

## Back to the future: should we live in a post-QALY world?

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Presenters:

- Andrew Walker
- Ad Rietveld
- Oriol Solá Morales

ISPOR Barcelona

12<sup>th</sup> November 2018

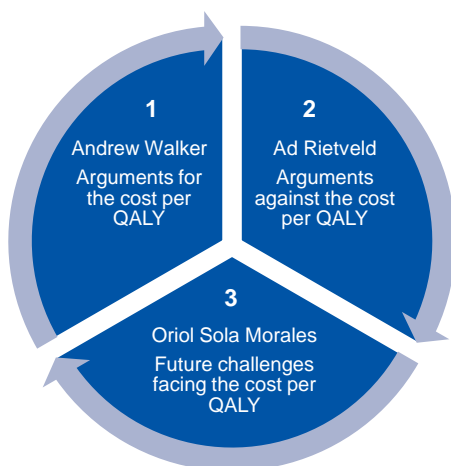
17:00 – 18:00

### Session Structure

3 x ~10-minute presentations



Debate and Q&A

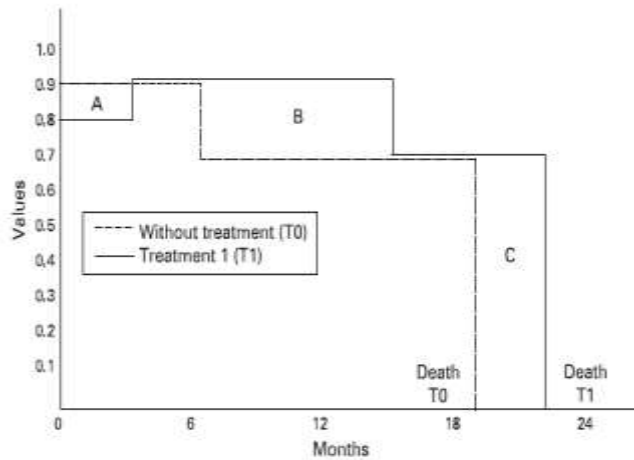


*"I hate QALYs! They are entirely logical, when you build up the argument for them. Until you realise that what you are doing is, putting a number between 0 and 1 (and in some cases less than 0), on a person's life. That is when it all falls apart."*  
**(Former NHS Commissioner)**

*"The cost per QALY approach is not perfect, but it is based upon a robust, transparent and established methodology which is intertwined with the utilitarian nature of the NHS"* **(NHS Policy Lead)**

“No issue in HTA provokes such passion as the QALY”

Andrew@salusalba.com



## In common with other systems

Focus on patient relevant endpoints such as survival and 'quality of life'

Evaluates new medicines using a common framework

A system decision-makers can grow familiar with and becomes a second language for them

Provides a definition of value to guide medicines developers when making decisions on study design and 'go/no go' investments

## Spotlight on value claims

'Value' can be one or more of improved quality life, longer survival or savings to health care budget

Shines light on case for a new treatment:

- Unmet need
- Disease-modifying
- Patient centric
- Innovative
- Granted breakthrough designation by [whoever]

## Opportunity cost

If we say yes to this new treatment what do we gain (measured in QALYs)?

And, by combining with data on net cost, how many QALYs do we have to give up from the money we have to take from other services?

Potential to evaluate all new treatments in the same terms

Hence maximise health gain from the health care budget

## Ask those involved ...

The cost per QALY is not about how much we are willing to spend but how much we are willing to take from someone else.

One man's QALY is another man's opportunity cost, (or even several men's opportunity cost.)

It's impossible to capture the value of human life in a single number like the QALY. But without it, choices are made by history and politics, and rarely with wisdom.



