The CardiAMP Heart Failure Trial: Efficacy Outcomes from Roll-In Phase

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Disclosures

P.V. Johnston: Consultant/Advisory Board; Modest; Biocardia, Inc.
H.J. Duckers: Employment; Significant; BioCardia, Inc. Ownership Interest; Significant; BioCardia, Inc.
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C.J. Pepine: Consultant/Advisory Board; Modest; Biocardia, Inc.

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CardiAMP-HF Study Rationale

Stem cell therapy shows promise in experimental models of ischemic cardiomyopathy, but results from clinical trials are variable.

Potential reasons include:
- Variable potency of autologous cells
- Poor cell retention and survival following delivery
- Sub-optimal dosing

The CardiAMP Heart Failure Trial (CardiAMP-HF) was designed to address these shortcomings through:
- Prospective assessment of cell potency
- Intramyocardial delivery using helical injection catheter to maximize retention
- High cell dose (200M bone marrow mononuclear cells)
Clinical Trial Design

Prospective, randomized, blinded, sham-controlled multi-center clinical trial

250 patients + 10 patient “Roll-In” Cohort (open-label)

Enrollment criteria
- Ischemic cardiomyopathy with EF 20-40%
- NYHA Class II-III symptomatic CHF
- Optimal medical therapy +/- CRT
- Sufficient cell potency score on screening bone marrow aspiration

Outcomes
- 1º Endpoint: Change in 6MWD at 12 months
- 2º Endpoints: NYHA HF Class, MLWHFQ, Echocardiography: LVEF, Wall motion

Roll-In Cohort follow-up complete to 12 months
1º Endpoint: Change in 6-Min Walk Distance

Change in 6MWD Relative to Baseline

- 8/10 patients had improved 6MWD at 12 months

Δ6MWD (m)

- +33.7
- +47.8
- +58.1
- +46.4

TAC HFT
+14.3 m#

- p=0.21
- *p=0.01
- p=0.36
- p=0.06

Mean +/-SEM; n=10, except at 9 months, when n=9
8/10 patients had improved 6MWD at 12 months

#Wong Po Foo et al, World Congress of Regenerative Medicine 2015
Clinical Assessment of Heart Failure Severity

Distribution NYHA Heart Failure Class

Mean +/-SEM; n=10 for NYHA HF Class at 3 & 6 mos, n=9 at 9 & 12 mos

*p=0.015   *p=0.037   p=0.194   p=0.183

#Wong Po Foo et al, World Congress of Regenerative Medicine 2015
LV Function Assessment at 12 months

Global LV Function

<table>
<thead>
<tr>
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<th>Baseline</th>
<th>6 Months</th>
<th>12 Months</th>
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</thead>
<tbody>
<tr>
<td>LVEF (%)</td>
<td>28.9</td>
<td>29.1</td>
<td>32.9</td>
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</table>

Change in LV Function

<table>
<thead>
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<th>Baseline</th>
<th>6 Months</th>
<th>12 Months</th>
</tr>
</thead>
<tbody>
<tr>
<td>Change in LVEF (%)</td>
<td>+1.0</td>
<td>+4.1</td>
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</table>

p=0.49

Mean +/-SEM; n=10; Transthoracic echocardiograms assessed by blinded readers in Echo Core Lab (Yale School of Medicine)
Decrease in Akinetic Wall Segments

Mean +/-SEM; n=10; Transthoracic Echocardiograms assessed by blinded readers in Echo Core Lab (Yale School of Medicine)

Pre-specified 2º Endpoint: Recruitment of myocardial segments

- Baseline
  - Akinetic Segments: 6.1
  - Change: -1.1 (p=0.08)

- 6 Months
  - Akinetic Segments: 5.0
  - Change: -1.9 (*p=0.04)

- 12 Months
  - Akinetic Segments: 4.2

Bar charts showing the decrease in akinetic wall segments over time.
Improvement in Wall Motion Score

**Total Wall Motion**

<table>
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<tr>
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<th>Mean Wall Motion Score</th>
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<tbody>
<tr>
<td>Baseline</td>
<td>36.5</td>
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<tr>
<td>6 Months</td>
<td>33.5</td>
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<tr>
<td>12 Months</td>
<td>30.6</td>
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**Change in Wall Motion Score**

<table>
<thead>
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<th>ΔWall Motion Score</th>
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<tbody>
<tr>
<td>Baseline</td>
<td></td>
</tr>
<tr>
<td>6 Months</td>
<td>-3.0</td>
</tr>
<tr>
<td>12 Months</td>
<td>-5.9</td>
</tr>
</tbody>
</table>

- Mean +/-SEM; n=10; Transthoracic Echocardiograms assessed by blinded readers in Echo Core Lab (Yale School of Medicine)
- Pre-specified 2º Endpoint: Recruitment of myocardial segments

*p=0.01*
Summary

12-month Results from CardiAMP-HF Roll-In Phase Show:

- Improved 6MWD at 6 mos (p=0.01); borderline at 12 mos (p=0.06)
- Trends for improved NYHA HF Class and MLWHFQ Score
- Recruitment of akinetic wall segments and improved wall motion at 12 mos
- Low MACE (0 treatment related, 1 f/u MACE event, All patients alive at 12 month f/u)

Limitations

- Open label (all patients received cells)
- Small size (n=10)

CardiAMP-HF Randomized Study is actively enrolling

- 250 patients to be enrolled at 40 centers in US
- 3:2 Autologous BMMNCs : Sham
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