RTI HEALTH SOLUTIONS®

W3
Orphan Drug Funding: A Model for Personalized Medicine?

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LEADING RESEARCH…
MEASURES THAT COUNT

Dee Dee Mladsi
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Introduction and Exercises

Agenda

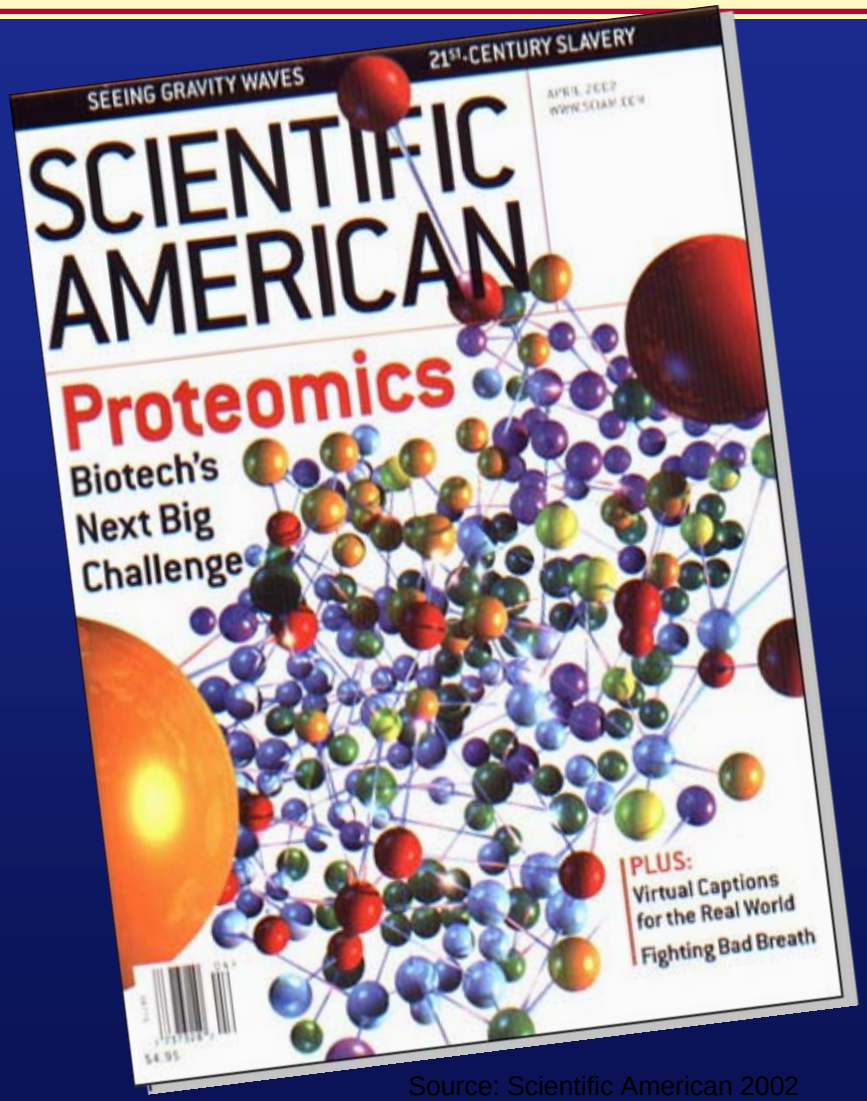
- **Introduction**
- **Exercises**
- **Orphan Drug Reimbursement in Europe**
- **Case Study: Catalonia**
- **Wrap-up and Questions**

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?



Source: Scientific American 2002

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

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

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

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

	Traditional Drug	Personalized Medicine
Size of patient population		500,000
Cost per test		€ 500
% Results of Test		20%
Cost to screen patient population		€ 250,000,000
Cost to identify 1 responder		€ 2,500
Size of treated population	500,000	100,000
Cost per patient for 1 year of treatment	€ 2,000	€ 2,000

Scenario 2: Economics of Personalized Medicine: Change 1 Factor—Introduce Test to Identify Likely Respondents

	Traditional Drug	Personalized Medicine
Total budget impact to diagnose and treat population	€ 1,000,000,000	
Percent of patients likely to respond	20%	100%
QALY/response	0.50	0.50
Total QALYs gained over population	50,000	50,000
Cost per QALY	€ 20,000	
Savings resulting from test		

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

	Traditional Drug	Personalized Medicine
Total budget impact to diagnose and treat population	€ 1,000,000,000	*€ 450,000,000*
Percent of patients likely to respond	20%	100%
QALY/response	0.50	0.50
Total QALYs gained over population	50,000	50,000
Cost per QALY	€ 20,000	*€ 9,000*
Savings resulting from test		*€ 550,000,000*

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

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

Sources: EMEA, London.

