



MINISTÉRIO DA  
SAÚDE



CENTRO COLABORADOR DO SUS  
AVALIAÇÃO DE TECNOLOGIAS  
& EXCELÊNCIA EM SAÚDE



Generalitat de Catalunya  
Departament de Salut



# Risk Sharing Agreements for Health Technology Assessment in the Brazilian National Health System

## The experience in Catalonia

Marta Roig, Joaquim Delgadillo  
Medicines Area, Catalan Health Service

# The Catalan Health System



# Spanish Healthcare System Funding

**National Health Service  
(Sistema Nacional de Salud,  
SNS)**



**Ministry of Health responsible for healthcare policy**

**Regions responsible for healthcare provision and funding**

- 17 autonomous communities
- Responsible for healthcare resource allocation and budget planning, healthcare service planning, health technology assessment

**MAIN principles**

- Decentralized system
- Universal and equal access to healthcare

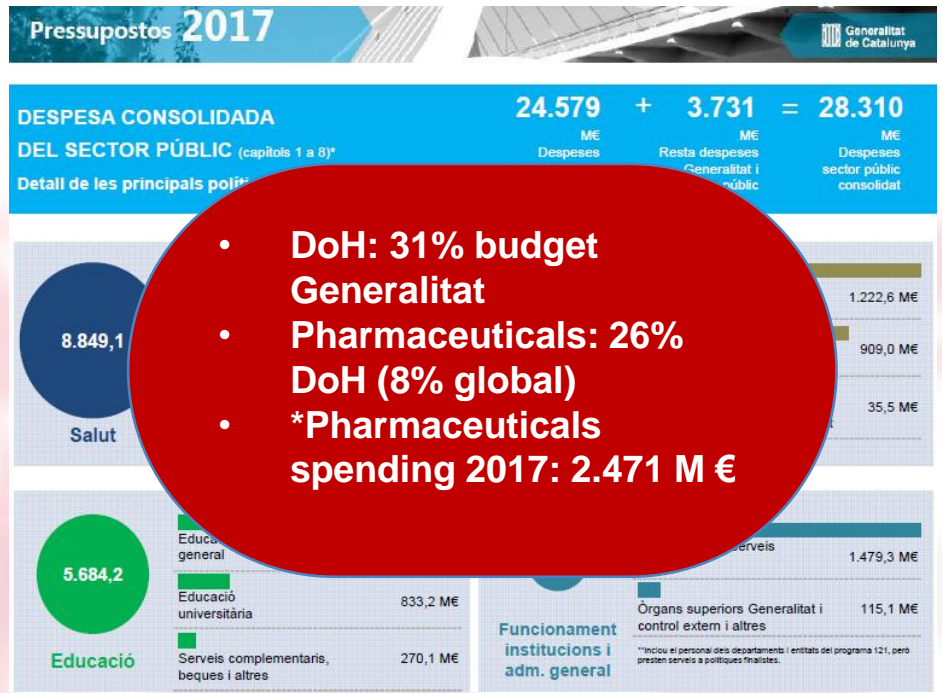
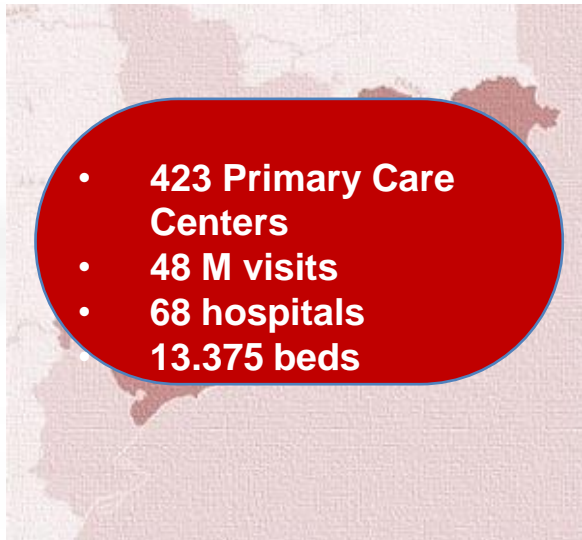
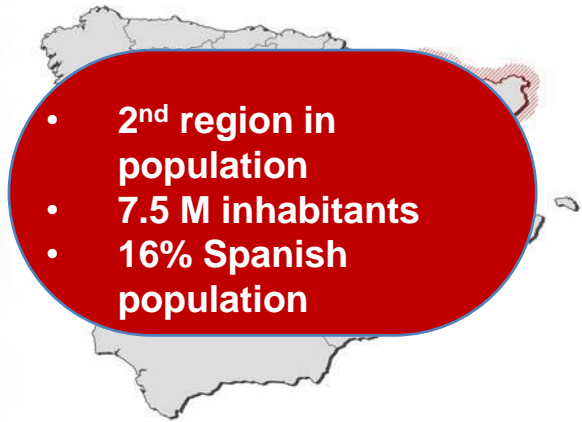
# Catalan Health Service

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- **Catalan Health Service - CatSalut**
- Universal coverage and free at the point of use
- Funded by taxes
- Multi-provider system
- Relationship between Catalan Health Service (public insurance) and providers contractually full accounted (health objectives, activity, economic amount, rate [pricing], invoicing system, evaluation system). Providers have the **duty to share information** with both CatSalut and other providers

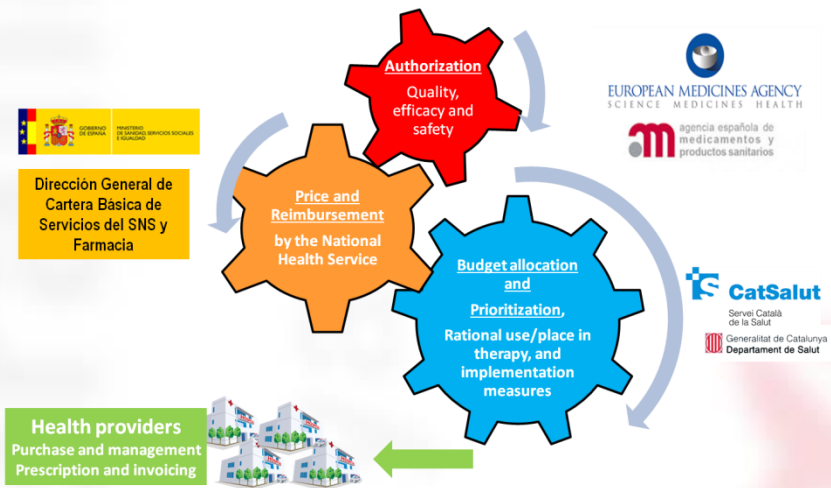
# Some data on Catalan Health Service



- DoH: 31% budget Generalitat
- Pharmaceuticals: 26% DoH (8% global)
- \*Pharmaceuticals spending 2017: 2.471 M €

\*[www.hacienda.gob.es/es-ES/CDI/Paginas/EstabilidadPresupuestaria/InformacionAAPPs/Indicadores-sobre-Gasto-Farmac%C3%A9utico-y-Sanitario.aspx](http://www.hacienda.gob.es/es-ES/CDI/Paginas/EstabilidadPresupuestaria/InformacionAAPPs/Indicadores-sobre-Gasto-Farmac%C3%A9utico-y-Sanitario.aspx)

