

**PIP DATA FOR
MARKET ACCESS**

By

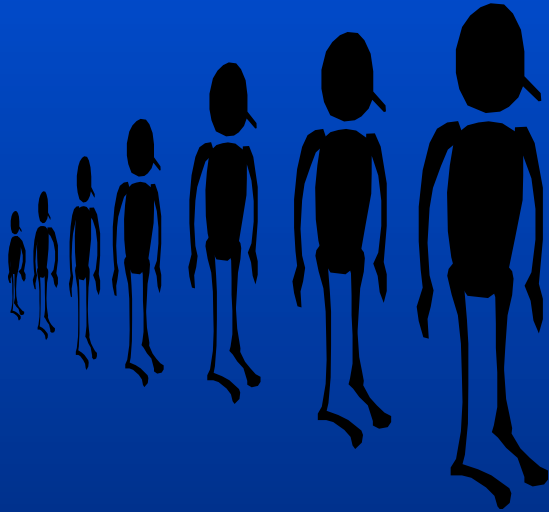
Mark Nuijten, PhD, MD, MBA

Maart 13, 2012

London, UK

**GENERAL TRENDS
IN HEALTH CARE SYSTEMS
IN EUROPE**

Health Care Systems

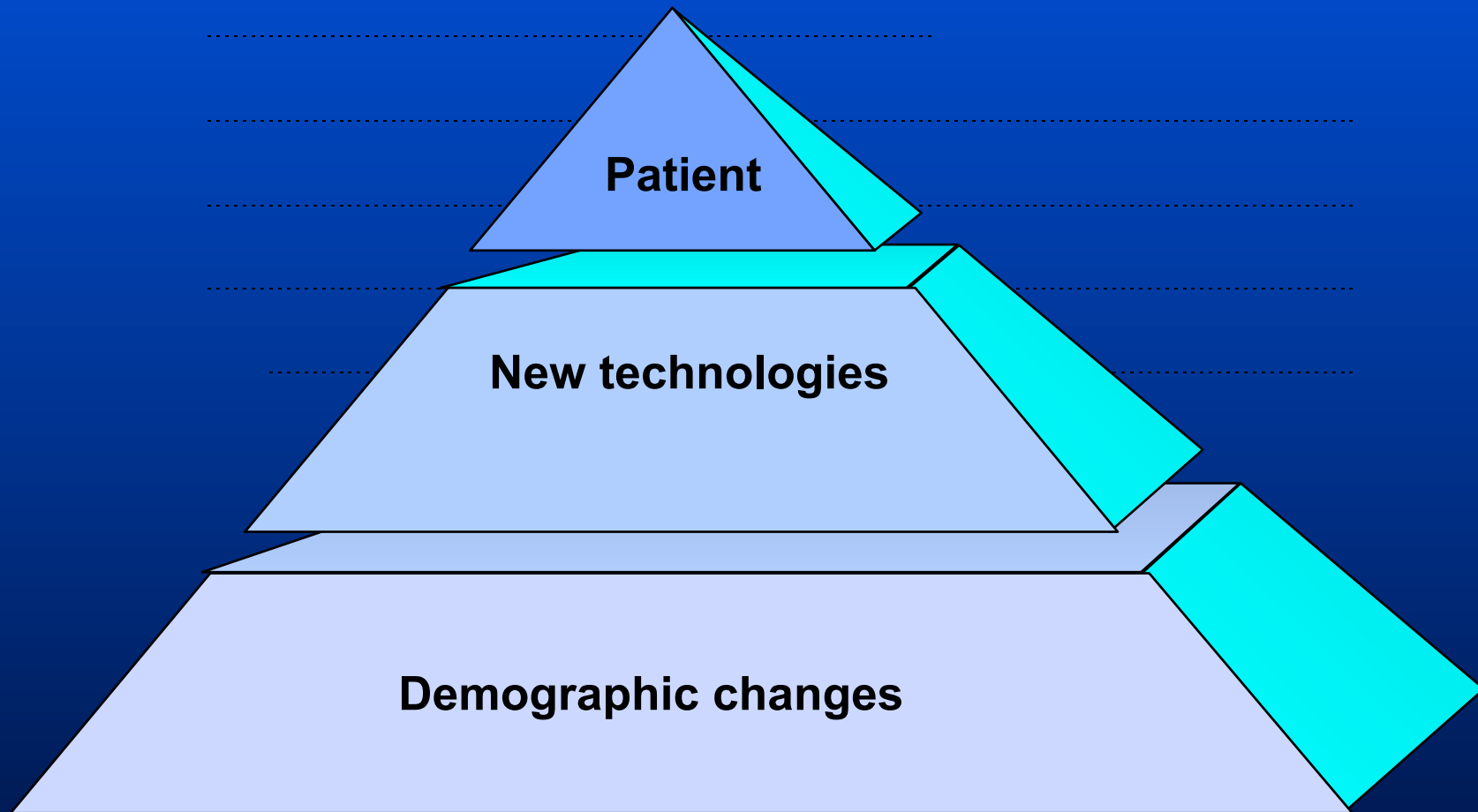


**Increasing demand for
health care**

Budget Constraints



Health Care Systems



Trends in Health Care Systems

Free market development

