Orphan Drug Funding: A Model for Personalized Medicine?

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Eric Faulkner

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RTI Health Solutions
Introduction and Exercises
Agenda

• Introduction
• Exercises
• Orphan Drug Reimbursement in Europe
• Case Study: Catalonia
• Wrap-up and Questions
Session Objective

- Can orphan drug reimbursement serve as a model for drugs developed in an era of personalized medicine?

- To answer, we will address the following:
  - How might the economics of orphan drugs and drugs developed in an era of personalized medicine compare?
  - What are the similarities between orphan drugs and drugs developed in an era of personalized medicine?
  - What are the differences?
  - How is orphan drug funding changing in Europe?
What is Personalized Medicine?

**Personalized Medicine:**
Use of genetic or other molecular biomarker information to improve the safety, effectiveness and health outcomes of patients via more efficiently targeted risk stratification, prevention and tailored treatment management approaches

**Pharmacogenomics:**
Use of genomic tests to inform patient treatment selection and dosing by predicting drug response

Several examples thus far...

- HER2/neu: Herceptin
- KRAS/EGFR: Vectibix
- OncoType Dx: breast cancer chemo
- PGx Predict: warfarin

Source: Scientific American 2002
Historical Context for Orphan Drug Development

- Tax incentives to encourage R&D
- Patent protection
- Acceptance of high prices
- Special funding mechanisms in some markets

- Small patient populations and relatively low budget impact despite high costs of therapy, so less focus on cost containment and ensuring value for money (in typical ways)
Exercises
### Scenario 1: Historical Economics of Orphan Drugs vs Traditional Drugs

<table>
<thead>
<tr>
<th></th>
<th>Orphan Drug</th>
<th>Traditional Drug</th>
<th>Magnitude of difference (Traditional vs Orphan)?</th>
</tr>
</thead>
<tbody>
<tr>
<td>Size of treated population</td>
<td>500</td>
<td>500,000</td>
<td>1000x more treated patients</td>
</tr>
<tr>
<td>Cost per patient for 1 year of treatment</td>
<td>€ 50,000</td>
<td>€ 2,000</td>
<td>1/25th the drug cost</td>
</tr>
<tr>
<td>Total budget impact to treat population</td>
<td>€ 25,000,000</td>
<td>€ 1,000,000,000</td>
<td>40x greater budget impact</td>
</tr>
<tr>
<td>Percent of patients likely to respond</td>
<td>100%</td>
<td>20%</td>
<td>1/5th the response rate</td>
</tr>
<tr>
<td>QALY/response</td>
<td>0.50</td>
<td>0.50</td>
<td></td>
</tr>
<tr>
<td>Total QALYs gained over population</td>
<td>250</td>
<td>50,000</td>
<td>200x more QALYs gained</td>
</tr>
<tr>
<td>Cost per QALY</td>
<td>€ 100,000</td>
<td>€ 20,000</td>
<td>5x better CE</td>
</tr>
</tbody>
</table>
Scenario 1: Historical Economics of Orphan Drugs vs Traditional Drugs

- Q1: How have orphan drugs achieved high price, reimbursement, and market access?

- Q2: What is the relationship of the number of patients treated and the total budget impact for the traditional drug versus the orphan drug? What explains the relationship?

- Q3: Comment on the effectiveness of the traditional drug.
Scenario 1: Historical Economics of Orphan Drugs vs Traditional Drugs

• Q1: How have orphan drugs achieved high price, reimbursement, and market access?
  – The budget impact of the orphan drug is small relative to that of the traditional drug (€ 25 mi vs € 1 billion); therefore, although the ICER for the orphan drug is higher (€ 100,000/QALY gained vs € 20,000/QALY gained), it may still be granted high price, reimbursement, and market access.

• Q2: What is the relationship of the number of patients treated and the total budget impact for the traditional drug versus the orphan drug? What explains the relationship?
  – 1000 times more patients are treated with a traditional drug than with an orphan drug; however, because of the lower price for the traditional drug vs the orphan drug, the impact on the drug budget is only 40 times greater for the traditional drug than the orphan drug.