## Debates with politicians

- A way to explain the decision made
- Transparent in the sense the calculations can be put on Powerpoint
- Helps to ensure consistency as decision-makers come and go and decisions are made by different agencies
- Helps with accountability of the agencies to government
- Partial defence when media asks why we don't pay for every new medicine

## Questions about 'special cases'

Includes an ethical assumption health gains are of equal value irrespective of the disease and recipient

What about 'special cases'

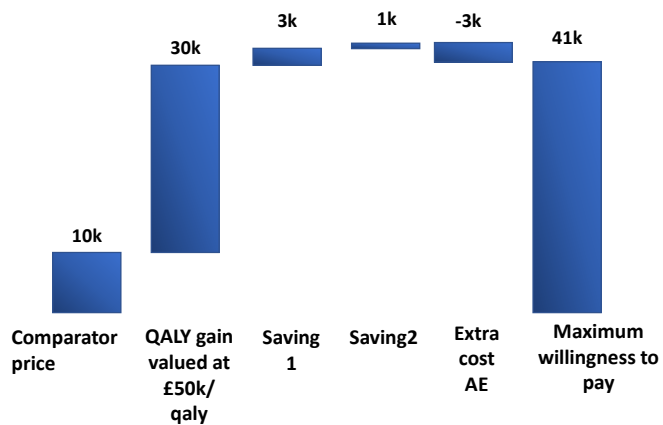
- Cancer
- End of life
- Start of life
- Rare diseases
- Diseases that are a bit rare but not ultra rare

Do we have a higher willingness to pay? Framework to decide

Based on  
society's  
values

- Members of UK public took part
- TTO exercise on EQ-5D states
- Produced value set
- EQ-5D measured in clinical study, then values of UK public applied
- Shows the values used in decisions take (some) account of the opinions of the public

### Economic value of a new oncology medicine



## Practical advantage

- Can take a TPP
- Use predicted endpoint differences to estimate QALY gain and savings
- Convert QALY gain to £-value using guidance on willingness-to-pay
- Estimate a total willingness to pay (QALYs-in-£ plus savings)
- Consider what this needs to cover (monitoring, admin, etc)
- Divide what is left by treatment duration to give a maximum willingness to pay per unit of time

QALYs raise uncomfortable issues

Raises key questions (e.g. about health benefit from achieving surrogate endpoint) and gives framework for discussion

Predicting long-term benefits from RCT with limited follow-up

Or with single-arm clinical study

Value of certain benefits today vs uncertain benefits 20y from now

‘Subjectiveness of valuing health’

