Augment-HF Discussion

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Disclosures

• Research
  – Amgen
  – AstraZeneca
  – AHRQ
  – Bayer
  – Merck
  – Novartis
  – NHLBI
  – PCORI

• Honorarium
  – Amgen
  – Janssen
  – Merck
  – Myokardia
  – Novartis
  – Pluristem
  – Sensible
Key Goals for Extended Follow-up

• Durability of effects
• Long-term safety
• Considerations for the next study
3 Questions

Are the results biologically possible and consistent?

Are there substantive methodological concerns?

Is there sufficient evidence of safety to proceed with larger studies?

Felker GM, Eur Heart J. 2015 Sep 7:2276-8
Methodological concerns?

• **Strengths**
  – Randomization
  – Objective primary endpoint
  – Core labs to ensure quality and consistency

• **Weaknesses**
  – Unblinded (Potentially too difficult?)
    • Problematic for NYHA & quality of life
  – Missing data
    • CPX is difficult for follow-up
    • High mortality
  – External validity outside the highly select sites
Is there sufficient evidence to proceed with larger studies?

YES, but....

- Will need to be a larger study to understand the benefits vs. risks (ideally with blinding)
  - Functional status vs. mortality

- Will need to consider a primary endpoint that is a patient-centered outcome (with regulatory approval)
  - What do patients want?

- Will need to have complete data

- Will need to be generalizable to the intended markets
Summary

• **Augment-HF showed potentially durable effects of algisyl in patients with severe heart failure**

• **Small number of events make it difficult to evaluate risk:benefit ratio**

• **One year follow-up data keeps the door open for a potentially new treatment pathway**