• Q3: Comment on the effectiveness of the traditional drug.
  – The traditional drug has only 1/5 the probability of response than the orphan drug, yet produces 200 times more QALYs for the population.
### Scenario 2: Economics of Personalized Medicine: Change 1 Factor—Introduce Test to Identify Likely Respondents

<table>
<thead>
<tr>
<th></th>
<th>Traditional Drug</th>
<th>Personalized Medicine</th>
</tr>
</thead>
<tbody>
<tr>
<td>Size of patient population</td>
<td></td>
<td>500,000</td>
</tr>
<tr>
<td>Cost per test</td>
<td></td>
<td>€ 500</td>
</tr>
<tr>
<td>% Results of Test</td>
<td></td>
<td>20%</td>
</tr>
<tr>
<td>Cost to screen patient population</td>
<td></td>
<td>€ 250,000,000</td>
</tr>
<tr>
<td>Cost to identify 1 responder</td>
<td>€ 2,500</td>
<td></td>
</tr>
<tr>
<td>Size of treated population</td>
<td>500,000</td>
<td>100,000</td>
</tr>
<tr>
<td>Cost per patient for 1 year of treatment</td>
<td>€ 2,000</td>
<td>€ 2,000</td>
</tr>
</tbody>
</table>
### Scenario 2: Economics of Personalized Medicine: Change 1 Factor—Introduce Test to Identify Likely Respondents

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<tbody>
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<td>Total budget impact to diagnose and treat population</td>
<td>€ 1,000,000,000</td>
<td></td>
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<td>Percent of patients likely to respond</td>
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<td>Total QALYs gained over population</td>
<td>50,000</td>
<td>50,000</td>
</tr>
<tr>
<td>Cost per QALY</td>
<td>€ 20,000</td>
<td></td>
</tr>
<tr>
<td>Savings resulting from test</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

© 2009
Scenario 1: Historical Economics of Orphan Drugs vs Traditional Drugs

- Q4: What is the total budget impact to diagnose and treat the population, assuming use of the diagnostic?

- Q5: What is the savings to the health care system resulting from use of the test, ceteris paribus?

- Q6: While not reaching the level of budget impact associated with the orphan drug, use of the test has drastically reduced the budget impact of the traditional drug. In what other ways does this personalized medicine scenario come closer to the orphan drug scenario?

- Q7: In this example, in what ways do the economics of personalized medicine not draw closer to those of orphan drugs?
## Scenario 2: Economics of Personalized Medicine: Change 1
Factor—Introduce Test to Identify Likely Respondents

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<td>€1,000,000,000</td>
<td>€450,000,000</td>
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<tr>
<td>Percent of patients likely to respond</td>
<td>20%</td>
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<td>50,000</td>
<td>50,000</td>
</tr>
<tr>
<td>Cost per QALY</td>
<td>€20,000</td>
<td>€9,000</td>
</tr>
<tr>
<td>Savings resulting from test</td>
<td></td>
<td>€550,000,000</td>
</tr>
</tbody>
</table>
Scenario 1: Historical Economics of Orphan Drugs vs Traditional Drugs

• Q6: While not reaching the level of budget impact associated with the orphan drug, use of the test has drastically reduced the budget impact of the traditional drug. In what other ways does this personalized medicine scenario come closer to the orphan drug scenario?
  
  – Size of treated population has gotten much smaller (from 500,000 down to 100,000).
  – The likelihood of responding to treatment has gotten much higher (from 20% to 100%). The same patients are responding, but because of test, we can identify and treat only them.

• Q7: In this example, in what ways do the economics of personalized medicine not draw closer to those of orphan drugs?
  
  – The drug cost for the traditional drug is still 1/50th the cost of the orphan drug.
  – The cost-effectiveness for the traditional drug is much improved.
Additional Questions for Later…

• Q8: In this example, the health care system captures the value of personalized medicine (by reducing the budget impact of the traditional drug). In what other ways and by which other parties may the value be captured?

• Q9: What is the relationship between the treatment response rate and the value of a test to identify patients likely to respond?