Dislike of reducing things to numbers

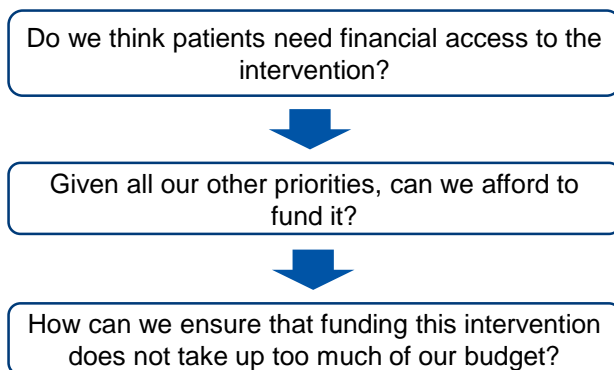
Adding QALYs across individuals

## The QALY and payer decision-making: a good fit?

By Ad Rietveld  
RJW&partners

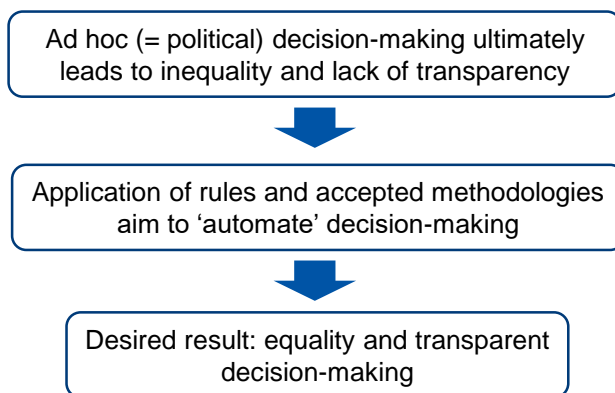
12 November 2018

### Fundamental questions that drive healthcare *funding* decision-making





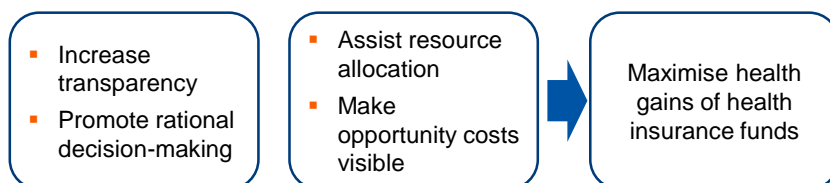
## Regulations, rules and methodologies have been developed to assist in healthcare funding decision-making



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## Expectations at time of introduction of the use of the QALY for drug reimbursement decision-making

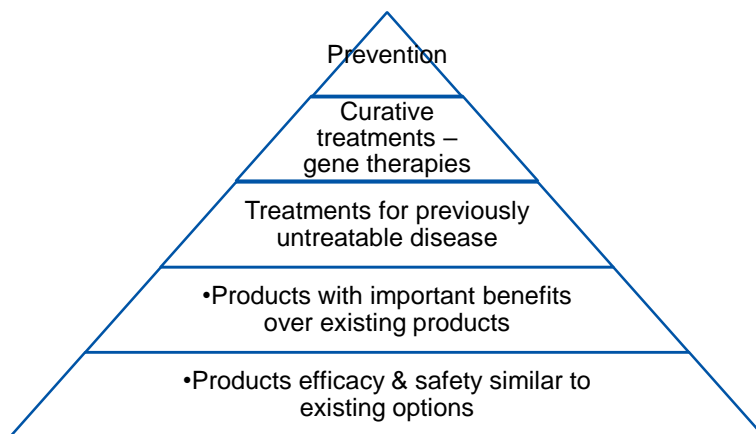


- Provide a framework for further discussions
- Stable & predictable methodology
- Give insights in length & quality of life trade-offs
- Industry perspective – another way for supporting the value of products.

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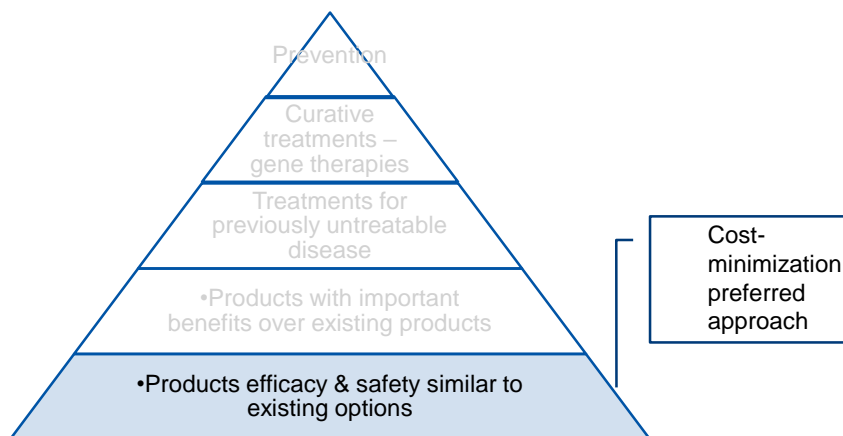
Let's see what the value of the cost/QALY approach is for deciding on the funding of different types of new treatments



