Performance-Based Risk-Sharing Arrangements for Drugs and Other Medical Products: An Overview

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Performance-Based Risk-Sharing Arrangements: A Variety of Names

- outcomes-based schemes
- risk-sharing agreements
- coverage with evidence development
- access with evidence development
- patient access schemes
- conditional licensing
- managed entry schemes
- pay-for-performance programs
- And others?

The New York Times

Business

Pricing Pills by the Results

Illustration by The New York Times

By ANDREW POLLACK
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Drug companies like to say that their most expensive products are fully worth their breathtaking prices. Now one company is putting its money where its mouth is — by offering a money-back guarantee.

Johnson & Johnson has proposed that Britain's national health

Why the "sudden" interest? Fad or trend?

- Understanding the cause should be helpful to predict long-term adoption and impact.
- Two general explanations:
  1) Innovation—it's a new invention.
  2) Environment has changed.
- Some "trends" may be fads:
  - Example: disease management (carve-outs)
- But we are seeing more formalized processes
  - Key principles for Patient Access Schemes in the UK

Cost Pressures

- Increasing drug expenditures attributed to:
  - Use of high cost biopharmaceuticals for common, chronic conditions (RA, Asthma, Psoriasis, etc)
  - Expensive, combination biopharmaceutical treatments in oncology and infectious disease
  - Prescribing beyond evidence and approved indications
  - Other factors (aging population, fewer resources, etc)

Real cost of new branded drugs is rising—fewer approvals and higher R&D spending.

Why the interest in performance-based risk-sharing?

- **Health care production is complex:**

  Economists think of it as a "Health Production Function":
  - Output = f(Inputs)
  - Health = H(hospital stays, doctors visits, drugs, OTHER)

- The physician acts as the "patient's agent" in organizing and advising on this process.
- Historically, either (1) all of these inputs were provided by a system of care or (2) each input is purchased on a fee-for-service basis—or some mix of these.

But the process is so complex that no one has offered guarantees of good outcomes.

Well, that's not entirely true:

Greater interest in "pay for performance"

1. In the U.S, for health plan processes and for physician services.

2. Internationally, for branded drugs.

Why the new interest? What has changed?

<u>Working hypothesis:</u> Performance-based agreements are a market response to increasing cost pressures: manufacturers have incentive (esp. in US) to push prices to limit of willingness to pay, and payers are pushing back.

Definitions

- **Performance-based agreement:**
  - An agreement between a payer and a pharmaceutical, device, or diagnostic manufacturer where the ***coverage, price, and/or revenue*** received is related to the ***future performance*** of the product in either a ***research or real-world environment***.
- **Risk-sharing agreement** (de Pouvourville, 2006):
  - "a contract between two parties who agree to engage in a transaction in which there are ***uncertainties*** concerning its final value. Nevertheless, one party has ***sufficient confidence*** in its claims of either effectiveness or efficiency that it is ready to ***accept a reward or a penalty*** depending on the ***observed performance***." *(emphasis added).*

We use these terms interchangeably.

Key Elements of Performance-Based Risk-Sharing Arrangements

1. There is an agreement about a program of data collection to reduce uncertainty about the expected cost-effectiveness of the drug (or device or diagnostic).
2. The coverage, price, and/or revenue is linked to the outcome of this program of data collection. This may be prospective or retrospective.
3. It can be about health outcomes and cost-effectiveness or about budgets.
4. These arrangements provide a ***different distribution of risk*** as between the payer and the manufacturer than "conventional" arrangements*.

** de Pouvourville EJHE, 2006*

Two Analytic Approaches

- **Economic Theory (deductive)**
  - What does theory say about the historical risk-sharing between manufacturers and payers? Why might this have changed?
- **Empirical Practice (inductive)**
  - What examples have arisen in the real world? What do their characteristics tell us about potential factors?

Some Theory

LEADING ARTICLE

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Can't Get No Satisfaction? Will Pay for Performance Help?

