Remaining obstacles towards optimal pharmaceutical development of rare cancer treatments

Andras Fehervary

Head Pricing Market Access & External Affairs Region Europe Oncology

Rare Cancers Europe Brussels, February 10th 2012

NOVARTIS ONCOLOGY

Drug Discovery and Innovation
The Core Dilemma

A. PHARMA INDUSTRY

1. Higher R&D Costs
2. Falling R&D Productivity
3. Smaller Return on Investments
4. Declining number approvals

B. HEALTH AUTHORITIES

1. Increasing/ Higher costs of drugs
2. Higher epidemiology in many cancer types
3. Uncertainty in outcomes

European Medicines Agency

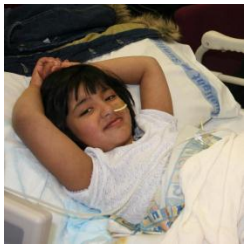
Discover, develop and make accessible effective therapies for rare tumors

C. PHYSICIANS

1. Need more effective use of treatments
2. Need for high caliber Scientific Projects
3. CoE/Reference networks

D. PATIENTS (ASSOCIATIONS)

1. Access to effective and safe treatments
2. Information
3. Quality care



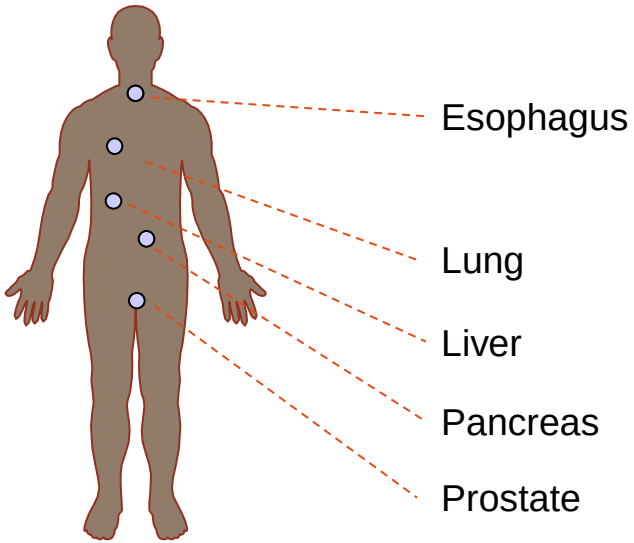
They all have a common goal ...

Major obstacles to develop rare cancers therapies

Old vs new paradigm ... Has regulatory framework kept up?

OLD PARADIGM

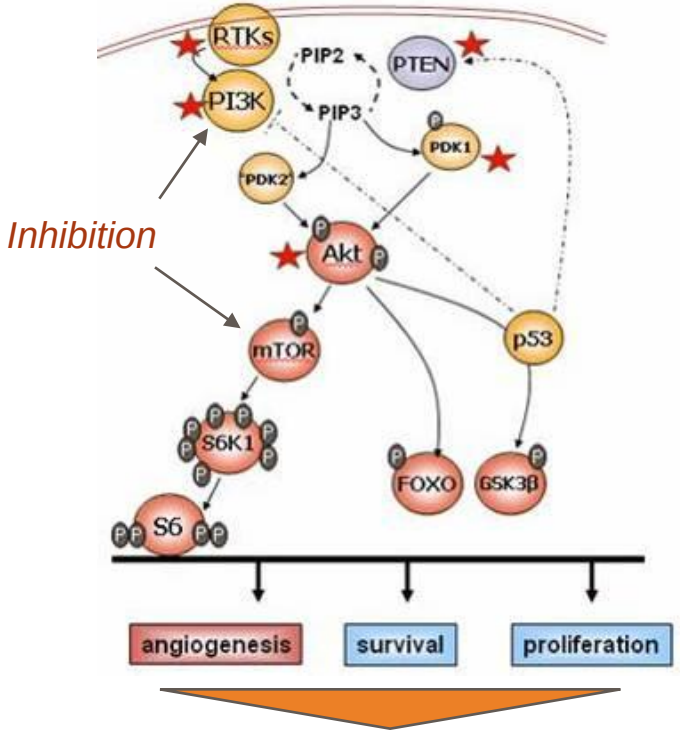
Targeting localized tumors with chemotherapy, combinations or use of specific drugs



