International Perspectives.

The UK's Experience

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IHE Innovative Approaches to Industry-Payer Agreements: National Roundtable

Vancouver, 3rd April 2011

Research OHE Consulting
Office of Health Economics

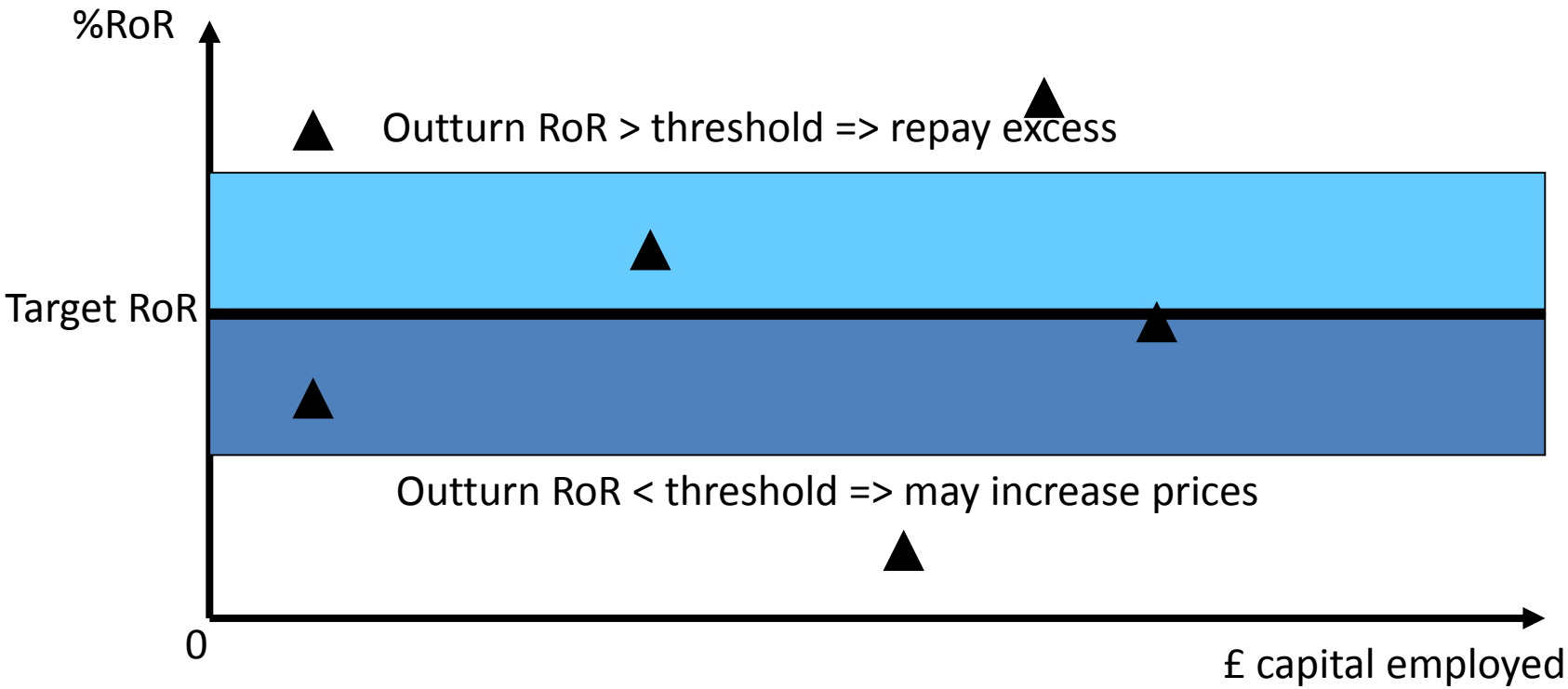
Agenda

1. Historic Context
   - The PPRS
   - NICE
     - Ad-hoc ‘risk sharing schemes’
   - OFT Market Study
2. Patient Access Schemes
3. VBP Consultation Document
4. Conclusions

