EUROPEAN ORGANIZATION OF REGIONAL AUDIT INSTITUTIONS

SINDICATURA DE COMPTES DE LES ILLES BALEARS

XI CONGRESO Y ASAMBLEA GENERAL DE EURORAI

"Las auditorías de los órganos regionales de control externo en el ámbito de la sanidad pública"

UNIVERSITAT POMPEU FABRA

CENTRE DE RECERCA EN ECONOMIA I SALUT·CRES
UNIVERSITAT POMPEU FABRA

barcelona gse graduate school of economics

Palma de Mallorca 19 October 2022

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To serve public interests and the objectives of social cohesion, efficiency is a necessary but not sufficient condition

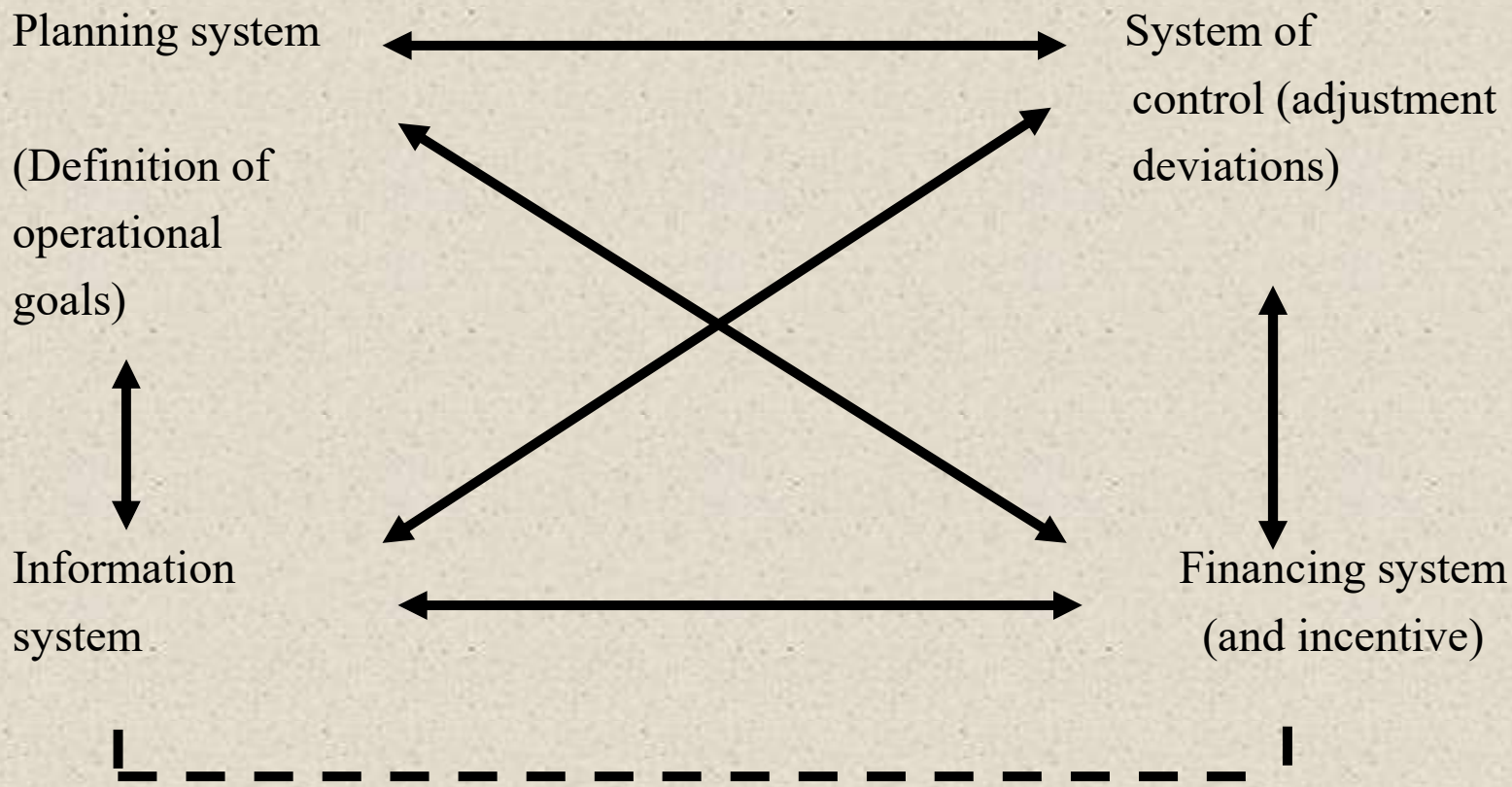
Residual surplus always exists in all human activity. *If not for profit for what?*

Health as a breeding ground for difficulties for an efficient management of public resources

In public health...

- Who is a patient in the public system?
- Opportunity costs are not visible either among beneficiaries or with respect to taxpayers.
- Utilisation does not signal: supply induces demand with a bias in response.
- Little play on the demand side. Rationing by queues rather than prices.
- The provision of services is highly corporatized.
- For managers, innovation does not save.
- No incentive in evaluation in the face of potentially systemic inefficiency.
- Without clear limitation of health policy referents.

STRATEGIC DESIGN OF ORGANIZATIONS (HAX, MAJLUF)



Iter evaluator

- *Input, throughput, output, outcome...* And their insertion in the social value chain (intersectorality) *and when the activity is not always the objective (prevention!) Against false productivity.*
- Health in all policies, *one-health*, etc.
- Costs from budgetary accounts
- Differentiated factors: complexity/ specialization of cases dealt with. *Case-mix*
- Rationality algorithms by health functions to be finally aligned
- Ex ante/ ex post/ concomitant strategies. Audit

DECISION-MAKING ALGORITHMS ACCORDING TO ROLE ASSIGNMENT AND ALLOCATION OF RESPONSIBILITIES IN HEALTHCARE

HEALTH PROVISION CONTEXTS AND STAGES OF DEVELOPMENT

Generic starting-point:

Planning / Financing/ Insurance coverage/ Purchase of services/ Production: Health departments that integrate all supplier entities as budgetary units (cost centres)

***Development*:**

A)

Planning / Insurance/ Financing	Purchase	Production
Health departments	Central service units	Producers

B)

Planning / Insurance/ Financing	Purchase	Production
Health departments	Regional service units	Producers

C)

Planning / Financing	Insurance/	Purchase	Production
Health departments	Regional Health departments	Health geographic areas	Producers

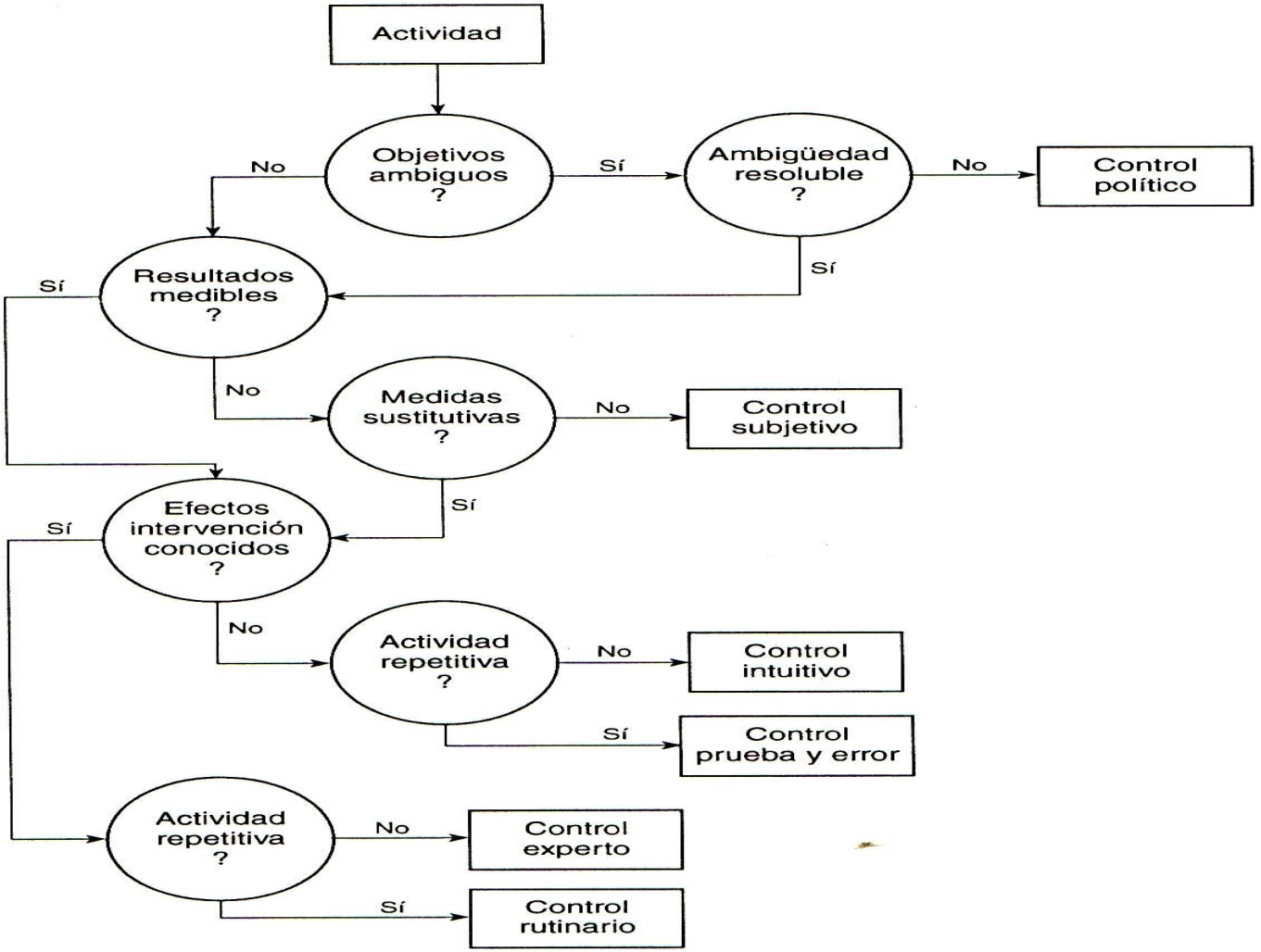
D)

Planning / Financing	Inssurance/	Purchase / Production	
Health departments	Regional Health departments	Health / geographic/ areas	Family doctors/	Integrated supplier networks

E)

Planning / Insurance/ Financing	Inssurance management/Purchase	Production
Health departments Regional Health departments	Integrated / Private sector supplier networks / insurers	Producers

FRAMEWORK AND LIMITS OF EVALUATION BY COMPARISON



Fuente: Hoffstede (1981) y elaboración propia.

