Priorizar en nuevos medicamentos, sin pagar más por lo mismo

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Madrid, 12/02/2013

20 AÑOS DE C/E, ¿HA SERVIDO DE ALGO? (1)

- **Cambios en el PROCEDIMIENTO de reembolso:**

1. **Informes de EE sometidos:**

- **Seguimiento de las guías y estándares: baja calidad; 50% entre 2001-2006 cumplen con 9 aspectos metodológicos de las guías canadienses.**
- **Evaluación de los informes: por staff estatal o de AET; o por expertos externos (académicos).**
- **NICE empezó financiando informe propio a grupos académicos independientes ($$$); infra-estimación del RCEI en los informes de la industria.**
- **Pero NICE evoluciona hacia solicitar una revisión crítica del informe recibido de la industria y no un nuevo informe (como la mayoría). Parece que informes nuevos indep. resultan más restrictivos.**

20 AÑOS DE C/E, ¿HA SERVIDO DE ALGO? (2)

- **Cambios en el PROCEDIMIENTO de reembolso:**

2. Cambios con relación directa con la EE:

a) Cada vez más, el PRECIO se considera como una variable y no como un dato (hacia precio basado en el valor); potencial endegeneización de P.

b) Acuerdos de riesgo compartido: reembolso condicionado a resultados; acuerdos de gestión complicada; el riesgo del risk sharing [Neumann et al 2011]: costes de transacción; medida del resultado; falta de IT adecuada, etc.

c) Extensión de la EE a tecnologías médicas e intervenciones de salud pública (NICE; no siempre…)

20 AÑOS DE C/E, ¿HA SERVIDO DE ALGO? (3)

- **Cambios en el PROCEDIMIENTO de reembolso:**

2. Cambios acompañados por la EE:

a) Necesidad de medidas de resultado final y desconfianza de las medidas intermedias y de progresión de la enfermedad (EE requiere medidas de supervivencia y calidad de vida).

b) Necesidad de evidencia sobre efectividad comparada (métodos de síntesis de la evidencia cuando no se dispone de comparaciones directas).

c) El ICER varía según subgrupos de pacientes; aumento del uso restringido a subgrupos dentro de la indicación aprobada.

d) Mayor transparencia de la toma de decisiones, aunque las decisiones basadas en ICER no son fáciles de explicar.

PRECIO BASADO EN EL VALOR

- **FRANCIA: a mayor valor terapéutico (SMR; *Service Médical Rendu*) mayor cobertura; a mayor ASMR (*amelioration du SMR*) mayor precio. Pequeña mejora: 15% por encima de competidor. Sin mejora: por debajo.**
- **SUECIA: si el precio es demasiado elevado para el valor aportado, se excluye o se reduce P; en algún caso, P distinto según subgrupos.**
- **En algunos países con PR: C/E para determinar inclusión dentro del mismo grupo de referencia (sin o con poco valor adicional respecto comparadores) y premium price cuando hay valor añadido.**
- **Contratos de riesgo compartido: una forma indirecta de P basado en el valor.**

CONTRATOS DE RIESGO COMPARTIDO (1)

- **CONCEPTO:**
  - **PAGO final no depende sólo de Q (unidades vendidas),**
  - **depende de ciertos OBJETIVOS en términos de: EFECTIVIDAD, EFICIENCIA o IMPACTO PRESUPUESTARIO.**

- **El nombre no hace la cosa: ARCs, Performan-based reimbursement schemes, Patient Access Schemes, etc.**

- **Experiencia internacional con intensidad creciente en UE y especialmente en ONCOLOGÍA.**