• Q10: Is the average cost to identify a likely responder a good predictor of the value of a test?
Salomé de Cambra
Senior European P&R Consultant
RTI Health Solutions
Orphan Drug Reimbursement in Europe
Rare diseases and orphan indications, an inequitable situation

- **International regulatory organizations and government’s reaction**
  - Research promotion (taxes, incentive/support research, pricing)
  - Regulatory centralization
  - Patients social support / local laws
  - Accessibility

- **Pharmaceutical response**
  - ~ 500 orphan designations registered in EMEA, during 2000-2008
  - ~ During S1 2009, 64 new orphan designations registered
  - ~ 51 authorizations issued 2000-2009
  - Expected ~10 new OD per year ...
  - Drugs may prove effective in other conditions and patients
Access to orphan drugs

<table>
<thead>
<tr>
<th>Country</th>
<th>Early access</th>
<th>Access</th>
<th>Comments</th>
</tr>
</thead>
<tbody>
<tr>
<td>GERMANY</td>
<td>No</td>
<td>Easy</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>AUSTRIA</td>
<td>UC/NP</td>
<td>Slow</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>BELGIUM</td>
<td>UC/NP</td>
<td>Slow++</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>DENMARK</td>
<td>UC/NP</td>
<td>Complex</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>FINLAND</td>
<td>UC/NP</td>
<td>Complex</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>FRANCE</td>
<td>TUA</td>
<td>Rapid</td>
<td>Coordination at OMS level</td>
</tr>
<tr>
<td>SPAIN</td>
<td>UC/NP</td>
<td>Classic</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>GREECE</td>
<td>UC/NP</td>
<td>Classic</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>IRELAND</td>
<td>UC/NP</td>
<td>Classic</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>ITALY</td>
<td>TUA</td>
<td>Classic</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>LUXEMBOURG</td>
<td>UC/NP</td>
<td>Classic</td>
<td>Nothing particular</td>
</tr>
<tr>
<td>THE NETHERLANDS</td>
<td>UC/NP</td>
<td>Classic</td>
<td>Improvement to be discussed</td>
</tr>
<tr>
<td>PORTUGAL</td>
<td>Depends on the case</td>
<td>Depends on the case</td>
<td>Special funds awarded</td>
</tr>
<tr>
<td>THE UNITED KINGDOM</td>
<td>UC/NP</td>
<td>Slow</td>
<td>Considered as expensive</td>
</tr>
<tr>
<td>SWEDEN</td>
<td>UC/NP</td>
<td>Easy</td>
<td>Nothing particular</td>
</tr>
</tbody>
</table>

Sources: EMEA, London.

UC: Compassionate Use
NP: Nominal Procedure
ATU: Temporary Use Authorization
OLU: Out labe Use
### Governments’ reaction to an inequitable situation

<table>
<thead>
<tr>
<th>Institutional Context</th>
<th>Pricing &amp; Reimbursement</th>
<th>Specific Processes &amp; Funds</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>BELGIUM</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• No specific funded research network</td>
<td>• Class I / subgroup</td>
<td>• Belgian Medical Need program (CU)</td>
</tr>
<tr>
<td>• 32 centers recognized by NIHDl</td>
<td>• Class A, 100% rb.</td>
<td>• Special solidarity fund 2007:</td>
</tr>
<tr>
<td>• Group for Orphan Drugs</td>
<td>By End 2008</td>
<td>• 141 Patients</td>
</tr>
<tr>
<td>• Rare Diseases and Orphan Drugs of the King Baldouin Foundation.</td>
<td>• 35 Indications</td>
<td>• €505.818 – €2.167 (per patient)</td>
</tr>
<tr>
<td></td>
<td>• 31 OD</td>
<td></td>
</tr>
<tr>
<td><strong>ITALY</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• National Network for RD and National Registry of RD: Hospitals and Regional Centers</td>
<td>• Class A or H 100%</td>
<td>• Fondo AIFA 5% (CU)</td>
</tr>
<tr>
<td>AIFA 45M€/yr :</td>
<td>• OD - unmet needs</td>
<td>• Off label use (OLU) In 2008</td>
</tr>
<tr>
<td>• Reimbursement of OD and live saving</td>
<td>In 2008</td>
<td>• 4 molecules (CU)</td>
</tr>
<tr>
<td>• Independent research and incentives</td>
<td>• 21 molecules</td>
<td>• 40 molecules (OLU)</td>
</tr>
<tr>
<td><strong>FRANCE</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• Institutte des Maladies Rares</td>
<td>• Same P&amp;R criteria</td>
<td>• ATU (CU)</td>
</tr>
<tr>
<td>• French Nat’l Plan for Rare Diseases</td>
<td>In 2007 ASMR:</td>
<td>• OLU for any drug for a RD</td>
</tr>
<tr>
<td>• Reference + Qualified Centers</td>
<td>I II III IV V</td>
<td></td>
</tr>
<tr>
<td>• Special fund in hospitals</td>
<td>3 13 8 4 2</td>
<td></td>
</tr>
<tr>
<td>• Taxes exemptions</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>UK</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• No specific funding for RD or promoting development</td>
<td>• Free price / PPRS</td>
<td>• CU / OLU, under the Prescription Price Authority</td>
</tr>
<tr>
<td></td>
<td>• 2009: end of life</td>
<td>• Funding specific mechanisms for OD</td>
</tr>
<tr>
<td></td>
<td>SMC: 50% neg. 50% rst.</td>
<td></td>
</tr>
<tr>
<td></td>
<td>NICE: Imitinab + £48 000</td>
<td></td>
</tr>
</tbody>
</table>
Payers’ reaction to an uncertain and budget threatening situation