FIG. 4.3. *Tipología del control de gestión.*

TRAINING MATTERS: SIX INSTRUMENTAL APPLICATIONS

- HAX/MAJLUF ORGANISATIONAL SYSTEM
- ROLES AND ASSIGNMENT OF RESPONSIBILITIES
- ASSESSMENT AND CONTROL MODULES
- REVERSE ENGINEERING
- DYNAMIC BENCHMARKING FOR COUNTERFACTUALS
- MACRO, MESO, MICRO INTEGRATION

TASKS (I)

- LACK OF INSTITUTIONALISATION FOR THE DEFINITON OF THE BASIC CATALOGUE
- AIREF Fiscal issues. Partial (for instance, Hospital Pharmacy)
- REGIONAL ASSESSMENT AGENCIES ‘Dos and undos’ (health agencies and similar...)
- Medical Products Agency (‘*a la EMEA*’: safety, efficiency)
- Ministry: REVALMED FOR IPTs. Guidelines
- Ex-ante assessment: internal audit body for social ecurity, health provision

  ex post: regional audit offices and courts of audit

TASKS (II)

- Ex-post assessment: regional audit offices and courts of audit.
- Ex-post assessment focuses more on the financial, budgetary and legality control area/sphere (procurement, human resources, etc.)
- Is there a place for operational assessment, or for assessment of efficiency and effectiveness in public sector audit institutions?
- During the presentation I will refer to the difficulties involved in the assessment of efficiency and effectiveness which is carried out from the discipline of Health Economics and I will talk about the need precisely for the development of an independent health policy evaluation agency, so that you can draw your own conclusions.

Designing an **independent authority for the evaluation of health practices and policies** for Spain

- 300 signers!
- December 2022
- No response

HISPANICE PROPOSAL: Authority (obligation) Independent (autonomous within its sphere of competence)

- Clarify: who is calling for the evaluation and for what purpose

- Scope... recommendation/ optimization/ limited recommendation/ recommendation for examination only/ no recommendation. Mandatory in 3 months vs prudential, etc...

- Decide when the price is displayed: during (in the *Patient access* sphere), *ex post* (only to produce guidelines and budgetary impact!). Joint decision

- Involvement of industry, professionals, patients,...

Keys for the implementation of the new evaluation entity

- Separating the assessment from the decision
- Binding decisions
- Clear and transparent methods
- Stable financing (beyond the funds for its constitution)
- Multidisciplinary teams. Training

…..

TO HAVE PRINCIPLES (and make them explicit)

Criteria decide reimbursement

The LFN's task is to decide whether a prescription drug for out-patient care should be reimbursed or not.

There are three criteria which must be fulfilled if a medicine should be reimbursed:

The *human value principle*; which underlines the respect for equality of all human beings and the integrity of every individual. We may not discriminate against people because of sex, race, age and so on when making decisions on reimbursement.

The *need and solidarity principle*; which says that those in greatest need take precedence when it comes to reimbursing pharmaceuticals. In other words, people with more severe diseases are prioritised over people with less severe conditions.

The *cost-effectiveness principle*; which states that the cost for using a medicine should be reasonable from a medical, humanitarian and socia-economic perspective.

These three criteria should all be considered and weighed together by the Board when making its decisions.

REGULATORY DEVELOPMENT

GENERAL REGULATION	
The 1978 Spanish Constitution The 2003 General Budgetary Law Organic Law 2/2012 of April 27 on Budgetary Stability and Financial Sustainability 1986 General Health Law	Appeal to the general principle of efficient allocation of public resources and sustainability of public expenditure
MEDICATIONS	
Medicine Act 1990	Pharmaceutical provision by the NHS should be carried out through targeted funding of medicines according to available resources. It incorporated criteria for financing and pricing related to basic economic aspects.
Strategic Plan of Pharmaceutical Policy of the NHS, 2004	Proposal to incorporate pharmaco-economic studies (cost-effectiveness analysis) to inform the Directorate General of Pharmacy and the Interministerial Commission on Medical Products Prices in their decisions on financing and pricing.
The Medicines (Guarantees and Rational Use) Law 2006	Appeal to an efficient use of medicines. The proposals contained in the 2004 strategic plan disappear.
The National Health System's Inter-territorial Council Agreement of March 2010	The agreement refers to the advisability of expressly including cost-effectiveness criteria in decisions on the incorporation of new medicines in the NHS service portfolio, as well as incorporating this type of information in pharmacotherapeutic guidelines.
Royal Decree-Law 9/2011	For the first time, the consideration of the social value of the health product and its incremental clinical benefit is mentioned as a funding criterion, taking into account its cost-effectiveness ratio. It also mentions the creation of a committee on the cost-effectiveness of medical products and medical devices to advise the inter-ministerial committee on medicine prices.
Royal Decree-Law of April 20 on urgent measures to guarantee the sustainability of the National Health System and improve the quality and safety of its benefits	It maintains the same principles as the RDL 9/2011, practically unchanged, and introduces the role of economic evaluation and the analysis of the budgetary impact as information to be taken into account by the Inter-ministerial Price Commission for Medicines, as well as the provision for the creation of an advisory committee for the pharmaceutical provision of the national health system (created in 2019 by Agreement of the Council of Ministers).
Plan for the consolidation of the Therapeutic Positioning Reports for medicines in the National Health Service	It expressly states that economic information must be included in the procedure for drawing up Therapeutic Positioning Reports. The objective of IPTs shall be to provide a comparative therapeutic and economic assessment of medicinal products in order to provide relevant information, based on scientific evidence, on the position of the new medical product, or its new indication, compared to existing pharmacological or non-pharmacological therapeutic alternatives.

(...) Lobo et al Funcas 2022

Other health technologies	
Law on Cohesion and Guarantees of the National Health Service, 2003	Efficiency is included as one of the relevant criteria in the development of service portfolios.
Ministerial Order SCO/3422/2007	Order for the development of the procedure for updating the common services portfolio of common services the -National Health System. It includes practically unchanged the articles of the law on cohesion and quality.
The National Health System's Inter-territorial Council Agreement of March 2010	Agreement to strengthen the role of health technology assessment agencies by working to strengthen guarantees and security in the authorisation procedure for new technologies in the National Health System, improving the availability of scientific evidence and cost-effectiveness as a basis for decision-making, through the creation of an organisational model in a network with the state and regional agencies.
Royal Decree Law 16/2012 of 20 April, on urgent measures to guarantee the sustainability of the National Health System and to improve the quality and safety of its benefits	The establishment of this Spanish network of agencies for the evaluation of health technologies and National Health System services. Updating of the Cohesion and Quality Law in 2012: the evaluation of the benefits of the service portfolio lies with the ETS network. Its functioning is regulated by Order SSI/1833/2013 of 2 October creating and regulating its board.

What is being asked for today?

Independent authority for the evaluation of health practices and policies

The aim would be to create **an independent evaluation body,** in the style of the Independent Authority for Fiscal Responsibility, with **its own legal personality, functional autonomy and with well-defined procedures and functions**, that would be responsible for the examination of healthcare services, their technologies, medicines and therapeutic indications, interventions for prevention and public health, and other health policies, combining health results with the costs they entail for the National Health Service and for society as a whole.

An Independent Authority for the Evaluation of Health Practices and Policies which, from the analysis of scientific evidence available at any given time, **determines whether the health and social benefits of a health intervention are worth their cost**.

ANEXO

CASE STUDY

PHARMACOECONOMIC EVALUATION

LA PRESCRIPCION ECONÓMICA
Incremental Cost Effectiveness Ratio COST UTILITY ANALYSIS: QALYs!!

- The Incremental Cost Effectiveness Ratio plays an important role in assisting NICE reach a recommendation

$$ICER = \frac{COST\ new - COST\ old}{QALY\ new - QALY\ old}$$

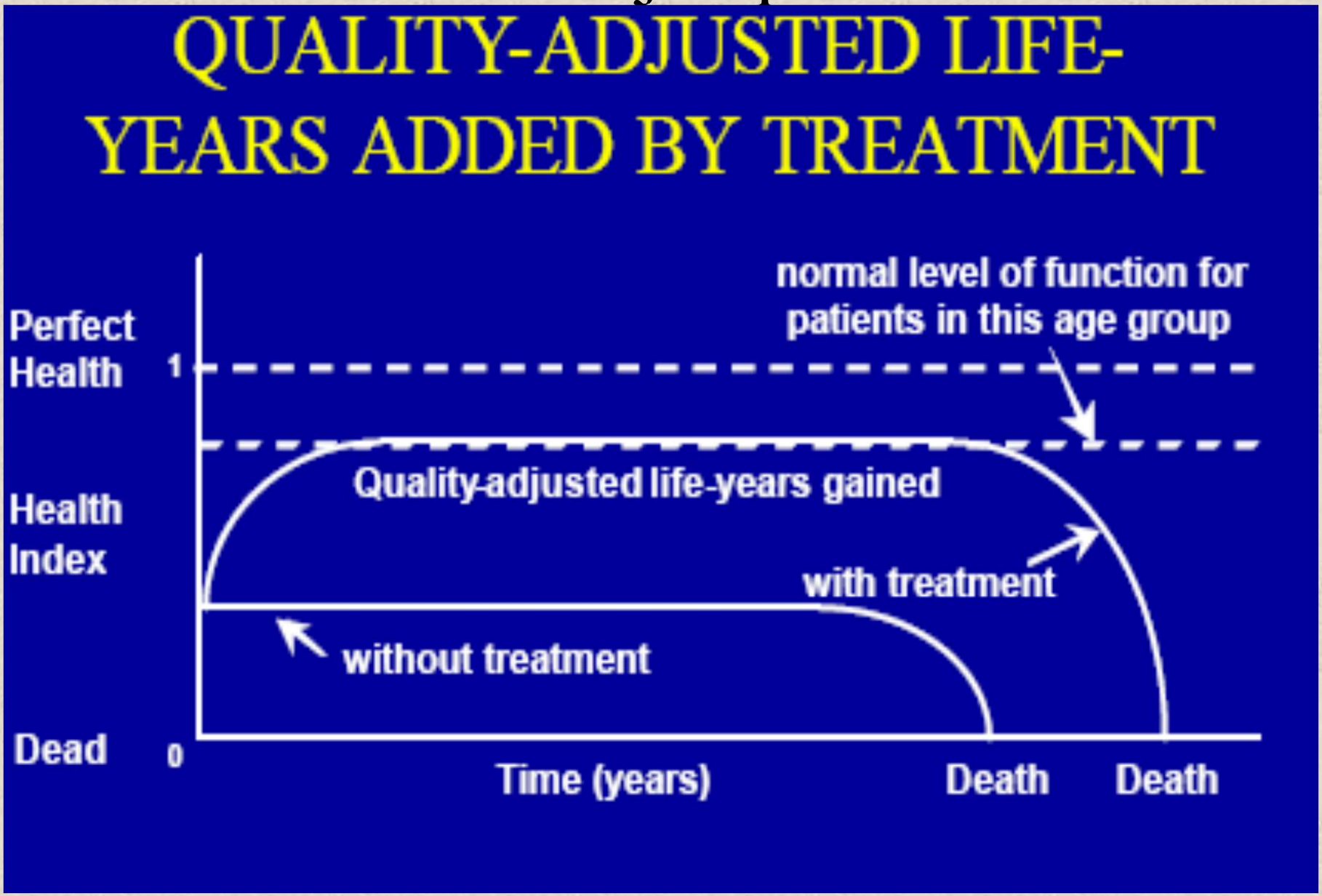
- Produces an estimated cost per quality-adjusted life-year gained

EN ESPAÑA, CUAN LEJOS PODEMOS IR EN LA UTILIZACIÓN DEL COSTE - EFECTIVIDAD?

OTROS HEALTH CARE VALUES (más allá de CUA)

- Prevention
- Quality of Life
- Cost Effectiveness
- Ability to Function
- Equity
- Effectiveness of treatment
- Benefits to many
- Mental Health
- Personal Choice
- Community Compassion
- Impact on society
- Length of Life
- Personal Responsibility

Como ejemplo...