Figure 2: Taxonomy of Risk Sharing Agreements

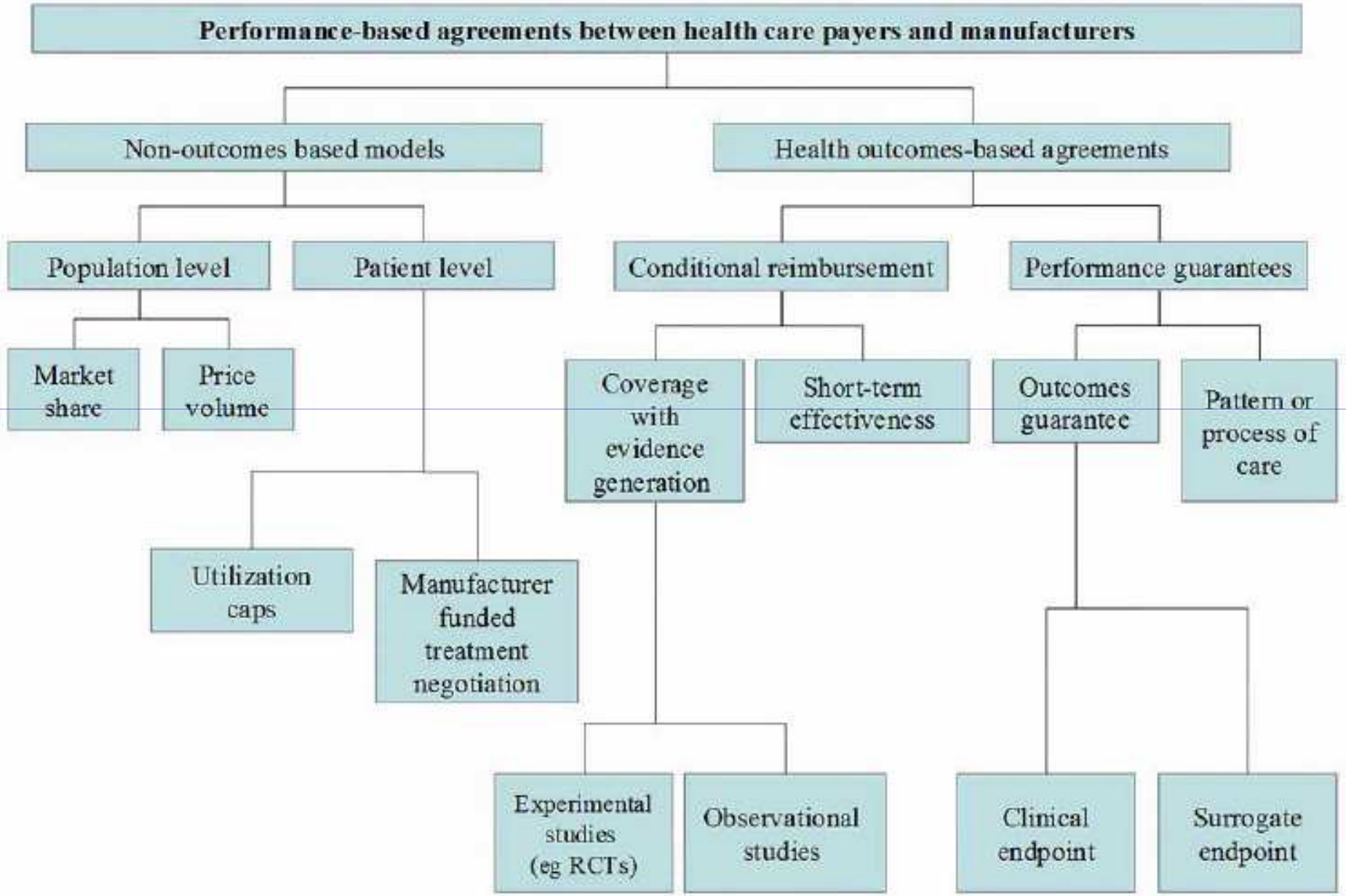
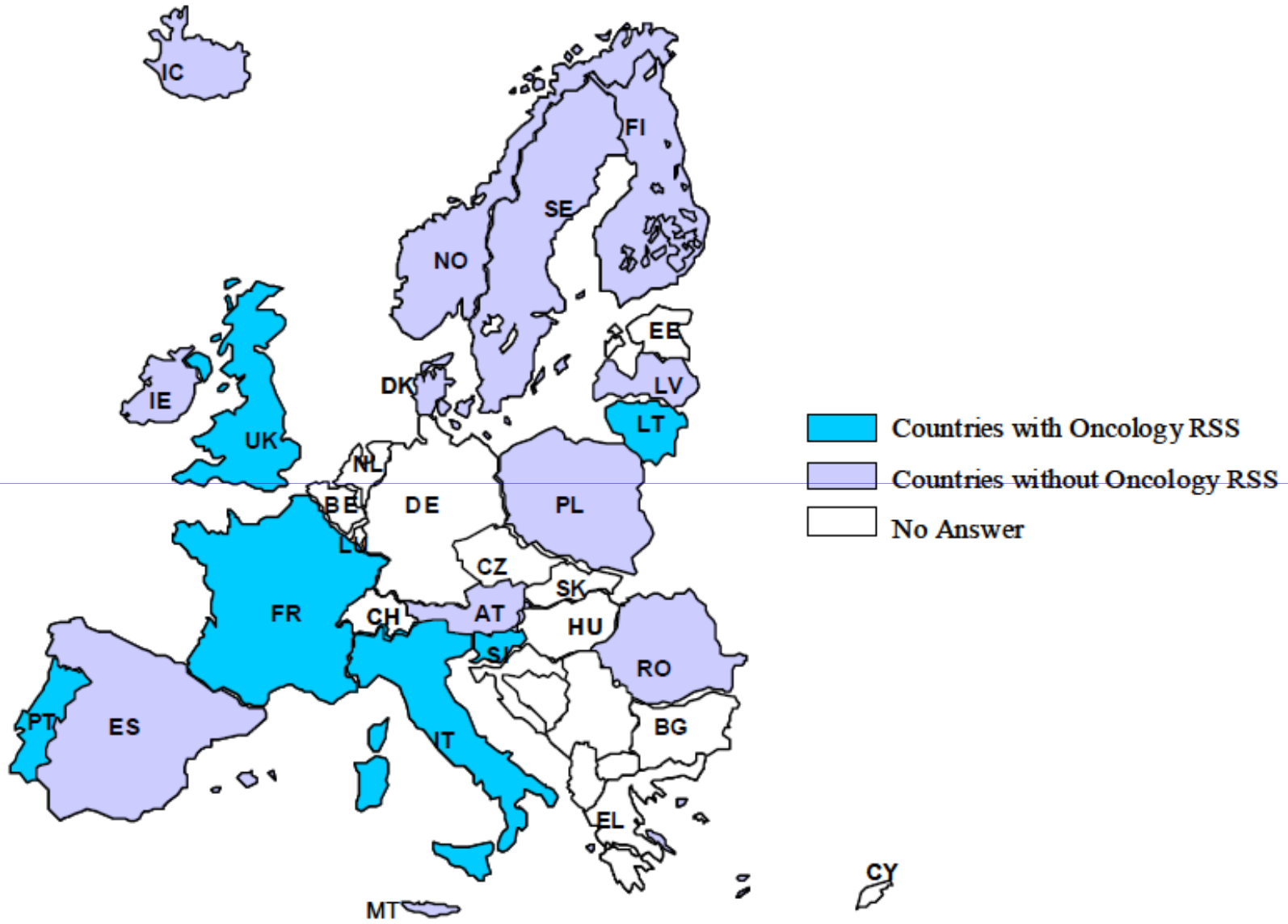