Toward an Economic Framework for Understanding Performance-Based Risk-Sharing Agreements for Innovative Medical Products

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Some Important Economic Terms and Concepts

- **"Market failure"**—when "free markets" do not provide an "optimal" allocation of resources—often when the conditions for a free market are not met.
  - For example, public goods, externalities, informational asymmetry, uncertainty, etc. —patent protection to incentivize investment and risk-taking

- **"Public good"**—a good for which one person's usage or consumption does not keep other from using it, e.g., national defense.
  - The free market can be expected to undersupply public goods.
  - We address this with interventions, such as **"intellectual property"** and **public subsidies**.
  - There can be a **"free rider"** problem.

- Information and scientific advances can be public goods—even **"global public goods"**
  - The whole world has a stake in innovative pharmaceutical R&D.
  - Economists agree that **"differential pricing"** would be an improvement: the challenge is how to implement it.

Basics: The Pervasiveness of Uncertainty

- Drugs are approved, launched, and reimbursed under conditions of uncertainty, affecting many key parameters:
  - Efficacy (heterogeneity)
  - Effectiveness in real world
  - Risks (safety)
  - Models, including links between surrogate markers and long-term outcomes
  - Cost-effectiveness
  - Budget impact.

1. **Variability→Uncertainty (=Risk)**
2. **Gathering more evidence to reduce uncertainty is costly.**

The Historical Risk-Sharing "Equilibrium"

- **Risk to manufacturer:** we operate with a blockbuster financing model for R&D.
  - Intellectual property—patent protection to incentivize investment and risk-taking
  - There is no *ex ante* clause to share innovation cost or to purchase drugs.

- **Risk to payer:** The payer negotiates a price and/or use.
  - The payer bears the risks of making a bad buy (i.e., when incremental health benefits are not worth the additional cost).
  - The payer is free to collect post-launch data. Manufacturers will only do this if it is in their competitive interests.

- Individual countries strike different types of deals with manufacturers
  - Range of country environments: negotiated prices < -- > free pricing
  - All of this provides an incentive for manufacturers to seek highest justifiable price at launch. Manufacturers would like to price for future (larger) indications.

An Economic Framework: The Marginal Condition

- Equilibrium condition:

Demand Price = expected net monetary benefit (ENMB)

= ($\lambda \cdot$ QALY gain) + nondrug cost offsets

where λ is willingness to pay for QALY gains.

However, if the buyer is risk-averse, and the demand price should be lower the greater the uncertainty.

Four Relevant Theoretical Approaches

1. **Value of information (Eckermann and Willan, 2008, 2009)**
   - **AN**: to **A**dopt with **N**o additional evidence collection
   - **DT**: to **D**ecline to adopt and seek further evidence for example from a **T**rial
   - **AT**: to **A**dopt but seek /require further evidence (for example via a **T**rial), e.g., coverage with evidence collection.
     - **New option: AT + RS**

2. **Real option theory**
   - Uncertainty and irreversibility make real options theory relevant.
   - We can view DT (delay and trial) as a *call option for the buyer, i.e., the payer is given* the right to buy at a point in the future at the current price (i.e., to take up the option of AN).

Four Relevant Theoretical Approaches (cont'd)

3. **Money-back guarantees and warranties**
   - Can get full rebate for non-performance: The consumer (payer) can "sell" the product back to the seller/manufacturer at the price paid.

4. **Portfolio theory (O'Brien and Sculpher, 2000)**
   - A risk-averse payer should only be concerned about risk they cannot diversify away by "portfolio diversification": that is, covariance rather than variance matters.
   - But covariance is an issue in medical care.

What insights do they provide?

- Importance of “efficient risk-bearing.”
- Importance of the value of information—weighing benefits and cost of delay
- Different risk preferences of public vs. private payers may matter.
- Diversifiability of risk matters.
- Importance of the public good nature of information.