EQ-5D

Mobility

I have no problems in walking about ☐
I have some problems in walking about ☐
I am confined to bed ☐

Self-Care

I have no problems with self-care ☐
I have some problems washing or dressing myself ☐
I am unable to wash or dress myself ☐

Usual Activities (e.g. work, study, housework, family or leisure activities)

I have no problems with performing my usual activities ☐
I have some problems with performing my usual activities ☐
I am unable to perform my usual activities ☐

Pain/Discomfort

I have no pain or discomfort ☐
I have moderate pain or discomfort ☐
I have extreme pain or discomfort ☐

Anxiety/Depression

I am not anxious or depressed ☐
I am moderately anxious or depressed ☐
I am extremely anxious or depressed ☐

EQ-5D HEALTH STATES

Health states: 245 in total

Health State 11111:

No problems walking about
No problems with self care
No problems performing usual activities
No pain or discomfort
Not anxious or depressed

Health state 21111:

Some problems walking about
No problems with health care
No problems performing usual activities
No pain or discomfort
Not anxious or depressed

VALUING HEALTH STATES WITH EQ-5D

- Interviews with 3395 randomly selected members of the public
- Values for each health state on 0-1 preference/utility scale
- Some examples (TTO):
  - Health state 11111 = 1.0
  - Health state 21111 = 0.85
  - Health state 22222 = 0.52
  - Health state 31133 = -0.286

FIG 1: EXPECTED VALUE OF QUALITY AND LENGTH OF LIFE GAINED FOR PATIENTS WITH SEVERE ANGINA AND LEFT MAIN VESSEL DISEASE

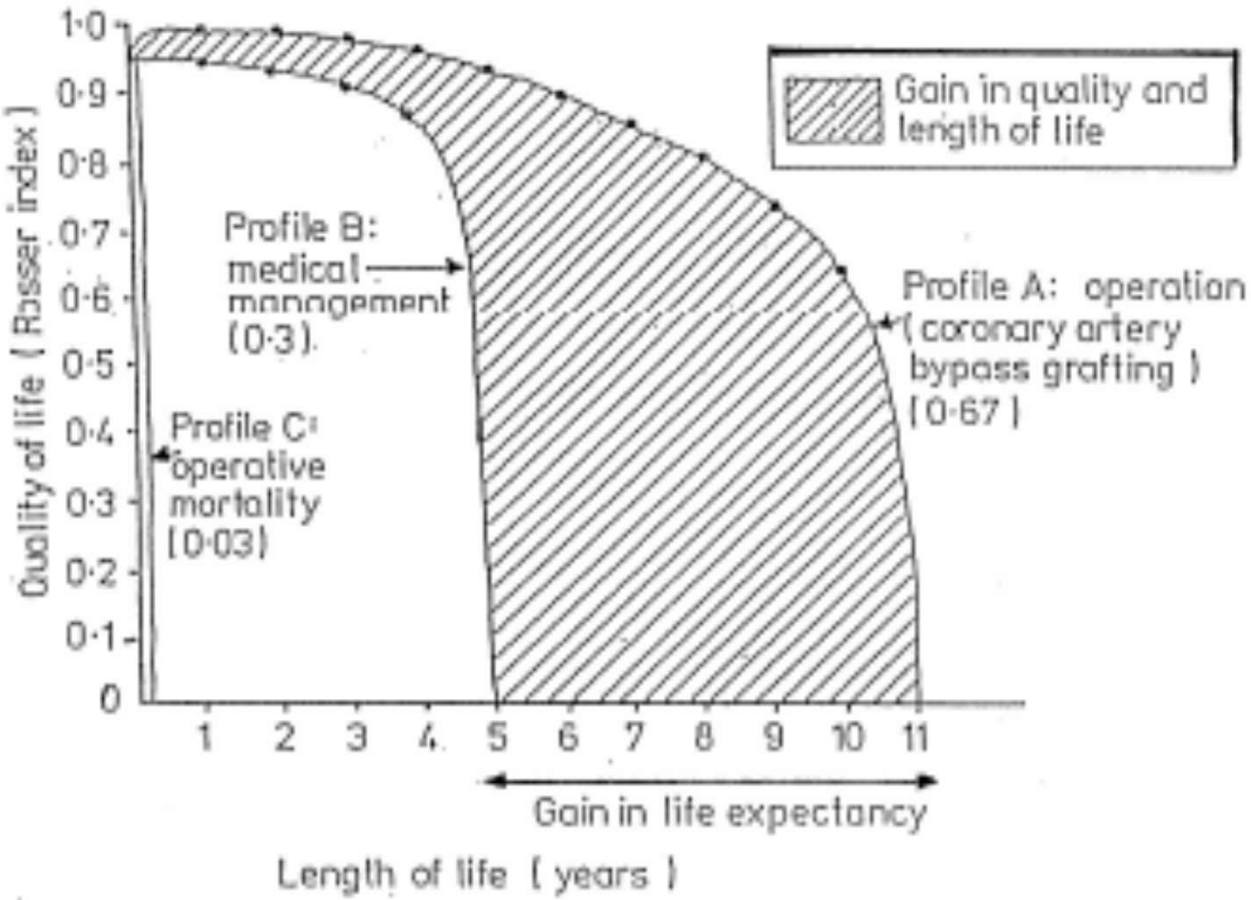
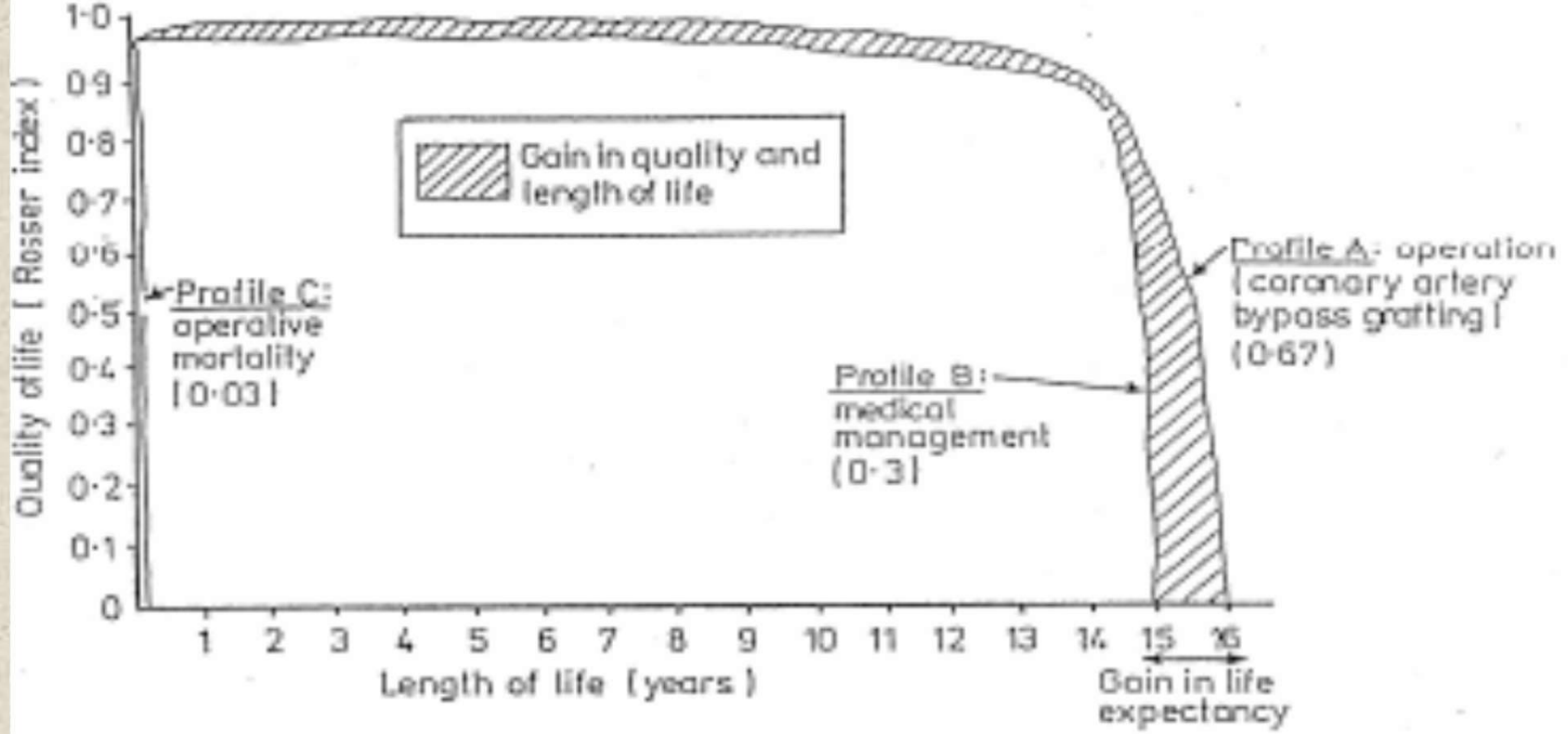


FIG 2: EXPECTED VALUE OF QUALITY AND LENGTH OF LIFE GAINED FOR PATIENTS WITH SEVERE ANGINA AND ONE VESSEL DISEASE



COST PER QALY GAINED FOR SELECTED HEALTH CARE INTERVENTIONS (£, 1989-90 PRICES)

GP advice to stop smoking	260
Hip replacement	1140
CABG for severe angina LMD	1590
GP control of total serum cholesterol	2600
Breast cancer screening	5340
CABG for mild angina 2VD	19250
Hospital haemodialysis	21500

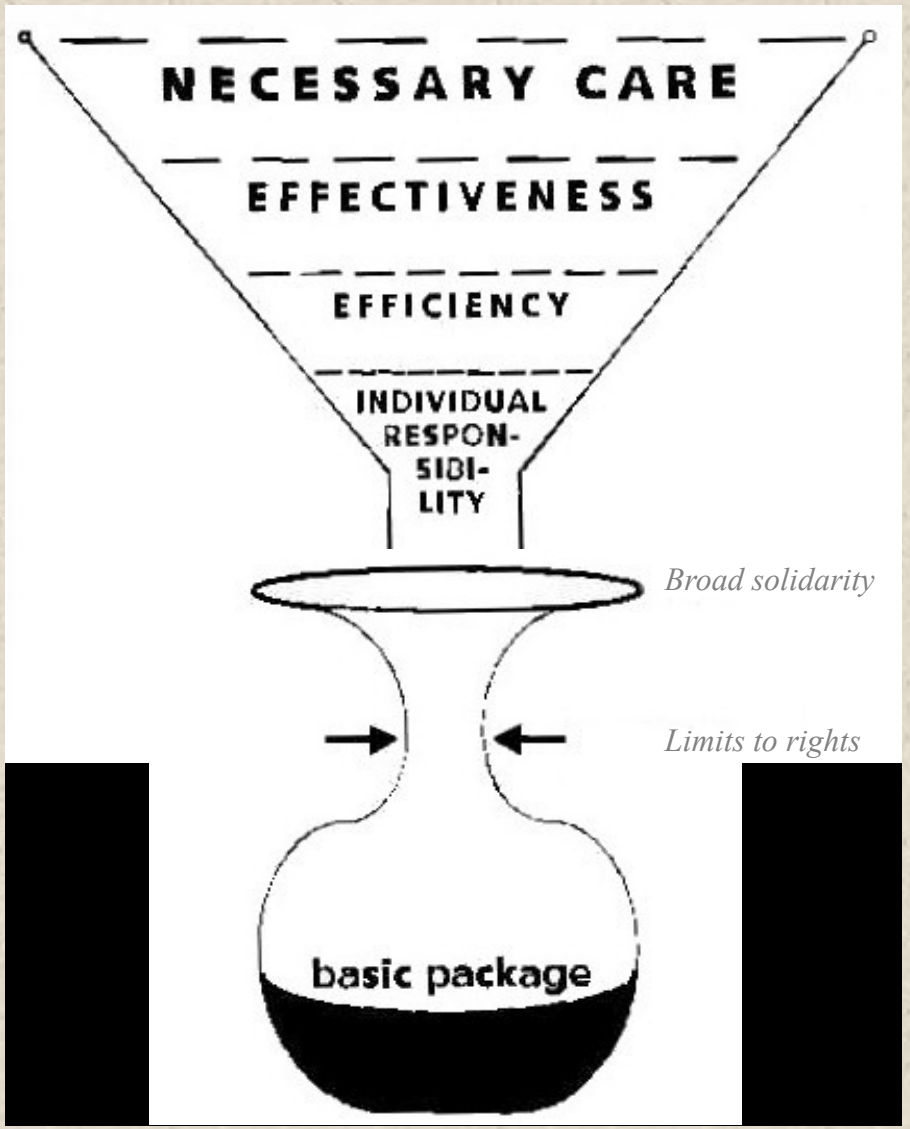
Adapted from Williams (1985).

