CAP-1002: Cardiosphere-Derived Cells
PPMD End Duchenne Tour – Birmingham, AL

NASDAQ: CAPR

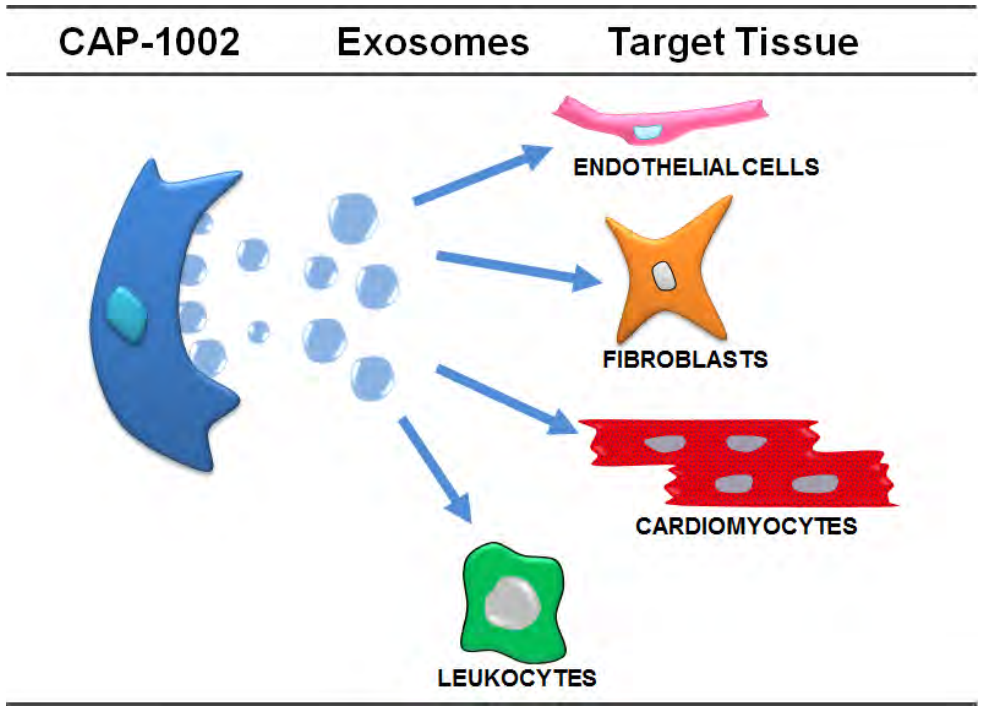
June 2018

Forward-Looking Statements

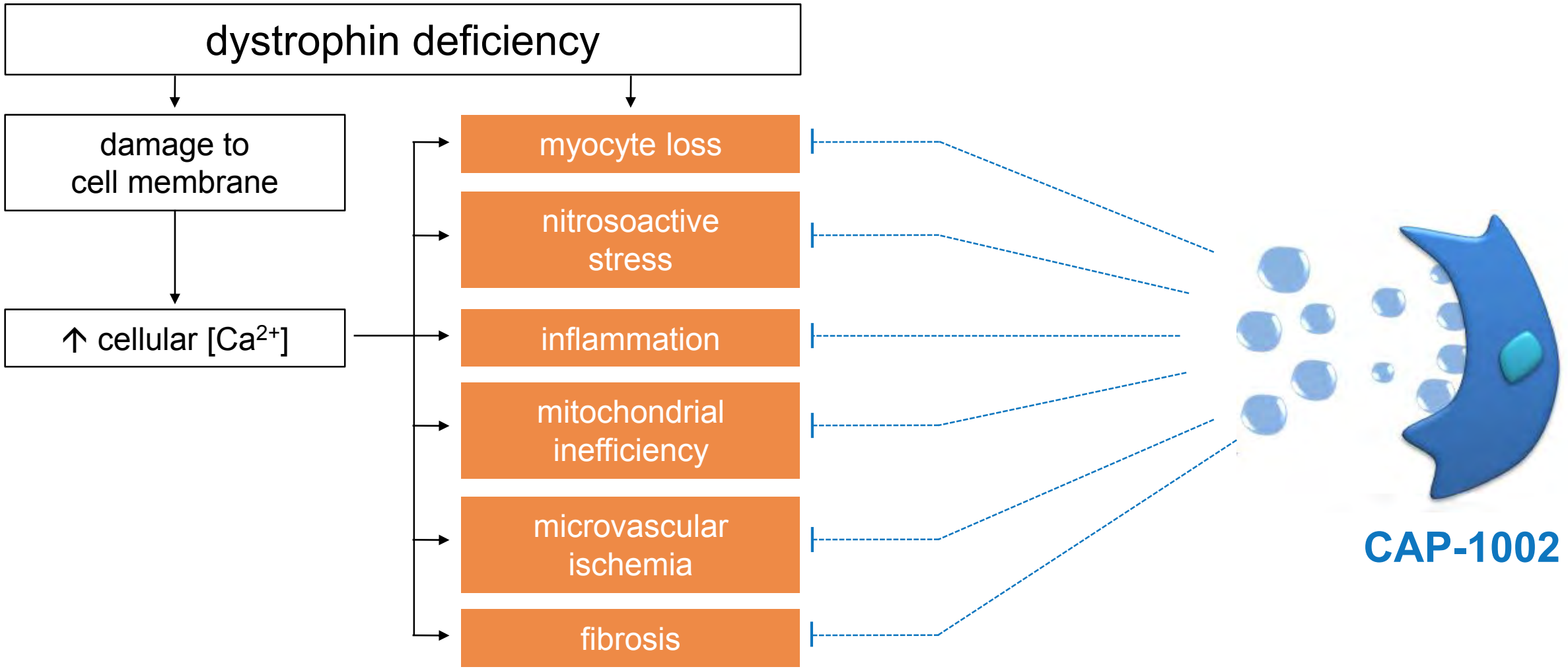
Statements in this presentation regarding the efficacy, safety, and intended utilization of Capricor's product candidates; the initiation, conduct, size, timing and results of discovery efforts and clinical trials; the pace of enrollment of clinical trials; plans regarding regulatory filings, future research and clinical trials; regulatory developments involving products, including the ability to obtain regulatory approvals or otherwise bring products to market; plans regarding current and future collaborative activities and the ownership of commercial rights; scope, duration, validity and enforceability of intellectual property rights; future royalty streams, expectations with respect to the expected use of proceeds from the recently completed offerings and the anticipated effects of the offerings, and any other statements about Capricor's management team's future expectations, beliefs, goals, plans or prospects constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Any statements that are not statements of historical fact (including statements containing the words "believes," "plans," "could," "anticipates," "expects," "estimates," "should," "target," "will," "would" and similar expressions) should also be considered to be forward-looking statements. There are a number of important factors that could cause actual results or events to differ materially from those indicated by such forward-looking statements. More information about these and other risks that may impact Capricor's business is set forth in Capricor's Annual Report on Form 10-K for the year ended December 31, 2017 as filed with the Securities and Exchange Commission on March 22, 2018, in its Registration Statement on Form S-3, as filed with the Securities and Exchange Commission on September 28, 2015, together with the prospectus included therein and prospectus supplements thereto. All forward-looking statements in this press release are based on information available to Capricor as of the date hereof, and Capricor assumes no obligation to update these forward-looking statements.

CAP-1002 Background

- CAP-1002 is a biologic product consisting of allogeneic cardiosphere-derived cells (CDCs) derived from donated heart muscle
- Do not act by “stemness” – do not engraft into host tissue
- Acts by releasing extracellular vesicles, or exosomes
  - ✓ Contain non-coding RNAs and proteins
  - ✓ Internalized by target cells
  - ✓ Stimulate diverse and lasting changes in cellular behavior
- CAP-1002 has been investigated in several clinical trials and more than 130 human volunteers