- Health Technology Assessment
- Registers
- Distribution / drug delivery
- Prescription
- Agreements / Risk sharing
<table>
<thead>
<tr>
<th></th>
<th>Health Technology Assessment</th>
<th>Drug dispensing</th>
<th>Prescription</th>
<th>Registers</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>BELGIUM</strong></td>
<td>Exempt of cost effectiveness analysis</td>
<td>Hospital</td>
<td>• Approval of Medical Advisor of the Sickness Fund</td>
<td>Rare Diseases and Orphan Drugs of the King Baldouin Foundation.</td>
</tr>
<tr>
<td><strong>ITALY</strong></td>
<td>Within the P&amp;R process</td>
<td>• Hospital, Community or ASL</td>
<td>• Reference center specialist</td>
<td>National Network for RD and National Registry of RD</td>
</tr>
<tr>
<td><strong>FRANCE</strong></td>
<td>Improvement of the clinical added value (ASMR), in the P&amp;R. 80% faster access/price</td>
<td>• Conditions: Dispensation only upon registration</td>
<td>• Condition for some drugs: Registration into a national register</td>
<td>Centers of reference</td>
</tr>
<tr>
<td><strong>UK</strong></td>
<td>NICE, SMC, AWMSG OD Appraisal Proposal 30,000/QALY Exceptions: 48,000 (Imatinib for ML)</td>
<td>• Hospital(1/3) or pharmacies</td>
<td>• Initiated by a reference center (refill receipt by any physician)</td>
<td>No specific</td>
</tr>
</tbody>
</table>
The “equity issue”

- **Orphan drugs and rare diseases policies have proved to be an effective strategy (early 2000’s)**
  - Available resources for patients
  - Research
  - Health care improvement
  - Harmonization

- **However, a new paradigm and formulas are required**
  - Instruments for HTA, clinical management, research and funding
  - Relationship among stakeholders (payers, healthcare providers, professionals and the healthcare products industry).
  - Involvement of society / patients and management of media.

... to support personalized medicine implementation
Antoni Gilabert-Perramon
Managing Director of Pharmaceutical Care
and Complementary Services
Catalan Health Service
Case Study in Catalonia
Orphan drugs

Current challenges facing payers

Antoni Gilabert-Perramon
Managing Director of Pharmaceutical Care and Complementary Services
Catalan Health Service

ISPOR Paris, 25th October 2009
Approaching the subject

- A quick presentation of the Catalan Health Service (CatSalut) within the Spanish context

- Current situation of rare diseases in Spain and strategical approach in Catalonia

- Dealing with orphan drugs in Catalonia: evaluation, monitoring and financing approach

- Challenges and recommendations
Health System in Catalonia

1. Parliament
   - Assigns budgets
2. Department of Health
   - Draws up the Health Plan
   - Transfers financial Resources
3. Catalan Health Service (CatSalut)
   - Guarantees Health care
   - Provides health services
4. Supplier network (ICS, consortiums, etc)
5. Financing
   - Resources /Health policies
6. Insurance
   - Purchase / payment Service policies
7. Supplies
Current model of P & R pharmaceutical decisions

\[
\text{COST} = \text{REIMBURSEMENT} \times \text{PRICE} \times \text{CONSUMPTION}
\]