Cost-utility analyses published from 1976 to 2001, with ratios converted to 2002 US dollars

Ref #	Year of Publ.	Intervention vs. Comparator in Target Population	$/QALY in 2002 US$
(Note: AMI = acute myocardial infarction, CAD = coronary artery disease, CHF = congestive heart failure, DBP = diastolic blood pressure, ECG = electrocardiogram, EMS = emergency medical services, HIV = human immunodeficiency virus, ICD = implantable cardioverter defibrillator, ICU = intensive care unit, LAD = left anterior descending artery, LVEF = left ventricular ejection fraction, NSAID = nonsteroidal anti-inflammatory drugs, PTA = percutaneous transluminal angioplasty, PTFE = polytetrafluorethylene graft.)			
Allergy / Immunology			
157	1989	Use of new contrast media for high risk patients vs Old contrast media for all in radiographic procedures using contrast media	27,000
157	1989	Use of new contrast media for all patients vs Old contrast media for all in radiographic procedures using contrast media	76,000
157	1989	Use of new contrast media for all patients vs Use of new contrast media for high risk patients in radiographic procedures using contrast media	240,000
266	1996	Protein A immunoadsorption column followed by splenectomy vs Column alone in 30-yo woman with chronic immune thrombocytopenic purpura who failed corticosteroid therapy	150,000
266	1996	Splenectomy followed by column vs Protein A immunoadsorption column in 30-yo woman with chronic immune thrombocytopenic purpura who failed corticosteroid therapy	180,000
266	1996	Splenectomy alone vs Protein A immunoadsorption column in 30-yo woman with chronic immune thrombocytopenic purpura who failed corticosteroid therapy	380,000
266	1996	Protein A immunoadsorption column followed by splenectomy vs Splenectomy followed by column in 30-yo woman with chronic immune thrombocytopenic purpura who failed corticosteroid therapy	Cost-saving
266	1996	Protein A immunoadsorption column followed by splenectomy vs Splenectomy alone in 30-yo woman with chronic immune thrombocytopenic purpura who failed corticosteroid therapy	Cost-saving

Digestive diseases

Digestive diseases			
151	2000	Proton pump inhibitor (PPI) on demand vs. Lifestyle modification in 40-year-old patients with severe symptoms of gastroesophageal reflux (GERD)	23,000
151	2000	Proton pump inhibitor (PPI) on demand vs. Lifestyle modification in 40-year-old patients with mild symptoms of gastroesophageal reflux (GERD)	41,000
313	2001	Laprascopic groin hernia repair vs. Open groin hernia repair in patients in the UK and Ireland presenting for elective groin (inguinal or femoral) hernia repair	45,000
313	2001	Laprascopic groin hernia repair vs. Open groin hernia repair in patients in the UK and Ireland presenting for elective groin (inguinal or femoral) hernia repair	100,000
464	1998	Omeprazole vs. Ranitidine 150 mg in patients with peptic stricture who require esophageal dilation	57,000
15	2001	3 infliximab infusions with episodic reinfusion as 2nd-line vs. 1st-line 6-mercaptopurine + metronidazole with 2nd-line infliximab in adult Crohn's disease patients with symptomatic perianal fistulae	Dominated
15	2001	1st-line 6-mercaptopurine + metronidazole with 2nd-line infliximab vs. 1st-line 6-mercaptopurine + metronidazole in adult Crohn's disease patients with symptomatic perianal fistulae	200,000
195	2000	Diagnostic strategy using upper gastrointestinal series vs. Empiric initial trial of proton pump inhibitor in 45-year-old men presenting with symptoms consistent with uncomplicated heartburn but otherwise healthy	Dominated
195	2000	Diagnostic strategy using initial esphagogastroduodenoscopy (EGD) vs. Empiric initial trial of proton pump inhibitor in 45-year-old men presenting with symptoms consistent with uncomplicated heartburn but otherwise healthy	Dominated
195	2000	Empiric initial trial of proton pump inhibitor vs. Empiric initial trial of histamine-2-receptor antagonist in 45-year-old men presenting with symptoms consistent with uncomplicated heartburn but otherwise healthy	11,000
395	1999	Surveillance every 1-5 years vs. No surveillance in patients with Barrett's esophagus	120,000
342	1998	Budesonide controlled ileal release capsules 6 mg/day as maintenance vs. No active maintenance treatment in patients with Crohn's disease of distal ileum & ascending colon with recent exacerbation brought into remission with budesonide controlled ileal release or prednisolone	16,000
25	1993	Laparoscopic cholecystectomy vs Open cholecystectomy in patients with a history of acute or chronic biliary pain and documented gallbladder stones eligible for laparoscopic & open cholecystectomy	Cost-saving
134	1993	Pancreatic surgery & subsequent management vs No treatment for pancreatic necrosis in patients who require operative intervention for pancreatic necrosis	5,000
394	1994	5-yr. endoscopic surveillance; esophagectomy for high-grade dysplasia vs No surveillance; esophagectomy for high grade dysplasia in 55-yo men with Barrett's esophagus	38,000
394	1994	4-yr. endoscopic surveillance; esophagectomy for high-grade dysplasia vs 5-yr. endoscopic surveillance; esophagectomy for high-grade dysplasia in 55-yo men with Barrett's esophagus	380,000
394	1994	Endoscopic surveillance; esophagectomy for high-grade dysplasia vs Endoscopic surveillance; esophagectomy for cancer in 55-yo men with Barrett's esophagus	Cost-saving



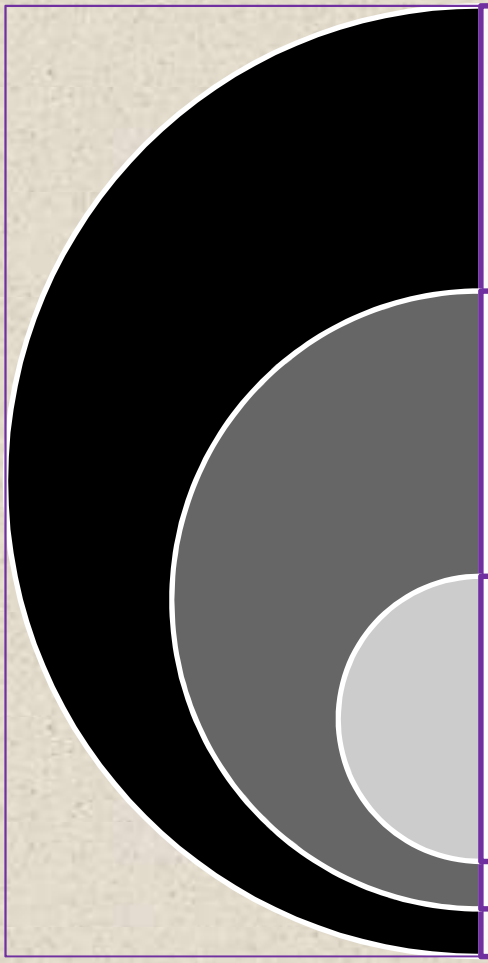
OTRAS PRIORIZACIONES SENSATAS

Dunning's funnel

CRITERIA SCORE

Healthy life years	• to what degree will the condition impact the health of the individual if left untreated? • **0** (no impact) to **10** (high impact)
Impact on suffering	• to what degree does the condition result in pain and suffering? • **0** (no impact) to **5** (high impact)
Population effects	• the degree to which individuals other than the person with the illness will be affected • **0** (no effects) to **5** (widespread effects)
Vulnerable population	• to what degree does the condition affect vulnerable populations? • **0** (no vulnerability) to **5** (high vulnerability)
Tertiary prevention	• to what degree does early treatment prevent complications of the disease? • **0** (doesn't prevent complications) to **5** (prevents severe complications)

COMBINACIONES: Effectiveness & need



Effectiveness	• to what degree does the treatment achieve its intended purpose? • **0** (no effectiveness) to **5** (high effectiveness)
Need for Medical Services	• the proportion of cases in which medical services would be required after the diagnosis has been established • **0** (services never required) to **1** (services always required).
Net Cost	• the cost of treatment for the typical case (including lifetime costs associated with chronic diseases) minus the expected costs if treatment is not provided • **0** (high net cost) to **5** (cost saving).

PROPÓSITO: SEPARAR SOSTENIBILIDAD FINANCIERA DE LA SOLVENCIA SANITARIA (ESPECIALMENTE POST PANDEMIA!!)

- DATOS DEL SECTOR Y CONTEXTUALES: Uffff!!!
- CONSIDERACIONES: QUÉ PODEMOS ESPERAR EN MATERIA DE GESTIÓN DEL GASTO E INGRESOS
- TAREAS PENDIENTES.... DESDE EL LASTRE POST COVID
- CUAN LEJOS PUEDE IR LA EVALUACIÓN ECONÓMICA EN LOS NUEVOS ESCENARIOS

ALTERNATIVAS

- O SE ANCLA EL GASTO, O SE AUMENTA LA FINANCIACIÓN
- SI EL GASTO, CON CRITERIOS DE COSTE EFECTIVIDAD, LA EFECTIVIDAD MEDIDA COMO... EL COSTE CONSIDERADO EN SU TOTALIDAD
- SI EL INGRESO, EN SU CONSIDERACIÓN DE TOTAL O PUBLICO, PRESUPUESTARIO, IMPOSITIVO, CUAL?.
- RELEVANCIA DEL CATÁLOGO EN LA DEFINICIÓN DE MARCOS ESTABLES PARA LA SANIDAD (TAMBIÉN DE LA PRIVADA)

EL ATERRIZAJE DE LAS NUEVAS TECNOLOGIAS

Llegadas

- Vuelos regulares (catálogo)
- Charters fletados (asociaciones)
- Vuelos ambulancia (end of life, sin alternativas)
- Low cost (listas de espera, pacientes)
- Jets privados (high tech, disposición a pagar)

Aeropuertos

- Trenes de aterrizaje
- Ampliaciones de pistas y terminales conectados
- Desviación a otros aeropuertos cercanos

Torre de control

- *First arrived first served*
- Programados
- Según retraso acumulado
- Con escaso ya combustible