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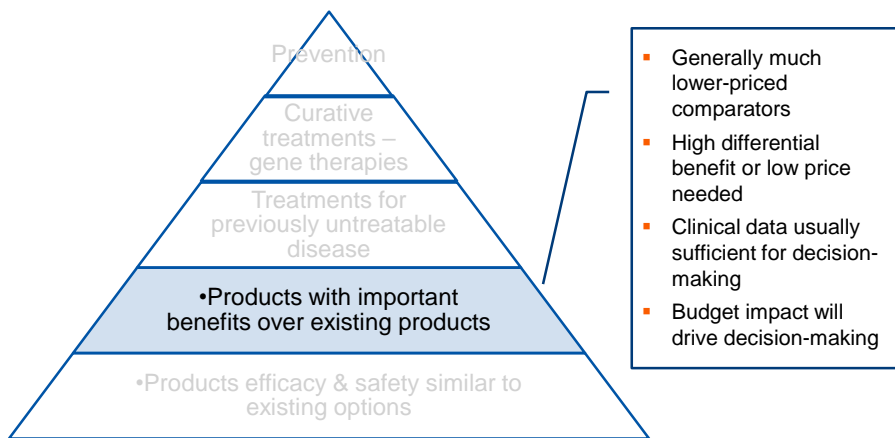
For non-innovative products cost minimization is the preferred approach



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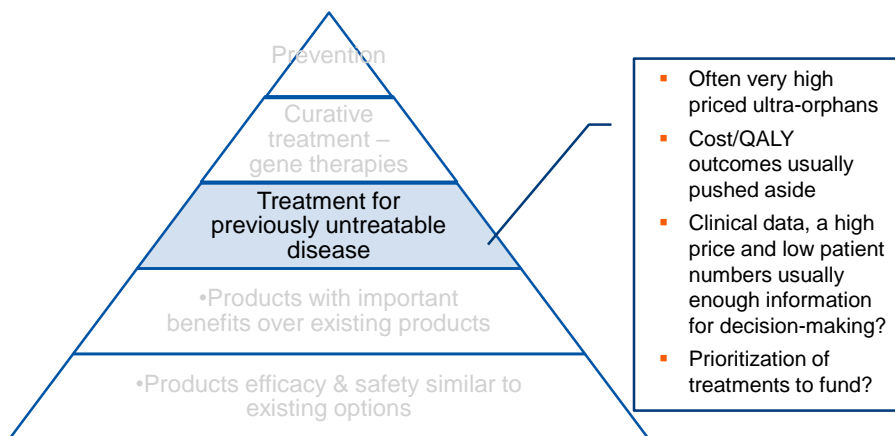
For products with important benefits, achieving an acceptable cost/QALY is usually hampered by a low comparator price



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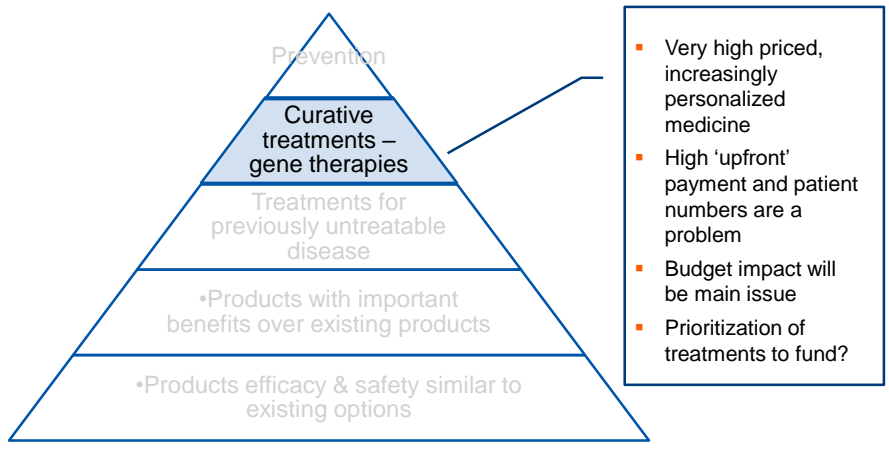
For products for previously untreatable diseases, product prices are such that cost/QALYs are usually disregarded



