Afprøvning af medicin på sjældne områder: hvad er udfordringerne

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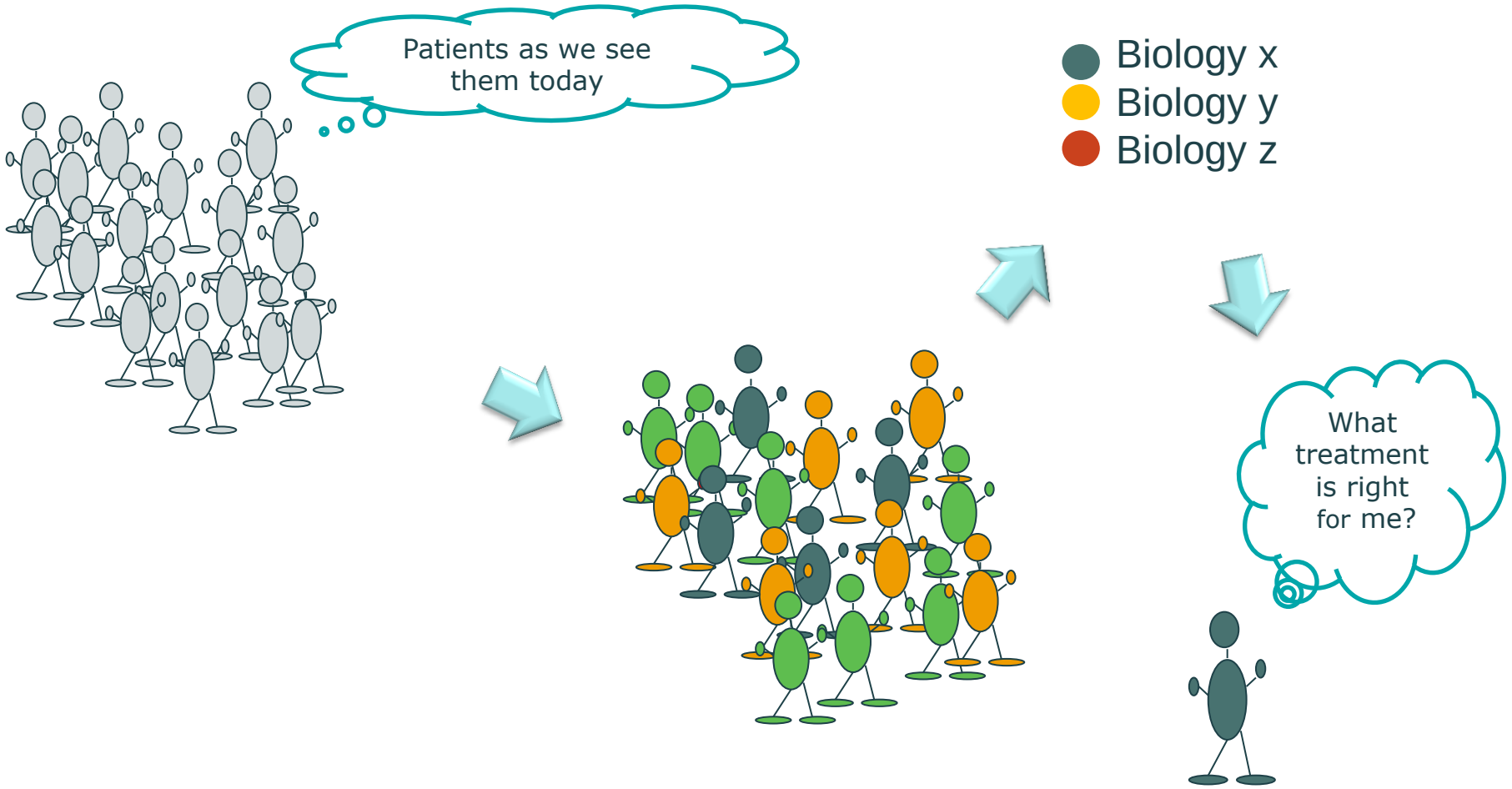
What is a “Rare Disease”

There is no single, widely accepted definition of rare (or orphan) diseases.