The PPRS

- Have been variants of the Pharmaceutical Price Regulation Scheme (PPRS) since 1960s
- Department of Health acts as regulator for whole UK
- Objectives of 2009 PPRS:
  - **Deliver value for money**
  - **Encourage Innovation**
  - **Promote access and uptake for new medicines**
  - **Provide stability, sustainability and predictability**
- Voluntary – but statutory alternative scheme for firms that opt out
- Negotiated every 5 years or so
- Indirectly controls price by regulating profits earned by these firms (branded medicines)

The PPRS



Freedom of pricing at launch, subject to constraints

NICE

- National Institute for Health and Clinical Excellence – founded 1999
- Initial objective: reduce 'postcode prescribing'
- Provides advice on 'value for money' – offers recommendations
- Covers three aspects:
  - Technology Appraisals
  - Clinical guidelines – *increasing importance*
  - Quality standards – *more recently*

NICE

- Explicit about cost-effectiveness thresholds – *since 2004*
- Range of £20,000 to £30,000 per quality adjusted life year (QALY)
  - Below a most plausible ICER of £20,000 per QALY gained - decision based on cost-effectiveness estimate
  - Above a most plausible ICER of £20,000 per QALY gained – following factors are important:
    - The degree of certainty around the ICER
    - HRQL inadequately captured
    - The innovative nature of the technology
  - Above a most plausible ICER of £30,000 per QALY gained, the case for supporting the technology on these factors has to be increasingly strong

NICE – 'Ad hoc' risk sharing schemes

Reason

- NICE deemed 4 drugs for multiple sclerosis not cost-effective– but recommended further action (2002)

Outcome

- Risk-sharing agreement (after round of price revisions - £36,000/QALY)

Monitoring

- Detailed monitoring over 10 years of a cohort of patients to confirm the cost-effectiveness of the MS treatments
- Formal reviews

Funding

- All companies participating in the scheme, and the health departments collectively, are expected to make an equal contribution to funding the administrative arrangements for the scheme

NICE – 'Ad hoc' risk sharing schemes

Some earlier critiques…

Problems with UK government's risk sharing scheme for assessing drugs for multiple sclerosis

BMJ VOLUME 326 15 FEBRUARY 2003 bmj.com

Cathie L M Sudlow, Carl E Counsell

The government plans to make interferon beta and glatiramer available to patients with multiple sclerosis through a risk sharing scheme, despite lack of evidence of cost effectiveness. Sudlow and colleagues argue that the money would be better spent on independent research

And some earlier results….

BMC Neurology

BioMed C

Research article

Open Ac

The Multiple Sclerosis Risk Sharing Scheme Monitoring Study – early results and lessons for the future

Mark Pickin*†1, Cindy L Cooper†1, Timothy Chater1, Anthony O'Hagan2, Keith R Abrams3, Nicola J Cooper3, Mike Boggild4, Jackie Palace5, George Ebers5, James B Chilcott6, Paul Tappenden6 and Jon Nicholl1

Abstract

Background: Risk sharing schemes represent an innovative and important approach to the problems of rationing and achieving cost-effectiveness in high cost or controversial health interventions. This study aimed to assess the feasibility of risk sharing schemes, looking at long term clinical outcomes, to determine the price at which high cost treatments would be acceptable to the NHS.

Methods: This case study of the first NHS risk sharing scheme, a long term prospective cohort study of beta interferon and glatiramer acetate in multiple sclerosis (MS) patients in 71 specialist MS centres in UK NHS hospitals, recruited adults with relapsing forms of MS, meeting Association of British Neurologists (ABN) criteria for disease modifying therapy. Outcome measures were: success of recruitment and follow up over the first three years, analysis of baseline and initial follow up data and the prospect of estimating the long term cost-effectiveness of these treatments.

Results: Centres consented 5560 patients. Of the 4240 patients who had been in the study for a least one year, annual review data were available for 3730 (88.0%). Of the patients who had been in the study for at least two years and three years, subsequent annual review data were available for 2055 (78.5%) and 265 (71.8%) patients respectively. Baseline characteristics and a small but statistically significant progression of disease were similar to those reported in previous pivotal studies.

