Pilot Studies

Michael L. Terrin, M.D., C.M., M.P.H.
University of Maryland School of Medicine
Claude D. Pepper Older Americans Independence Center (OAIC)

Pilot Study (Meinert) –
A small preliminary study performed as a possible prelude to a full-scale study and intended to provide training and experience in carrying out such a study, if undertaken.

Pilot Definition

Pilot (Webster) –
A guide; a director or a leader.

Pilot (Meinert) –
A guide or leader.

Purpose Beyond Definition

• Estimate Parameters of Design
• Feasibility of Component Efforts
• Develop, Test and Practice Procedures
• Provide Preliminary Data
• Make Decisions on Proceeding to Full-Scale Study

Estimate Parameters of Design

• Screening Yield – FOCUS
• Measure Scale or Event Rates – TIMI I
• Variability – MSH
• Cross Overs/ Adherence – FOCUS
• Losses to Follow-Up/ Missing Data – N-TA^CT
• Retention – TIMI I, TIMI II
• Treatment Effect – IMPACT
• Correlation Structures - N-TA^CT

Feasibility of Component Efforts

• Recruitment – FOCUS
• Treatment Assignment – TIMI II
• Most Challenging Intervention – TIMI II
• Outcome Ascertainment – ACIP
Develop, Test and Practice Procedures

- New Approaches – TIMI II
- Logistics – NHLBI/NINDS

Provide Preliminary Data

- Necessary for Seeking Support
- New NHLBI Initiative (R34)
- Traditional R21
- Center Grants

Design

May Not Have to Be the Same as the Full-Scale Study

- Open Label – MSH
- Uncontrolled – TIMI II
- Parallel/ Cross Over/ Factorial – TIMI I
- Multiple Treatment – CDPA

Make Decisions on Proceeding to Full-Scale Study

- Pre-Determined Rules – DOVCAR

Types of Pilot Studies – Uncontrolled

- Phase I – AZIP
- Phase IIa – Routine Industry
- External – MSH

Types of Pilot Studies – Controlled

- Phase IIb – IMPACT
- External – ACIP; FOCUS
- Internal – DCCT
Controls

Necessary for Comparisons Only

Kinds of Controls
- Historical
- Concurrent (Observational)
- Randomized

Desirable Feature of Controls
- Good Estimate of the Parameter to Be Compared

Pilot Design

- Patient Population
- Exposure(s)
- Assignment(s)
- Number of Observations
- Duration
- In vivo/ In Silico
- Outcomes

Conclusions

The need for and design of any pilot study is determined by the specific information necessary and the purpose to which that information will be put.

With purposeful planning, a pilot study can be valuable either as a controlled or uncontrolled effort.

The closer a pilot is to the full-scale study anticipated, the more definitive it can be.