- **Central processes, but a shift to decentralisation:**
 - hospitals
 - regional authorities
- **Creating market mechanisms in order to increase efficiency:**
 - purchasers versus providers: UK, Italy
 - Hospital budgets from “per diem” to cost per case”

PRICING AND REIMBURSEMENT OF DRUGS

Trends for Pharmaceuticals

Safety, Efficacy & Quality



Clinical & Cost effectiveness



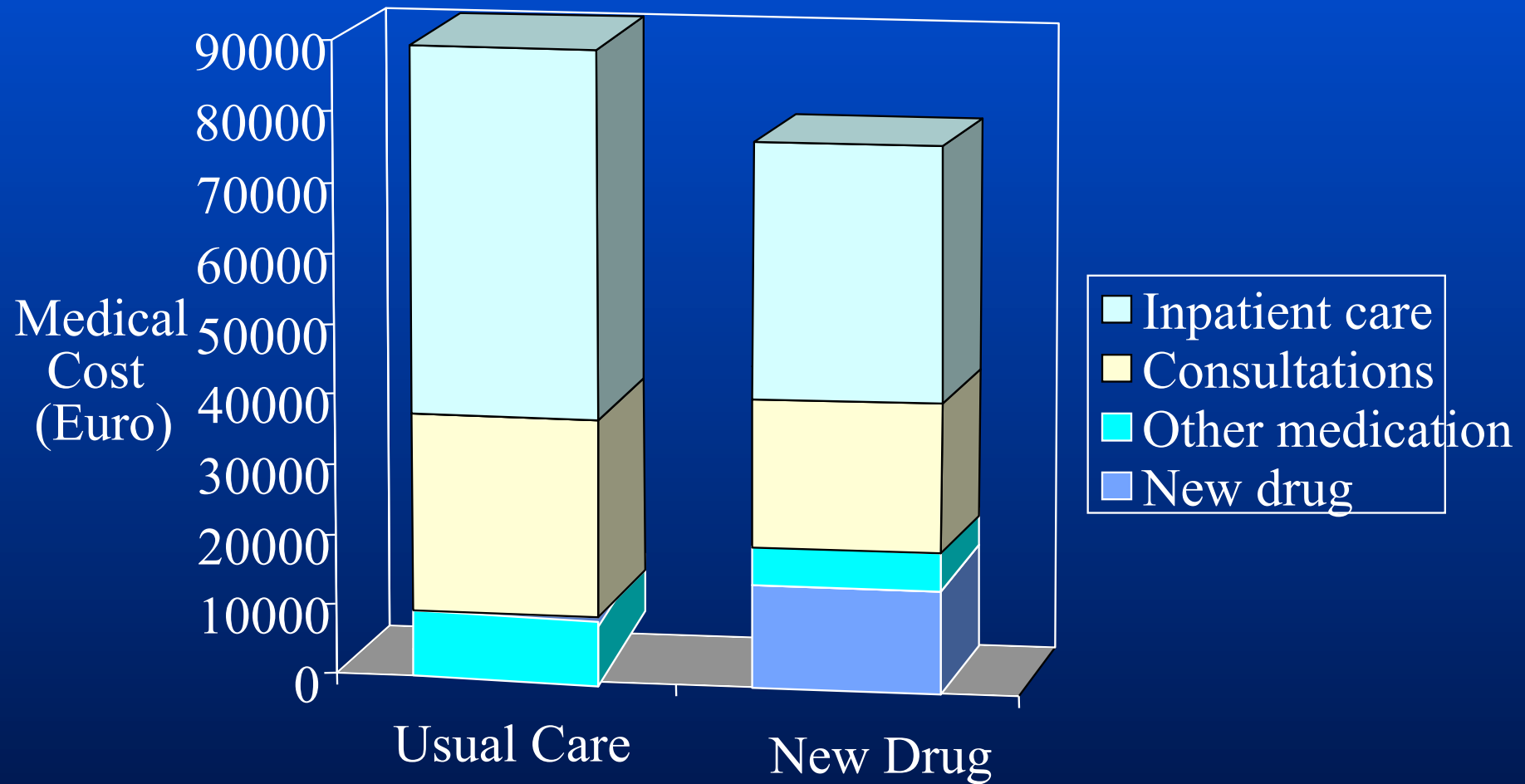
'4th Hurdle'

