Don’t Do Different Things...
Do Things Differently!
Drug Development in Rare Diseases

ASCPT Annual Meeting
11 March 2016
Introductions and Housekeeping

• Joan Korth-Bradley, PharmD, PhD (Pfizer Inc)
• Michelle Rudek, PharmD, PhD (Johns Hopkins)

• Brief clarifying questions after each speaker
• Question and answer session after last speaker
Why Are We Here?

• Urgent need for treatment options
  – more than 7000 rare diseases
  – 95% of these have no approved drug therapy
• Drug product development high standards
• Bridging the gaps
  – Collaboration with community
  – Innovative development plans
  – Innovative study designs
  – Innovative regulatory consultation and review
  – Innovative supply chain and distribution
One Patient

- I-Cell (ML2)
- Inherited lysosomal storage disease, first described in 1967
- 1:640,000 live births
- mannose-6-phosphate missing from lysosomes
- phase-dense cytoplasmic inclusions (I-cell) in fibroblasts

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Symposium Agenda

• David Swinney, PhD, Institute for Rare and Neglected Diseases
  – The Challenges and Hurdles to Business as Usual in Drug Development for Treatment of Rare Diseases
• Indranil (Neil) Bhattacharya, PhD, Pfizer Inc
  – Making Every Subject Count: a Case Study of the Drug Development Path for a Medication in a Pediatric Rare Disease
• E. Dennis Bashaw, PharmD, CDER, FDA
  – Regulatory Perspectives on the Approval of Rare Diseases
• Lorna Speid, PhD, Rare Diseases Patients First!
  – A Patient’s Perspective of Rare Diseases