<table>
<thead>
<tr>
<th>COMPETENCE</th>
<th>State</th>
<th>State</th>
<th>Auton. Community</th>
</tr>
</thead>
</table>
Drug policy

Aims:

- To provide quality and efficiency of, and accessibility to pharmaceutical care, focusing on patients and their health outcomes
- To promote a safer and more rational use of drugs
- To rationalize pharmaceutical expenditure and stimulate efficient management (stay within the budget to ensure the sustainability and viability of the health system)
- To promote an integral and integrated vision of the drug chain
Current strategic plan and measures (2007-2010)

Sharing responsibilities
- Sharing risk
  - Providers (PCT/hospitals)
  - Industry
- Capitation financing system
- Professional incentives
- Health education
- Generics and RP
- Drug utility evaluation
- Economic evaluation
- High complexity treatments
- Advisory Committees
- Chronic prescription
- Clinical practice guidelines

Information Systems
- DataMart
- Patient records DB
- Patient analysis prog.
- Benchmarking
- Bulletins and reviews
- Drug utilization studies
- Electronic prescrip.

Redefinition of services
- Pharmaceutical care
- Coordination PC-HC
- Primary care pharmacy
- Geriatric/home care
- Community pharmacy services
- Contract objectives evaluation
- Invoicing control
- Promotion control
- Farmacovigilance
- Fraud control

Evaluation and control
1) **Information on rare diseases:** information, health registers, classifications, codification

2) **Prevention and early diagnosis:** screening of newly born, genetic diagnosis...

3) **Health care:** coordination between specialized and primary care

4) **Treatment:** advanced therapies, orphan drugs, health products

5) **Long-term health care**

6) **Research:** prioritization research of rare diseases

7) **Training:** professionals (under-graduate, post-graduate, and continuous training) and patients
Rare diseases: initiatives in Catalonia (I)

Health Plan 2010: “...improve knowledge, information about needs, diagnoses, treatment of, and resources for neurological rare diseases...”

Long-term care Leading Plan: “...preserve and maintain the autonomy and the ability of patients with rare neurological diseases and improve their quality of life...”

Public Health Law: “...consider as a public health service the prevention of disability risk factors caused by rare diseases...”
Resolution of the Parliament of Catalonia on rare diseases: “...urges the Government to tackle the main needs of people with rare diseases and take measures in research, diagnosis, treatment, and follow up...”

Advisory Committee for rare diseases: “...to implement and promote health policies on rare diseases...”
Catalan Advisory Committee for rare diseases
October 1st, 2008

- Promote training and information on rare diseases
- Suggest measures to be taken to improve services and care of patients with rare diseases
- Establish registers of rare diseases
- Propose home care rehabilitation interventions
- Coordinate different levels of care
- Financial initiatives for orphan drugs
Resolution of the Parliament of Catalonia on rare diseases: “...urge the Government to tackle the main needs of people with rare diseases and take measures in research, diagnosis, treatment, and follow up...”

Advisory Committee for rare diseases: “...to implement and promote health policies on rare diseases...”

Evaluation, Monitoring and Financing Programme for High Complexity Treatments: “...to guarantee access and rational use of HCT, including orphan drugs...”
What is a high complexity treatment (HCT)?

- EMEA authorized drugs under “Conditional approval” or “Exceptional circumstances”
- Drugs under an additional risk minimisation plan
- Advanced therapy: gene therapy, tissue engineering therapy... including advanced cancer therapy
- Drugs for rare diseases (orphan drugs)
Orphan drugs in Catalonia: situation

- Constant setting up of new orphan drugs (7 new OD in 2008) mostly as conditioned drug approval or drugs approved under exceptional circumstances
- Limited and poor information about orphan drugs effectiveness and safety in normal clinical practice
- Extremely high cost per treatment (20,000€/patient/year)
- Very few cases for each rare disease but a high number in total (30 drugs, 2% hospital out-patients, 8% hospital drug budget)
- High expenditure growth rate (60% increase from 2007 to 2008)
- Problems of access and potential inequality
- High social awareness and pressure put on Government
Orphan drug treatments in Catalonia: aims