One specific therapy for each localized tumor

NEW PARADIGM

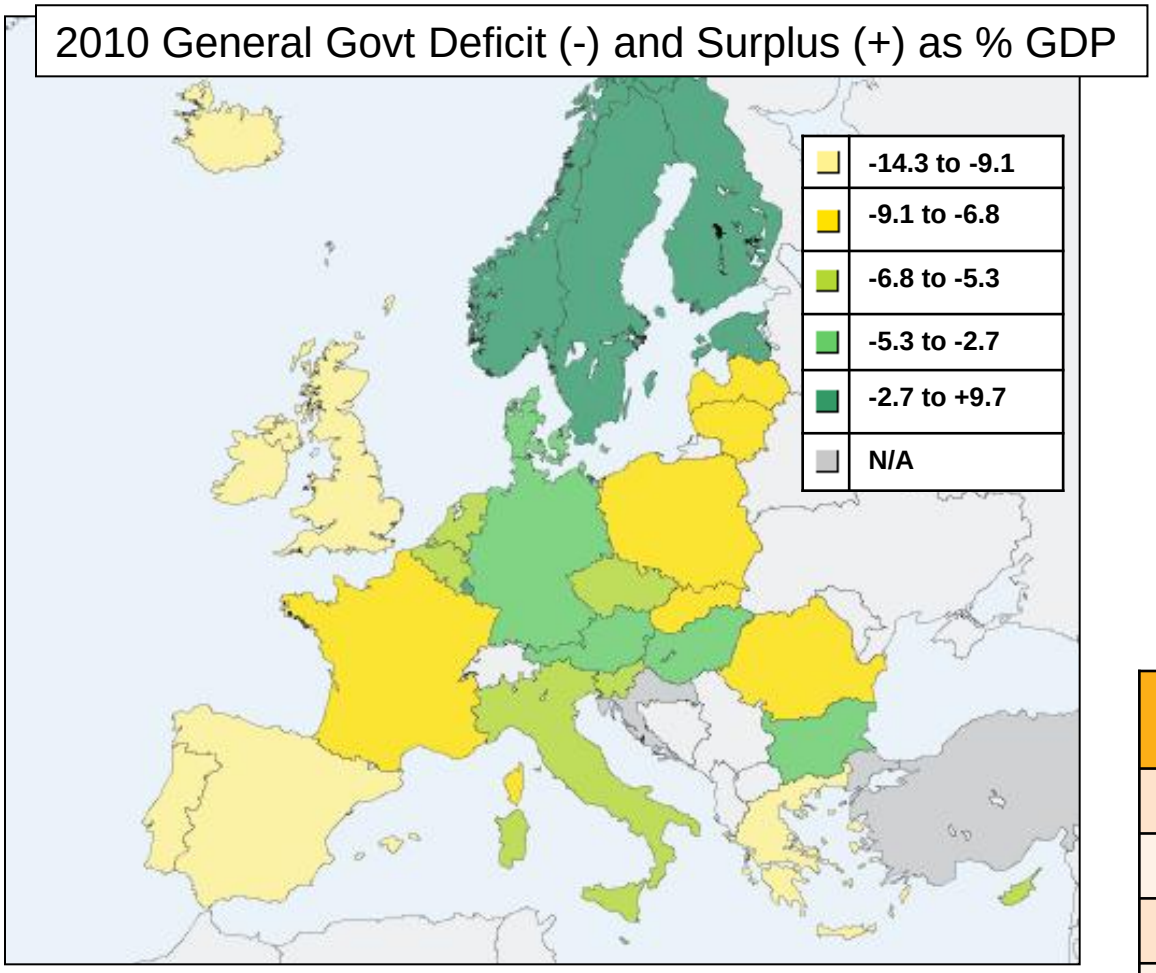
Targeting the mutational pathway may inhibit different tumors type (below an example of the PI3K pathway)