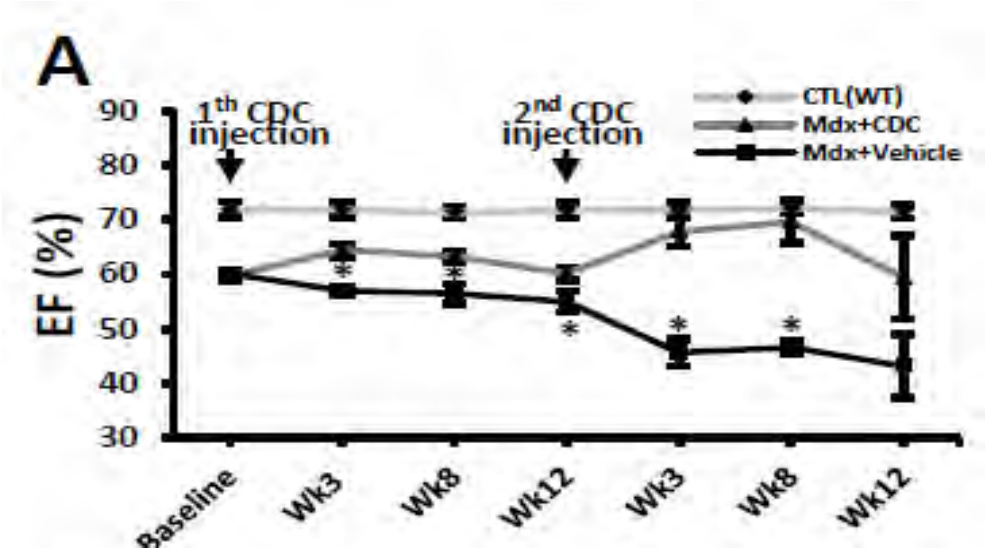


CAP-1002 Targets Multiple Disease Processes in DMD



Effects of CDCs in mdx Mouse Model

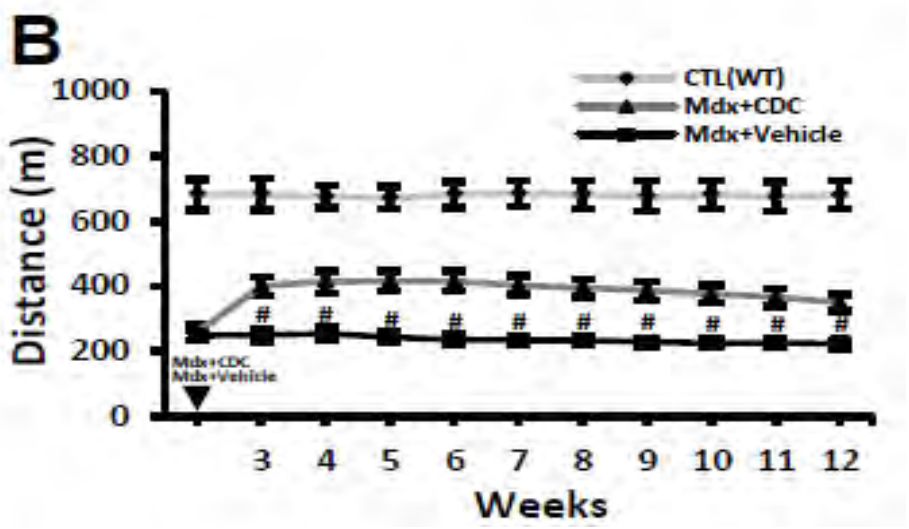
Improved cardiac function



- Left ventricular ejection fraction markedly improved vs. control

p<0.05 at all timepoints through Week 12

Increased exercise capacity

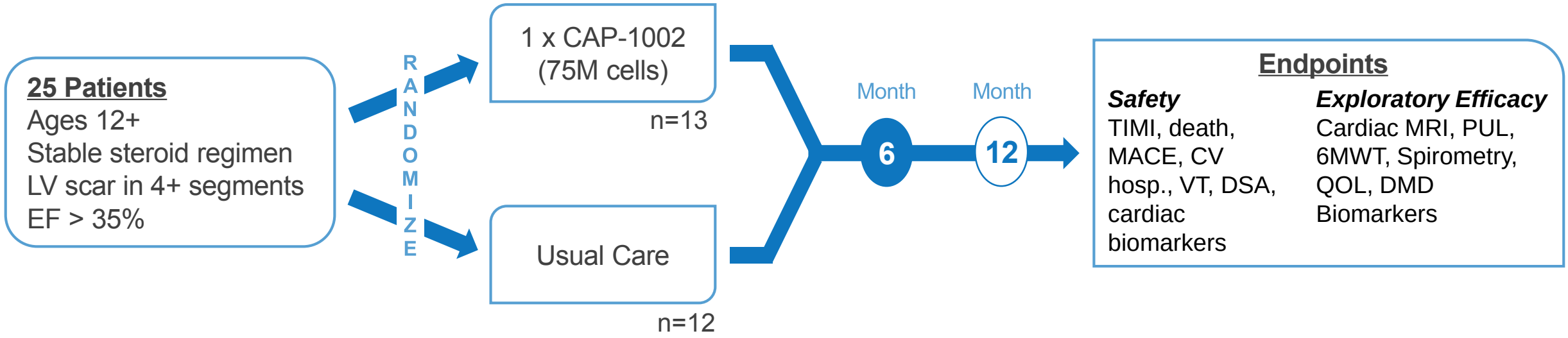


- Exercise performance approximately doubled vs. control

p<0.005 at all timepoints through Week 12

Aminzadeh MA et al. (2018). Stem Cell Reports 10(3):942-955.