UC: Compassionate Use

NP: Nominal Procedure

ATU: Temporary Use Authorization

OLU: Out labe Use

Governments' reaction to an inequitable situation

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

Payers' reaction to a uncertain and budget threatening situation

- **Health Technology Assessment**
- **Registers**
- **Distribution / drug delivery**
- **Prescription**
- **Agreements / Risk sharing**

Payers' reaction to an uncertain and budget threatening situation

	Health Technology Assessment	Drug dispensing	Prescription	Registers
BELGIUM	Exempt of cost effectiveness analysis	Hospital	•Approval of Medical Advisor of the Sickness Fund •College of Medical Doctors for Orphan Drugs (CMOCD) advice on a case by case basis	Rare Diseases and Orphan Drugs of the King Baldouin Foundation.
ITALY	Within the P&R process	•Hospital, Community or ASL •Conditions: Dispensation only upon registration	•Reference center specialist •Condition for some dugs : Registration into a national register	National Network for RD and National Registry of RD
FRANCE	Improvement of the clinical added value (ASMR), in the P&R. 80% faster access/price	•Hospital(1/3) or pharmacies	•Initiated by a reference center (refill receipt by any physician •Reimbursed at a level	Centers of reference
UK	NICE, SMC, AWMSG OD Appraisal Proposal 30,000/QALY Exceptions: 48,000 (Imatinib for ML)	• Hospital pharmacies or specialists centers	•Initiated by an specialist, •PPA monitoring.	No specific

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

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Managing Director of Pharmaceutical Care and Complementary Services
Catalan Health Service

Case Study in Catalonia

Orphan drugs

Current challenges facing payers

Antoni Gilabert-Perramon

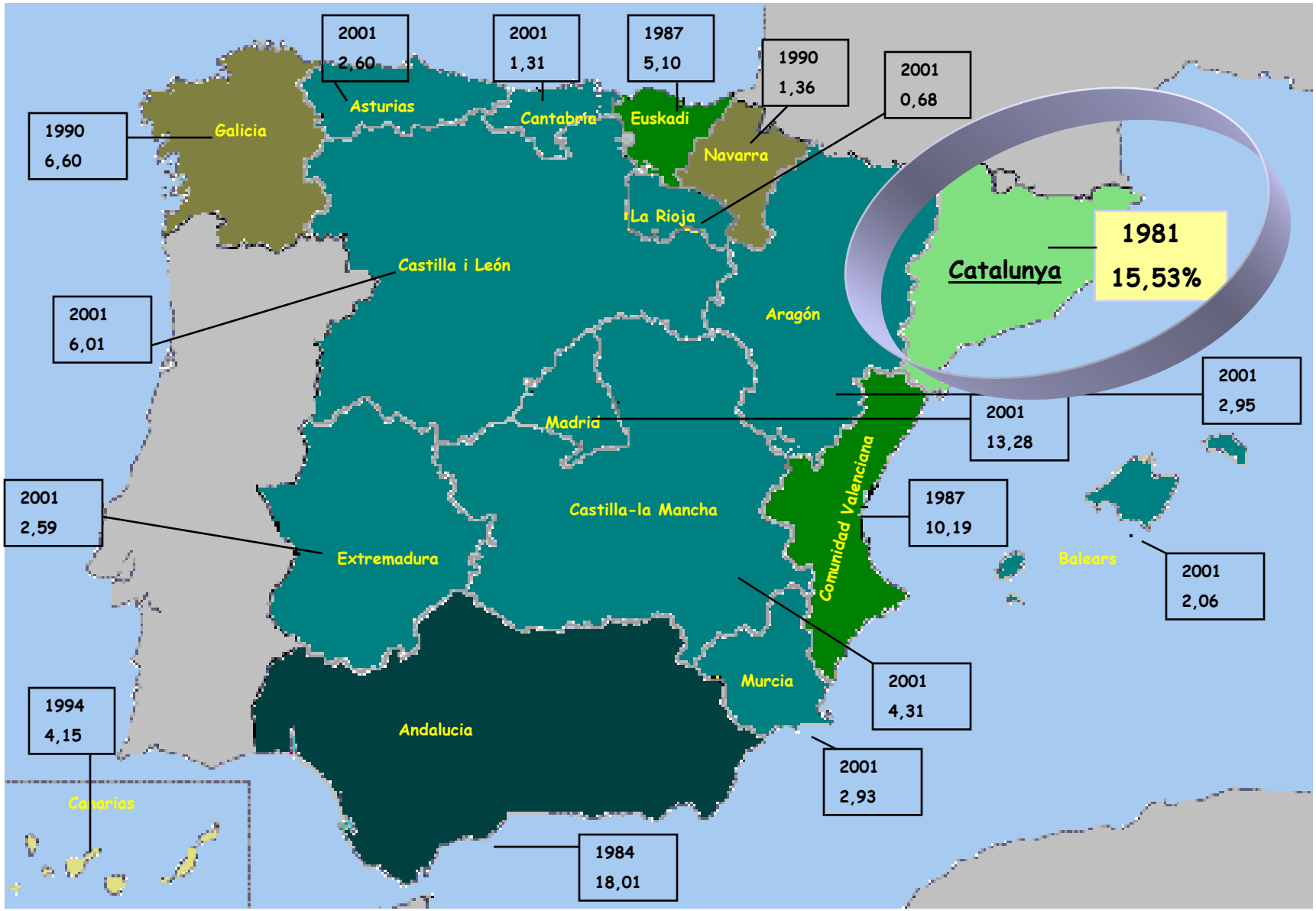
Managing Director of Pharmaceutical Care and Complementary Services