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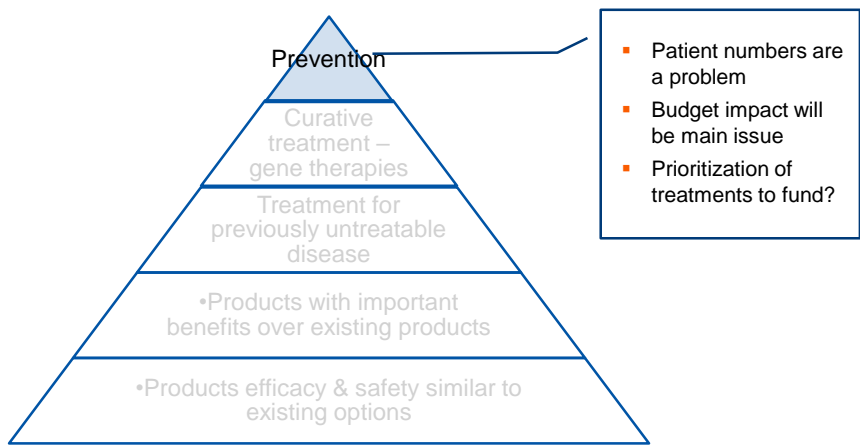
Irrespective of the cost/QALY, budget impact will be the main hurdle for curative treatments



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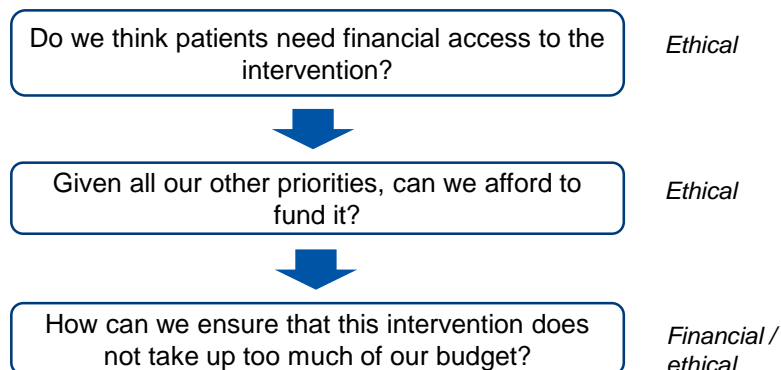
Irrespective of any cost/QALY outcome, budget impact will be the main hurdle for preventative treatments



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Healthcare decision-making is actually not so much about budgets as it is about ethics



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Usefulness of the cost/QALY is hampered by a fundamental contradiction.....

Cost/QALY analysis is a technological approach for assessing the monetary value of an investment in a medical intervention

but

Most of healthcare funding decision-making is not about investments but about ethics



Cost/QALY analysis is applied as a technological solution to an ethical problem



Technology cannot solve ethical dilemmas

**No universally accepted algorithms exist for healthcare prioritization**

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## If it is not cost/QALY, what then?

Focus on robust clinical data support allowing for clinical comparisons and assessment of clinical benefits

Use of conditional reimbursement as a way of dealing with uncertainty on benefits and supporting data at launch

More flexibility in price negotiations and contracting options with less importance attached to list prices

New pricing models urgently required to deal with personalized medicine and gene therapies

Prepare for increasing demand for transparency of setting prices

*Prioritization in decision-making will remain event driven and political*

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Let's stop asking industry to throw in more data and additional analyses – decomplicate!

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## QALY and future demands

Running title  
Client, date



## Personal position on CEA

- It is **far** from being perfect

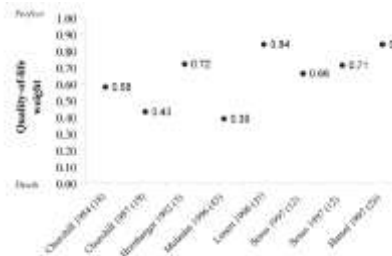


Figure 2 Quality-of-life weights published for haemodialysis at hospital.