# Medicines access in Catalonia



- Authorization and P&R at national level
  - P&R supported by national reports on therapeutic positioning
  - Reports coordinated, contributions of 17 regions
- P&R decision binding for all regions



- Budget allocation
- **Therapeutic positioning**
  - Pharmacotherapeutic Harmonization Program: technical appraisal
  - Catalan Pharmacotherapeutic Committee
    - Prioritization and clinical criteria for use
    - Invoicing system and requirements
    - Managed access: RSA
- **Patient and treatment registry (RPT)**
  - Tool for implementation (outpatient hospital medicines)
  - Real world data collection and analysis

# Key driver RSA in Catalonia



## Catalonia's Health Plan: Management centered on health results



Pla de Salut  
de Catalunya 2016-2020



Generalitat de Catalunya  
Departament de Salut

### COMPROMÍS I PARTICIPACIÓ

- |  |                                    |
|--|------------------------------------|
| 1<br>Les persones, la seva salut i el sistema sanitari | 2<br>Implicació dels professionals |
|--|------------------------------------|

### ATENCIÓ DE QUALITAT

- |                    |                                 |   |                                     |                                   |
|--------------------|---------------------------------|---|-------------------------------------|-----------------------------------|
| 3<br>Salut pública | 4<br>Accessibilitat i resolució | 5<br>Política farmacèutica i del medicament | 6<br>Atenció integrada i cronicitat | 7<br>Recerca i innovació en salut |
|--------------------|---------------------------------|---|-------------------------------------|-----------------------------------|

### BON GOVERN

- |                               |   |                     |                              |
|-------------------------------|---|---------------------|------------------------------|
| 8<br>Excel·lència i seguretat | 9<br>Avaluació de resultats i transparència | 10<br>Salut digital | 11<br>Integració territorial |
|-------------------------------|---|---------------------|------------------------------|

### SALUT A TOTES LES POLÍTIQUES

- |  |
|--|
| 12<br>Polítiques interdepartamentals i intersectorials |
|--|

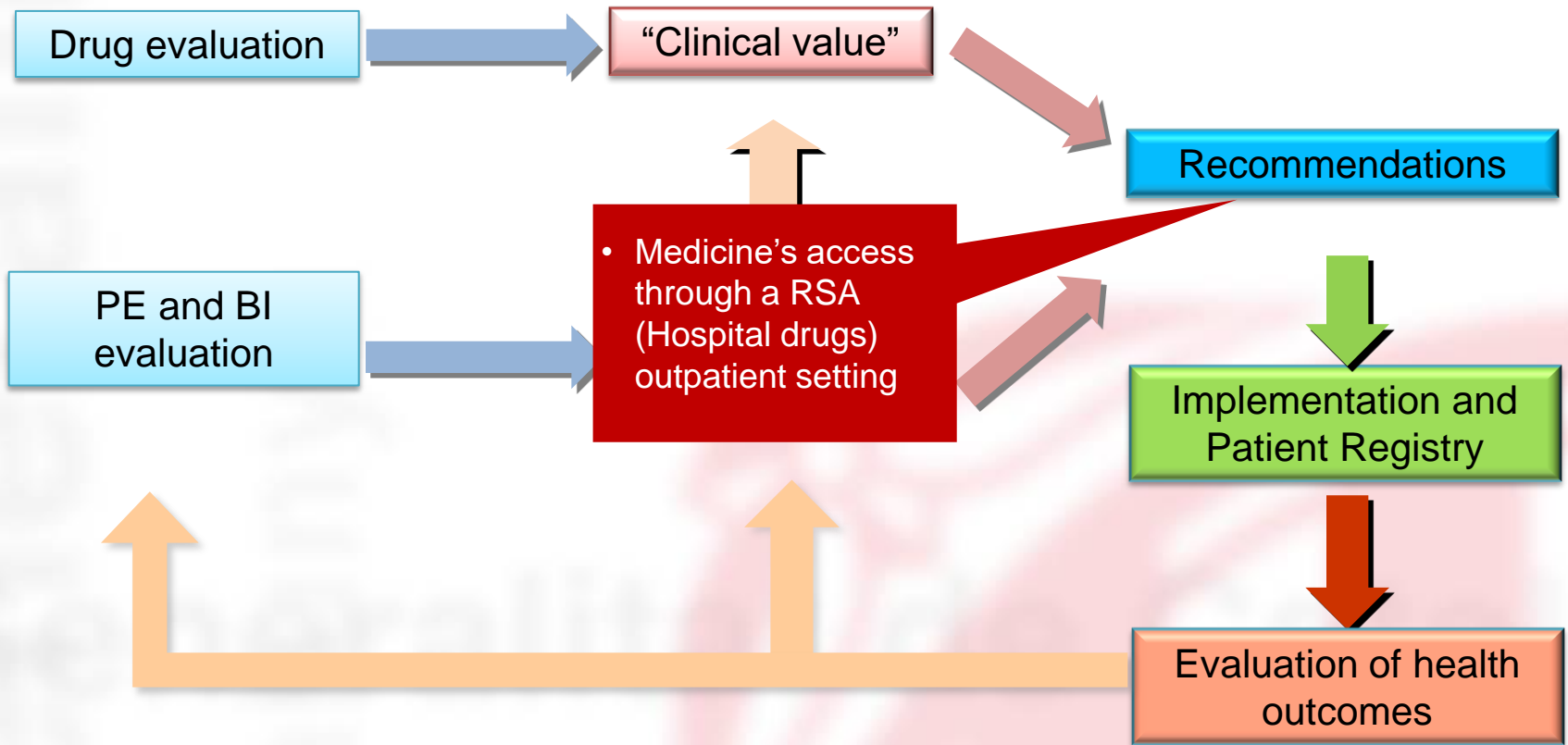
### ÀREES PRIORITÀRIES DE SALUT I PROJECTES SINGULARS

- |                                   |                          |              |                        |                          |                   |                     |                  |        |
|-----------------------------------|--------------------------|--------------|------------------------|--------------------------|-------------------|---------------------|------------------|--------|
| Infants i adolescents vulnerables | Geni gran i discapacitat | Salut mental | Malalties minoritàries | Malalties transmissibles | Aparell locomotor | Aparell respiratori | Sistema vascular | Càncer |
|-----------------------------------|--------------------------|--------------|------------------------|--------------------------|-------------------|---------------------|------------------|--------|

**Project 5.3.**  
**Procurement and financing of medicines based on results**

**Project 5.5.**  
**Assessment of medicines use based on health results**

# Pharmacotherapeutic Harmonization Program



2 (+ 2 alternates)  
patients out of 24  
members participating in  
each committee



# Guidance documents

## GUÍA Y RECOMENDACIONES PARA LA REALIZACIÓN Y PRESENTACIÓN DE EVALUACIONES ECONÓMICAS Y ANÁLISIS DE IMPACTO PRESUPUESTARIO DE MEDICAMENTOS EN EL ÁMBITO DEL CATSALUT