Figure 3. Answers to the Online Survey



Source: EMINet online survey on RSS

CONTRATOS DE RIESGO COMPARTIDO (4)

❑Italia (oncología)

paid by the manufacturers. The *Cost-Sharing scheme* consists of a discount on the (initial) treatment costs *for all eligible patients*, whereas in the scheme more properly defined as *Risk-Sharing* the discount is applied to the cost of the initial therapy cycle(s) *for non-responder patients*. The multiple innovative access schemes implemented in Italy are either variations or modalities of these financially-based schemes (cost sharing scheme), or based on health outcomes schemes (payment by results or risk sharing schemes), and they are shown in Table 2.

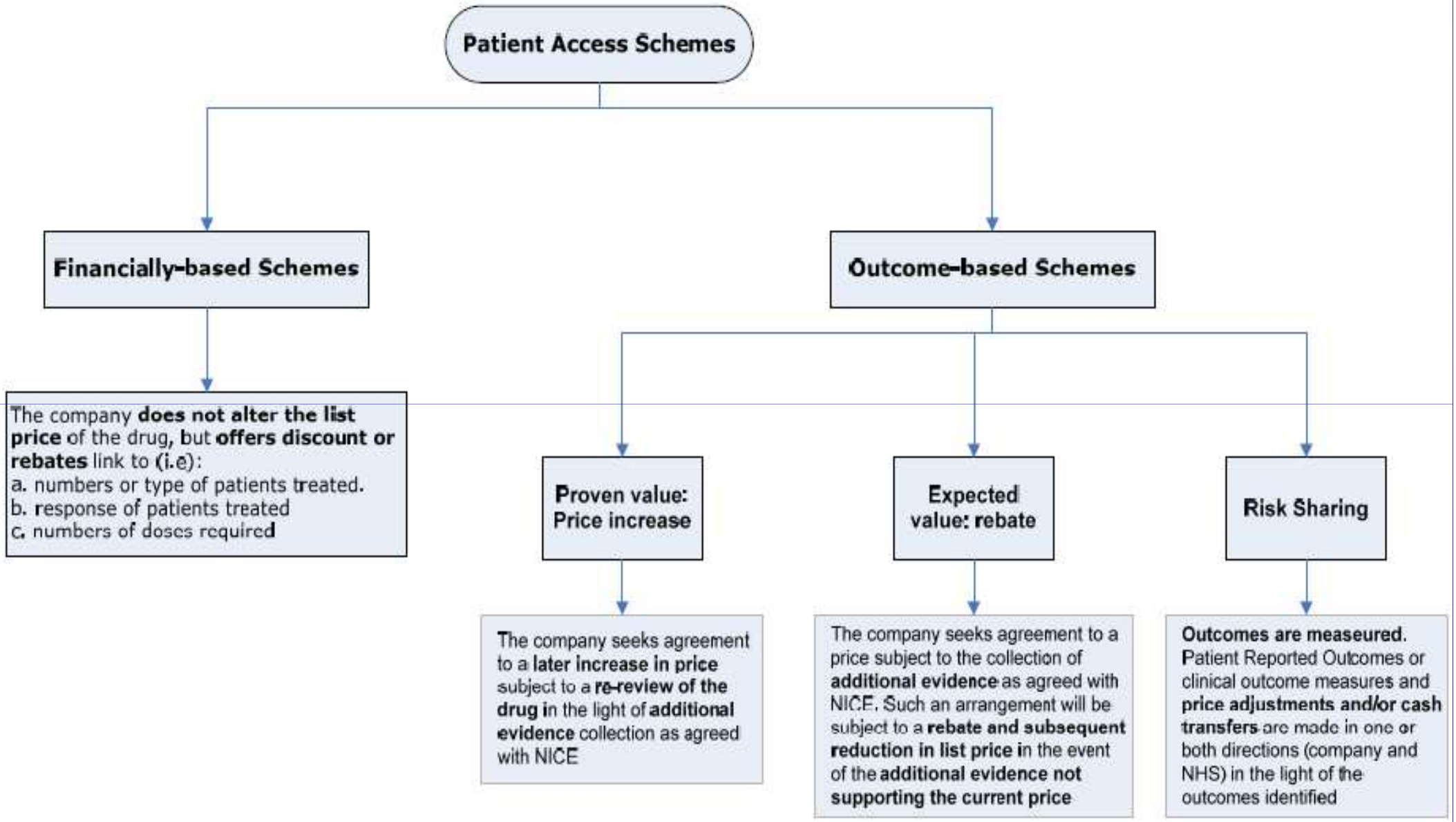
Payment by results	Cost Sharing Scheme	Risk Sharing Scheme
Dasatinib (Leukemia) - 2007 Nilotinib (Leukemia) - 2008 Temsirolimus (RCC) - 2008 Sorafenib (HCC) – 2008 Pegaptanib (AMD) – 2009 Ranibizumab (AMD) - 2009 Trabectedin (STS) - 2009 Lapatinib (mBC) - 2009	Erlotinib (NSCLC) -2006 Sunitinib (RCC) -2006 Sorafenib (RCC) -2006 Bevacizumab – 2008 Bortezomib (Myeloma) - 2009	Panitumumab (mCRC) -2009 Cetuximab (CRC) - 2009