La evaluación en los nuevos escenarios

- Reaparece la regla del rescate
- Los AVACs funcionan mal en tratamientos sin alternativas, de final de vida o raras. A veltas con el umbral
- El coste, el de oportunidad colectivo, ignorado
- ¿Quien teme a los silos?: inter = transparente; intra = eficiencia relativa imperante
- La desazón en la constatación de la salud global sus determinantes.
- Conocimiento global, restricción presupuestaria local

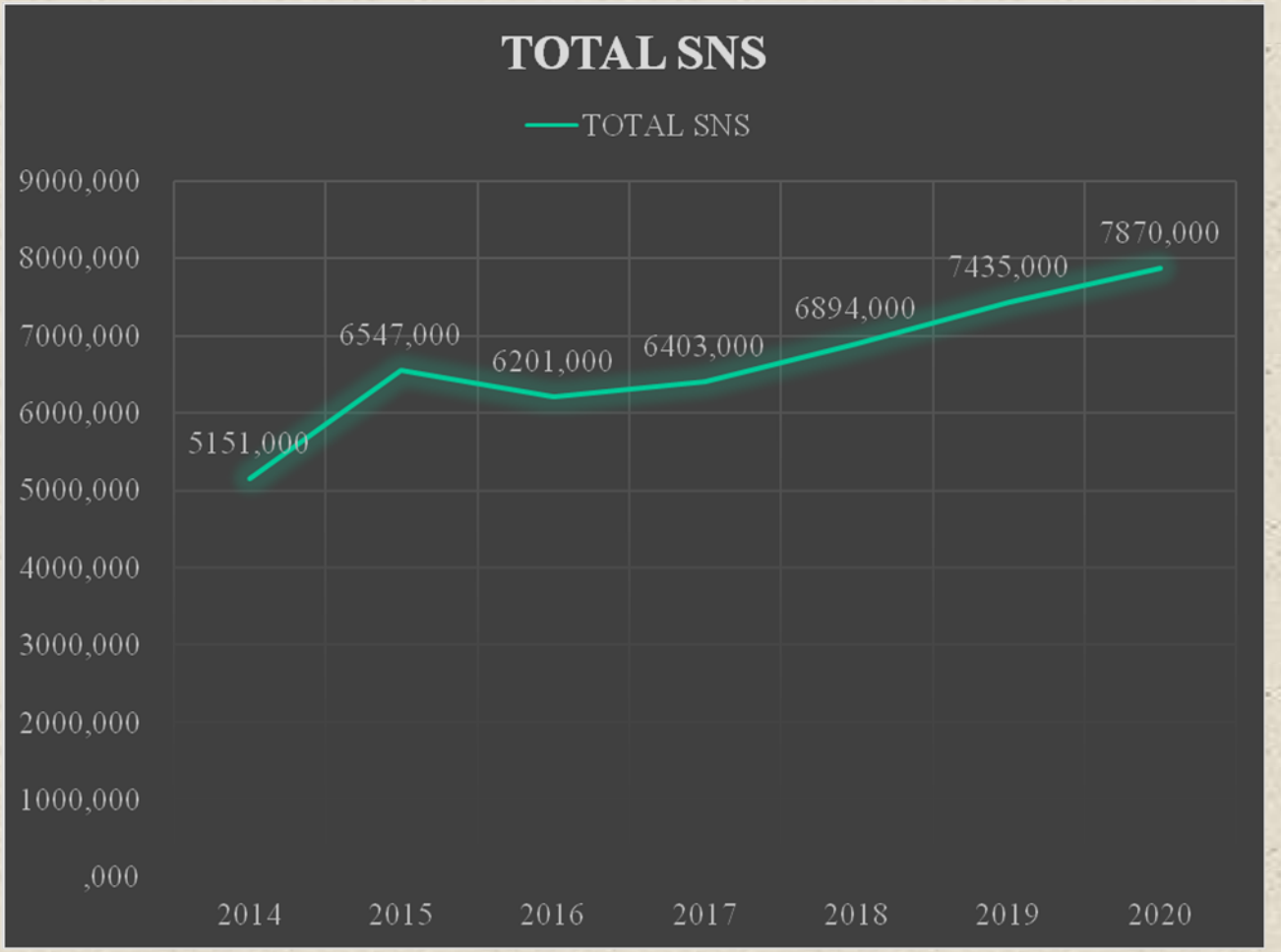
DATOS de partida

	Año 2014	Año 2015	Año 2016	Año 2017	Año 2018	Año 2019	Año 2020
Gasto farmacéutico público (miles €)	15.110.568,15	16.639.863,21	16.686.527,72	17.162.625,92	17.957.409,66	18.741.658,04	19.561.002,34
Gasto sanitario público (miles €)	61.950.561,37	65.741.717,65	66.696.315,70	68.507.032,93	71.090.267,62	75.056.107,35	83.811.368,19
Peso gasto farmacéutico (%)	24,39%	25,31%	25,02%	25,05%	25,26%	24,97%	23,34%

Tabla 4. Peso del gasto farmacéutico respecto al total.

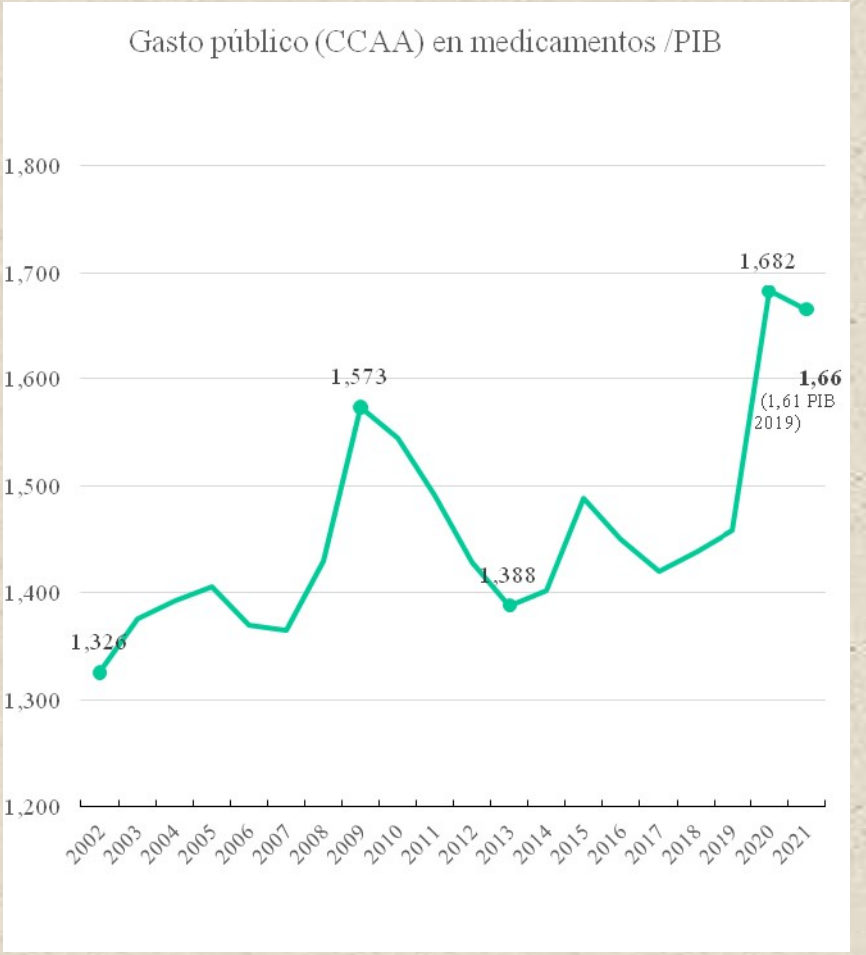
Fuente de información: Elaboración propia. Dirección General de Cartera Común de Servicios del SNS y Farmacia.

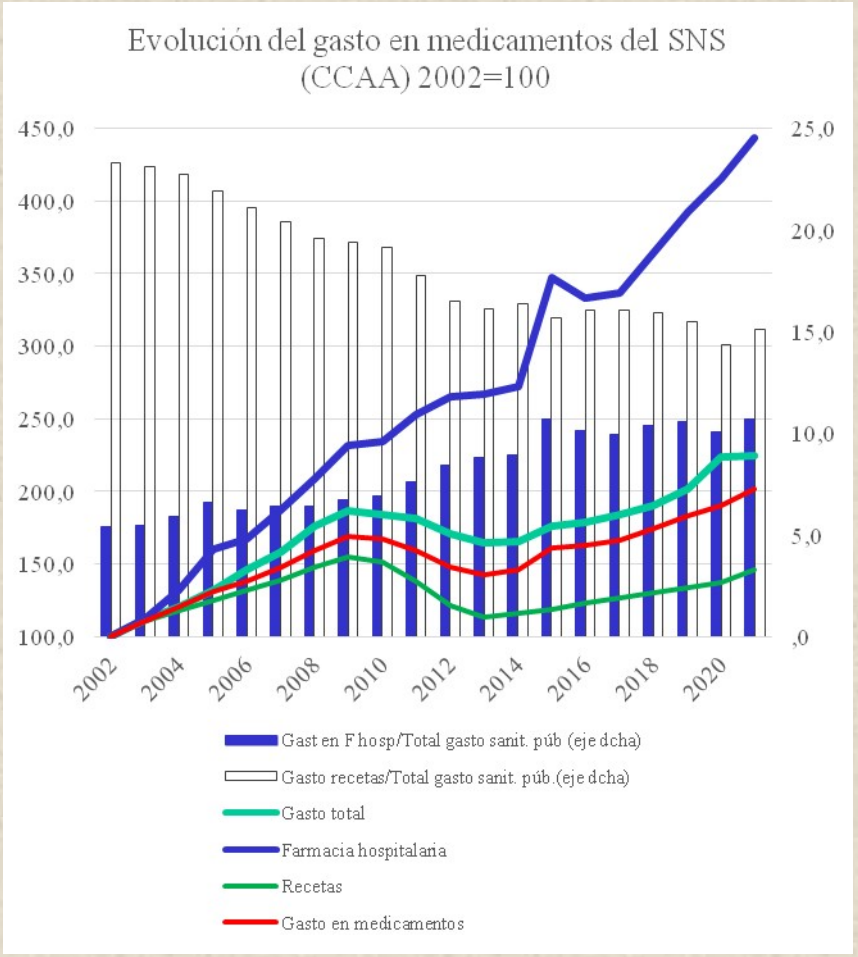
GASTO FARMACÉUTICO HOSPITALARIO 2014 – 2020 en LANZADERA