OCTUBRE 2014

 Generalitat de Catalunya  
Departament de Salut

 **CatSalut**  
Servei Català  
de la Salut

Versión 1.0

© Servei Català de la Salut (CatSalut), 2014

[https://catsalut.gencat.cat/web/.content/minisite/catsalut/proveidors\\_professionals/medicaments\\_farmacia/farmaeconomica/caeip/documents/gaeip\\_publica\\_castellano\\_octubre\\_2014\\_catsalut.pdf](https://catsalut.gencat.cat/web/.content/minisite/catsalut/proveidors_professionals/medicaments_farmacia/farmaeconomica/caeip/documents/gaeip_publica_castellano_octubre_2014_catsalut.pdf)

## GUÍA PARA LA DEFINICIÓN DE CRITERIOS DE APLICACIÓN DE ESQUEMAS DE PAGO BASADOS EN RESULTADOS (EPR) EN EL ÁMBITO FARMACOTERAPÉUTICO (ACUERDOS DE RIESGO COMPARTIDO)

MAYO 2014

Versión 1.0

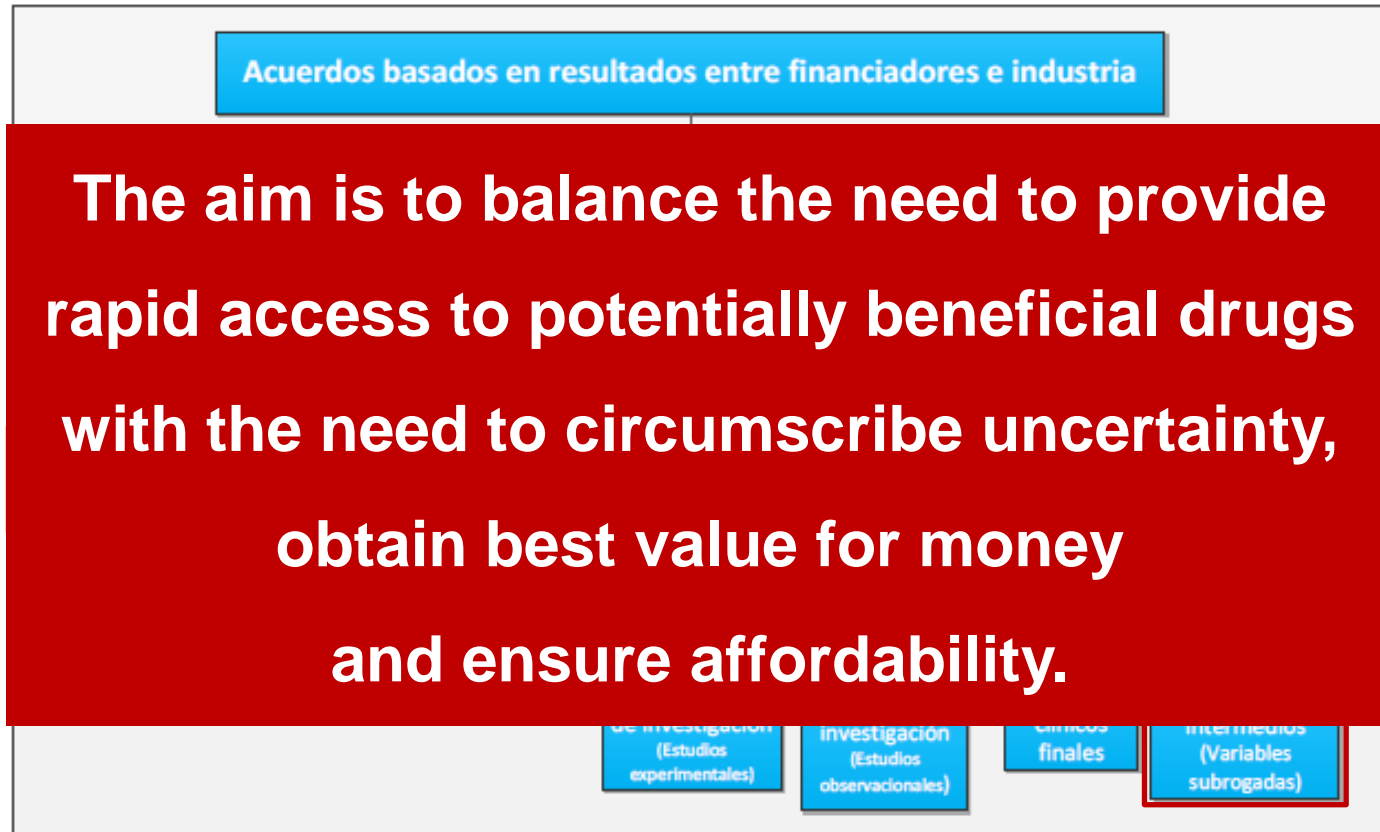
 Generalitat de Catalunya  
Departament de Salut

 **CatSalut**  
Servei Català  
de la Salut

[https://catsalut.gencat.cat/web/.content/minisite/catsalut/proveidors\\_professionals/medicaments\\_farmacia/acords-risc-compartit/guia\\_epr\\_castellano\\_1.0.pdf](https://catsalut.gencat.cat/web/.content/minisite/catsalut/proveidors_professionals/medicaments_farmacia/acords-risc-compartit/guia_epr_castellano_1.0.pdf)