Conclusion: Successful recruitment, follow up and early data analysis suggest that risk sharing schemes should be able to deliver their objectives. However, important issues of analysis, and political and commercial conflicts of interest still need to be addressed

NICE – 'Ad hoc' risk sharing schemes

Multiple sclerosis risk sharing scheme: two year results of clinical cohort study with historical comparator

Mike Boggild, consultant neurologist,[1] Jackie Palace, consultant neurologist,[2] Pelham Barton, senior lecturer in mathematical modelling,[3] Yoav Ben-Shlomo, professor of clinical epidemiology,[4] Thomas Bregenzer, director, biostatistics,[5] Charles Dobson, senior projects officer,[6] Richard Gray, director[7]

BMJ

Conclusions It is too early to reach any conclusion about the cost effectiveness of disease modifying treatments from this first interim analysis. Important methodological issues, dataset impact address essenti the cohort are likely to be more informative, not least because they will be less sensitive to short term fluctuations in disability.

More recent results and analysis (2009/10)

Analysis

Multiple sclerosis risk sharing scheme: a costly failure

James Raftery, *professor of health technology assessment*

Analysis

Commentary: Outcome measures were flawed

G C Ebers, *Action Research professor of clinical neurology*

Editorials

The multiple sclerosis risk sharing scheme

A clever achievement, which despite being flawed, has had unintended beneficial consequences

Analysis

Continuing the multiple sclerosis risk sharing scheme is unjustified

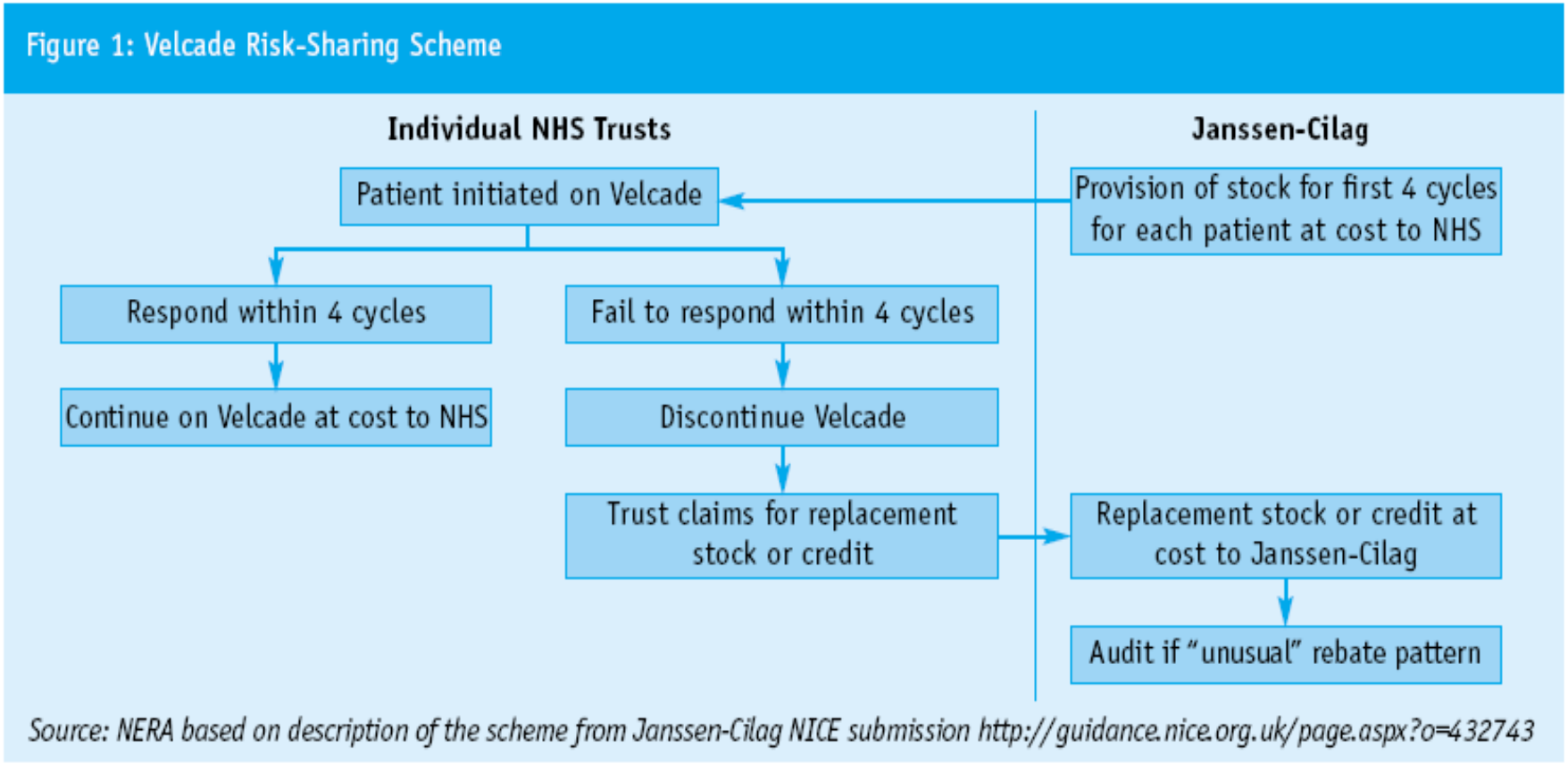
Christopher McCabe, *professor*[1], Jim Chilcott, *senior research fellow*[2], Karl Claxton, *professor*[3], Paul Tappenden, *senior research fellow*[2], Cindy Cooper, *senior research fellow*[2], Jennifer Roberts, *professor*[4], Nicola Cooper, *senior research fellow*[5], Keith Abrams, *professor*[5]