- Guarantee equity of access
- Achieve rational use
- Improve health outcomes
- Manage to make the health system sustainable
- Promote research and innovation
Evaluation, Monitoring and Financing Programme for High Complexity Treatments (EMFP-HCT)
CatSalut Resolution November 10th, 2008

1. Evaluation: EC-HCT

Criteria for use and outcomes

2. Monitoring: AC-HCT

3. Financing: FC-HTA

Authorisation, renewal, registration

Financing conditions
Evaluation Committee (EC-HCT)

Responsibilities

- Evaluation of HCT and elaboration of reports and recommendations of:
  - Therapeutic utility: recommended drug, restricted use, or exceptional utilization
  - Criteria for prescription, dispensation, and follow up

Members

- Catalan HTA Agency
- Hospital pharmacists
- Doctors
- Health economists
- Experts ad hoc

Drug evaluation

<table>
<thead>
<tr>
<th>Drugs evaluated</th>
<th>Indicació</th>
</tr>
</thead>
<tbody>
<tr>
<td>Eculizumab (O)</td>
<td>* Paroxismal Hemoglobinuria</td>
</tr>
<tr>
<td>Laronidasa (O,E)</td>
<td>Hurler syndrome</td>
</tr>
<tr>
<td>Lapatinib (C)</td>
<td>Breast cancer metastasis</td>
</tr>
<tr>
<td>Trabectedina (O,E)</td>
<td>Sarcoma</td>
</tr>
<tr>
<td>Ambrisentan (O)</td>
<td>Lung HT</td>
</tr>
<tr>
<td>Azacitidina (O)</td>
<td>Leukemia</td>
</tr>
<tr>
<td>Miglustat (O,E)</td>
<td>* Niemann Pick/ Gaucher</td>
</tr>
<tr>
<td>Mifamurtide (O)</td>
<td>Sarcoma</td>
</tr>
<tr>
<td>Dihodrocloruro de histamina (O, EC)</td>
<td>Leukemia</td>
</tr>
</tbody>
</table>
Advisory Committee (AC-HCT)

• **Responsibilities:**
  – To advise CatSalut on HTC
  – Receive and assess the applications for treatment
  – Authorise or deny treatments
  – Register the cases and follow up the outcomes
  – Revise treatments and renew or stop them

• **Members:**
  – Members of CatSalut, of Catalan HTA Agency, hospital pharmacists, doctors, experts “ad hoc”
**ECULIZUMAB**

- Creation of a multidisciplinary group of experts (November 2008)

- Produce Clinical criteria for the treatment with eculizumab of people diagnosed from paroxysmal hemoglobinuria on CatSalut account

  - 6 authorized patients. 1 denied. 1 in course.

  - Cost/treatment/year = 340,000€
**MIGLUSTAT**

- Creation of a multidisciplinary group of experts (may 2009)
- Draw up the report of *Clinical criteria for the treatment with Miglustat to patients diagnosed of Niemann-Pick type C*
- 2 authorised patients
- Cost/treatment/year = 156,000€

---

CatSalut Resolution for miglustat treatments (26.5.2009)
Financing Committee (FC-HCT)

- **Responsibilities:**
  - Proposals for the purchasing, supplying and financing conditions of HCT for CatSalut
  - Assessment of pharmacoeconomic issues of new HCT, according to the CatSalut Committee for Economic Evaluation and Budgetary Impact of Drugs (EEC)
  - Define strategies for sharing responsibilities (CatSalut Bio-Workshop)

- **Members:**
  - Members of CatSalut board of directors, health economists support
Financing Committee (FC-HCT)

Economic Evaluation and Budgetary Impact Committee (EEC)
http://www10.gencat.cat/catsalut/cat/prov_farmacia_economia.htm
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http://www10.gencat.cat/catsalut/cat/prov_farmacia_economia.htm

- Responsibilities:
  - Elaborate economic evaluation reports for new drugs
  - Make pharmacoeconomical research reviews of pharmacological groups
  - Propose and monitor cost-effectiveness studies
  - Carry out budgetary impact studies for new drugs
  - Deliver opinions about drug pharmacoeconomic studies presented by pharmaceutical laboratories
  - Deliver opinions about drug financing decision-making process