Table 3: Description of RSS in Italy

Active Ingredient	Description	Date
Bortezomib	Cost-sharing scheme that requires manufacturers to pay back 50% of the treatment cost for all eligible patients during the first treatment cycle (6 weeks of treatments).	2009-2011
Erlotinib	50% price reduction for the first two cycles of therapy.	2006-2011
Nilotinib	Manufacturers must assume all costs for the first month of treatment for non-responder patients	2008-2011
Panitumumab	Risk sharing scheme that requires the manufacturer to pay-back 50% of the cost for non-responders (evaluation after 2 months of treatment).	2008 - 2012
Sorafenib	Treatment of advanced renal cell carcinoma: Risk sharing consisting of a 50% price reduction for the first 3 months of treatment. Treatment of hepatocellular carcinoma: Full price reduction for the first 2 months of treatment. Later reimbursed through credit notes for non-responders (2008)	2009-2011
Sunitinib	50% price reduction for the first three months of treatment.	2006 - 2011
Temsirolimus	The medicine is freely provided by the manufacturer for the first two months of treatment but only for non-responders.	2008 - 2011

Source: AIFA (Italian Medicines Agency) – Ministry of Health

Figure 4: Patient Access Schemes in the UK



Source: Pharmaceutical Price Regulation Scheme (PPRS) 2009

Design of Patient Access Schemes in the UK

Influence of Health Technology Assessment by the National Institute for Health and Clinical Excellence

Szymon Jarosławski[1] and *Mondher Toumi*[2]

Appl Health Econ Health Policy 2011; 9 (4): 209-215
1175-5652/11/0004-0209/$49.95/0

NICE HTA is the key driver of Patient Access Scheme (PAS) design, with the schemes constructed to address uncertainty surrounding cost effectiveness

All PAS were financially based but, rather than reducing list prices, cost reductions were via various discounts or rebates on a per-patient basis

Manufacturers' motivations to put forward PAS are unclear, and a more transparent process might be necessary to protect against a perverse impact of PAS on international reference pricing and to enable impartial monitoring and evaluation of the schemes