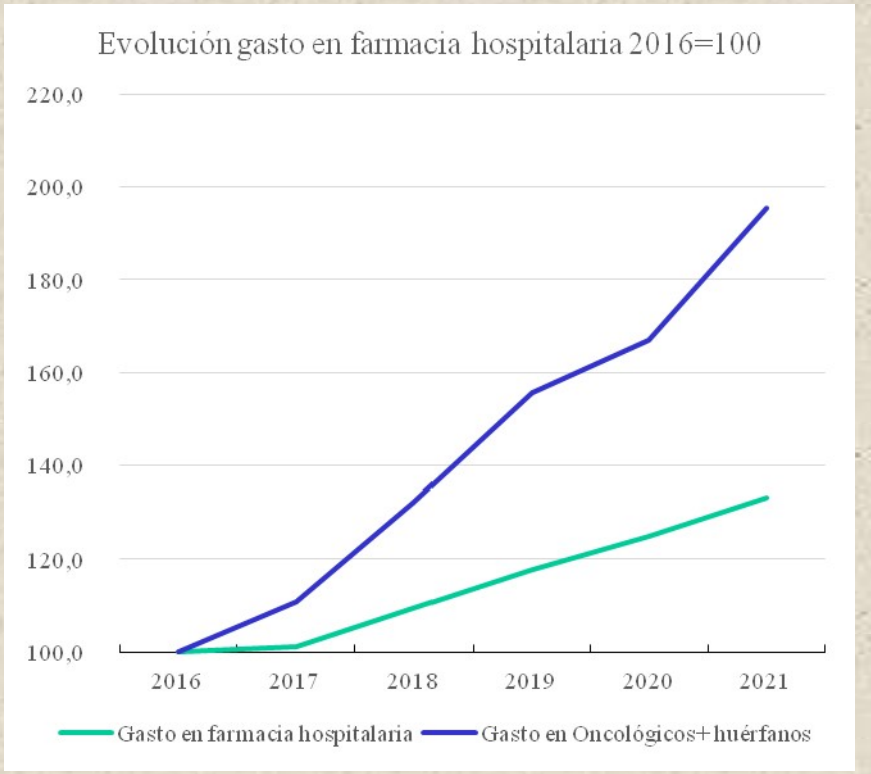
https://www.hacienda.gob.es/es-ES/CDI/Paginas/EstabilidadPresupuestaria/InformacionAAPPs/Indicadores-sobre-Gasto-Farmac%C3%A9utico-y-Sanitario.aspx

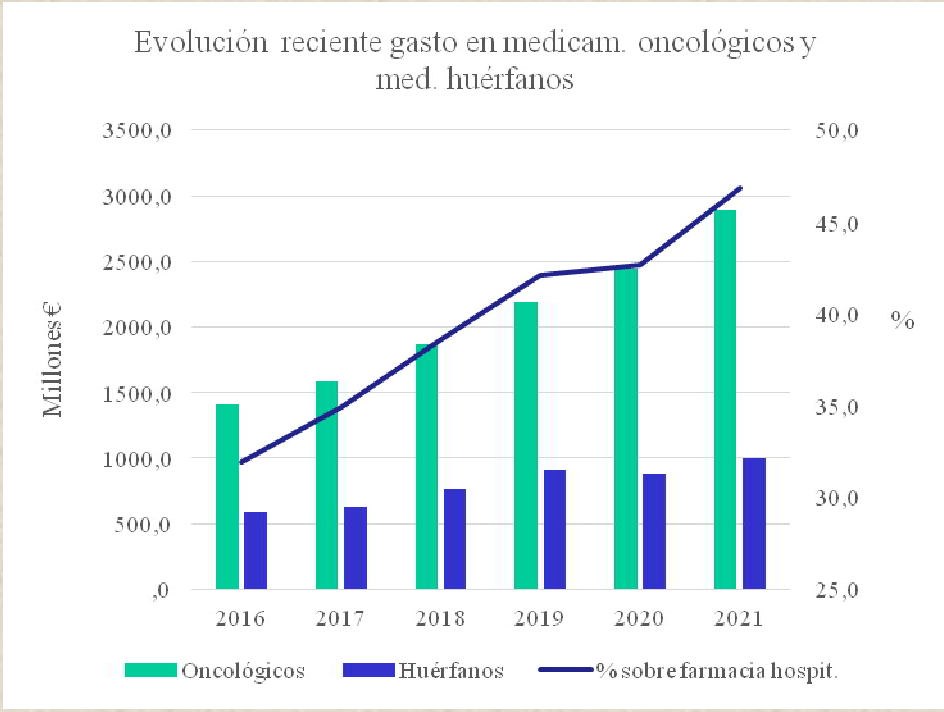
GASTO EN MEDICAMENTOS





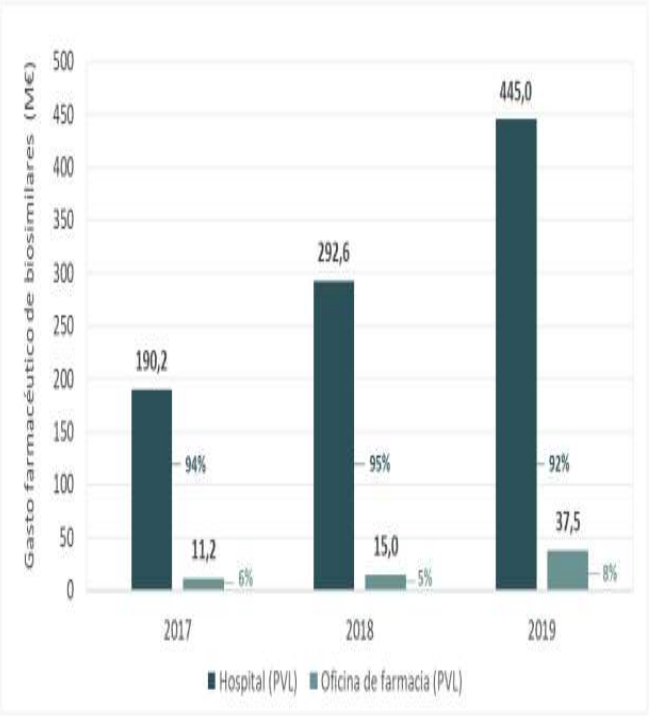
Patología	Coste anual (€)
Mieloma múltiple	130.000
Leucemia linfática crónica	79.000
Enfermedad de pompe	400.000
Distrofia muscular espinal	400.000
Fibrosis Quística	200.000
CAR-T Cell	350.000





Fuente: García Goñi, M. Análisis del impacto presupuestario de los medicamentos biosimilares en el Sistema Nacional de Salud de España, 2009-2022. Biosim , Sep 2020

Figura 9. Evolución del gasto farmacéutico del SNS en medicamentos biosimilares en los ámbitos hospitalario y de oficina de farmacia (*Fuente*: elaboración propia a partir de datos del Ministerio de Sanidad)



26/7/2022

EL PAÍS Sociedad SUSCRÍBETE

MEDICAMENTOS >

Sanidad negocia la adquisición del medicamento más caro del mundo: 2,47 millones por una única dosis

El Libmeldy puede curar por completo la leucodistrofia metacromática, una rara enfermedad genética mortal que frena el desarrollo de los niños al dañar las conexiones neuronales

Cuales son las terapias genéticas aprobadas o de próxima aprobación ?

1,5 m€

2,1 m€

Rare birds

Key gene therapies for rare diseases in development or approved

● Medicines approved in any jurisdiction

Company/therapy name	Disease
BioMarin/Roctavian ●	Haemophilia a
Disease description Blood-clotting disorder	
bluebird bio/eli-cel	Cerebral adrenoleukodystrophy
Rapid loss of neurological function. Often fatal	
bluebird bio/lovo-cel	Sickle-cell disease
Atypical haemoglobin molecules lead to painful condition with wide damage to body and organs	
bluebird bio/Zynteglo* ●	Beta thalassaemia
Blood disorder with reduced levels of working haemoglobin	
CRISPR Therapeutics and Vertex Pharmaceuticals/CTX001	Sickle-cell disease
Atypical haemoglobin molecules lead to painful condition with wide damage to body and organs	
Gensight Biologics/Lumevoq	Leber hereditary optic neuropathy
Mitochondrial genetic disease that causes irreversible and severe vision loss, leading to blindness mostly in teens and young adults	
Novartis/Zolgensma ●	Spinal muscular atrophy
Causes weakening muscles and can be fatal	
Orchard Therapeutics/Libmeldy ●	Metachromatic leukodystrophy
A disorder which degrades the nervous system	
Orchard Therapeutics/Strimvelis ●	ADA-SCID
Inability to fight infections due to lack of white blood cells	
PTC Therapeutics/Upstaza ●	AADC deficiency
Rare genetic disorder of nervous system, interferes with way nerve cells talk to each other	
Roche/Luxturna ●	Inherited retinal disease
Eye disorder that causes vision loss or blindness	
UniQure/EtranaDez†	Haemophilia b
Blood-clotting disorder	
UniQure/Glybera* ●	Lipoprotein lipase deficiency
Disease description Inability to digest fats	

*Withdrawn from Europe †Approval expected in 2022

Source: *The Economist*

The Economist

1,9 m€

2,4 m€

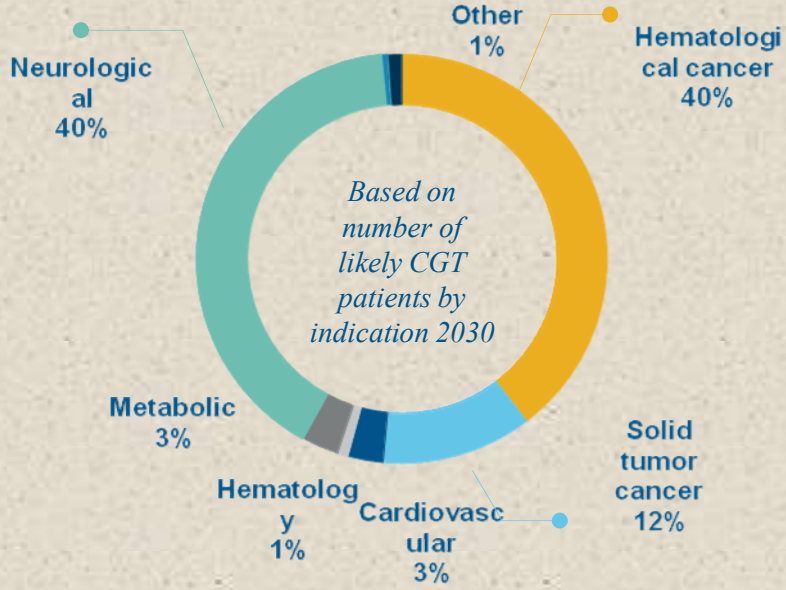
0,59 m€

3m $

0,85 m€

2,1 m$

APLICACIONES LO QUE VIENE: NUEVOS ACCESOS. TERAPIAS GÉNICAS



7000 rare disease identified and majority are genetic

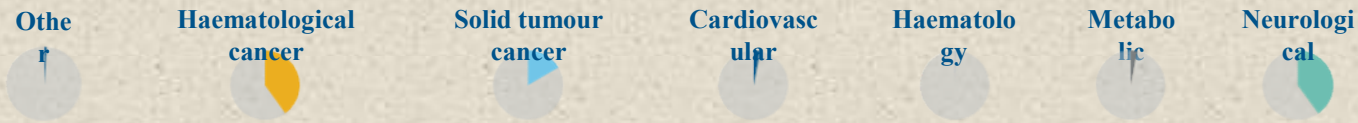
By 2030 there could be 60 Gene Therapies (GTx) on the market

195 USD billion anticipated investment by 2020, 16.5 invested so far

350,000 patients will undergo cell and gene therapies in the US by 2030

31,000 patents filed and granted

Reimbursement potential of up to 30 USD billion by 2031



Source

Gene therapy Market insights (3rd Edition) 2019-2030. Roots Analysis; Estimating the Clinical Pipeline of Cell and Gene Therapies and Their Potential Economic Impact on the US Healthcare system. Quinn et al. Centre of Biomedical Innovation, MIT NEWDIGS FoCUS Writing Group; FoCUS October 2019 Lab Design Highlights