- USA: “any disease with a prevalence about 1 in 1,500 people”. (Orphan Drug Act of 1983)
- Japan: “any disease that affects about 1 in 2,500 people.”
- EU: "life-threatening or chronically debilitating diseases ….with *low prevalence* defined as <1 in 2,000 people”. (eur-lex, 2009)
- Denmark ”a prevalens on 1 – 2 in 10.000 people” (sundhedsstyrelsen 2014)
- The medical literature …… definitions ranging from 1/1,000 to 1/200,000.

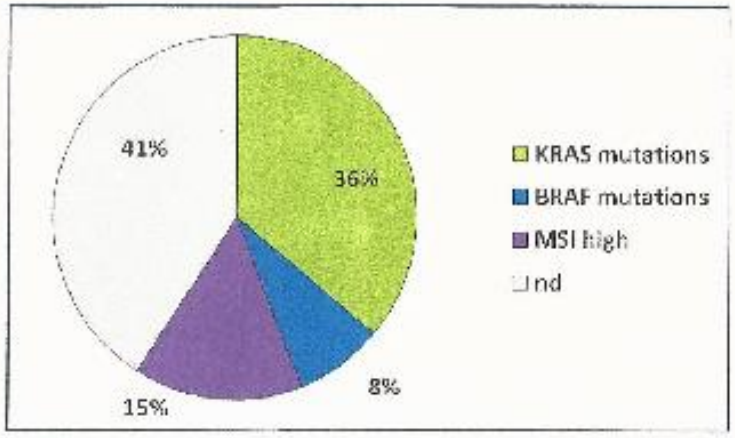
Nobody expected genetics to come around ……



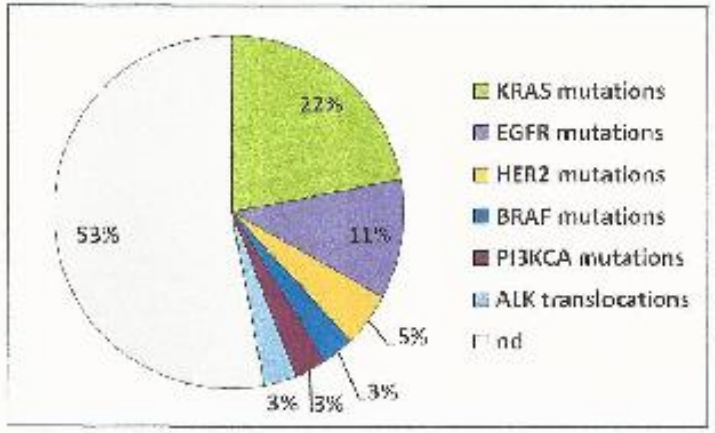
Molecular genetics deciphers severe frequent cancers into specific rare cancers