A Taxonomy

Contents lists available at ScienceDirect

Health Policy

journal homepage: www.elsevier.com/locate/healthpol

Review

Linking payment to health outcomes: A taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers

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COMMENTARY

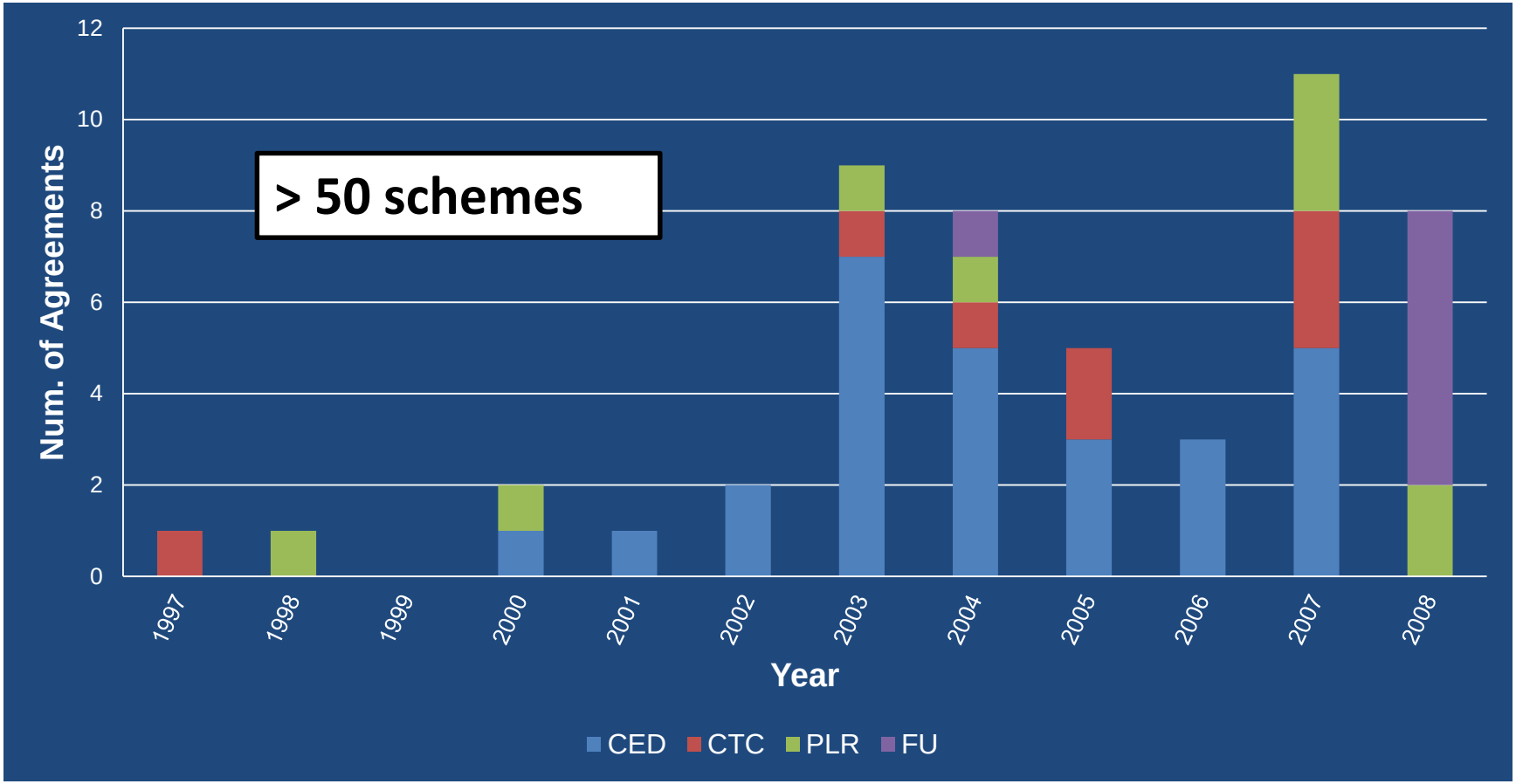
Paying for Outcomes: Innovative Coverage and Reimbursement Schemes for Pharmaceuticals