Analysis

Commentary: Scheme has benefited patients

Alastair Compston, *professor of neurology*

NICE – 'Ad hoc' risk sharing schemes



Source: Barham, PPR September 2007

OFT Market Study (2007)

Key recommendations

- **We recommend that Government reform the PPRS, replacing current profit and price controls with a value-based approach to pricing**, which would ensure the price of drugs reflect their clinical and therapeutic value to patients and the broader NHS.
- We believe this would provide **major benefits to patients and innovative companies** in the short and long term:
  - **value for money for the NHS:** we have identified hundreds of millions of pounds of expenditure per year that could be used more cost effectively under value-based pricing, allowing patients greater access to drugs and other healthcare benefits they are currently being denied. In short, the same level of expenditure could be used to produce greater benefits for patients.
  - **better incentives to invest:** more value-reflective prices would give companies much stronger incentives to invest in the drugs that are most beneficial to society, particularly in areas of unmet patient need. Given the international importance of UK prices, these benefits would be felt not just in the UK, but globally.
  - **a stable, sustainable system:** these reforms would improve stability for Government and industry in the long run, by avoiding reliance on increasingly arbitrary profit and price controls and ensuring instead that future pricing decisions are based on an informed, rational debate about how to make the best use of available NHS resources.
- International experience shows that value-based pricing can work well in countries that have fewer resources than we enjoy in the UK but companies have highlighted key issues that need to be addressed in ensuring effective implementation. We believe we have met these concerns in developing options for reform that will provide **a credible, practical pricing regime for the long term**.

- One key recommendation: replacement of profit and price controls with “VBP”
- Centralised price setting mechanism determined (or at least heavily influenced) by HTA, assuming:
  - ‘value = incremental cost effectiveness’, and
  - an explicit threshold can be determined.
- Risk-sharing schemes an exception, not the norm