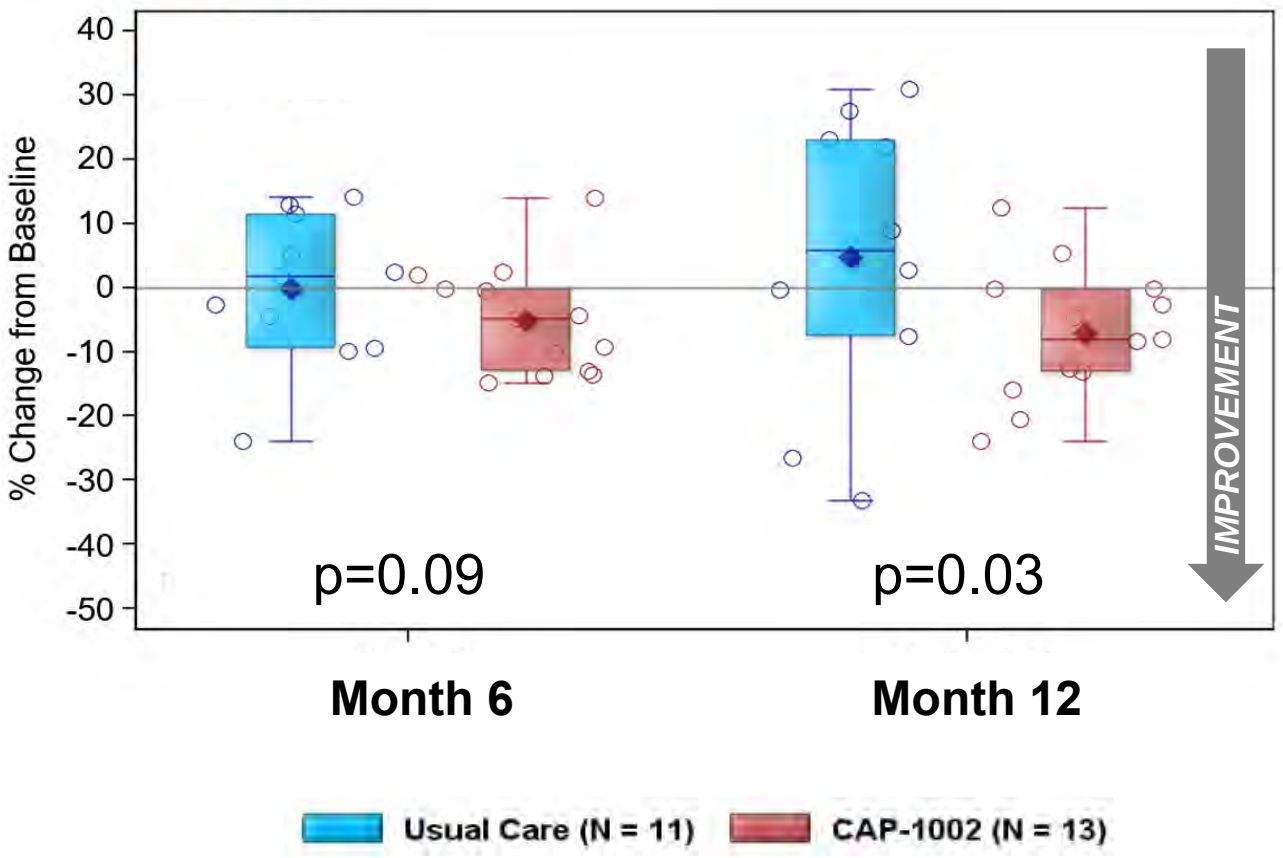
HOPE-Duchenne Trial Design



- One-time, multi-vessel, intracoronary delivery of 75M cells
- Safety trial with multiple exploratory efficacy endpoints
- Conducted at 3 clinical sites in the United States
- Enrollment population characterized by advanced disease
- Open-label extension anticipated to begin enrolling 2Q2018