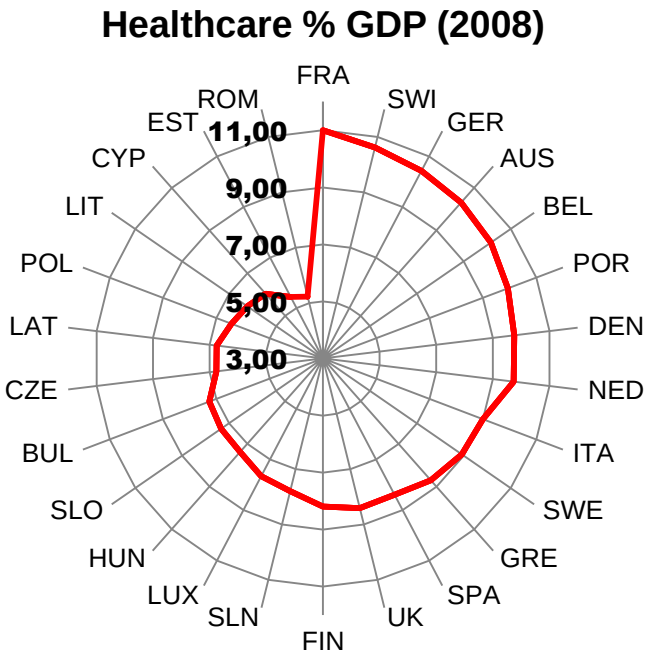


One drug for many different tumor types

Economic Challenges

But broad data obscures high variability across countries





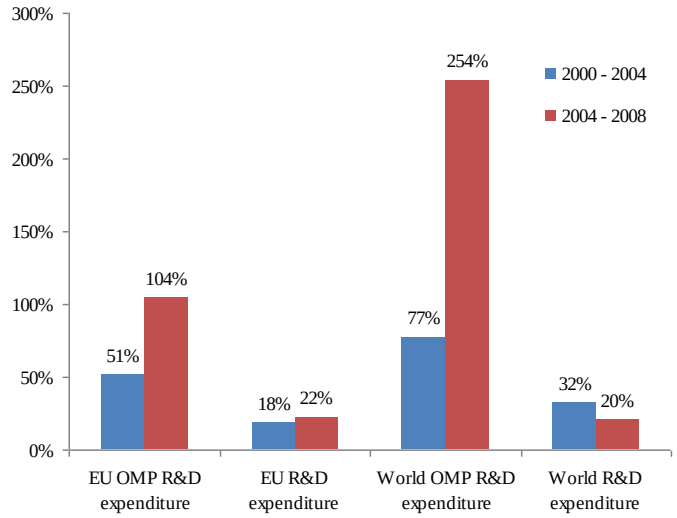
Country	Deficit % GDP (2009)	Healthcare % GDP (2008)
Greece	-13.6	9.7
Spain	-11.2	8.4
UK	-11.5	8.4
Italy	-5.3	9.0

Source: European Commission and OECD (underlying data)

NOVARTIS ONCOLOGY

.. But Orphan Drug budget impact remains low

budget impact <2.5% of pharma budget, <0.4% hc spend)

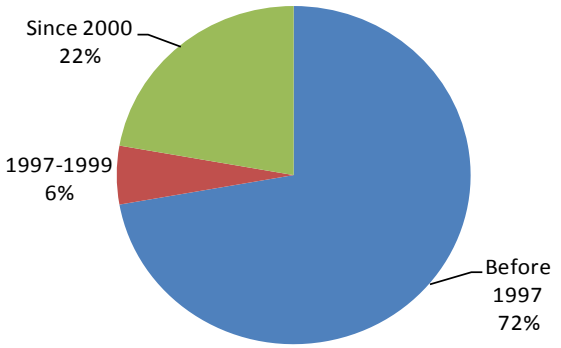


209% growth in R&D Investment

2009 budget impact of ODs*

- **Germany 2.5%**
- **France 2.4%**
- **Italy 2.5%**
- **Spain 2.5%**
- **UK 1.8%**
- **Poland 1%**

(% total pharma spend)