Josh J. Carlson, PhD; Louis P. Garrison, Jr., PhD; and Sean D. Sullivan, PhD

Review of Performance-Based Arrangements: Methods

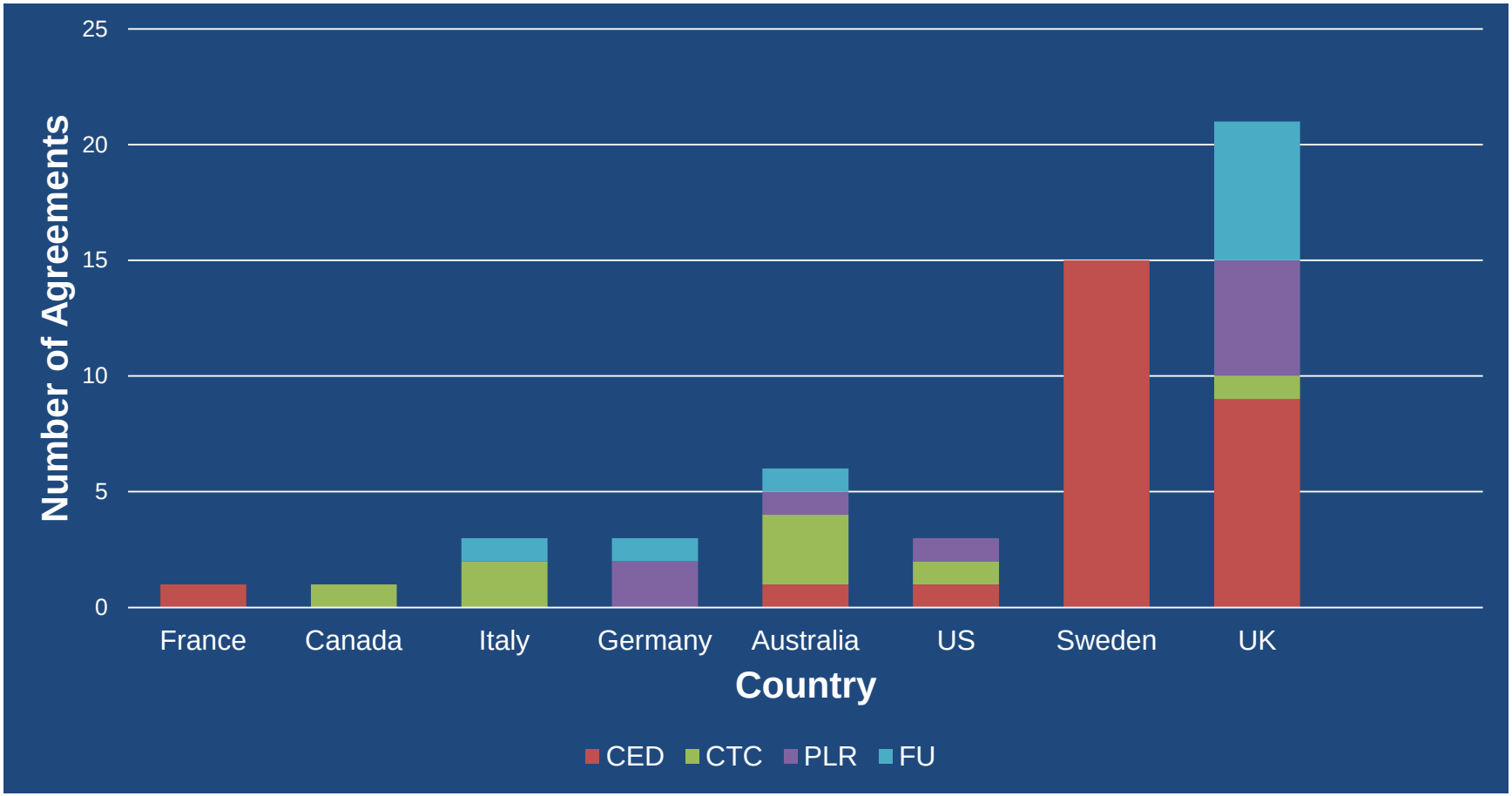
- Sources:
  - PubMed, Scrip, Embase, and Google
  - Experts and peers
- 20 year time frame
- Included: Health outcomes based agreements: price, level, or nature of reimbursement are tied to measures ultimately related to patient quality or quantity of life.
- Excluded: non-outcomes based models including price volume agreements, market share agreements, utilization caps
- Develop a taxonomy of agreements:
  - Inductive approach
  - Refined using a modified Delphi approach with experts in the area.