Baseline	Usual Care	CAP-1002
Age, median yrs.	17.5	18
Wheelchair Use, Always	58%	77%
Cardiac Scar, mean % (SD)	21.4 (10.8)	17.6 (6.8)
LVEF, mean % (SD)	48.4 (7.5)	49.6 (6.7)

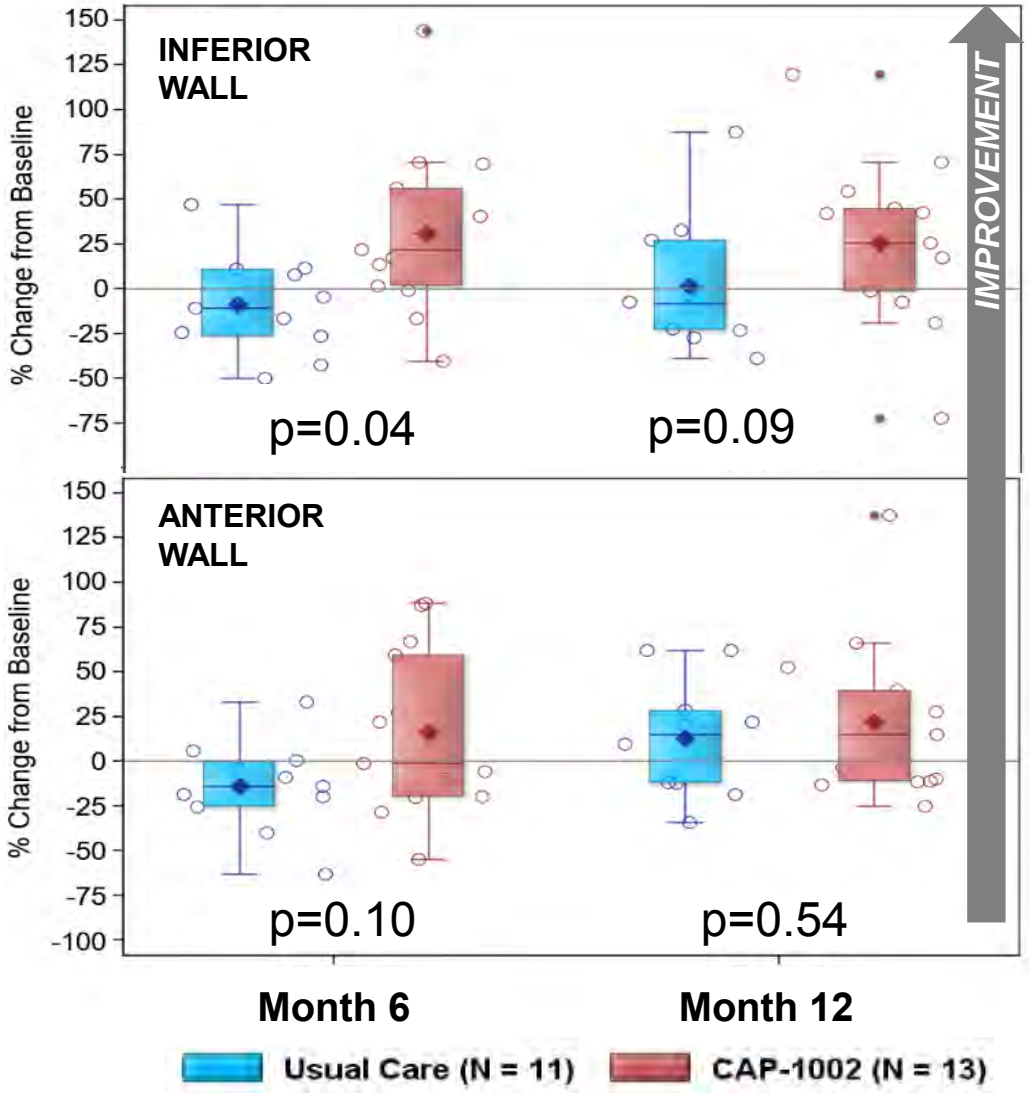
Reduction in Cardiac Scar



- Assessed by cardiac MRI with blinded analysis by core lab
- Scar increased in the Usual Care group, but decreased in the CAP-1002 group
  - 11.9% group difference in change score at Month 12 (p=0.03)
- Notable, since scar reduction is counter to the natural history of DMD