Affordability and impact on services



'5th Hurdle'

Trends for Pharmaceuticals



DATA REQUIREMENTS

Data Requirements

Decision Criteria

- **Clinical decision criteria are:**
 - Efficacy
 - Side effects
 - Route of administration/ease of use
 - Contra-indications/warnings
 - Mechanism of action/drug interactions
- **Non-clinical decision criteria are:**
 - Drug price and impact on drug budget
 - Other (less impact): cost-effectiveness, QoL
 - **Clinical Effectiveness – most important: NICE**

Health Economic Data

Cost-effectiveness ratios

- No explicit threshold in any country
- Proposed levels (per QALY) - \$20,000 Canadian
 - \$50,000 US
 - £10,000 UK
 - E 20,000
- Observed values - £20,000 - £30,000 UK
 - \$22,000 Australian

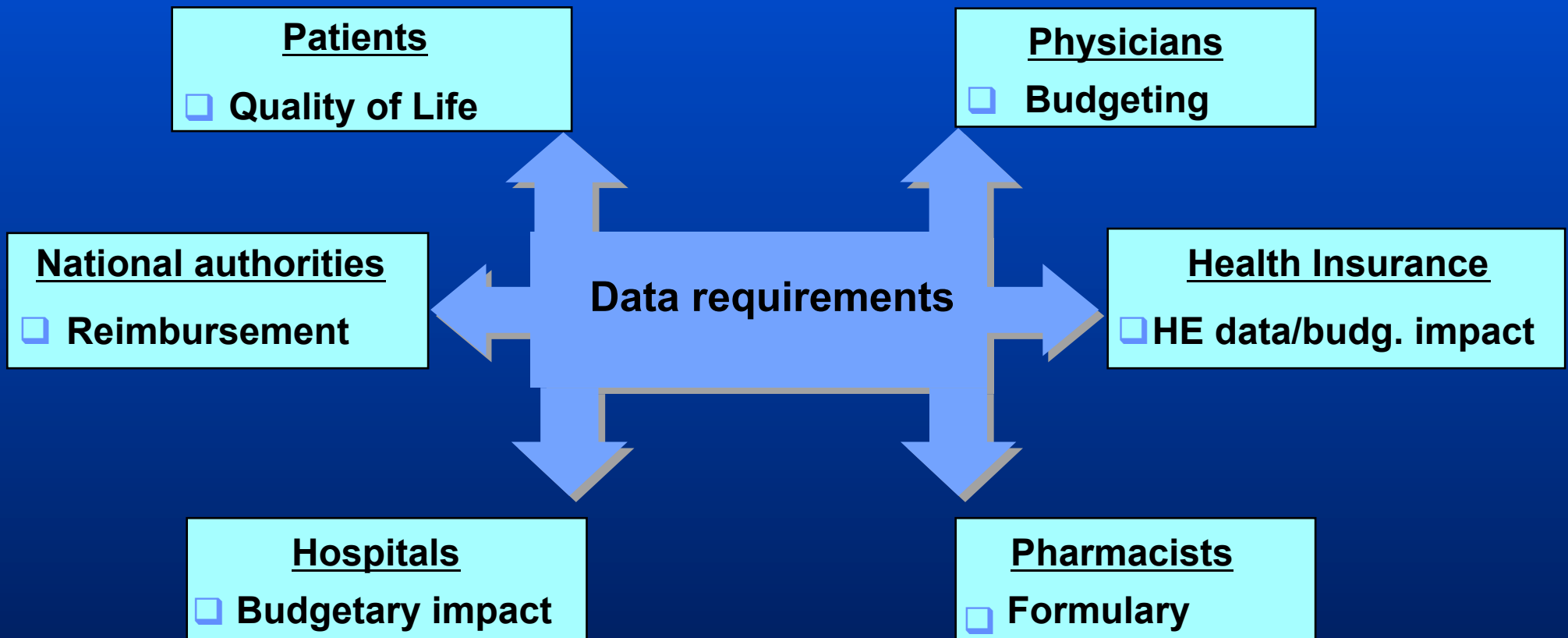