# Taxonomy RSA

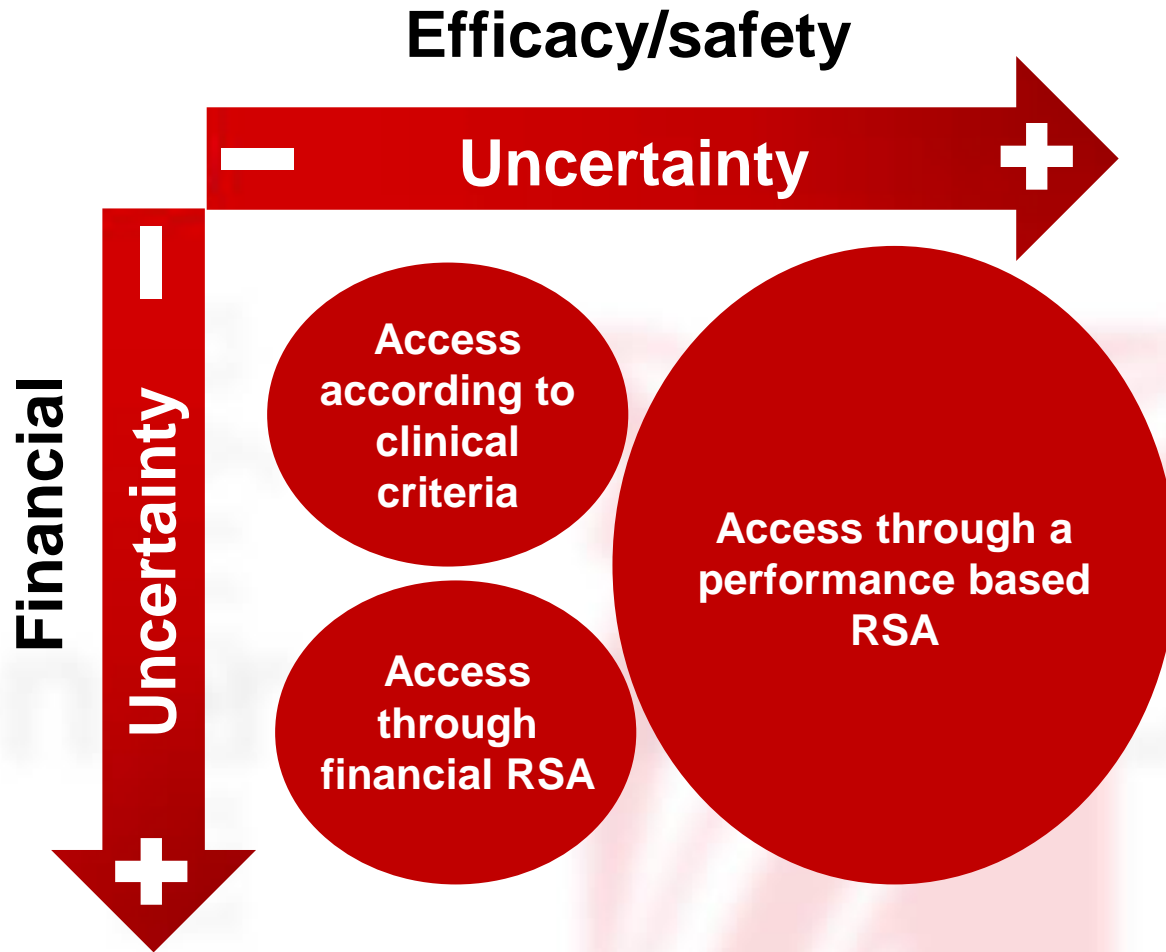
Figura 1. Tipologías de ARC



Fuente: Adaptado de Carlson J, et al. *Linking payment to health outcomes: a taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers*. Health Policy 2010, 96: 179-190

# Managing uncertainty

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# **Risk Sharing Agreements in Catalonia**



# Pilot RSA experience in Catalonia

JOURNAL OF MEDICAL ECONOMICS, 2016  
http://dx.doi.org/10.1080/13696998.2016.1215991  
Article 0038.R2/1215991

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ORIGINAL RESEARCH

## Financial consequences of a payment-by-results scheme in Catalonia: gefitinib in advanced EGFR-mutation positive non-small-cell lung cancer

Ana Clapes<sup>a</sup>, Montse Gasol<sup>b</sup>, Rosana Cajal<sup>c</sup>, Luis Segú<sup>d</sup>, Ricard Crespo<sup>a</sup>, Ramón Mora<sup>b</sup>, Susana Simon<sup>c</sup>, Luis A Cordero<sup>c</sup>, Candela Calle<sup>a</sup>, Antoni Gilibert<sup>b</sup> and Josep R Germà<sup>a</sup>

<sup>a</sup>Catalan Institute of Oncology, Barcelona, Spain; <sup>b</sup>Catalan Health Service, Barcelona, Spain; <sup>c</sup>AstraZeneca, Madrid, Spain; <sup>d</sup>Oblikue Consulting, Barcelona, Spain

### ABSTRACT

**Background:** In 2011 the first payment-by-results (PbR) scheme in Catalonia was signed between the Catalan Institute of Oncology (ICO), the Catalan Health Service, and AstraZeneca (AZ) for the introduction of gefitinib in the treatment of advanced EGFR-mutation positive non-small-cell lung cancer. The PbR scheme includes two evaluation points: at week 8, responses, stabilization and progression were evaluated, and at week 16 stabilization was confirmed. AZ was to reimburse the total treatment cost of patients that failed treatment, defined as progression at weeks 8 or 16.

**Objective:** To estimate the financial consequences of this PbR reimbursement model and determine the perception of the stakeholders involved in the agreement.

**Methods:** Differential drug costs between two scenarios, with and without the PbR, were calculated. A qualitative investigation of the organizational elements was performed by interviewing the parties involved in the agreement.

**Results:** Forty-one patients were included from June 2011 to October 2013 and assessed at two evaluation points. Clinical results were comparable to those observed in the pivotal studies of gefitinib. The difference in the cost of gefitinib using the PbR compared to the traditional purchasing scenario was 6.17% less at 8 weeks, 11.18% at 16 weeks and 4.15% less for the overall treatment. The PbR resulted in total savings of around €36,000 (€880 per patient). From an operational and organizational perspective, the availability of adequate data systems to measure outcomes and monitor accountability and the involvement of healthcare professionals were acknowledged as crucial.

**Conclusions:** Tangible and intangible benefits were identified with respect to the interests of the parties involved. This has led to the incorporation of innovation for patients under acceptable conditions.

### ARTICLE HISTORY

Received 19 March 2016  
Accepted 19 July 2016  
Published online 3 August 2016

### KEYWORDS

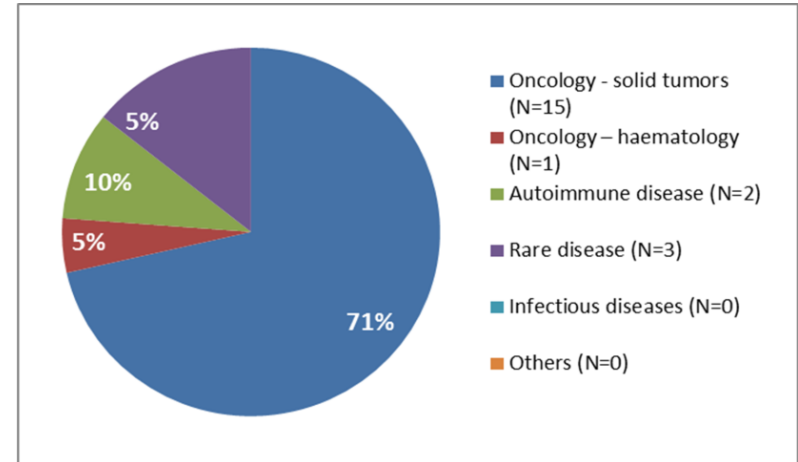
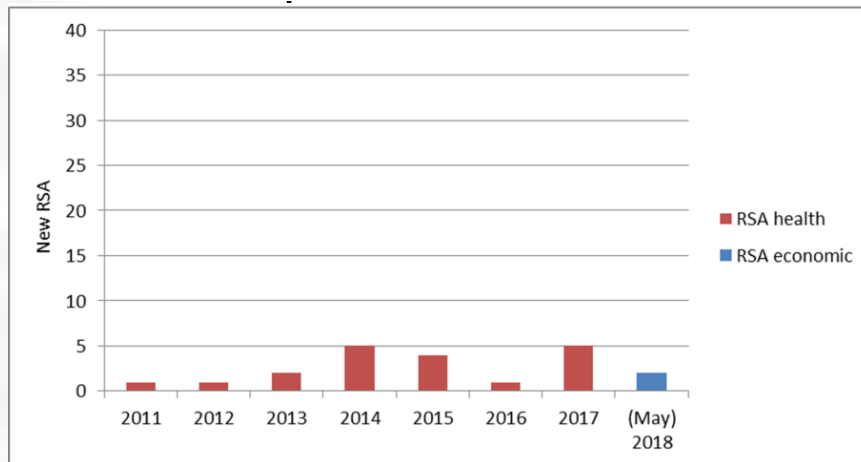
Catalonia; Financial consequences; Oncology; Payment by results