The 2009 PPRS

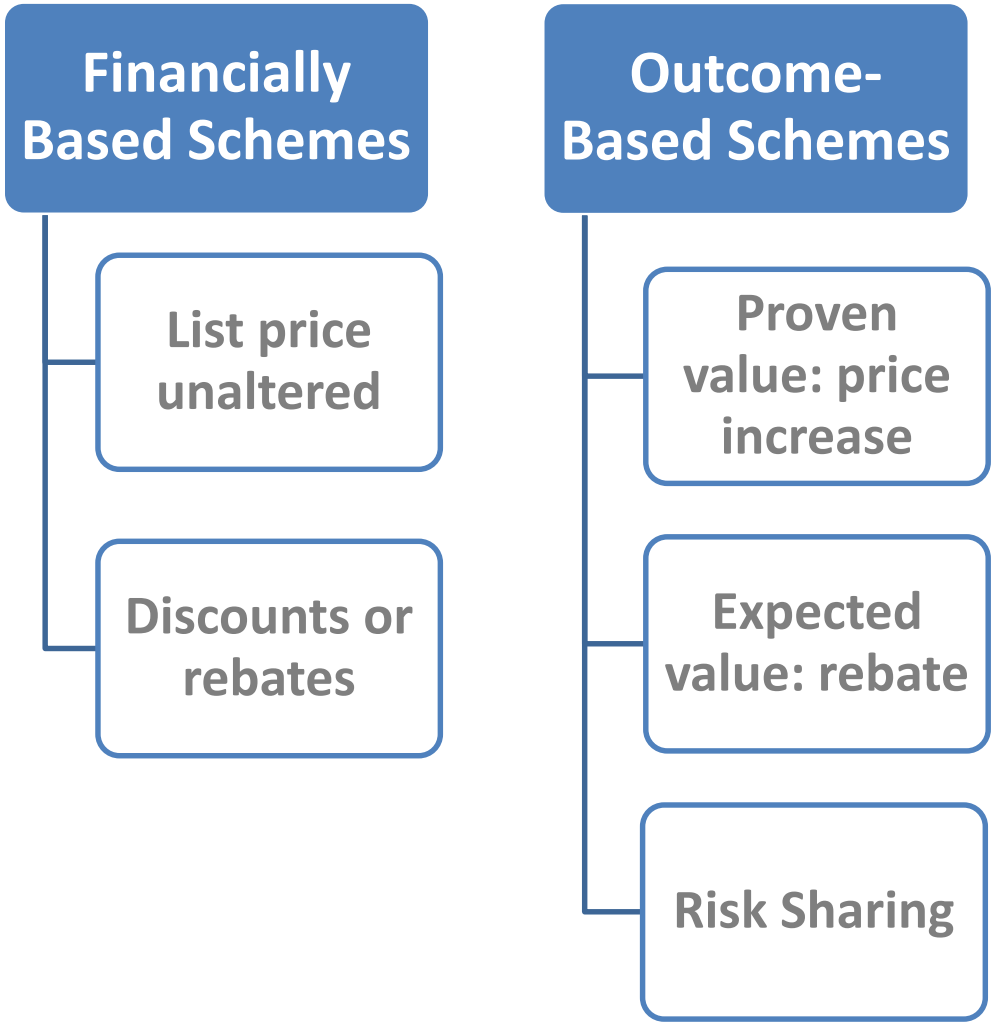
- **Flexible Pricing Schemes:** where a company can increase or decrease its original list price in light of new evidence or a different indication being developed (*NB none to date*)
- **Patient Access Schemes (PAS):** which will facilitate earlier patient access for medicines that are not in the first instance found to be cost and clinically effective by NICE within a framework that preserves the independence of NICE

PAS

(Some) Key principles:

- Arrangements must respect the role of NICE
- Schemes are to be discussed first and agreed in principle by the Department and the company
- Schemes should be clinically robust, clinically plausible, appropriate and monitorable
- Any scheme should be operationally manageable for the NHS without unduly complex monitoring, disproportionate additional costs and bureaucracy
- Schemes should be consistent with existing financial flows in the NHS and with local commissioning
- The more systematic use of such Schemes will need to be reviewed in light of experience. The timing of such a review will be jointly agreed but will be initiated not later than two years after the commencement of this Agreement – *currently on-going*

PAS



Outcome based schemes particularly risk sharing schemes are likely to be more burdensome - only to be appropriate in exceptional circumstance