Health Economic Data

Stringency of health economic requirements

	Health Economics
GERMANY	XX
FRANCE	XX
UK	XXX
ITALY	XX
SWEDEN	XXX
NETHERLANDS	XXX
SPAIN	XX
PORTUGAL	XXX
FINLAND	XXX
DENMARK	XX
HUNGARY	XX
POLAND	X

Low level of requirements	X
Medium level of requirements	XX
High level of requirements	XXX

Decentralisation-Target Audiences



Decentralisation

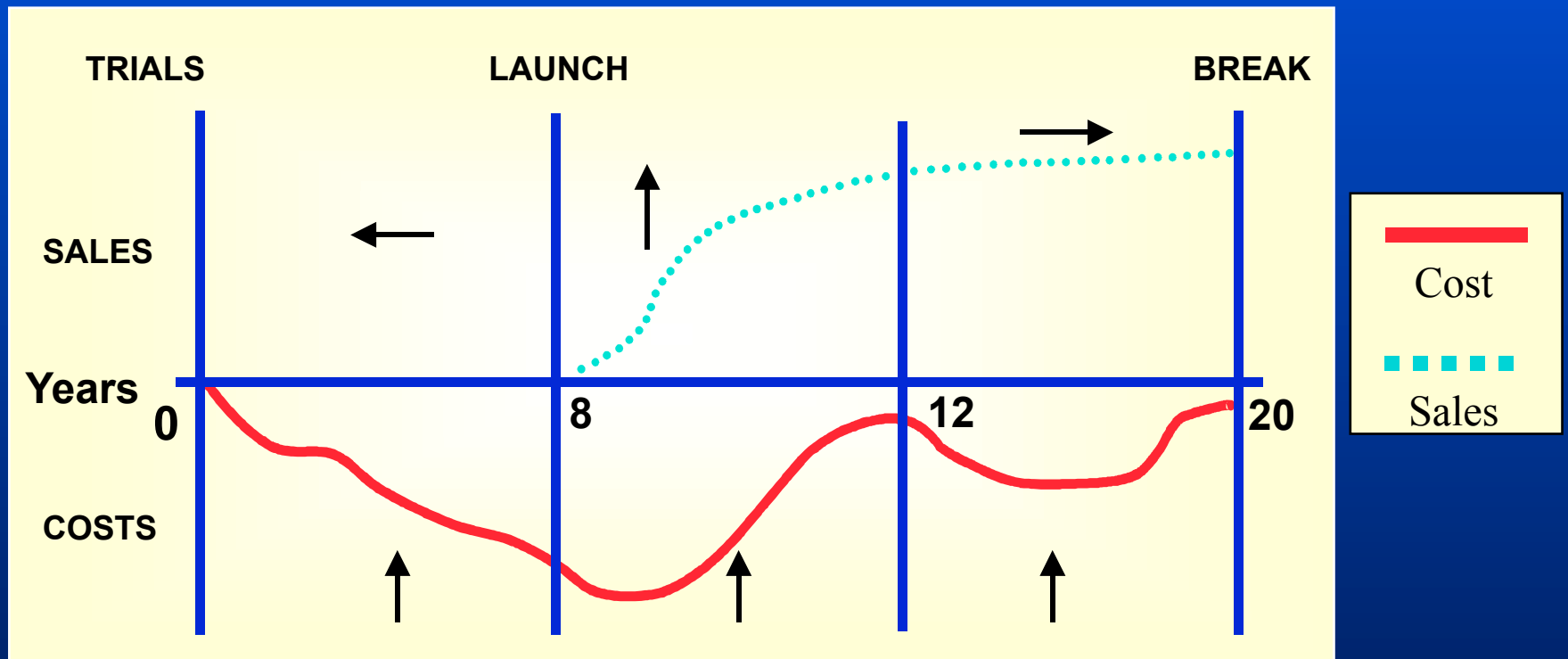
	patient	physician	pharmacist	management	insurer
Efficacy	2	3	3	2	2
Safety	2	3	3	2	2
Administration	2	2	3	2	2
Effectiveness	3	2	1	1	2
Quality of Life	3	2	1	1	2
Cost-effectiveness	1	1	1	1	2
Budgetary impact: drug costs	1	1-3	3	3	3
Budgetary impact: medical costs	1	1	2	3	3
Quality of care	3	2	2	2	3
Co-payment	3	1	1	1	2
Discounting	0	1	3	3	3
Price	1	1	3	3	3
Indirect costs	3	1	1	1	2