* p-values are based on absolute change from baseline

Increased Regional Systolic Wall Thickening

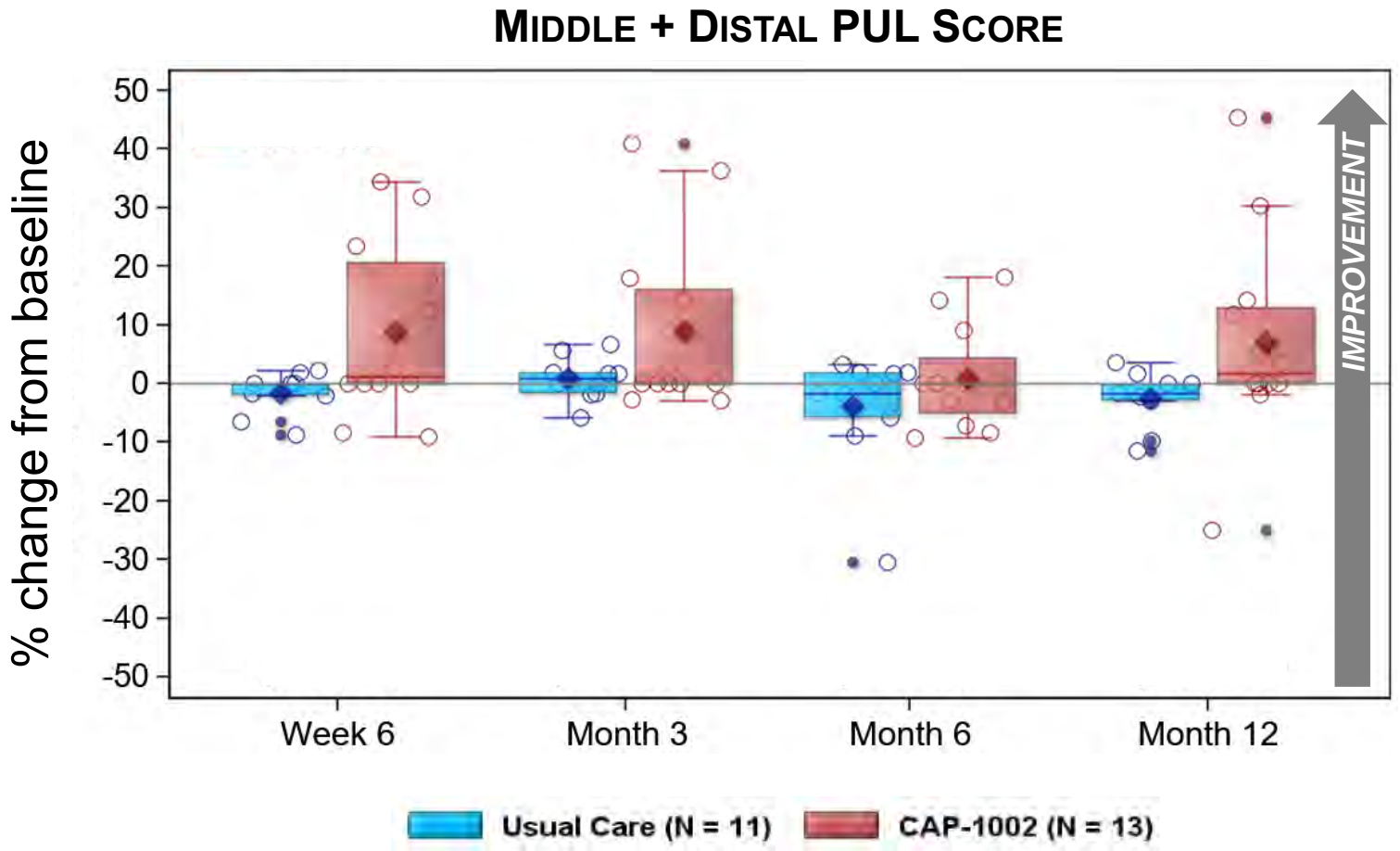


- Greatest evidence of improvement seen in inferior wall
- Similar trend in anterior wall
- Lesser trends in lateral & septal walls
- Consistent with natural history of scar progression in DMD
  - Inferior → Anterior → Lateral → Septal