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


2001
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Asturias
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Cantabria
1987
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2001
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La Rioja
1990
6,60
Galicia
Castilla i León
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6,01
Aragón
Catalunya
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15,53%
2001
2,95
Madrid
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13,28
Comunidad Valenciana
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Extremadura
Castilla-la Mancha
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Baleares
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Murcia
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1994
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Andalucia
2001
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Canarias
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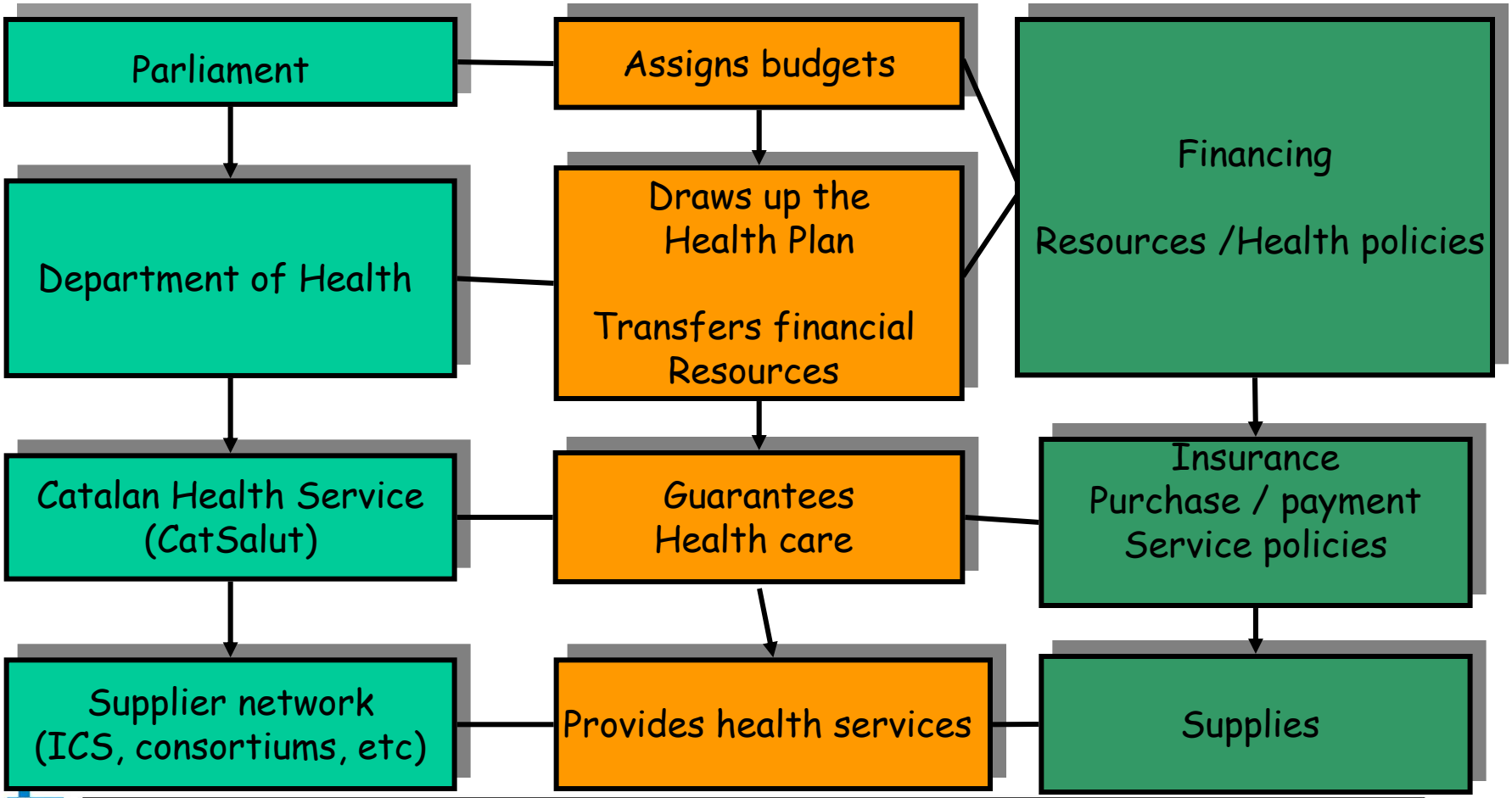
Health System in Catalonia