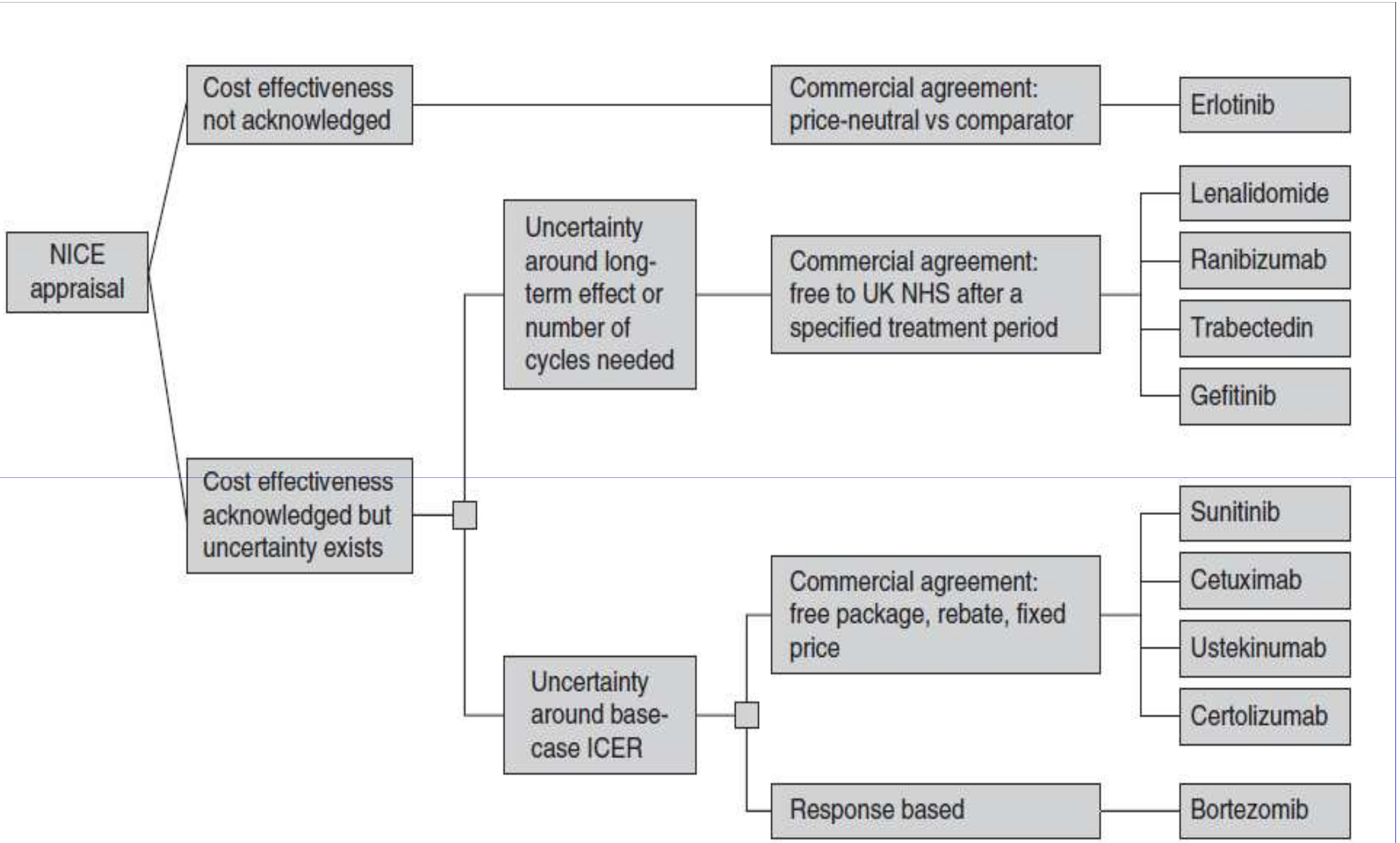


Fig. 1. Classification of Patient Access Schemes (PAS) according to UK National Institute for Health and Clinical Excellence (NICE) consideration of the cost-effectiveness evidence and the design of the PAS. **ICER** = incremental cost-effectiveness ratio.