# Pilot RSA experience in Catalonia

## COMPARING RISK SHARING AGREEMENTS IN CATALONIA WITH OTHERS EU COUNTRIES

Guarga L<sup>1</sup>, Fondevilla E<sup>1</sup>, Pastor M<sup>1</sup>, Roig M<sup>1</sup>, Cirera J<sup>1</sup>, Llop C<sup>1</sup>, Alonso E<sup>1</sup>, Obach M<sup>1</sup>, Fontanet M<sup>1</sup>, Gasol M<sup>1</sup>, Zara C<sup>1</sup>, Pontes C<sup>1</sup>  
Delgadillo J<sup>1</sup>

<sup>1</sup>Drug area in the Catalan Health Service (CatSalut)



Catalonia implemented 20 local RSA between January 2011 and May 2018, 86% were based on health outcomes and 14% on economic outcomes.

# Extended Financial RSA

Therapeutic area	Drug	Indication	Laboratory	Start	Conditions
Respiratory				June 2018	Patient subgroup Volume discount
Nephrology				January 2018	Expenditure cap
Gastroenterology				March 2018	Expenditure cap
Oncology				April 2018	Selected indication Direct discount
				April 2018	Selected indication Direct dicount
				January 2019	Expenditure cap

# Extended Performance based RSA

Therapeutic area	Drug	Indication	Laboratory	Start	Hospitals	Patients
<u>Onco-hematology</u>				April 2016	28	256
				March 2017	25	125
				March 2017	5	58
				March 2017	33	382
				June 2017	7	45
<u>Neurology</u>				March 2017	17	167



# Key point - Patients and treatment registry

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- Database that collects data of patients treated with hospital drugs. Mandatory for all hospitals.
- Designed in 2012, objectives:
  - Monitor the use of drugs linked to a diagnosis (basic information: indication & duration)
  - Find out the adherence to clinical criteria set by the Pharmacotherapeutic Harmonization Program → Guarantee the accomplishment of reports
  - Assess health outcomes

More than 500 indications registered  
(oncology, HIV, orphan drugs..)  
More than 230.000 treatments  
Almost 150.000 patients



# Key point - Patients and treatment registry

Patient identification – age, sex, prescription center, ...

Treatment variable

- Start – ATC code, indication, baseline characteristics, criteria of use, ...
- Follow up – treatment duration, health outcomes



- Follow up and monitoring drug prescription
- Evaluation of health outcomes
- Analysis of RSA based on health outcomes

Public reports



We use all of these for DECISION MAKING

**Tratament del càncer colorectal metastàtic amb anticòs monoclonals**

**RESUM ANÀLISIS RESULTATS**  
Programa d'Farmacovigilància  
Farmacovigilància (FV)  
Informe d'avaluació de resultats

**Antecedents**

El càncer colorectal (CC) és la segona causa de mort a causa de la malaltia i la primera causa de mort a causa de la malaltia oncològica (CC) en els homes i la segona causa de mort a causa de la malaltia oncològica (CC) en les dones. El CC és un tipus de càncer que pot afectar qualsevol part del colon i del recte, però que sol afectar més el colon. El CC és un tipus de càncer que pot afectar qualsevol part del colon i del recte, però que sol afectar més el colon. El CC és un tipus de càncer que pot afectar qualsevol part del colon i del recte, però que sol afectar més el colon.

**Metodologia**

El present informe té com a objectiu principal descriure el perfil de seguretat dels anticòs monoclonals utilitzats en el tractament del CC metastàtic. Els resultats es basen en les dades de seguretat recollides durant el període de seguiment de 12 mesos des de l'inici del tractament amb anticòs monoclonals.

**Resultats**

En el present estudi es van registrar 1.000 pacients tractats amb anticòs monoclonals, amb 1.000 pacients registrats i 1.000 pacients tractats. Els resultats es basen en les dades de seguretat recollides durant el període de seguiment de 12 mesos des de l'inici del tractament amb anticòs monoclonals.

**Figura 1. Flux de pacients**

**Compliment dels criteris i recomenacions de l'aford**

El 100% dels pacients complien amb els criteris d'afordabilitat i de seguretat establerts en el programa d'afordabilitat i de seguretat.

**Dades de seguiment i resultats en salut**

El 100% dels pacients complien amb els criteris de seguiment establerts en el programa d'afordabilitat i de seguretat.

# Agreement process



## Pharmacotherapeutic Harmonization Program



RECOMENDATIONS/  
CRITERIA OF USE, ACCESS  
AND PURCHASING



Servei Català  
de la Salut

Agreement hospita  
activity

Agreement CatSalut  
and Pharmaceutical  
Company

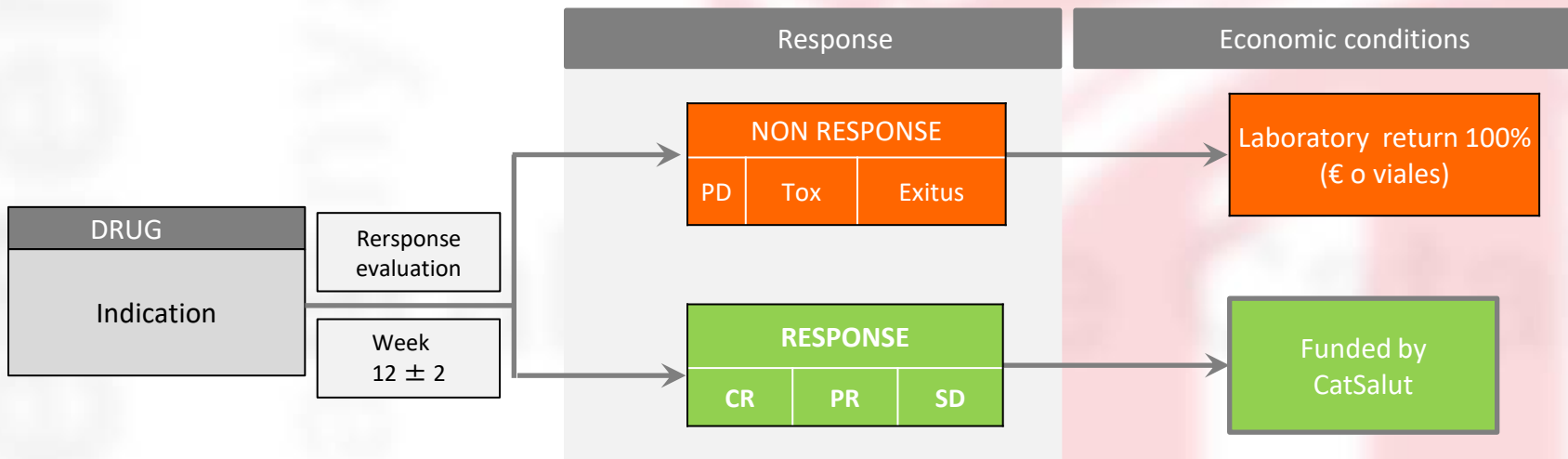
Agreement hospital and  
Pharmaceutical Company