- Costs and utilities are prone to many uncertainties / methodological limitations

Arnesen\_JHealthSerResPolicy\_2004

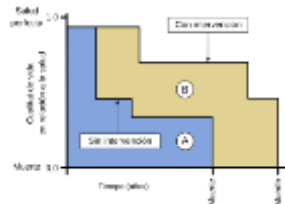


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## Personal position on CEA

- It is **far** from being perfect
  - Do we value equal paediatric and elderly care?
  - Is cancer equal to high blood pressure?
  - **QALYs do not relate to VALUE**

**EDITORIAL**  
**A QALY Is a QALY Is a QALY – Or Is It?**  
Milton C. WEINSTEIN  
School of Public Health, Harvard University, Boston, MA 02115, USA  
Final version received June 1988



Weinstein\_JHEcon\_1988



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## Personal position on CEA

- ..... But you may want to take an extra-welfarist approach
  - *It is not (pareto) optimal, but it is better than not having it*
- CEA is a good tool in combination with a full HTA analysis
  - Provides good framework analysis
  - Enables comparison
  - Facilitates repetition
  - **Obliges to make explicit decision-making, and therefore to justify deviations from the criterion**



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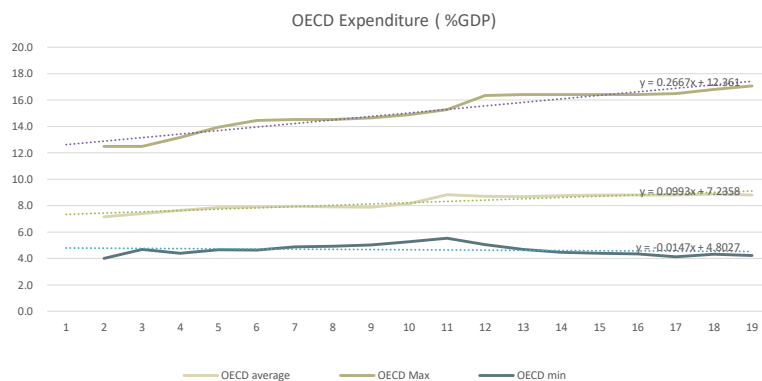
## Potential usages of Economic Evaluation

EE can / should still be used in

- Early Market Access
  - To model the potential outcomes of drug / device development
  - To model sensitivity analysis and complete investment decisions
- In developing economies
  - To ensure most efficient technologies are introduced
  - To avoid non efficient technologies
  - To improve transparency
  - To help build the healthcare system



## New Market Access Requirements



- What to do in an unsustainable situation? What are the new challenges? What to do?



## What can we do?



No one does  
Nothing  
(Business as Usual)

The Alzheimer's  
Paradox



Do Something  
Transformational

Demand Side  
Policies



Be Reactive

Cannibalism



## Cannibalism

What's in a loaf of bread?



Water	.....	10 €
Flour	.....	20 €
Heat	.....	15 €
Salt	.....	5 €
<b>TOTAL</b>	.....	<b>50 €</b>

- What happens when the government decides it has to introduce Flour in bread to prevent caries?



## Cannibalism

What's in a loaf of bread?



Water	.....	9 €
Flour	.....	18 €
Heat	.....	13 €
Salt	.....	4 €
Flour	.....	7 €
<b>TOTAL</b>	.....	<b>50 €</b>

- Everybody loses.
- The (public) insurer will limit its expenditure / ability to pay to a fixed amount (OECD minimum)
- It will maximise competition, driving production costs up.



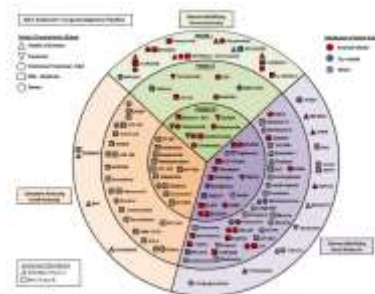
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## The Alzheimer's Paradox

- Under current market perspectives, manufacturers may want to maximise their revenue, and compensate for previous losses.
  - This is leading to 10% GDP average annual increase

- The potential market is huge
- The potential price is huge
- The potential risk for no-approval is huge