The shift of paradigm : towards molecular subsets of cancers

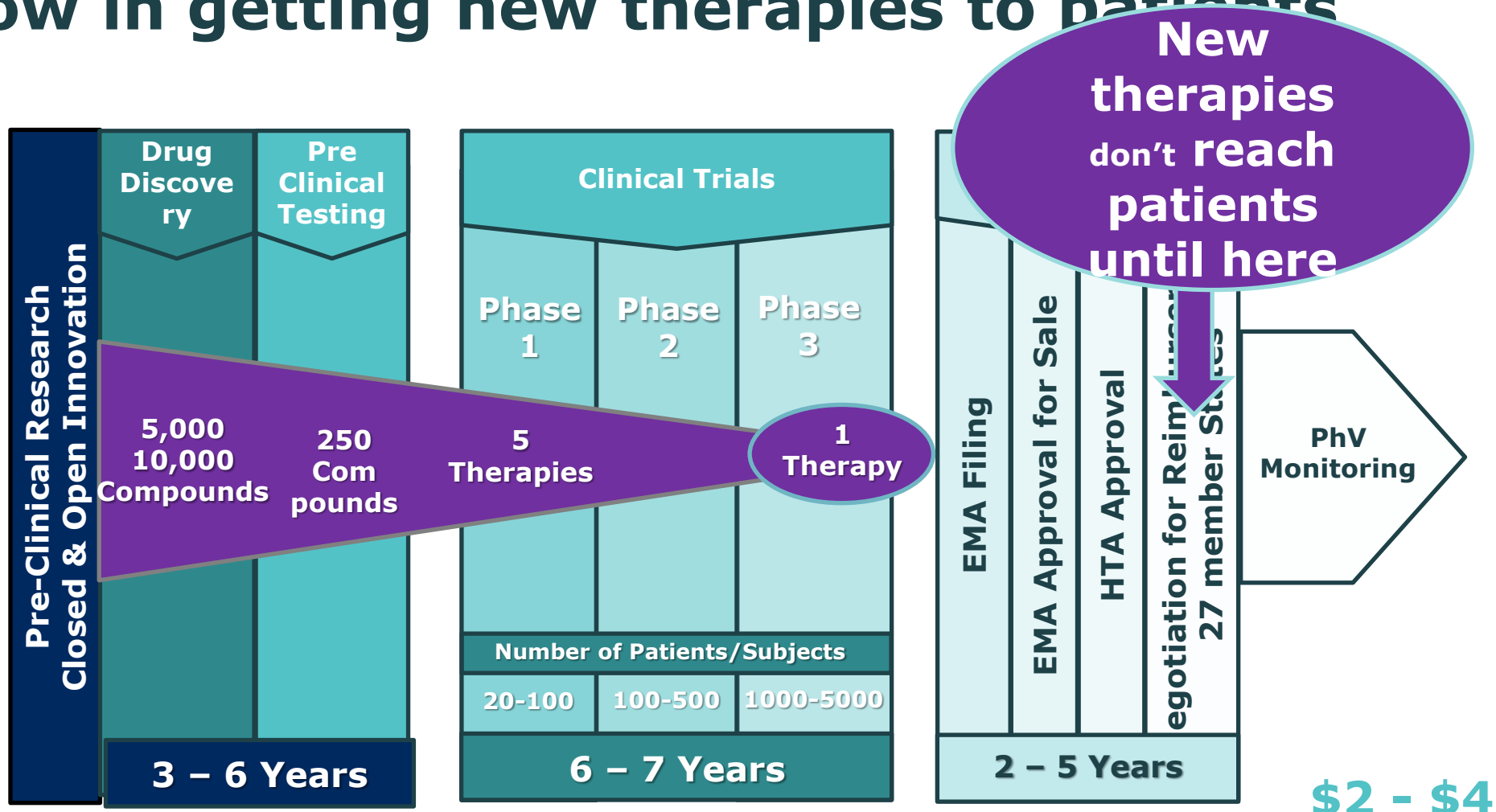
Molecular subsets of **colorectal cancer** : 18,000 patients



Molecular subsets of **non small cell lung cancer** : 16,000 patients



Current EU regulatory pathways are expensive and slow in getting new therapies to patients