Performance-based schemes by year



CED: Coverage with evidence development; CTC: Conditional treatment continuation; PLR: Performance linked reimbursement; FU: Financial or utilization based agreements

Performance-based schemes by country



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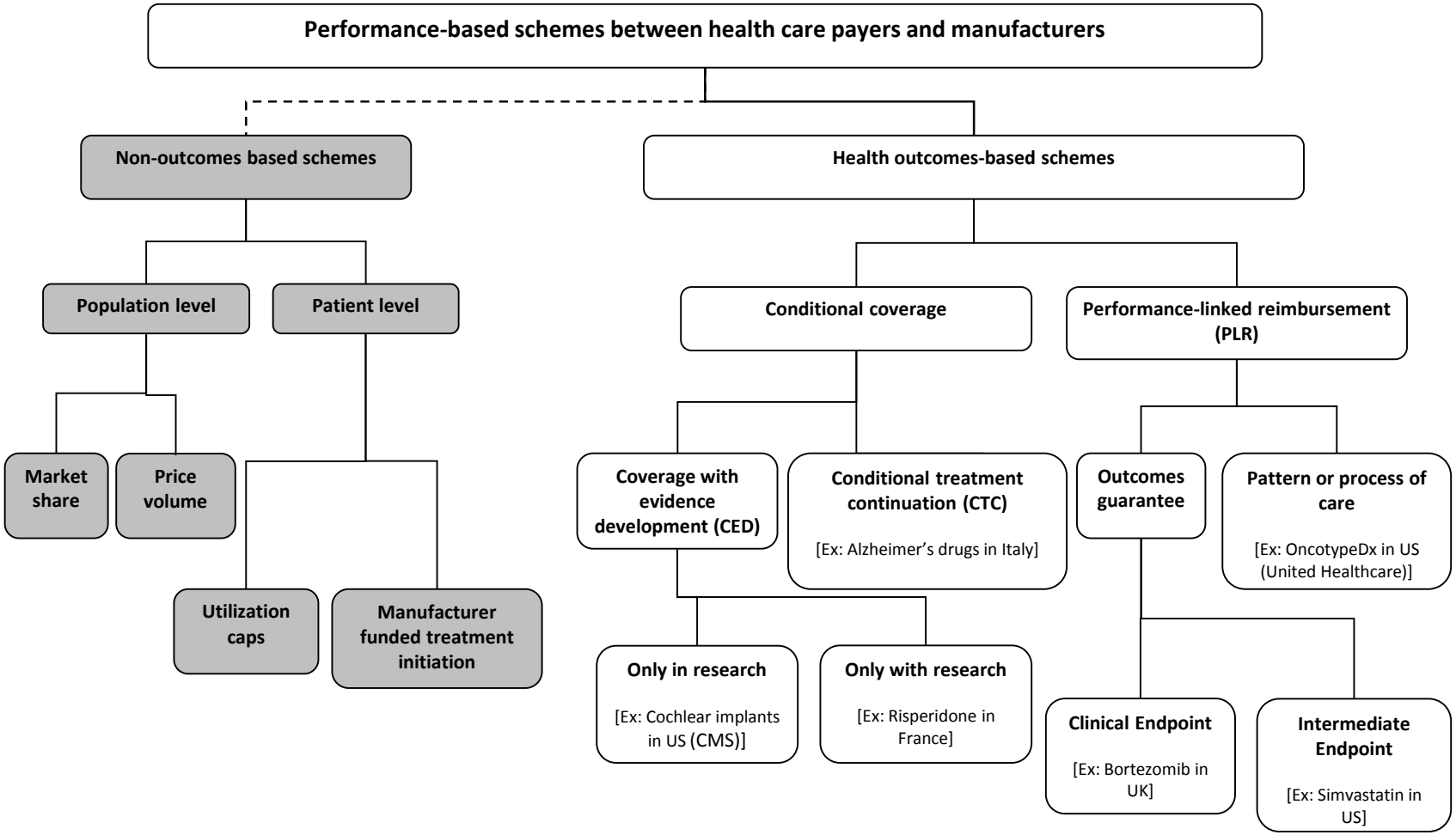
Performance-based schemes between health care payers and manufacturers
Non-outcomes based schemes
Health outcomes-based schemes
Population level
Patient level
Conditional coverage
Performance-linked reimbursement (PLR)
Market share
Price volume
Utilization caps
Manufacturer funded treatment initiation
Coverage with evidence development (CED)
Conditional treatment continuation (CTC)
[Ex: Alzheimer's drugs in Italy]
Outcomes guarantee
Pattern or process of care
[Ex: OncotypeDx in US (United Healthcare)]
Only in research
[Ex: Cochlear implants in US (CMS)]
Only with research
[Ex: Risperidone in France]
Clinical Endpoint
[Ex: Bortezomib in UK]
Intermediate Endpoint
[Ex: Simvastatin in US]