Cummings\_AlzDem17



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## The Alzheimer's Paradox

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## Managing Demand: Priority Setting Exercises



Dimension	Weight (%) 2010	Dimension	Weight (%) 2012
Impact on QoL	38	Clinical Impact	66
Risc of no Treatment	30		
Waiting time	8		
Clinical Effectiveness	13	Expected Benefit	12
Resource Use	11		
		Social Role	22

Setting priorities sends a clear message to the EcoSystem and allows alignment of policies to results



## Demand Side Policies



- Pre-commercial Procurement
- Innovative Public Purchasing



## Demand Side Policies

- Purchasers may sign pre-commercial agreements
  - Lowers the cost of (borrowing) money
  - Reduces commercial costs
  - Ensures 'early adopters' / experience (reduces commercial uncertainty)
- There are societal benefits
  - Needs are fulfilled
- And business benefits
  - Companies make the same profit at less cost /risk



## Demand Side Policies

- Under agreed developments ...
  - Incentives are possible
    - OMP legislation
    - Antibiotic development incentives
    - GAVI alliance
  - Externalities appear
    - New markets are developed
    - Triggers science
    - Rewards Universities / RnD
    - Fulfils Lisbon treaty (knowledge based society)

Table 5.5 | Study sample of orphan designations in on-going development (n=982), described by designation holder type and R&D phase

	Small or medium-sized enterprises (SMEs) n(%)	International companies n(%)	Contracting entities n(%)	Large companies n(%)	Very large companies n(%)	Private parties n(%)	Academic Public Sector n(%)
Preclinical	19 (48.0)	22 (18.7)	8 (8.3)	11 (8.8)	6 (4.9)	6 (4.9)	20 (8.1)
Phase I	17 (31.8)	11 (13.8)	18 (24.6)	8 (7.1)	11 (11.8)	1 (1.9)	1 (1.9)
Phase II	11 (19.7)	22 (18.8)	18 (21.4)	14 (8.3)	9 (6.0)	13 (7.1)	2 (8.7)
Phase III	11 (26.2)	7 (7.3)	7 (13.7)	8 (8.3)	9 (9.1)		
Not registered	11 (26.2)	4 (8.7)	9 (14.1)	9 (8.3)	4 (8.3)	8 (8.3)	2 (3.3)
<b>TOTAL n(%)</b>	<b>21 (19.8)</b>	<b>47 (18.1)</b>	<b>49 (11.8)</b>	<b>49 (18.8)</b>	<b>37 (7.4)</b>	<b>22 (14.4)</b>	<b>31 (2.4)</b>

Morel\_Nature2016



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## Demand Side Policies

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## Example: Hypercholesterolemia

- Need: high prevalence, high related mortality
- Gene- autosomal dominant pattern
  - Polygenic, but LDLR, APOB, or PCSK9 have been identified
- Can we develop a gene therapy?
  - Define potential targets
  - Define timings, expectations
  - Ensure access + reimbursement
  - Would outcomes be better than for PCSK9i



## Example: ADA-SCID

- The treatment was developed at **San Raffaele** Telethon Institute for Gene Therapy and developed by GlaxoSmithKline (GSK) through a 2010 **collaboration** with Fondazione Telethon and Ospedale San Raffaele.
- Italian authorities
  - co-develop it
  - accept to pay for it
  - Speed access
  - Price is 'better'
- **MOLMED** is a biotechnology company founded in 1996 in Milan.



## What shall we do?



High Cost  
Limited Value

High ICER



Low Cost  
High Value

Low ICER



High Cost  
Limited Value

High ICER



## Demand Side Policies



High Cost  
Limited Value

High ICER



Low Cost  
High Value

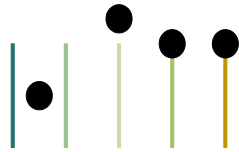
Low ICER



High ICER







Health Innovation  
Technology Transfer

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## Discussion and Q&A



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