Decentralisation - insurers

Decision criteria

- **Perspective: short-term (1 to 3 year): most drugs in chronic disease will only show cost-effectiveness after 5 years.**
- **Cost-benefit > cost-effectiveness > cost-utility**
- **Cost per month without symptoms and toxicity instead of cost/QALY:**
 - **insurers not familiar with QALY and cost/QALY concept**
 - **terminology: cost-effective = cost saving**
 - **cost-effectiveness: conceptual closer to medical community**
- **BUT: opportunities for Quality of Life:**
 - **closer to patient**
 - **competing claim towards other insurers**

Strategy



... to accelerate business performance across the entire product life cycle, while reducing fixed costs.

PIP DATA

Model

$T = t$

normal

ESRD

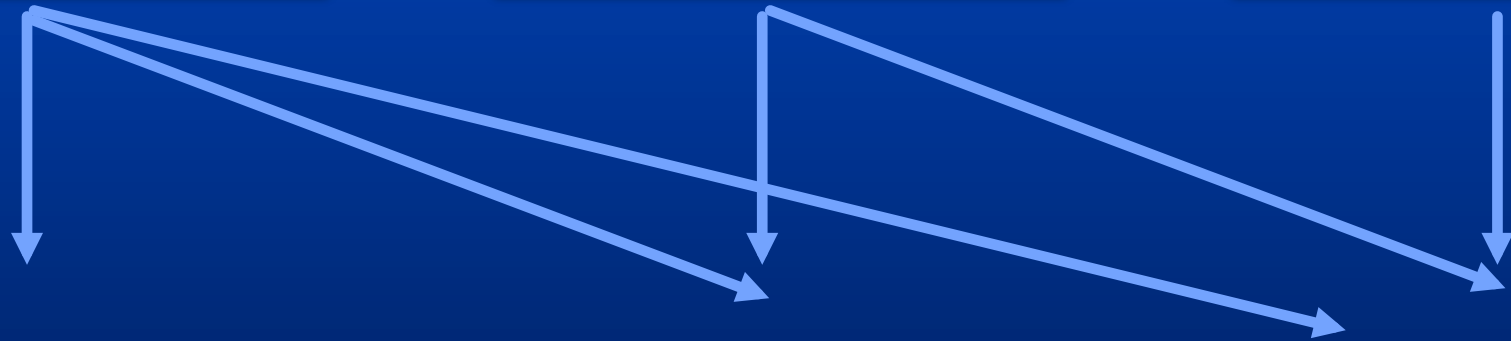
death

normal

ESRD

death

$T = t + 1$



Data Sources

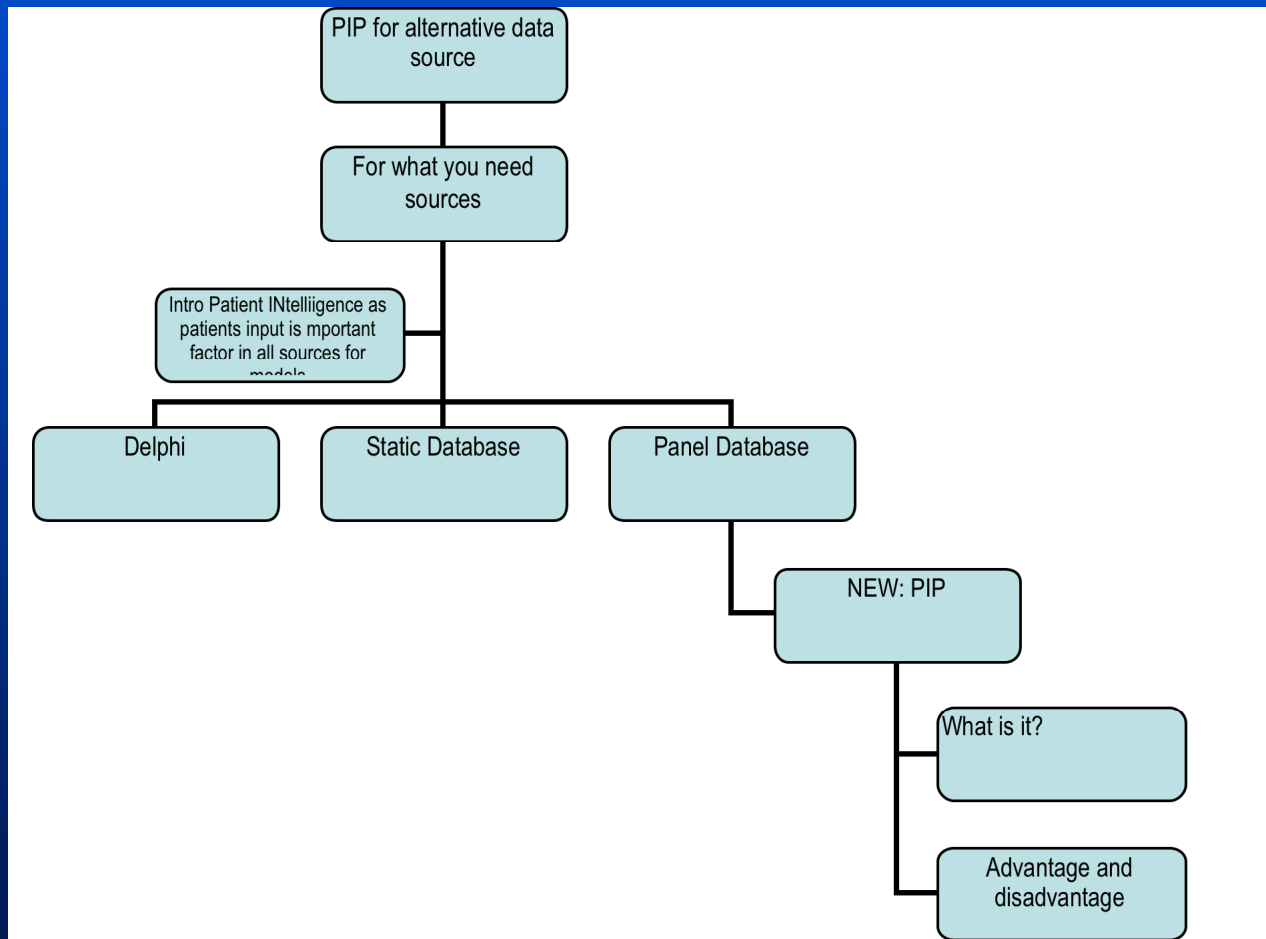
Types of data:

- Probabilities
- Treatment decisions
- Health care resource utilisation (e.g. consultations)

Data sources:

- RCT (Randomized Controlled Trial): cause-effect
- Observational studies, registers
- Cross-sectional studies, inc. QoL studies
- Claims databases
- Retrospective patient chart analyses
- Delphi panels

PIP



Data Sources

PIP: Nationline panel database (Internet access panel)

- Pre-screened respondents who have expressed a willingness to participate in surveys and/or customer feedback sessions.
- Respondents become "panelists" by completing a profiling questionnaire.
- The data collected in the profiling includes demographics and also health status characteristics.
- A patient specific online panel gives researchers access to patients worldwide is the Patient Intelligence Panel (PIP).
- Having globally on-line access to thousands of people who are willing to participate in research on healthcare and specific indications, all questions can be asked and a wide range of feedback can be obtained.

Data Sources

PIP: Nationline panel database (Internet access panel)

- The PIP dataset is not limited by power constraints as static databases, and especially clinical databases, which usually have a limited number of patients.
- The sample size of PIP data set can be adjusted based on a priori sample size calculations in order to show statistical significant results.
- PIP data set is the flexible sample size allows the inclusion of a representative patient population.

Data Sources

PIP: Nationline panel database (Internet access panel)

- **Patient data: medication, co-morbidity, socio-demographic**
- **Clinical data: response, side effects, treatment failure**
- **Quality of Life and PRO (e-surveys)**
- **Economic data: resource utilisation, productivity loss**

THUS

- **all data can be defined on beforehand – complete full data set for model**
- **high external validity – fully representative of daily care – and therefore relevant for decision makers.**

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- Measure the impact of a particular disease or condition on clinical and patient-specific outcomes.
- Document the outcomes associated with different treatments or settings of care in a quantitative matter.
- Patients can be followed prospectively and data are collected on disease severity and clinical outcomes, as well as resource use, functional status and quality of life as reported by the patient.
- PIP data reflect the current treatment patterns without influencing the treatments or interventions and consequently the PIP study is fully naturalistic without any intervention with real practice (e.g. no randomisation) and has a high external validity.

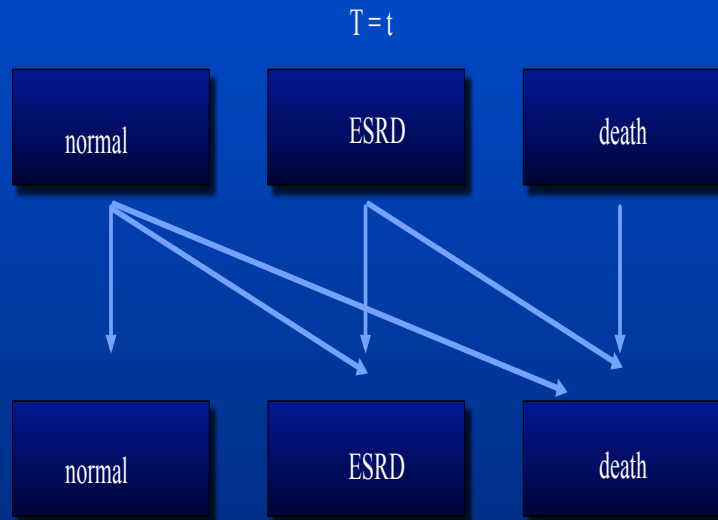
CONCLUSION

Conclusion

PIP: the Patient's Voice

- Integrating the patients' voice in the models, a more holistic outcome will be the result corresponding with the concept of cost-effectiveness requiring a high external validity and outcomes representing real life.
- The patients' voice can be considered the optimal data source for a health economic model as it has the highest representativeness of the effectiveness of a treatment in real-life.
- Specifically for perception sensitive factors in health economic models, like quality of life (QALYs), adherence, side-effect severity and discontinuation rational, the patients' voice should be integrated as the patient is sole source for outcomes related to the patients' experience with pharmaceutical therapy.

Conclusion



PIP DATA



**PIP DATA
BRIDGING PATIENT'S VOICE
WITH POLICY MAKING**

Conclusion



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