Taxonomy Definitions

- Performance-based health outcomes schemes: price, level, or nature of reimbursement are tied to future performance measures of clinical or intermediate endpoints that are ultimately related to patient quality or quantity of life

Taxonomy Definitions (cont.)

- Conditional coverage: coverage is granted conditional on the initiation of a program of data collection
  - Coverage with evidence development: coverage is conditioned on collection of additional population level evidence, from pre specified study, to support continued, expanded, or withdrawal of coverage
    - Only in research: coverage conditional on individual participation in research (i.e. only patients participating in the scientific study are covered)
    - Only with research: coverage conditional on agreement to conduct a study that informs the use of the medical product in the payer patient population

Taxonomy Definitions (cont.)

- Performance-linked reimbursement: reimbursement level for covered products is tied, by formula, to the measure of clinical outcomes in the "real world";
  - Outcomes guarantees: manufacturer provides rebates, refunds, or price adjustments if their product fails to meet the agreed upon outcome targets
    - Example: J & J agreed to reimburse the NHS in either cash or product for patients who do not respond (Response measure: 50% decrease in serum M protein) after 4 cycles of treatment with Velcade. Responding patients receive additional 4 cycles.
  - Pattern or process of care: reimbursement level is tied to the impact on clinical decision making or practice patterns
    - Example: UnitedHealthcare agreed to reimburse OncotypeDx test for 18 months while it and Genomic Health monitor the results.
    - If the number of women receiving chemotherapy exceeds an agreed upon threshold, even if the test suggests they do not need it, the insurer will negotiate a lower price.

*Of note, there were hybrid examples that had two or more of the above components.

Performance-based schemes in the U.S.

Year	Disease area	Product(s)	Manufacturer	Payer	Agreement
1998	High cholesterol	Simvastatin	Merck	Patients and Insurers	Merck promised to refund patients and insurers up to six months of their prescription costs if simvastatin plus diet did not help them lower LDL cholesterol to target concentrations identified by their doctors.
2007	Breast Cancer	OncotypeDx	Genomic Health	United Healthcare	United Healthcare agreed to reimburse the OncotypeDx test for 18 months while it and Genomic Health monitor the results. If the number of women receiving chemotherapy exceeds an agreed upon threshold, even if the test suggests they do not need it, the insurer will negotiate a lower price.
2009	Type 2 diabetes	Januvia, Janumet	Merck	Cigna	Merck has agreed to peg what the insurer Cigna pays for the diabetes drugs Januvia and Janumet to how well Type 2 diabetes patients are able to control their blood sugar.
2009	Osteo-porosis	Actonel	Proctor & Gamble, Sanofi-Aventis	Health Alliance	Two companies that jointly sell the osteoporosis drug Actonel agreed to reimburse the insurer Health Alliance for the costs of treating fractures suffered by patients taking that medicine.

Barriers to Risk-Sharing Schemes

(Carlson, Garrison, Sullivan; JMCP, 2010)

1. Associated transaction and administration costs;
2. Limitations of current information systems in terms of tracking performance;
3. Agreeing on the scheme details (e.g., the appropriate outcome measure or the financial adjudication process);
4. Physician push-back;
5. "Free-rider" problem—other manufacturer or payer competitors may benefit from the information or schemes developed; and
6. Lack of trust between payers and developers.

Conclusions

- Performance-based agreements are in line with healthcare trends
- They are intrinsically appealing
  - Align incentives toward realized value
- Substantial barriers to implementation that will limit both the short-term and long-term impact
  - Especially in the US.
- They will not apply to all medical products, but rather to a select group where the payer and manufacturer can find common ground
- Key principles for implementation of Patient Access Schemes in the UK are an indication of continued interest

Fad or a trend?

Stay tuned—yet to be determined.

Thanks!

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