Later price increase subject to re-review of drug using additional data collected

Price set subject to collection of additional data and subsequent price reduction if data do not support price

Price adjustments / cash transfers made subject to outcome measures (PROMs or clinical)

PAS to date

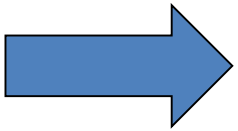
- 15 PAS in England and Wales
  - Only 10 have been part of positive (including 'restricted' or 'optimised') NICE appraisals
- All 15 are financially-based
  - Two: response related (1 accepted/1 rejected)
  - One: simple discount
  - 12: involve rebates or free replacement stock and require collection of patient level data
- Hospitals are not finding implementation easy

Source: Towse, 2010

- **DH view**: "PASs have led to NHS patients being provided with access to new drugs on more cost-effective terms" (PPRS 10th Report to Parliament, October 2009)

But big change (?) in prospect…

July 2010



December2010

Implementation of Value Based Pricing (VBP) replacing the PPRS by 2014 (when current PPRS expires)

VBP Consultation Document

Key points:

- The Government believes that there are significant shortcomings in the current system for branded drug pricing and access
- PAS not a long term solution - the cumulative administrative burden falling on front-line NHS staff from Patient Access Schemes must be managed
- Government would apply weightings to the benefits provided by new medicines => a range of price thresholds reflecting the maximum we are prepared to pay for medicines
- Basic threshold – but factors driving higher thresholds:
  - Burden of illness: severity + unmet need
  - Greater therapeutic innovation and improvements
  - Wider societal benefits
- Price mechanism: weighted cost per QALY
- NICE has important part to play in these longer-term plans

- NB We already have elements of VBP

What is VBP?

VBP requires 4 top level decisions:

1. What elements to include in “value”
2. How each element is measured – and from whose perspective
3. How the different elements are aggregated into a single measure of overall “value”
4. How assessed “value” then translates into price

OHE's Response to VBP Consultation

1. Value-based pricing (VBP) can, and should, exist side-by-side with other approaches such as the Patient Access Schemes.
2. No approach to assessing value as a basis for pricing can be wholly mechanistic; as demonstrated in every other country that assesses the 'value' of medicines, an element of negotiation about price always is present.
3. 'Innovation' is not a 'yes or no' variable, but occurs along a continuum of various degrees of innovation. This perspective must underlie any VBP.
4. Discussions must include whether the UK has an obligation to price in a way that encourages innovation, not 'free ride' on other countries that do.
5. Full assessment of value must take full account of patients' preferences and experiences as well as the wider benefits to society, not be limited to costs to the NHS.
6. A value assessment based on weighting quality-adjusted life years (QALYs), as suggested by the UK Department of Health, is not appropriate. Multiple criteria must be considered and include social value judgements, some of which will not be proportional to the incremental QALYs a medicine is judged to yield.

Source: More at: http://oheuk.wordpress.com/

Some Reflections

'Risk-sharing' agreements will grow in importance – and more flexible than direct price controls/cuts

- **Pros**:
  - May provide access where otherwise there would be none
  - Incentives for manufacturer/payer to run compliance schemes
  - Provides information on use in practice so adds to evidence base

- **Cons**:
  - Additional costs of collecting the information and setting up the scheme
  - Delays in patient access to new medicines as scheme is agreed – especially relevant for the otherwise 'free-pricing at launch' (i.e. no price negotiations) countries
  - Access delays as scheme is implemented on the ground
  - Long term impact on innovative environment
  - Not enough 'trust' between payers/industry to make them workable

Conclusions

What are the key factors for successful development of industry payer agreements?

- In UK: Willingness from both sides to have a regular, constructive dialogue (other initiatives that demonstrate this - PICTF, MISG…)
- But seems UK Department of Health puts significant weight in avoiding administrative costs - *proportionately too high*?
  - Is this just a short run constraint or a more fundamental issue: is the government a 'perfect' agent of societal interest?
- PAS are a working example of flexible, non-linear approaches to pricing => good thing!

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