Hospital/  
Providers

# Performance based RSA - SCHEME

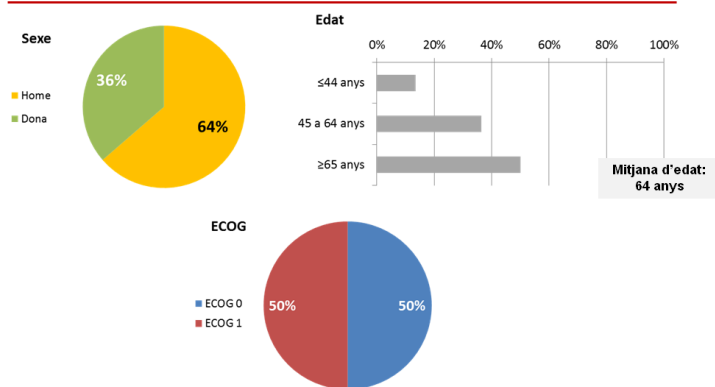
- ✓ **Clinical conditions:**
  - ✓ Eligibility criteria defined by Pharmacotherapeutic Harmonization Program
  - ✓ Response evaluation: e.g., Oncology drug - RECIST criteria at  $12 \pm 2$  weeks
- ✓ **Economic conditions**
- ✓ **Follow-up commissions:** hospital-laboratory and hospital-laboratory-CatSalut



Criteria RECIST: complete response (CR), partial response (PR), stable disease (SD) or progressive disease (PD); \*toxicity and stop

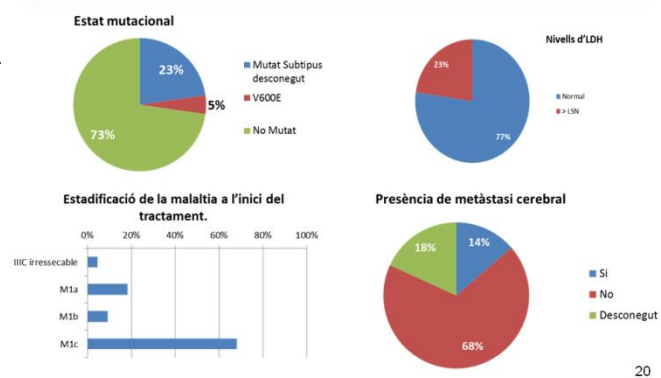
# Performance based RSA – Shared results

## Característiques basals dels pacients (n=22) 1L i 2L (1/2)



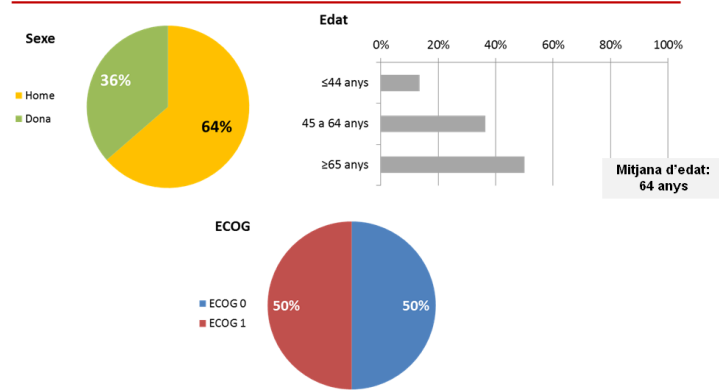
## PATIENTS' DATA

## Característiques basals dels pacients (n=22) 1L i 2L (2/2)



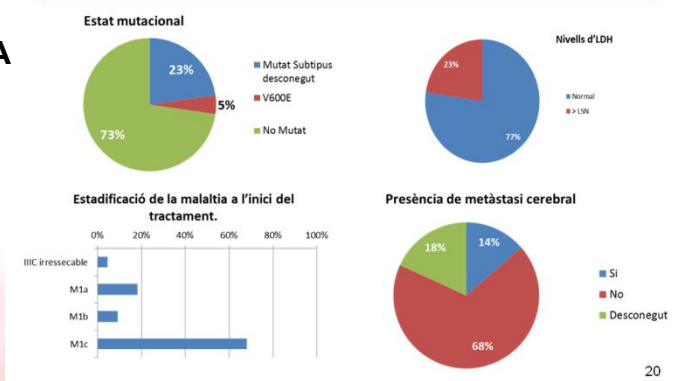
# Performance based RSA – Shared results

## Característiques basals dels pacients (n=22) 1L i 2L (1/2)



## PATIENTS' DATA

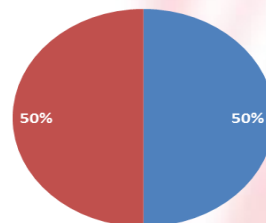
## Característiques basals dels pacients (n=22) 1L i 2L (2/2)



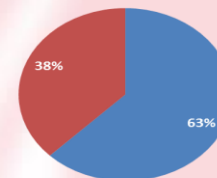
	TOTAL patients	Response	Non Response	Non evaluable
<b>TOTAL</b>	22	11	11	0
<b>1L</b>	16	10	6	0
<b>2L</b>	6	1	5	0

## OUTCOMES

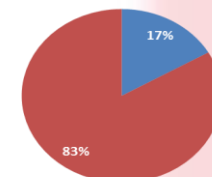
TOTAL (n=22)



1L treatment (n=16)



2L treatment (n=6)



■ Respondors  
■ No Respondors

# Performance based RSA – Overview

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## Outcomes assessment

- Alignment between RSA and CT responses.
- Validation and approval from health professionals.

## Implementation assessment

- Successful clinical management (e.g., follow-up meetings to ensure agreed conditions are met, effective stakeholder communication/engagement).
- Positive experience shared by health professionals.



