Elements of Successful Product Development for Rare Disease

• Patient/Family Advocacy
  – Awareness
  – Funding
  – Helping establish
    • Investigative network
      – Basic Science
      – Clinical
    • Industry partner(s)
    • Regulatory partnership
  – DEFINING THE CONDITION
    • NATURAL HISTORY
    • PATIENT-CENTRIC OUTCOMES
Translational Science

- Genomics
- Pathogenesis
- Model systems
  - Animal models
  - Cell-based models
- Defining/validating a “drug-able” target
  - Reagent discovery
  - Drug development (including formulation across age spectrum)
- Validating/qualifying biomarkers
- Defining/Validating clinical outcomes
  - The more patient centric, the more likely to yield
    - Meaningful clinical trials
    - Ultimate adherence to therapy