Remaining obstacles towards optimal pharmaceutical development of rare cancer treatments

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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

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

Discover, develop and make accessible effective therapies for rare tumors

They all have a common goal ...
Major obstacles to develop rare cancers therapies

- Scientific challenges
- Economic challenges
- Operational Framework/ rules
Old vs new paradigm ... Has regulatory framework kept up?

**OLD PARADIGM**

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

- Esophagus
- Lung
- Liver
- Pancreas
- Prostate

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

2010 General Govt Deficit (-) and Surplus (+) as % GDP

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<tbody>
<tr>
<td>Greece</td>
<td>-13.6</td>
<td>9.7</td>
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<tr>
<td>Spain</td>
<td>-11.2</td>
<td>8.4</td>
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<tr>
<td>UK</td>
<td>-11.5</td>
<td>8.4</td>
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<tr>
<td>Italy</td>
<td>-5.3</td>
<td>9.0</td>
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Source: European Commission and OECD (underlying data)
.. 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)

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

- Industry
- Patient Community
- Patients
- Regulators
- Ethical Committees
- Investigators
- Academics
- Practitioners
- Legislators
- Policy makers

- Better identify responders in clinical trials
- Involve more physicians/patients in clinical trial design
- Enhance transparency in clinical trials/data
- Reduce internal bureaucracy/processes burden
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 Bayesian 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

- 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
THANK YOU