## Reports

- Confidential
- Overview of key highlights and results obtained by the RSA
- Shared with hospitals



## Room for improvement

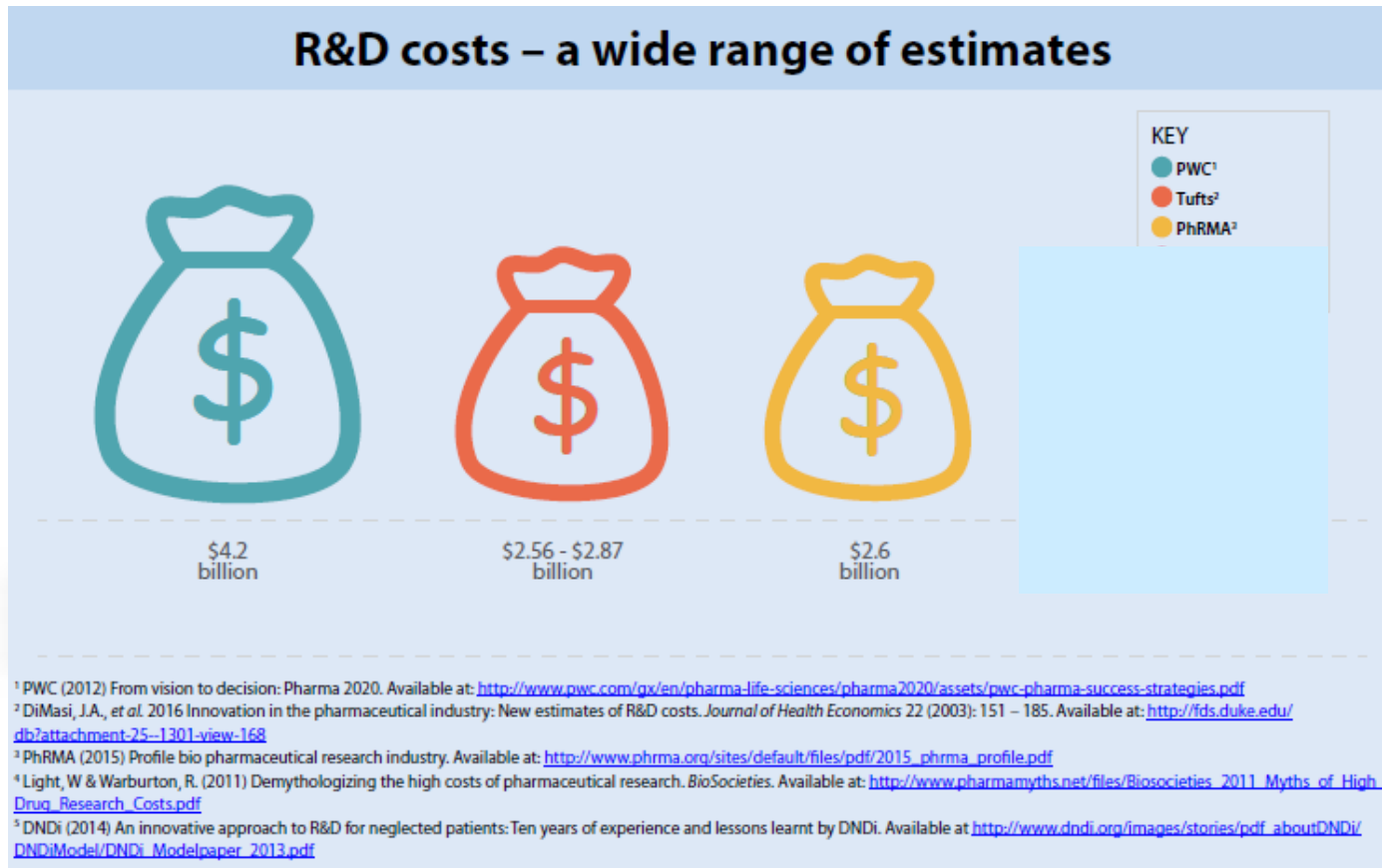
- Eligibility and follow-up criteria
- Timelines
- Better case analysis

# **Key considerations and recommendations**



# But before some thoughts...

It is really so expensive to develop a new drug?



Report of the United Nations Secretary-General's High-Level Panel on Access to Medicines. Promoting innovation and access to health technologies. September 2016

# It is really so expensive to develop a new drug?

JAMA Internal Medicine | Original Investigation

## Research and Development Spending to Bring a Single Cancer Drug to Market and Revenues After Approval

Vinay Prasad, MD, MPH; Sham Mailankody, MBBS

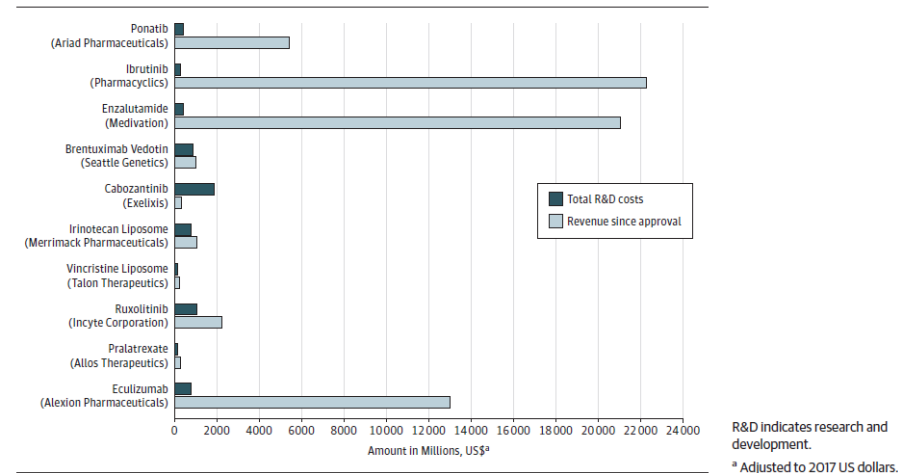
### Key Points

**Question** What is the estimated research and development spending for developing a cancer drug?

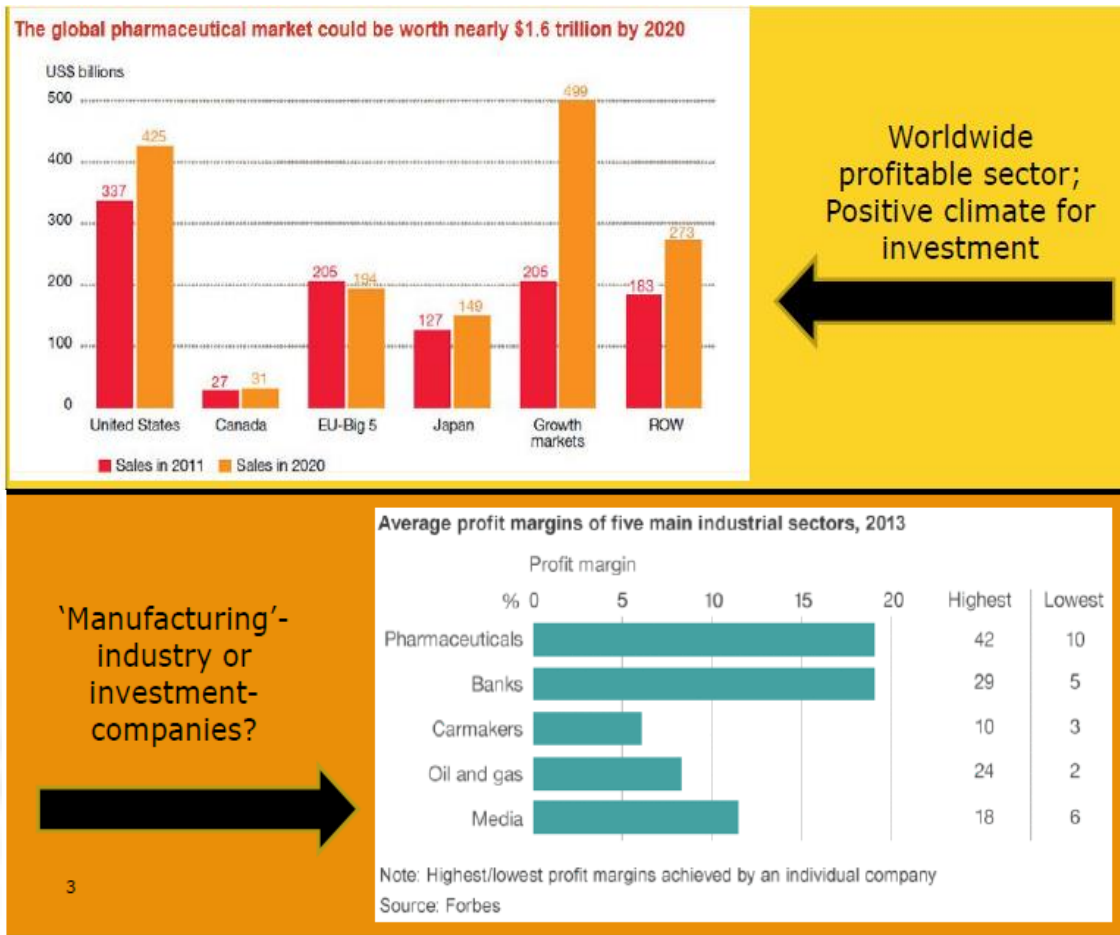
**Findings** In this analysis of US Securities and Exchange Commission filings for 10 cancer drugs, the median cost of developing a single cancer drug was \$648.0 million. The median revenue after approval for such a drug was \$1658.4 million.