Current model of P & R pharmaceutical decisions

COST =	REIMBURSEMENT	X	PRICE	X	CONSUMPTION
COMPETENCE	State		State		Auton.Community

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

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

Quality/Efficiency

- Generics and RP
- Drug utility evaluation
- Economic evaluation
- High complexity treatments
- Advisory Committees
- Chronic prescription
- Clinical practice guidelines

Evaluation and control

- Contract objectives evaluation
- Invoicing control
- Promotion control
- Farmacovigilance
- Fraud control

Servei Català de la Salut

Strategy for rare diseases in the Spanish NHS

June 20th, 2009

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..."*

Rare diseases: initiatives in Catalonia (II)

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

Rare diseases: initiatives in Catalonia (II)

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…"*

Evaluation, Monitoring and Financing Programme for High Complexity Treatments (EMFP-HCT)

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

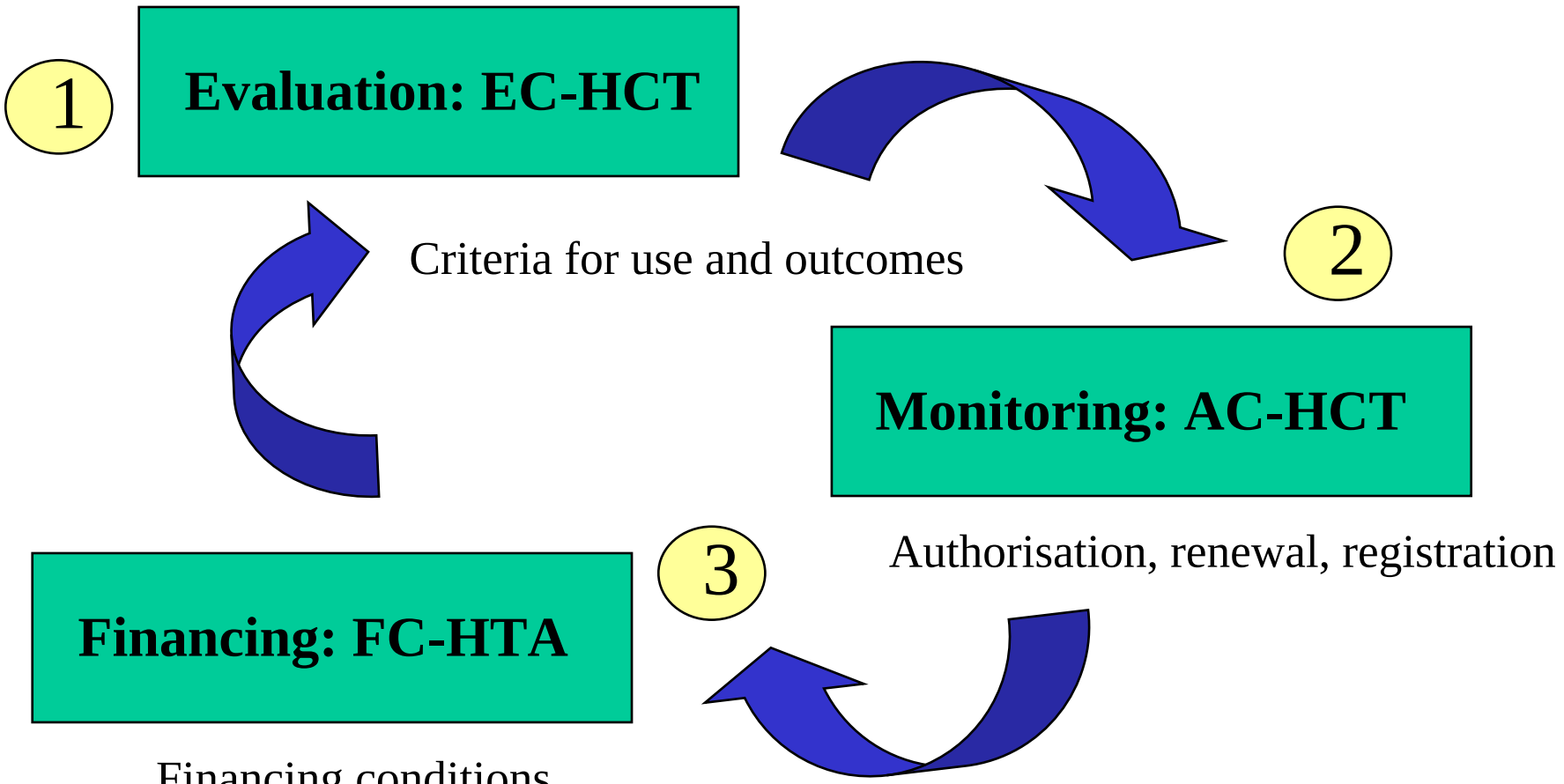
- Constant setting up of new orphan drugs (7 new OD in 2008) mostly as conditioned drug approval or drugs approved under exceptional circumstances
- Limited and poor information about orphan drugs effectiveness and safety in normal clinical practice
- Extremely high cost per treatment (20.000€/patient/year)
- 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



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Evaluation Committee (EC-HCT)

Responsabilities

- 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

Drugs evaluated	Indicació
Eculizumab (O) *	Paroxismal Hemoglobinuria
Laronidasa (O,E)	Hurler syndrome
Lapatinib (C)	Breast cancer metastasis
Trabectedina (O,E)	Sarcoma
Ambrisentan (O)	Lung HT
Azacitidina (O)	Leukemia
Miglustat (O,E) *	Niemann Pick/ Gaucher
Mifamurtide (O)	Sarcoma
Dihodrocloruro de histamina (O, EC)	Leukemia

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”

2

Advisory Committee (AC-HCT)

Expert Group for Paroxysmal Hemoglobinuria

- **ECULIZUMAB**

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

  - Produce C*linical criteria for the treatment with eculizumab of people diagnosed from parozysmal hemoglobinuria on Catsalut account*

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

  - Cost/treatment/year = 340.000€

CatSalut

Criteris clínics en relació amb l'eculizumab en el tractament de persones malaltes diagnosticades d'hemoglobinúria paroxismal nocturna (HPN) en l'àmbit del CatSalut

