8 treated, 12 placebo) through functional assessment of upper limb performance, cardiac measurements, pulmonary tests, myometry, quality of life indicators and biomarkers of inflammation and muscle damage.

FIGURE 1

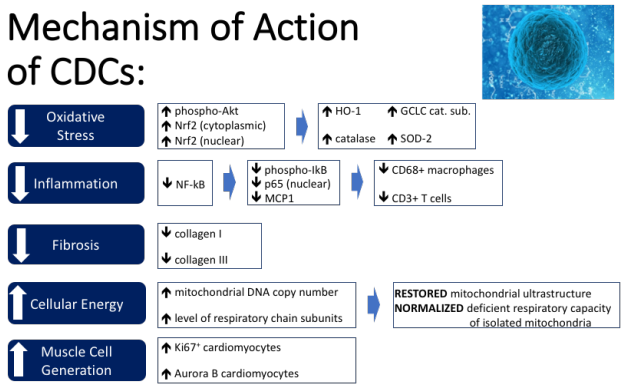


FIGURE 2

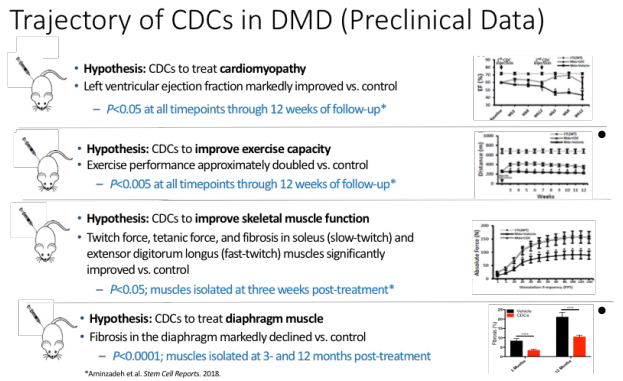


FIGURE 3

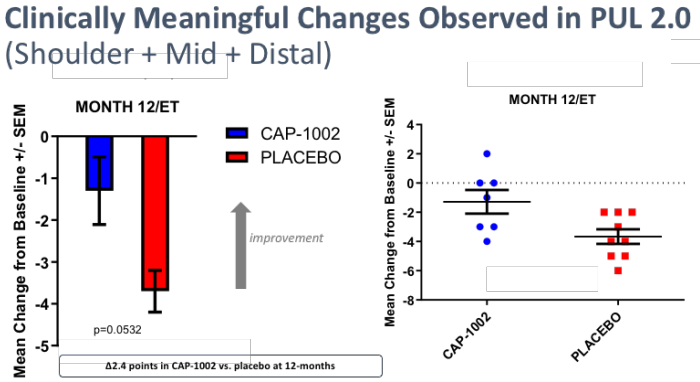
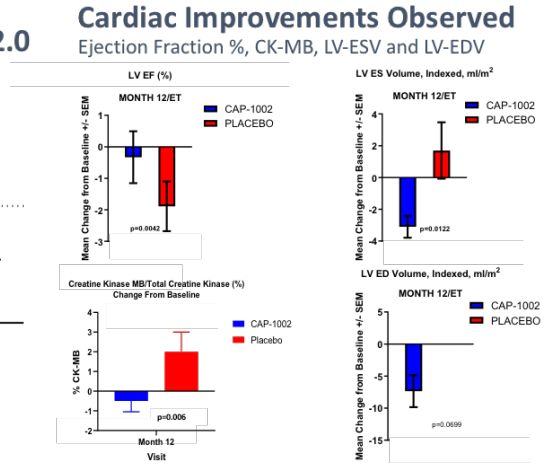


FIGURE 4



RESULTS

- In the ITT Population, both the full PUL 1.2 and full PUL 2.0 showed significant and meaningful differences between CAP-1002 and Placebo groups in favor of the treatment (PUL 1.2: Δ=4.1; p=0.034; PUL 2.0: Δ=2.4; p=0.0532, see **FIG 3**).
- CAP-1002 at 12 months resulted in statistically significant differences versus Placebo by cardiac MRI in mean left ventricular (LV) ejection fraction (mean change from baseline: -0.33% versus -1.89%; Δ =1.56%; p=0.0042, see **FIG 4**).
- Greater reductions in indexed LV end-systolic volume (4.80 mL/m2; p=0.0122, see **FIG 4**), and indexed LV end-diastolic volume (Δ=7.35 mL/m2; p=0.070, see **FIG 4**). LV end-systolic volume is often used as a surrogate for LV remodeling in clinical trials evaluating cardiac outcomes.
- There was a marked decrease in the CK-MB isozyme biomarker (**FIG 4**) in CAP-1002-treated compared to the Placebo arm indicative of reduced cardiac muscle damage over the course of the study.
- A total of 69 infusions (CAP-1002 or placebo) were performed in HOPE-2.
- Generally safe and well tolerated throughout the study With the exception of hypersensitivity reactions, no safety signals were identified.

CONCLUSIONS

The HOPE-2 study demonstrated that CAP-1002 therapy is safe and effective in treating deterioration of upper limb and cardiac function in DMD patients.

UCDAVIS

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