* p-values are based on absolute change from baseline

Skeletal Muscle: PUL Results Indicate Functional Benefit

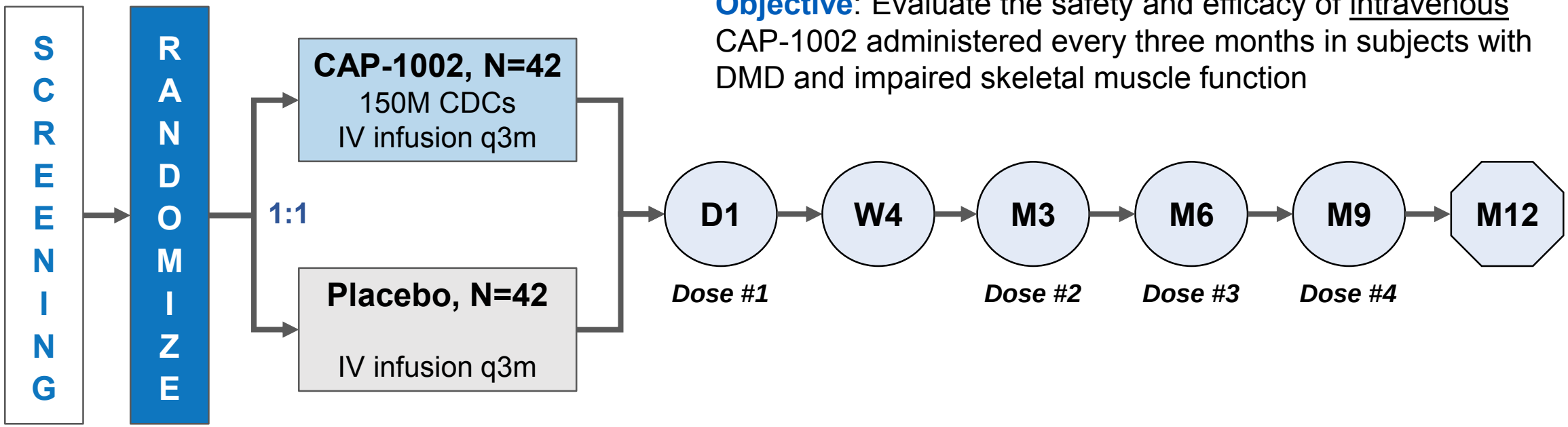
- Performance of the Upper Limb (PUL) test is a validated instrument in DMD
  - Relates to patients' ability to perform common activities of daily living
- Trends towards improvement observed throughout follow-up



Key Conclusions from HOPE Trial Results

- Early clinical data demonstrated that CAP-1002 benefits both cardiac (scar & thickening) and skeletal (PUL) muscle in DMD
- CAP-1002 (75M cells) generally safe and well-tolerated
  - Adverse events consistent with an intracoronary infusion procedure
- Sustained benefit likely to require repeat doses

HOPE-2 Trial Design



Objective: Evaluate the safety and efficacy of intravenous CAP-1002 administered every three months in subjects with DMD and impaired skeletal muscle function

- Phase 2, randomized, double-blind, placebo-controlled trial for patients 10 years and older
- Total of 4 doses of CAP-1002 or placebo solution administered via outpatient IV infusion
- About 6-7 site visits over 13 months
- Approximately 84 participants will be randomized at 10-15 medical centers in the United States

HOPE-2 Endpoints

Primary

- Upper-limb function at Month 12 by PUL
- Pre-specified safety events

Secondary

- Upper-limb function at Months 3, 6, & 9 by PUL
- Cardiac function by MRI
- Incidence and severity of AEs

Exploratory

- Elbow, grip, & pinch strength
- Pulmonary function testing
- NSAA
- Blood biomarkers
- Quality of life
- Resource utilization

HOPE-2 Eligibility

Selected Inclusion Criteria

- Genetic confirmation of DMD
- Reduced upper limb strength as measured by PUL
- Reduced ability to walk/run
- Systemic glucocorticoids for at least 12 months, and stable dose for at least 6 months