- Members:
  - Members of CatSalut, Catalan HTA Agency, experts on health economics
Interdisciplinary workshop for debate on new biological therapies and to generate knowledge related to risk sharing agreements between pharmaceutical industry and health administration.
Financing Committee (FC-HCT)

CatSalut Bio_Workshop: Main proposals

- To promote the creation of patient registers by payers
- To explore risk sharing agreements for some new drugs
- To make pharmacoepidemiology studies
- To manage the expectations of patients
- To focus on low incident diseases with high needs and cost
Our challenge: a complex balance

- Meet the expectations and needs of patients with rare diseases
- Realistic and sustainable funding of orphan drugs
- Profitability of research and development in orphan diseases
Recommended actions

• Create centres of excellence
• Develop registers of diseases and patients
• Promote research and support
• Generate sustainable funding
• Take into consideration the role of patients
Thank you!

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and Market Access
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Wrap up and Questions
## Comparing Orphan Indication to Personalized Medicine Scenarios

<table>
<thead>
<tr>
<th>Key Consideration</th>
<th>Orphan Indication</th>
<th>Personalized Medicine</th>
</tr>
</thead>
<tbody>
<tr>
<td>Held to same clinical requirements as larger population products</td>
<td>Not fully, but becoming more stringent in some markets</td>
<td>Yes</td>
</tr>
<tr>
<td>Held to same economic requirements as larger population products (e.g., cost-effectiveness)</td>
<td>Not fully, but becoming more stringent in some markets</td>
<td>Yes</td>
</tr>
<tr>
<td>Individual financial impact may be small, but payers are considering budget impact of multiple entrants</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Pricing is being evaluated with greater scrutiny</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Conventional health economic modeling approaches “fit”</td>
<td>Debatable</td>
<td>Yes</td>
</tr>
<tr>
<td>Conventional large population technology assessment approaches “fit”</td>
<td>Debatable</td>
<td>Debatable</td>
</tr>
</tbody>
</table>
**Key Challenges & Unanswered Questions**

- **Products for orphan indications face increasingly restrictive policies and may be held to the same standards as non-orphan products**
  - Question: What criteria are appropriate for selecting among orphan products with comparable value propositions, particularly in a scenario where all products under consideration fill an important unmet need?
  - Question: As other markets evolve more restrictive policies for orphan products, what key issues should manufacturers consider in assessing product development risks?
  - Question: Is there a minimum value proposition that an orphan product should meet (aside from safety and effectiveness) to support reimbursement?
Key Challenges & Unanswered Questions

- **PM or orphan treatments may reach a “price threshold” above which reimbursement is uncertain/unlikely**
  
  - Question: As more of these treatments enter the market over time, should we establish a different acceptable “threshold” for high cost BUT small population or individual budget impact products?

- **Are different modeling approaches needed? What attributes should these modeling approaches incorporate or avoid?**
  
  - Societal values, unmet medical need, cost-effectiveness exceptions?
  
  - Question: Is there a certain level of cost/volume tradeoff at which a product is no longer viable/worth developing from a manufacturer’s perspective?

- **If so, should government subsidize conditional coverage/value assessment efforts in certain scenarios? Under what circumstances?**
Key Challenges & Unanswered Questions

- Depending on the scenario, personalized medicine products may face challenges similar to orphan drugs for demonstrating cost-effectiveness
  - E.g.: small patient population that requires a high price for market viability and/or sufficient ROI
  - Question: Should PM products be subject to different evidentiary requirements as broader population or blockbuster counterparts?
    - What about scenarios where a large proportion of the original target population are responders vs. very few?
    - How should the cost of the diagnostic figure into the assessment?
      - Question: Should PM products be subject to comparative effectiveness?
    - How would you ideally compare a PM product to a blockbuster product? What modeling considerations are relevant?
Key Challenges & Unanswered Questions

• In some markets, follow-on products for orphan indications may be subject to more stringent review of clinical & economic evidence than the innovator product.

  – Question: How should the value be estimated for products with different amounts of supporting evidence, particularly in the emerging era of comparative effectiveness?
Thank you

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