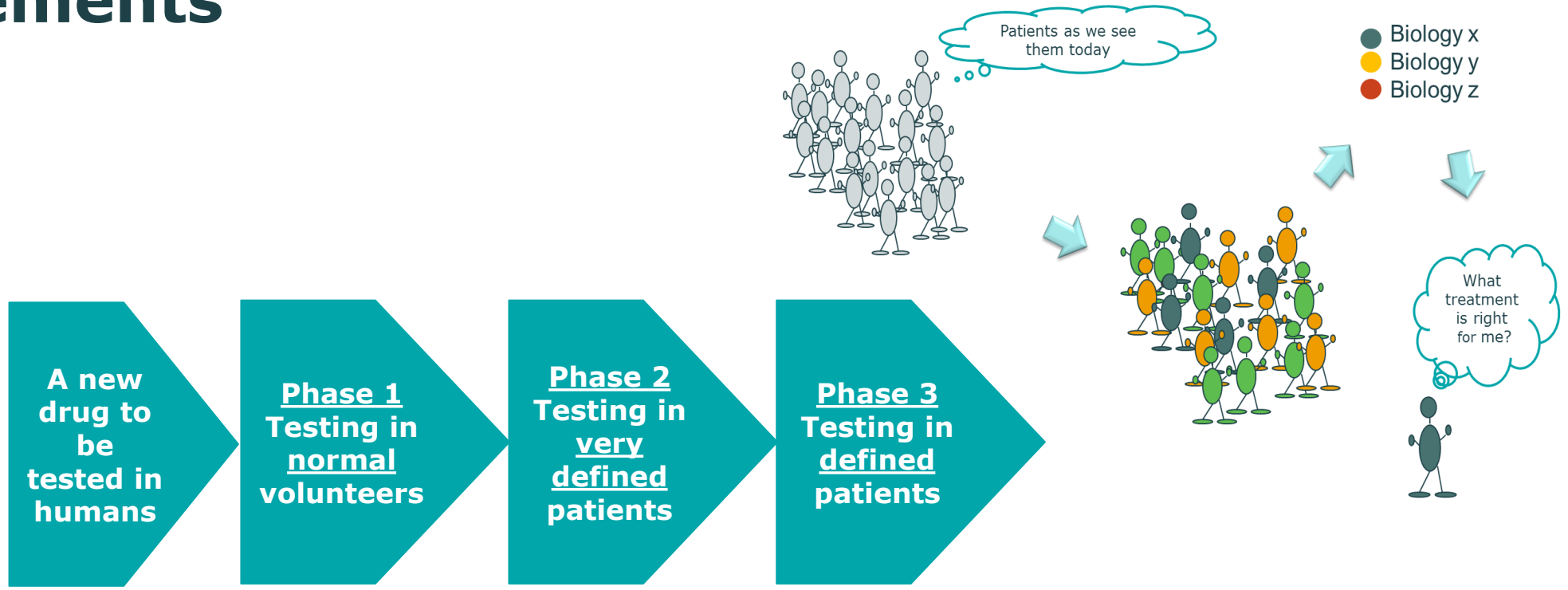
Sources: Drug Discovery and Development: Understanding the R&D Process, www.innovation.org;

CBO, Research and Development in the Pharmaceutical Industry, 2006;

Forbes, Matthew Herper, "The Truly Staggering Cost Of Inventing New Drugs", February 10, 2012

"The average drug developed by a major pharmaceutical company costs at least $4 billion, and it can be as much as $11 billion."

