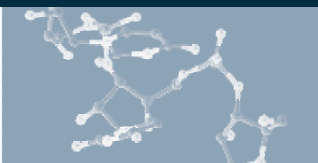


Challenges in Drug Development for Muscle Disease

Introduction to Drug Development

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Drug Development Issues in Muscle Disease

- Unique natural histories for different diseases
- Few clinical or regulatory therapeutic precedents establishing endpoints
 - What are endpoints that document changes in patient well-being based on natural history?
 - What are endpoints that measurably, reliably, detectably and rapidly change in response to pharmacological intervention?
 - What are endpoint changes that predict or document “clinical benefit”?
- Unique population characteristics
 - Small numbers of patients
 - Pediatric patients
 - Sick patients
- Unique benefit:risk equations
- Return on investment concerns
 - Risks, costs, time versus potential returns (clinical and commercial)
 - Patients, MDA, NIH, FDA, industry (big and little pharma)

Addressing Developmental Needs

- Resources

- Funding
- People
- Effort

- Communication

- Patients/patient advocates
- Investigators
- Facilitators
- Regulators
- Drug developers

- Reflection

- Knowledge
- Assessment
- Organization
- Innovation and planning

- Common Understanding

- Viewpoints
- Opinions
- Conjectures
- Hypotheses

Solutions!!!

Challenges in Drug Development for Muscle Disease

- Wilson Bryan, MD
Center for Drug Evaluation and Research, FDA
- Marc Walton, PhD
Center for Drug Evaluation and Research, FDA
- Edward Kaye, MD
Clinical Research, Genzyme
- Cristina Csimma, RPh, MHP
Translational Research, Wyeth

