EFFECTING Heart Failure

Adrian F. Hernandez, MD, MHS
Duke Clinical Research Institute
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Trial Design

• Cardiopulmonary exercise testing
  • Prognostically important
  • Objective measure of functional capacity

• However….CPX is often difficult test in a clinical trial setting
  • Quality
  • Reproducibility
  • Ensuring maximal effort
  • Completeness
Meeting the Guiding Principles

• **Right Patient:** Real World vs. Clinical Trial World
  • Able to do a CPX
  • Screening led to a bias population? (525→174)

• **Right Intervention:** Complete, blinding…
  • Open label, assessor blinded
  • On good background therapy

• **Right Outcomes:** Complete without bias
  • Missing from primary analysis?
  • Missing from per-protocol analysis?
Outcomes Matter!

- Heart Failure is a syndrome characterized by DYSPNEA, FATIGUE.…
  - Exercise capacity is an outcome that matters to patients!

- What about the imbalance in readmissions?
  - Small numbers, small numbers.…
  - Perhaps imbalance in baseline characteristics
What should be done next?

• Ferric carboxymaltose has been shown to improve...
  • Global assessment
  • NYHA class
  • 6 minute walk
  • Peak V02

• Now what?
  • Outcomes Patients Prefer
    • Survival
    • Worsening heart failure
    • Every day functional status…

• Larger, longer trials