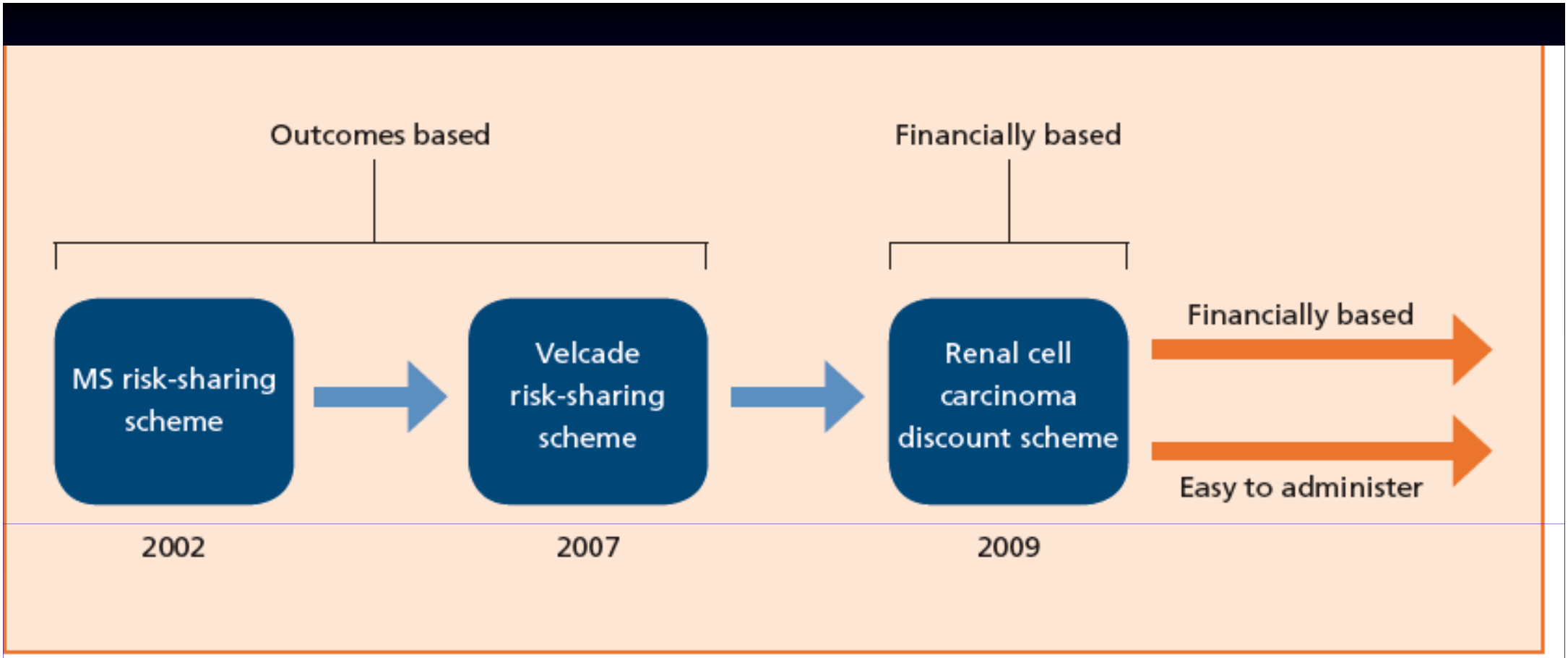


Figure 1. The evolution of PAS

Patient access schemes in the new NHS

British Journal of Healthcare Management 2012 Vol 18 No 8

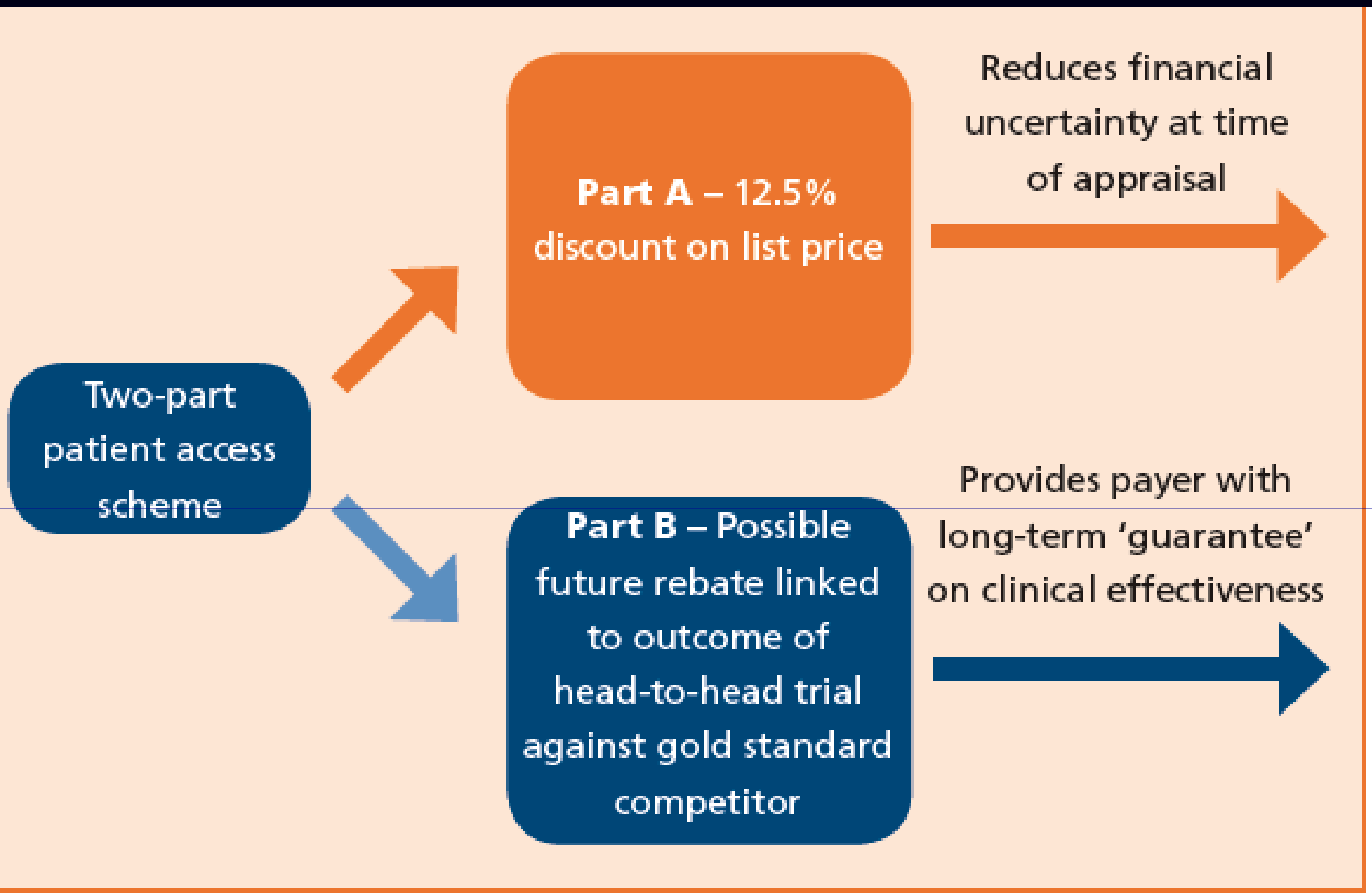


Figure 2. Reducing uncertainty at launch: the Votrient® PAS

Source: RJW and Partners, 2012

- Patient access schemes (PAS) have become an integral part of the UK pharmaceutical environment
- PAS have shifted from outcomes-based schemes to financially-based discounts. Bureaucratic schemes which are hard to administer are not welcomed by the NHS
- The Cancer Drugs Fund (CDF) may act as a potential disincentive for manufacturers to engage with PAS
- Integrating PAS and value propositions into pharmaceutical strategy as early as possible is crucial
- The key to the long-term future of effective value propositions such as PAS is to provide a stable pricing environment in the UK

- **PRINCIPALES RIESGOS en los contratos de RIESGO compartido:**

- **1. ELEVADOS COSTES DE TRANSACCIÓN: PUEDEN SER ELEVADOS (protocolos recogida datos; diseño acuerdos contratuales; recogida datos; evaluación resultados; procedimientos caso desacuerdo, etc.)**
- **2. PROBLEMAS DE MEDIDA: medida de efectos del tratamiento en entorno no aleatorizado; medidas intermedias poco adecuadas excepto si son precictores válidos de resultado final; influencia de otros factores (por ej. Sobre mortalidad); mejor is medidas de eventos clínicos poco ambiguos (fracturas) o biomarcadores bien establecidos (Velcade)**
- **AUSENCIA DE TI y SI ADECUADOS.**

Key Success Factors For Risk-Sharing Agreements

Stakeholder	Factor
Manufacturers	Ability to measure outcomes in short time frame, with clear indicator (biomarker) Undeveloped evidence base, opportunity to gather real-world evidence Product with clinical advantage over lower-cost competitors Few comorbid conditions, limited size of target patient population Availability of multiyear clinical data (mid–life cycle rather than newly launched products) IT infrastructure to track and audit data and manage patient registries
Payers	IT infrastructure to track outcomes and switched patients, simple audit systems Clear payment or reimbursement mechanism (free initial therapy preferable to later rebates if outcomes are not reached) Unequivocal outcome measure (for example, valid biomarker) Clear outcome-reporting flow from physicians
Physicians	Objective outcomes measures (for example, MRI or biomarkers) Opportunity to standardize dosing requirements and minimize overuse through physician training Small patient population, few comorbid conditions, system to monitor adherence
Patients	Rapid enrollment process, simple data-release authorization Clear clinical advantage of product, lack of alternatives Participation in outcome reporting Opportunity to improve adherence

SOURCE Authors' analysis. **NOTES** IT is information technology. MRI is magnetic resonance imaging.