Exclusion Criteria

- LVEF < 35%
- FVC < 35%
- BMI > 45
- Mutations in DMD gene
  - Exon 44 skip-amenable
  - Deletion in exons 3-7
- FDA-approved DMD exon-skipping therapy if on stable dose for less than 24 months
- Cell therapy product within 12 months
- Investigational product within 6 months
- Ambulant if ≥ 18 years of age

*For more detailed eligibility criteria, go to clinicaltrials.gov (NCT03406780)

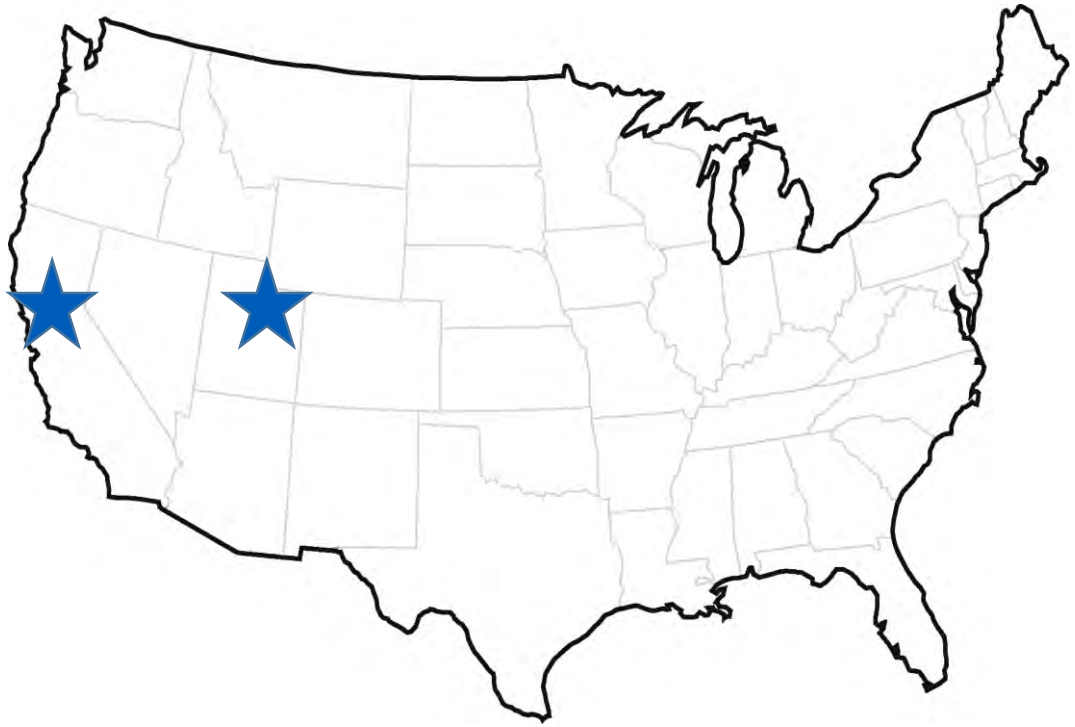
HOPE-2 Study Activity

- **Actively screening patients**
- First patient was treated in April 2018
- Currently enrolling site(s):

  University of California, Davis
  PI: Craig McDonald, MD
  Coordinator: Colleen Anthonisen
  916-734-4307
  canthonisen@ucdavis.edu

  University of Utah
  PI: Russell Butterfield, MD, PhD
  Coordinator: Kathleen O'Connor
  801-585-0892
  kathleeno@genetics.utah.edu

- More sites expected in Summer 2018



HOPE-2 Considerations

- Ambulatory and non-ambulatory boys and young men considered for enrollment
- Requires 4 intravenous infusions
- Estimated 10-15 clinical sites across the United States
- Robust travel policy to reduce burden on patients and their families

For more information, visit hope2trial.com or clinicaltrials.gov (NCT03406780)

Thank You!

- HOPE-1 trial patients
- Patient advocacy groups

- DMD Advisory Board
  - Barry Byrne, MD, PhD
  - Michelle Eagle, PhD
  - Richard Finkel, MD
  - Pat Furlong
  - Kan Hor, MD
  - John Jefferies, MD
  - Oscar Henry Mayer, MD
  - Craig McDonald, MD
  - Eugenio Mercuri, MD, PhD
  - Franceso Muntoni, MD
  - Michael Taylor, MD
  - Ron Victor, MD
  - Thomas Voit, MD