TERAPIAS AVANZADAS. ATERRIZADOS O EN PISTA

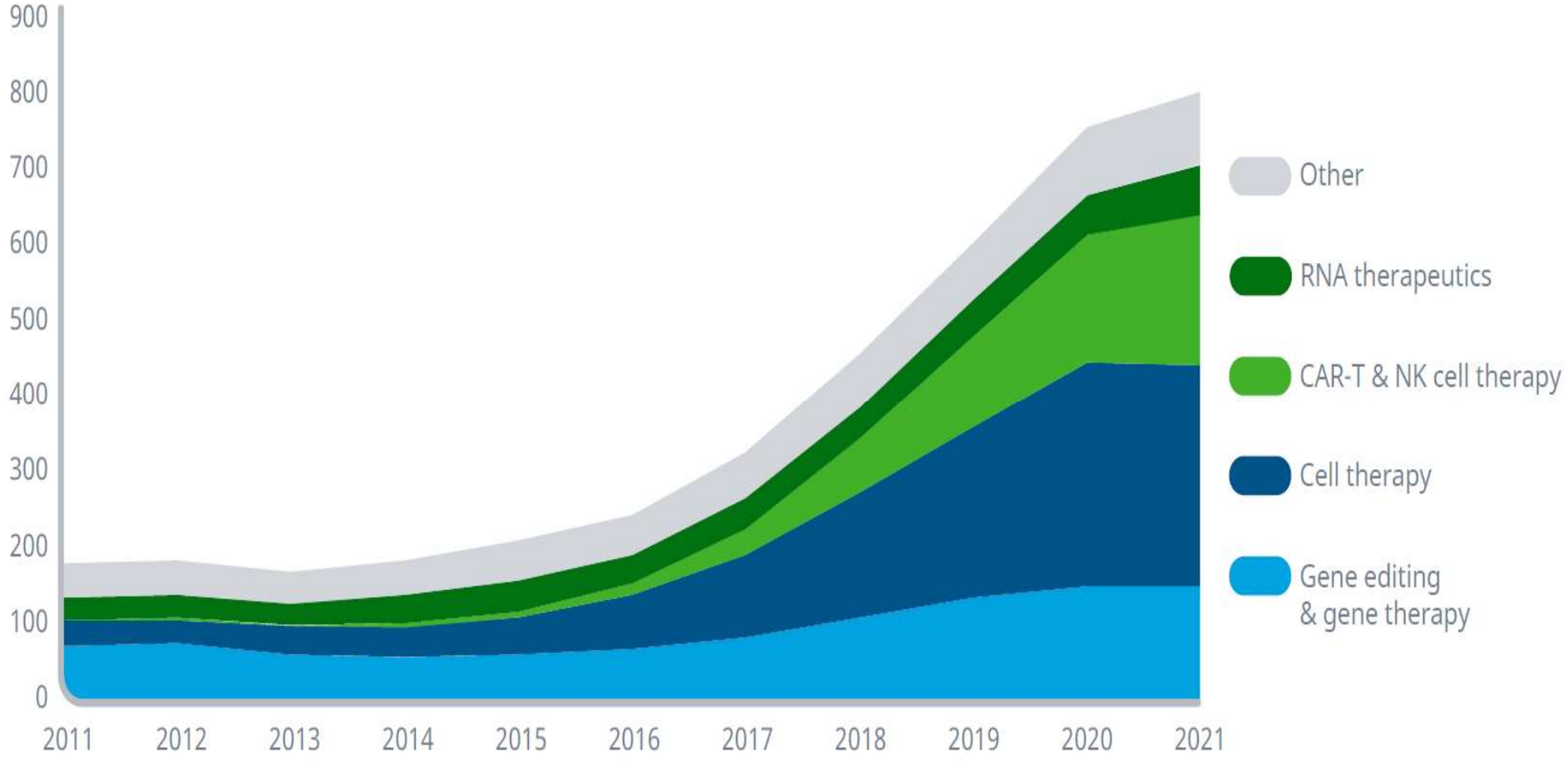
	zolgensma	TECARTUS (brexucabtagene autoleucel)	libmeldy	Abecma (idecabtagene vicleucel)	SKYSONA	BREYANZI ® (lisocabtagene maraleucel)	Ciltacabtagene autoleucel
Indicación	Atrofia muscular espinal	Linfoma manto	Leucodistrofia metacromática	Mieloma múltiple	Adreno - leucodistrofia cerebral	Linfomas	Mieloma múltiple
Titular AC	NOVARTIS	GILEAD	Orchard therapeutics	Bristol Myers Squibb	bluebird bio	Bristol Myers Squibb	janssen
Terapia	Génica	CAR-T	Génica	CAR-T	Terapia génica	CAR-T	CAR-T
Evaluación							

 Medicamento ya autorizado por la Comisión Europea, en fase de evaluación de oferta de precio y condiciones de financiación en España (Ministerio de Sanidad)

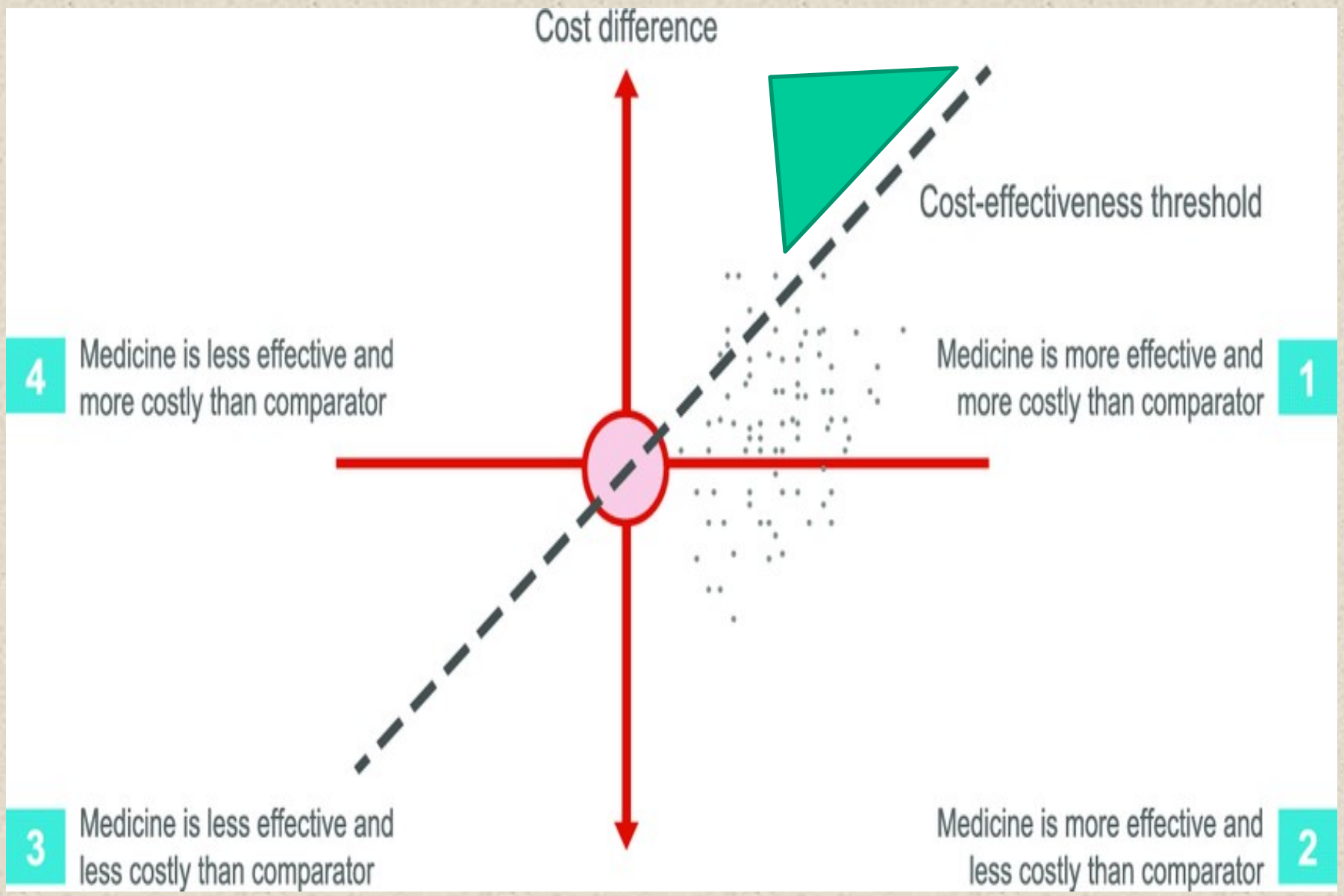
 Medicamento pendiente de autorización por la Comisión Europea, en fase de evaluación de eficacia/seguridad/calidad en Europa (Agencia Europea de Medicamentos)

Medicamentos de terapias avanzadas en fase 1

Exhibit 14: Next-generation biotherapeutics Phase I to regulatory submission pipeline by mechanism, 2011–2021



Source: IQVIA Pipeline Intelligence, Dec 2021; IQVIA Institute, Jan 2022.


Cost difference
Cost-effectiveness threshold
4
Medicine is less effective and more costly than comparator
Medicine is more effective and more costly than comparator
1
3
Medicine is less effective and less costly than comparator
Medicine is more effective and less costly than comparator
2

ESTRATEGIAS DE ABORDAJE

1. **Informes de posicionamiento terapéutico.** Posicionamiento en base a criterios clínicos y económicos.

2. **Restricción de la población**. Selección de los pacientes que más puedan beneficiarse del tratamiento: criterios de inicio, seguimiento y discontinuación de los tratamientos en protocolo farmacoclínico de obligado cumplimentación en todo el SNS. Biomarcadores y otros

3. **Acuerdos pagos por resultados**. Registro de datos (VALTERMED). Implica un Comité de Seguimiento en cada CCAA para la determinación del cumplimiento individual de las condiciones de pago. La información se trasladará a la DG de Cartera Común de Servicios del SNS y Farmacia con objeto de determinar la necesidad de revisión de precio

4. **Acuerdos financieros:**
   - Techo máximo de gasto (incertidumbre en la población a tratar)
   - Coste máximo de tratamiento (incertidumbre en la duración del tratamiento o nº de viales)
   - Pago fraccionado (no vinculado a resultados)
   - Acuerdos precio/volumen.

5. **Medidas para el seguimiento y control del gasto**. Registro de información (SEGUIMED) para controlar que las ventas no exceden el volumen establecido. Si exceden, revisión de PVL. Debate sobre como retornar

TORRE DE CONTROL: *Las alforjas para el viaje actual. REVALMED, IPTs*

La inclusión de medicamentos en la financiación del Sistema Nacional de Salud se posibilita mediante la financiación selectiva y no indiscriminada teniendo en cuenta criterios generales, objetivos y publicados y, concretamente, los siguientes:

a) Gravedad, duración y secuelas de las distintas patologías para las que resulten indicados.

b) Necesidades específicas de ciertos colectivos.

c) Valor terapéutico y social del medicamento y beneficio clínico incremental del mismo teniendo en cuenta su relación coste-efectividad.

d) Racionalización del gasto público destinado a prestación farmacéutica e impacto presupuestario en el Sistema Nacional de Salud.

e) Existencia de medicamentos u otras alternativas terapéuticas para las mismas afecciones a menor precio o inferior coste de tratamiento.

f) Grado de innovación del medicamento.