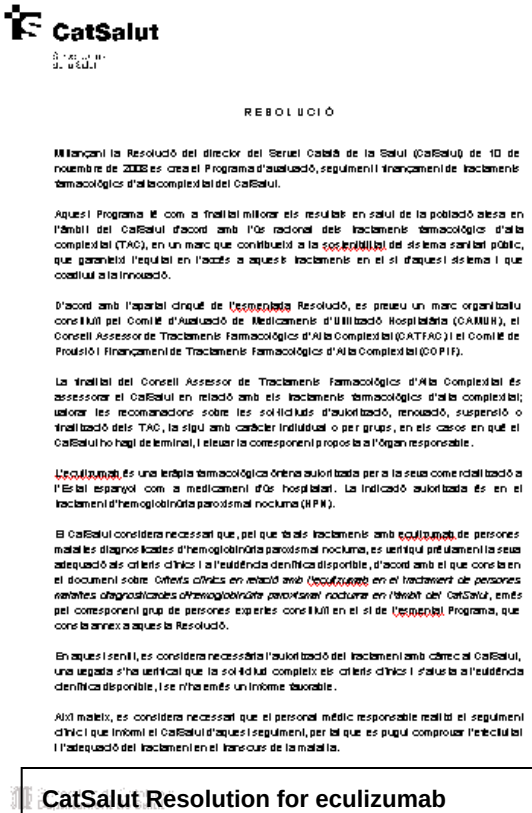
Versió 1

Grup de persones expertes en el tractament amb eculizumab

Barcelona, 2 de desembre de 2008

CatSalut Servei Català de la Salut

Generalitat de Catalunya Departament de Salut



CatSalut

RESOLUCIÓ

Mitjançant la Resolució del director del Servei Català de la Salut (CatSalut) de 10 de novembre de 2008 es crea el Programa d'avaluació, seguiment i finançament de tractaments farmacològics d'alta complexitat del CatSalut.

Aquest Programa té com a finalitat millorar els resultats en salut de la població atesa en l'àmbit del CatSalut d'acord amb l'ús racional dels tractaments farmacològics d'alta complexitat (TAC), en un marc que contribueixi a la sostenibilitat del sistema sanitari públic, que garanteixi l'equitat en l'accés a aquests tractaments en el si d'aquest sistema i que estimuli la innovació.

D'acord amb l'apartat cinquè de l'esmentada Resolució, es preveu un marc organitzatiu constituït pel Comitè d'Avaluació de Medicaments d'Utilització Hospitalària (CAMUH), el Consell Assessor de Tractaments Farmacològics d'Alta Complexitat (CATFAC) i el Comitè de Provisió i Finançament de Tractaments Farmacològics d'Alta Complexitat (COPIF).

La finalitat del Consell Assessor de Tractaments Farmacològics d'Alta Complexitat és assessorar el CatSalut en relació amb els tractaments farmacològics d'alta complexitat; valorar les recomanacions sobre les sol·licituds d'autorització, renovació, suspensió o finalització dels TAC, ja sigui amb caràcter individual o per grups, en els casos en què el CatSalut ho hagi determinat, i elevar la corresponent proposta a l'òrgan responsable.

L'eculizumab és una teràpia farmacològica òrfena autoritzada per a la seva comercialització a l'Estat espanyol com a medicament d'ús hospitalari. La indicació autoritzada és en el tractament d'hemoglobinúria paroxismal nocturna (HPN).

El CatSalut considera necessari que, pel que fa als tractaments amb eculizumab de persones malaltes diagnosticades d'hemoglobinúria paroxismal nocturna, es verifiqui prèviament la seva adequació als criteris clínics i a l'evidència científica disponible, d'acord amb el que consta en el document sobre *Criteris clínics en relació amb l'eculizumab en el tractament de persones malaltes diagnosticades d'hemoglobinúria paroxismal nocturna en l'àmbit del CatSalut*, emès pel corresponent grup de persones expertes constituït en el si de l'esmentat Programa, que consta annex a aquesta Resolució.

En aquest sentit, es considera necessària l'autorització del tractament amb càrrec al CatSalut, una vegada s'ha verificat que la sol·licitud compleix els criteris clínics i s'ajusta a l'evidència científica disponible, i se n'ha emès un informe favorable.

Així mateix, es considera necessari que el personal mèdic responsable realitzi el seguiment clínic i que informi el CatSalut d'aquest seguiment, per tal que es pugui comprovar l'efectivitat i l'adequació del tractament en el transcurs de la malaltia.

