

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