The thinking behind conventional regulatory requirements



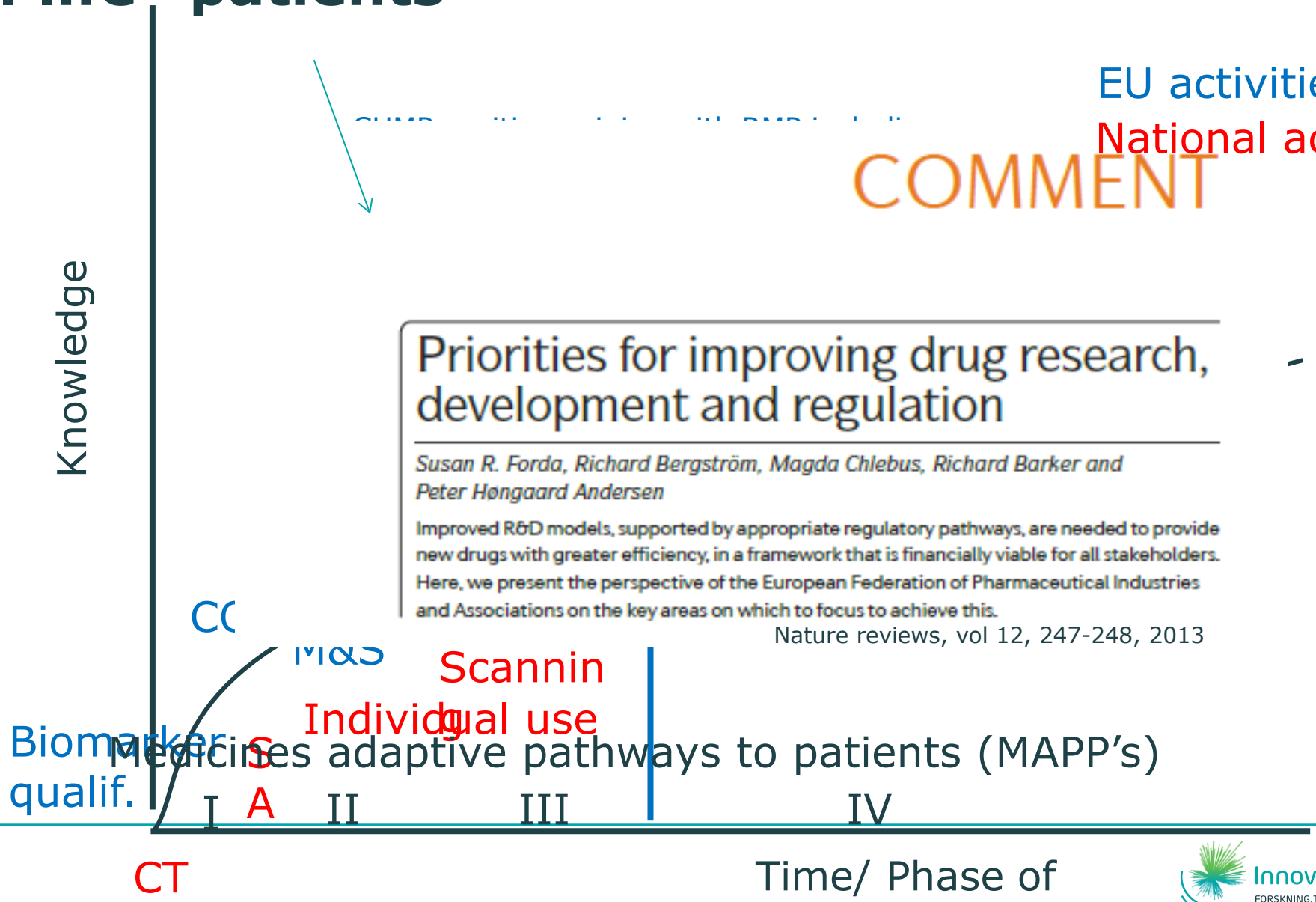
It's all about getting Benefit and Risk defined so your B/R ratio can be calculated with some certainty

