# Don’t Do Different Things… Do Things Differently!

## Drug Development in Rare Diseases

ASCPT Annual Meeting

11 March 2016

# Introductions and Housekeeping

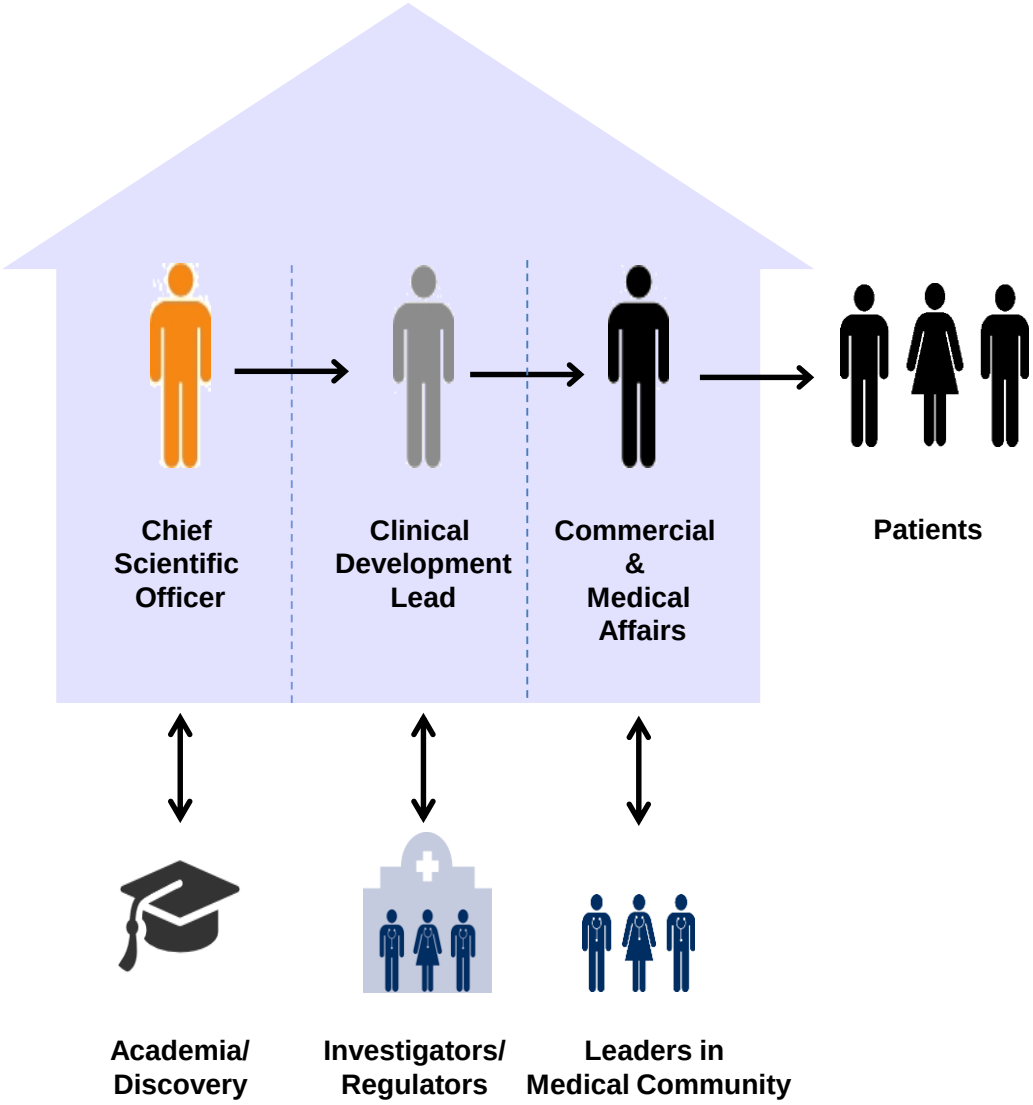
- Joan Korth-Bradley, PharmD, PhD (Pfizer Inc)
- Michelle Rudek, PharmD, PhD (Johns Hopkins)

- Brief clarifying questions after each speaker
- Question and answer session after last speaker