CatSalut Resolution for eculizumab treatments (13.11.2008)

2

Advisory Committee (AC-HCT)

Expert Group for Niemann-Pick disease

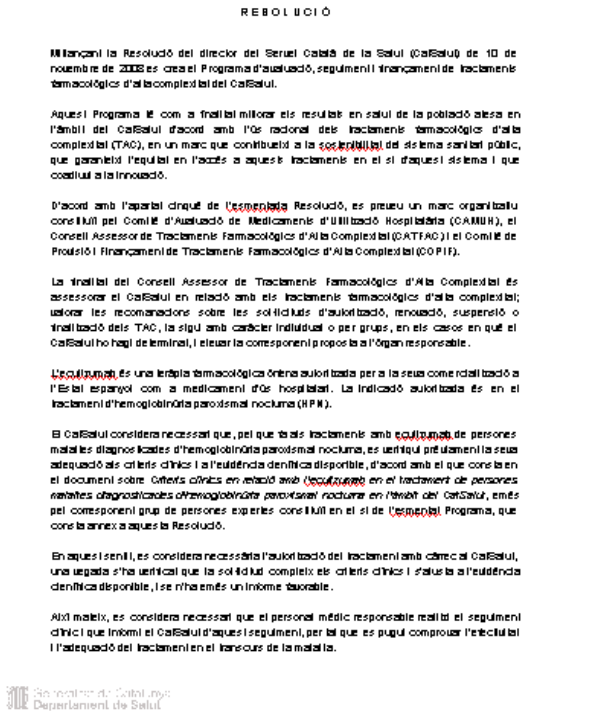
- **MIGLUSTAT**

  - 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€



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El CatSalut considera necessari que, pel que fa als tractaments amb eculizumab de persones malaltes diagnosticades d'hemoglobinúria paroxísmal nocturna, es verifiqui prèviament la seva adequació als criteris clínics i a l'evidència científica disponible, d'acord amb el que consta en el document sobre *Criteris clínics en relació amb l'eculizumab en el tractament de persones malaltes diagnosticades d'hemoglobinúria paroxísmal nocturna en l'àmbit del CatSalut*, emès pel corresponent grup de persones expertes constituït en el si de l'esmentat Programa, que consta annex a aquesta Resolució.

En aquest sentit, es considera necessària l'autorització del tractament amb càrrec al CatSalut, una vegada s'ha verificat que la sol·licitud compleix els criteris clínics i s'ajusta a l'evidència científica disponible, i se n'ha emès un informe favorable.

Així mateix, es considera necessari que el personal mèdic responsable realitzi el seguiment clínic i que informi el CatSalut d'aquest seguiment, per tal que es pugui comprovar l'efectivitat i l'adequació del tractament en el transcurs de la malaltia.

Generalitat de Catalunya
Departament de Salut

CatSalut Resolution for miglustat treatments (26.5.2009)

3

Financing Committee (FC-HCT)