Overview of Regulatory hurdles on the way to the "real life" patients

EU activities

National activities

COMMENT



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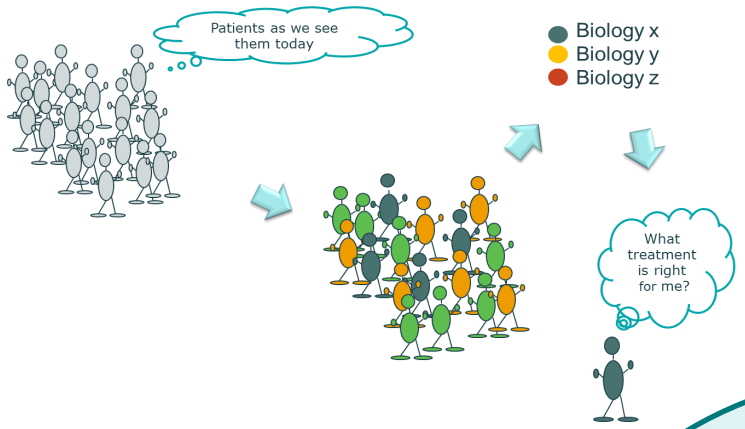
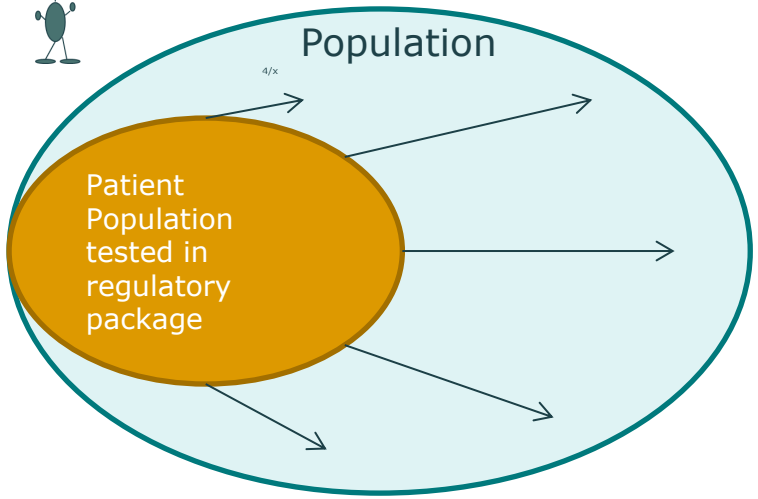
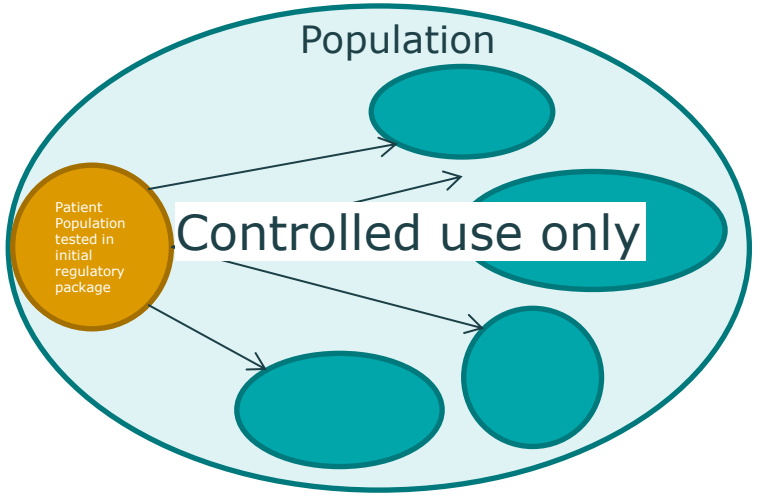
Medicine adaptive pathways to patients (MAPPs)



Today



Future



We need a new and sustainable business model for personalized medicine, which benefits both patient, society, industry and healthcare budgets

…And the problems

- Genetics will make all diseases (more or less) orphan diseases
- It takes too long time to translate scientific discoveries to new medicines
- New medicines are generally (too) expensive especially orphan drugs
- Regulators are typically “hyper-cautious” – in defining the benefit/Risk
- Few countries have systems in place for “controlled use” and even fewer countries have comprehensive patient registries in place
- Health Care systems are really disease care systems
- Generally genetics are not a ”standard discipline” in our Health Care system

…. And the solutions

MAPPs:

- Progressive Patient access – step-wise release of new medicines
- The regulatory systems needs to be adapted to "orphan disease" thinking:
  - Change of clinical trial set-up
  - Benefit/risk – based on patient input to get a "real-life" judgement
  - Controlled access – combined with patient registries
- Economics – it will cost switching to personalized medicine but:
  - We will only treat "responders" = major savings
  - We need to develop adaptive Pharma economic models based on value based prizing (prize and exclusivity timing)
- The Health Care system
  - Needs a technology upgrade – genetics and molecular biology should be "everyday life"
  - The internal setup of the Health Care system needs a major revision = "Cowboys and pit crews" (*A. Gawande, the New Yorker, 2011*)

Thx for your attention