Real Decreto Legislativo 1/2015, de 24 de julio, por el que se aprueba el texto refundido de la Ley de garantías y uso racional de los medicamentos y productos sanitarios. Art. 92.1

IMPACTO PRESUPUESTARIO

- SÓLO TENDRÍA SENTIDO CUANDO SE HA DEMOSTRADO LA UTILIDAD TERAPÉUTICA, EL VALOR TERAPÉUTICO AÑADIDO Y UNA RELACIÓN COSTE-EFECTIVIDAD INCREMENTAL POSITIVA.

- NO RESPONDE A LA PREGUNTA DE SI "PODEMOS" FINANCIAR UN MEDICAMENTO COSTE-EFECTIVA SINO A LA DE SI "DEBEMOS" FINANCIARLO EN LUGAR DE HACER OTRAS COSAS (USOS ALTERNATIVOS DE LOS RECURSOS Y PRIORIZACIÓN).

- ATIENDE A LOS COSTES DE OPORTUNIDAD DEL FINANCIADOR: ¿QUE TECNOLOGÍAS DEBEN FINANCIARSE (ENTRE LAS QUE SON COSTE-EFECTIVAS) PARA PRODUCIR EL MÁXIMO BENEFICIO SOCIAL?. SUBSTITUYEN?

Confluencia en gasto MDH y CARTs

- Procedimientos diferentes: quien contrata qué y cómo, pero con evaluación basada en *outcomes*
- Papel de la Farmacia Hospitalaria
- Generación de 'excedentes': desde el Servicio Regional a Gerencia para MDH y de SRS a Laboratorios previo paso por Servicios Asistenciales. Empoderamientos!
- Peligros de hipertrofia por un lado en Servicios y apropiación de activos específicos reputacionales por otro
- Conflictos Gerencia-FH vs Servicios, muy marcados por sus protagonistas

En la base, ante la incertidumbre, los acuerdos de riesgo compartido.

(S Peiró 2021)

- Transferencia parcial a la industria de los riesgos asociados a la **incertidumbre** sobre el impacto clínico y presupuestario.

- Los "**managed entry schemes**" limitan la exposición del financiador asociando descuentos a determinados riesgos, **financieros** (acuerdos precio-volumen, caps), o **clínicos** (coverage with evidence development, patient access schemes, risk-sharing agreements/schemes, conditional reimbursement), payment by results/performance)

- Las tipologías más **frecuentes**: acuerdos precio-volumen (40%), fórmulas con generación de evidencia (29%), y **programas de acceso restringido** (13%).

(…)

- Entre sus **ventajas**: enfocan la utilización inicial en las subpoblaciones con mayor beneficio potencial, minimizan el uso off-label, generan evidencia adicional en condiciones reales, reducen costes promocionales, ofrecen un marco predecible de ventas

- **Implementación práctica compleja**, requerimientos informativos, confidencialidad, desincentivo para las inversiones de alto riesgo, traslado de parte de los costes de desarrollo (investigación clínica) desde la industria al sistema de salud

- Importante incremento de los **costes de transacción**, información (y problemas de incentivos al cumplimiento en RPTs).

CUESTIONES de Financiación

Desde la aceptación de:

-Conocimiento global, presupuestos locales. Restricción global sobre la protección social en un sistema público

-Tratamientos

- Silos *inter* desde las decisiones políticas
- Silo *intra*: coste de oportunidad
- Onco (fondo específico) o gravedad (transversal) como conductores?
- NICE is benchmark? HISPANICE?
- Modificadores? (equidad de acceso incompatible con escalas y calidad? Tecnologías innovadoras sin desplazar alternativas?)
- Incluir la perspectiva social (remitido si acaso al análisis de sensibilidad)?.
- Mantener la exención hospitalaria en la financiación?

England's NICE: *EPPUR SI MUOVE*

- "Changes to the healthcare system. The healthcare system is changing. Products are becoming more complicated to evaluate due to innovations such as:
  - personalised medicine
  - digital health technologies
  - cell therapy.
- There's also demand for products to be made available more quickly, sometimes with a lower evidence base than was previously the case. This means there's more demand for the guidance and advice that we produce."
- ...
- "The voluntary scheme for branded medicines pricing and access was agreed by government and the Association of the British Pharmaceutical Industry (ABPI) in December 2018. It commits NICE to a review of its methods for technology appraisals and highly specialised technologies, including the process of guidance production for highly specialised technologies.
- We are taking this opportunity to extend this exercise to include the methods and processes of the Medical Technologies Evaluation Programme and the Diagnostics Assessment Programme as well and align them where appropriate."
- Source: https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation

National Institute for Health and Care Excellence

Review of methods, processes and topic selection for health technology evaluation programmes: conclusions and final update
31ST January 2022

aligning the methods across health technology evaluation programmes*severity of disease*

uncertainty
health inequalities
discounting
understanding and improving the evidence base.

Severity of disease

- At consultation for a quantitative modifier to give an additional weight to health benefits in the most severe conditions, while the additional considerations for life-extending treatments at the end of life (the 'end of life modifier') to be removed. The new severity modifier was developed with an overall size similar to the end of life modifier (referred to as 'opportunity cost neutral

- The proposed modifier used absolute and proportional QALY shortfall to measure severity and defined cut-offs at which additional QALY weights would apply, using 2 steps for different levels of severity.

- ***It is important to emphasise that NICE has critical responsibilities both in supporting innovative health technologies and in ensuring effective use of NHS resources***. In the context of the proposed severity modifier, alongside its advantages in supporting health benefits and innovation for the most severe diseases, ***we must recognise the effects of healthcare displacement and opportunity cost in the NHS***. A larger modifier would displace a greater amount of technologies, services, care and therefore health, for patients elsewhere in the NHS.

CONCLUSIONS from the review

- *We will give additional weight to health benefits in the most severe conditions (a 'severity modifier'), using the approach proposed in the consultation and using weights of 1.2 and 1.7 as the lower and higher weights ('option 1' in the consultation). The additional considerations for life-extending treatments at the end of life (the 'end of life criteria') have been removed.*

- No changes have been made to how we consider health inequalities in health technology evaluations. This is an important and complicated area to include in NICE methods, and more work is needed.

- *We have clarified how we will handle 'uncertainty', particularly in circumstances when evidence from research trials or elsewhere is especially difficult to generate. In favour of flexibility..*

DEBATE: **¿Una evaluación única y cerrada de la innovación?**

Abrir la puerta para que el evaluador apruebe innovaciones reembolsables sólo parcialmente por el financiador,

…ya desde estrategias de riesgo compartido o de descentralización territorial de decisiones (esto es, acepte implícitamente fórmulas de contribuciones adicionales) territoriales –bajo responsabilización fiscal-, o personal –copago-, o de simple financiación privada complementaria para tratamientos de prescripción pública), lo que descomprimiría la presión por el *'in' o 'out'* actual.

- Para ganar la confianza del financiador, también cabrían acuerdos "joint-venture" para compartir por ejemplo, riesgo financiero sobre los deslizamientos presupuestarios, con el pago de un fármaco según resultados (*Pay for performance*) u otras modalidades de devolución, vista la evidencia "ex post" de la efectividad práctica y la aceptación social de las innovaciones.

- Es en este terreno de experimentación donde la descentralización territorial y funcional pueden tener su papel más relevante en mejorar la flexibilidad del sistema.

LA PRESCRIPCION ECONÓMICA
Incremental Cost Effectiveness Ratio

- The Incremental Cost Effectiveness Ratio plays an important role in assisting NICE reach a recommendation

$$ICER = \frac{COST\ new - COST\ old}{QALY\ new - QALY\ old}$$

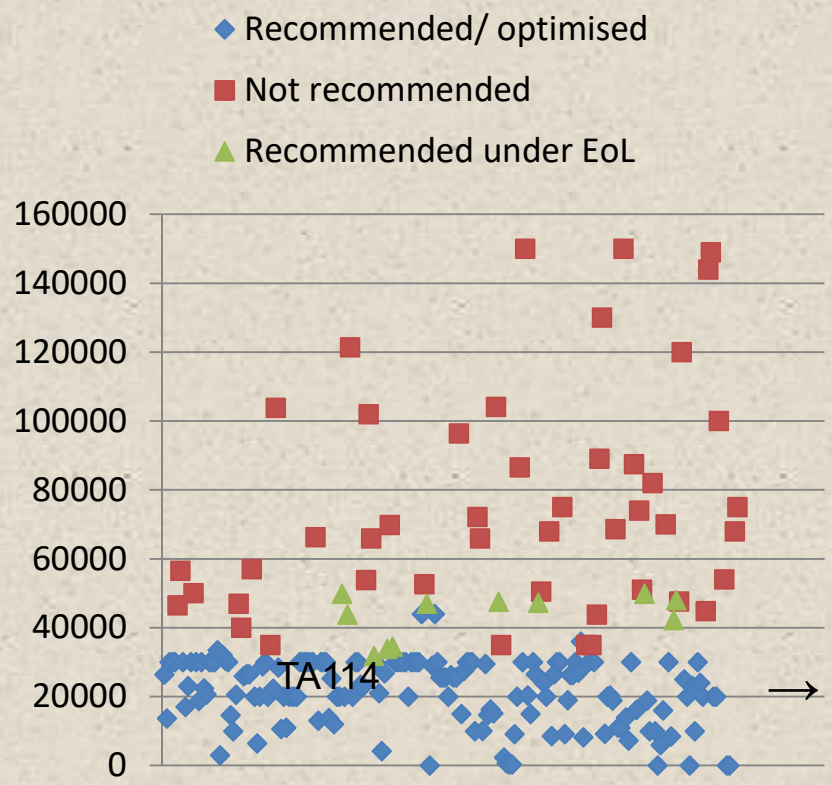
- Produces an estimated cost per quality-adjusted life-year gained

EN ESPAÑA, CUAN LEJOS PODEMOS IR EN LA UTILIZACIÓN DEL COSTE EFECTIVIDAD?

Respuesta

- **TODO LO QUE QUERAMOS PERO DESPACIO. ANTICUERPOS**
- **LAS ALFORJAS NO DAN HOY PARA MÁS. COSTES, OUTCOMES, CULTURA**
- **PERO EL NORTE ESTA BIEN DEFINIDO. PRIORIZACIÓN**
- **EN LOS MÁRGENES DEL CAMINO SE VISLUMBRA EL ABISMO**
- **Y LOS UTILLAJES HAN DE PODER MEJORAR. DE LOS IPTs al ACU**
- **ESPAÑA ES REMISA. UN AUTORIDAD INDEPENDIENTE PODRÍA AYUDAR. EUROPA EN FAVOR DE LA NEXT GENERATION LO DEBIERA DE PODER EXIGIR.**
- **HACER CAMINO MIENTRAS, PERO SIN HIPOTECRA UN FUTURO MEJOR**
- **SIN ENGAÑARSE. NUNCA HAY UN *AHORRO* DE COSTES EN EL SISTEMA PÚBLICO. EL GESTOR LO SABE. LA PREVENCIÓN, TAMPOCO AHORRA. PERO LOS BENEFICIOS A CONSEGUIR VALEN LA PENA. APLICAR A LO NUEVO**
- **PARA TODO ELLO, LA DESCENTRALIZACIÓN NO ES UN PROBLEMA SINO UNA OPORTUNIDAD**

Cut off points en perspectiva



Fuente:. NICE International

Fuente: https://news.cancerresearchuk.org/2021/12/02/how-will-new-cancer-medicines-be-approved-in-the-future/

CENTRE DE RECERCA
EN ECONOMIA I SALUT·CRES
UNIVERSITAT POMPEU FABRA

Gracias por su atención