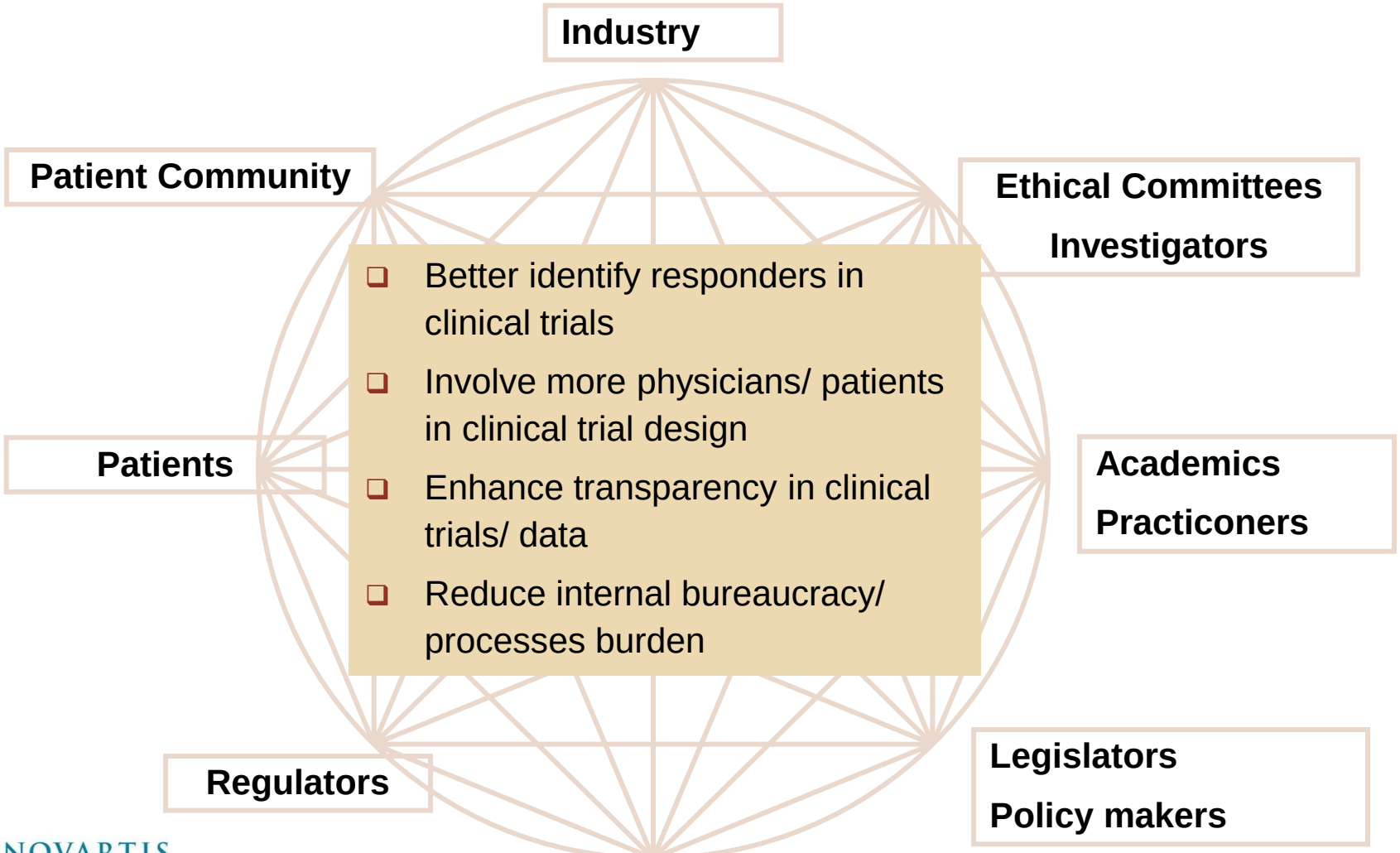
Impact on Company Creation

Sources: EU and World OMP R&D expenditure: OHE Consulting confidential survey; EU R&D expenditure: EFPIA (up to 2007); World R&D expenditure: PICTF
Note: EU OMP-specific R&D expenditure, in absolute terms, (obtained from our confidential survey) represents 1.01%, 1.30% and 2.16% of EU pharmaceutical R&D expenditure (from EFPIA) in 2000, 2004 and 2008 respectively.

Uncertainty in Assessing value - An example

- Drug for myelofibrosis
- Phase II data, no OS, non standard end-points
- High Burden of symptoms for the patients
- High unmet need (no drug approved for the disease)
- What is the value?
  - For the industry
  - For the patient
  - For the physician
  - For the payor

Stakeholders Involved in Clinical Trials Access Issues



NOVARTIS ONCOLOGY

Focus issues for this conference and related opportunities

- Rare cancers as an issue ➔ manage budget impact for sustainability
  - Generic entry, biosimilars
  - Outcomes based risk sharing schemes /pay for performance
  - Evidence and value based pricing
  - Explore new approaches: dynamic pricing; differential pricing
- Design of clinical trials ➔ revision of CT Directive
  - Acceptance Baysian statistical methods
- End-points of clinical trials ➔ alignment between payers & regulators
  - Expansion of expanded access, compassionate use prgs (ATU/648)
- Summarizing available evidence
  - Consistent framework for registries (EUCERD, EuropaBio)
  - Launch with evidence generation

Conclusions: Innovative Partnerships for a more effective approach towards cancer solutions

Holistic

Collaborative

Innovative

- **Address CT issues opportunities**
- **Exploit potential of CoE/ Reference networks**
- **Conditional reimbursement schemes**
- **Innovative access schemes (Risk Sharing)**
- **Expanded Access Programs / ATU**
- **Centers of Excellence/Networks**
- **Address access inequalities**

NOVARTIS ONCOLOGY

THANK YOU

NOVARTIS ONCOLOGY