**Meaning** These results provide a transparent estimate of research and development spending on cancer drugs and show that the revenue since approval is substantially higher than the preapproval research and development spending.

Figure. Comparison of Drug Development Costs With Revenue Earned After Approval



# It is really so expensive to develop a new drug?



The Dutch Policy position Timely Access to innovative Drugs but with Affordable Prices  
 Dr. Marcel van Raaij, Dutch Ministry of Health.  
 Affordable Drugs Congress Cinderella and Inspire2Live, Amsterdam April 15th 2016

# Managing uncertainty with RSA

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## Limiting budgetary uncertainty

- Financial agreements can be useful
  - Relatively simple tools, such as caps or price-volume agreements
  - Controlling budget impact on its own does not address uncertainty on value

## Limiting uncertainty on evidence

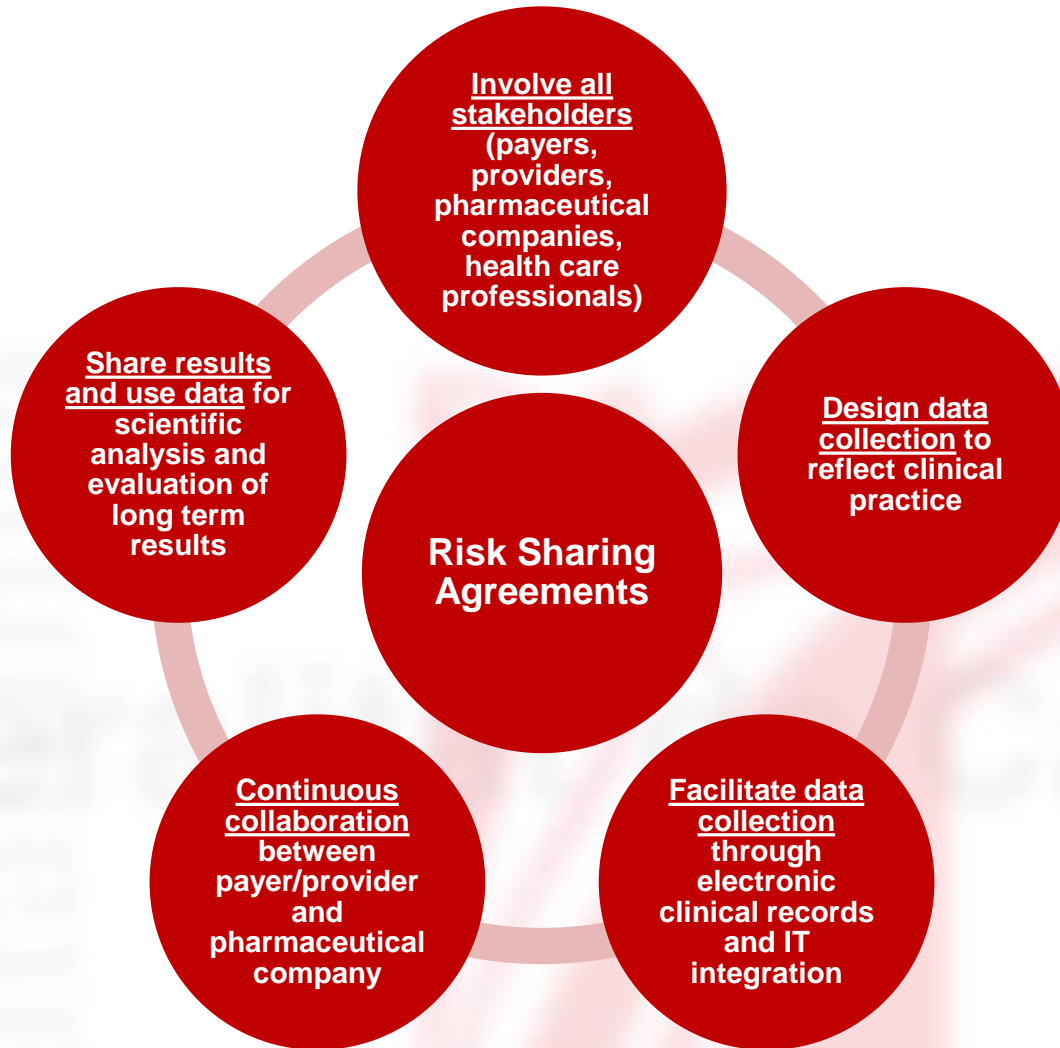
- Performance-based agreements can be useful
  - Implementation of studies or registries may be complex and costly in practice
  - Reliability of data, missing data
  - Definition of effectiveness based on surrogates of unknown clinical relevance as in trials – uncertainty may persist
  - Results available too late – useful to reverse decisions?

## • *Management of stakeholders' expectations*

- **Unfeasibility to conduct further controlled clinical trials**
  - Physicians' and patients' reluctance to enroll into randomized controlled studies if product is commercially available
- **Thus, difficult to gather robust risk/benefit evidence**
  - Bias of observational data (RWD), overestimation of effects
- **Authorization reversal may be not feasible**
  - Patients on treatment requiring continuation
  - Treatment availability becomes SOC
- **Difficulties for pricing revisiting**
  - Negotiation with MAH difficult since most eligible population already treated and product considered SOC

# Recommendations for RSA

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**Muito obrigado**

**Moltes gràcies**