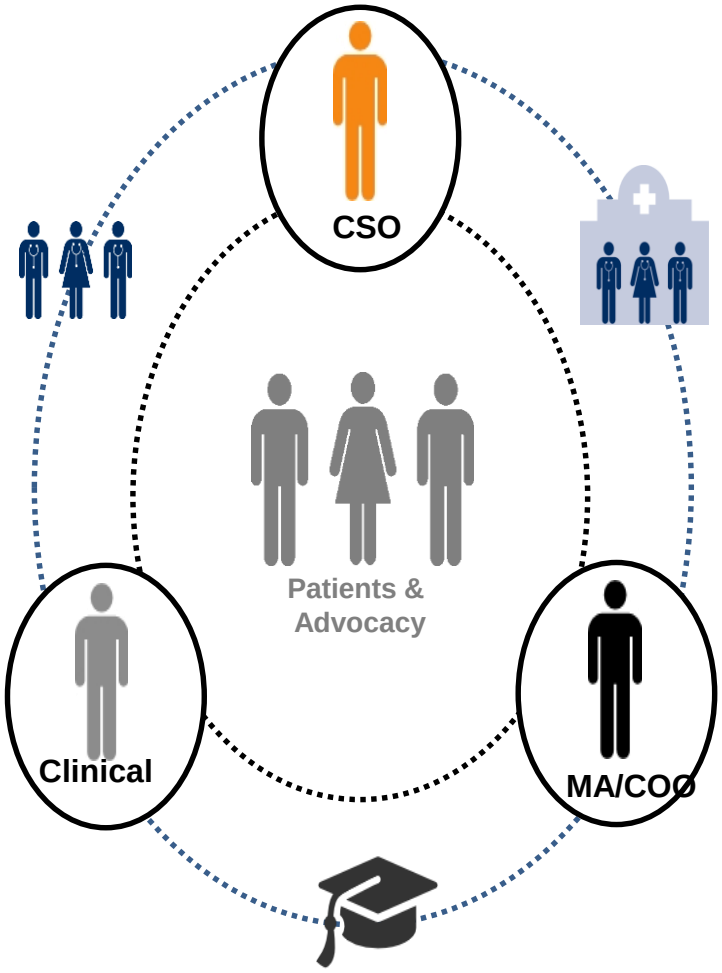
# Why Are We Here?

- 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

# Regular Development Paradigm

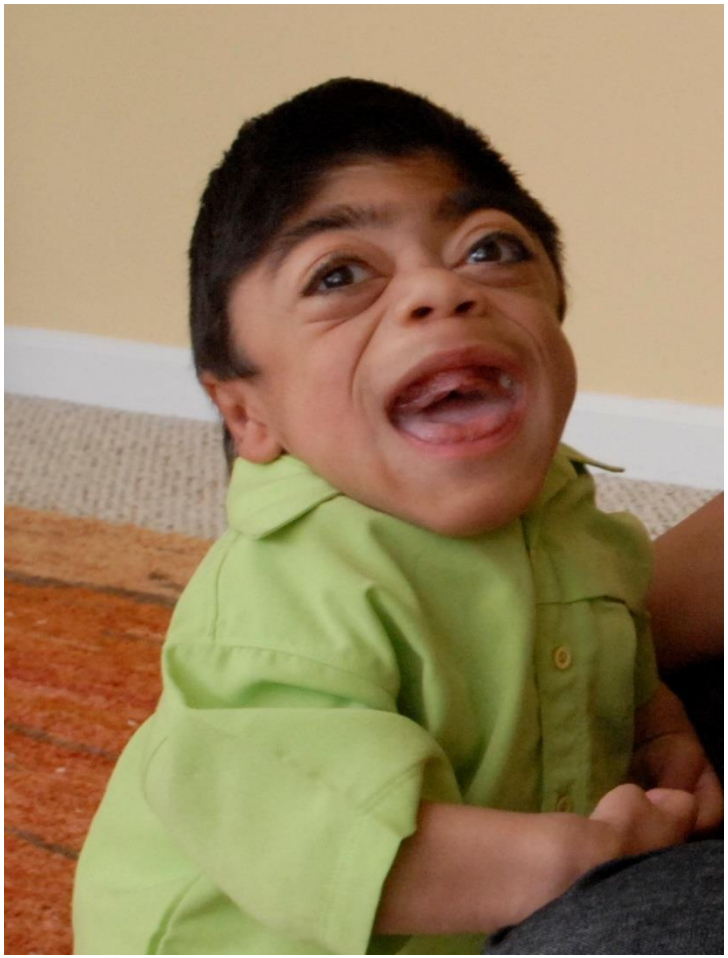


# Rare Disease Development



# 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