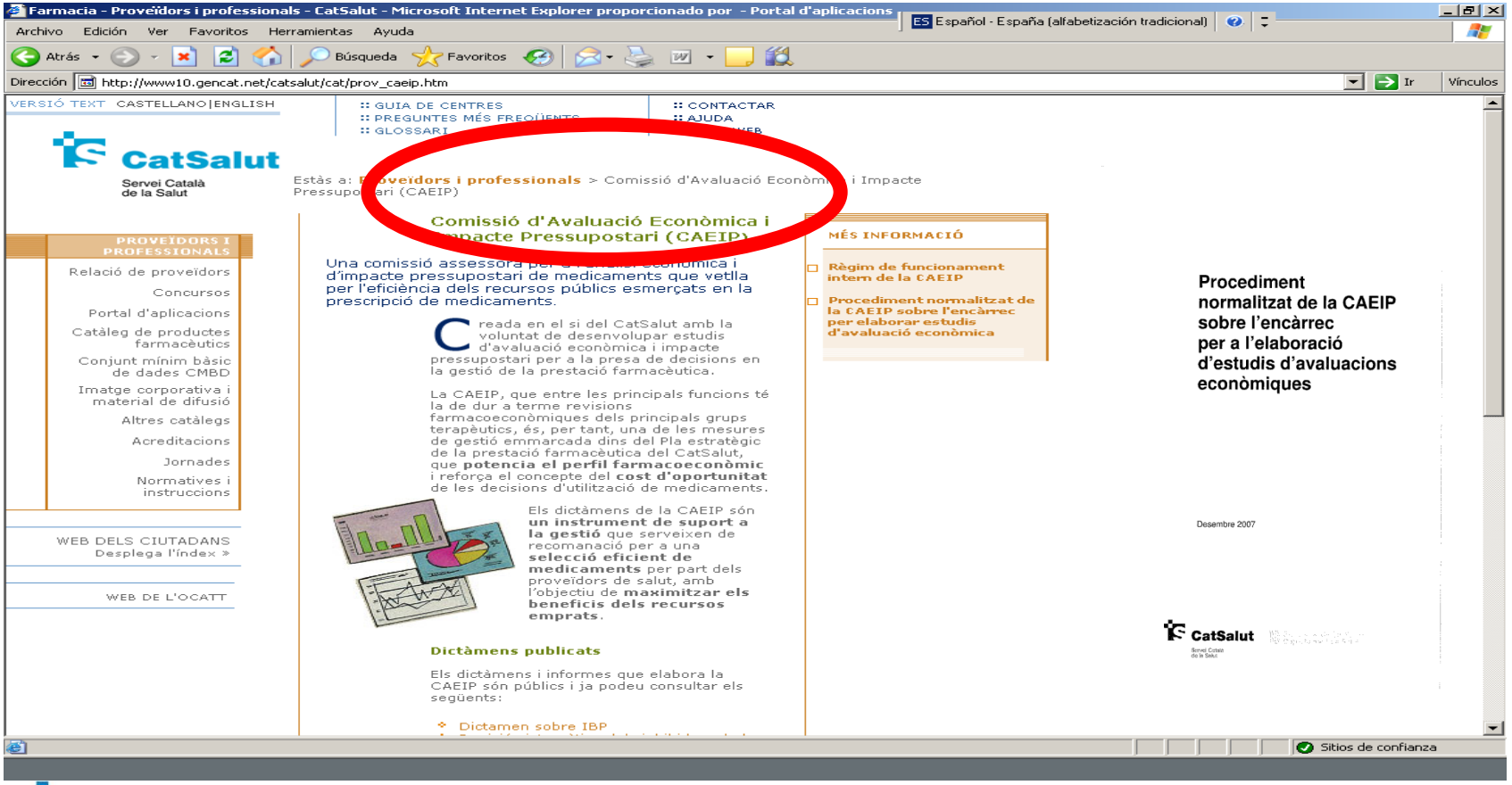
- Responsibilities:
  - Proposals for the purchasing, supplying and financing conditions of HCT for CatSalut
  - Assessment of pharmacoeconomic issues of new HCT, according to the CatSalut Committe for Economic Evaluation and Budgetary Impact of Drugs (EEC)
  - Define strategies for sharing responsibilities (Catsalut Bio-Workshop)
- Members:
  - Members of CatSalut board of directors, health economists support

3

Financing Committee (FC-HCT)

Economic Evaluation and Budgetary Impact Committee (EEC)

http://www10.gencat.cat/catsalut/cat/prov_farmacia_economia.htm



3

Financing Committee (FC-HCT)

Economic Evaluation and Budgetary Impact Committee (EEC)

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

CatSalut BIO_WORKSHOP

CatSalut Bio_WORKSHOP

"Biomedicina: nous reptes i oportunitats per a l'Administració"

Barcelona, 6 de juny de 2008

CatSalut Bio_WORKSHOP

"Risc compartit: nous reptes i oportunitats per al sistema sanitari"

18 de novembre de 2008, sala Picasso, Hotel Condes de Barcelona

CatSalut Bio_WORKSHOP_III

"Risc compartit: de la teoria a la pràctica"

Barcelona, 5 de maig de 2009

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 challange: 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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Senior Director, Reimbursement and Market Access
RTI Health Solutions

Wrap up and Questions

Comparing Orphan Indication to Personalized Medicine Scenarios

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

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?

RTI HEALTH SOLUTIONS®

Thank you

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