

# Novel business models for orphan drug development

Hans A. Büller, M.D. Ph.D  
ErasmusMC  
Rotterdam, The Netherlands

ICORD Ede 2014

# Need for new approach

- Orphan drug act is working
- Prices are very high
- Societal burden is increasing
- Many orphan drugs have been developed
- Many orphan drugs are too expensive (abuse)

# Need for new approach

- Extent of use and reimbursement differs
- Prices stay very high even after patent expiration
- Market failure
- Personalised Medicine; tsunami of orphan drugs
  - **Need for another approach**

# Drug development 'Classic'

- (Academic) research leads to patent
- Often public money
- License to pharma company
- All risks, finances and profit for company
- One party is in charge

# Orphan Drug Act 1983

- 'The pharmaceutical industry has taken advantage of the initiatives to charge excessive profits and to reap windfalls far in excess of their investments in the drug'
- Henry Waxman, primary sponsor of the ODA, 2013

# Drug development 'Classic'

- A 'pharmaceutical' crises
- Bank financial crises in 2008
- Classic businessmodel
- Double digit profits
  - **Need for another approach**

# New business model

- Not one entity is responsible
- Form coalition of important participants
- Patients, M.D's, UMC, investors
- Also including Pharma Companies

# New business model

- Bring participants together on the basis of trust
- With shared responsibility, shared financial input
- Shared risks and profits
  - **Fair Medicine Approach**

# New business model

- Form a social enterprise
- Investors are focussed on societal needs
- Not on share holder profits/value
- Prices should be transparent
  - **Fair Medicine Approach**

# New business model

- Prices are based on cost of goods
- Including investment and research costs
- With a societal acceptable profit margin
- Thus, drugs developed for and with patients
- Together with all other important participants

**Patient driven drug development**

# Novel business models for orphan drug development

- **SUMMARY I**
- Orphan drug act is working
- Prices are very high
- Tsunami of orphan drugs
- Classic drug development

# Novel business models for orphan drug development

- **SUMMARY II**
- Public money in research
- Patent
- All burden on Pharma Company
- All profits for company
- Prices are not transparent

# Novel business models for orphan drug development

- **SUMMARY III** Fair Medicine
- Coalition of all important Participants
- Trust, risk and profit sharing
- Investors with eye for societal need
- Transparent prices and acceptable profit margins