Risk sharing’ arrangements – considerations for the future

Piperska course on the managed Introduction of new drugs

Prepared by Brian Godman
1. **Introduction**

2. **Current situation**

3. **Future considerations**
Risk sharing schemes are growing to enhance the value of new drugs and their reimbursement

- New expensive drugs account for over 50% of in-patient drug budget in Marseilles hospitals - growing at over 20% per year

- New biological drugs with acquisition prices over $50,000 to $120,000/patient/year are adding to resource pressures - some with only marginal health gain. These resource pressures will intensify

- Consequently, new models are growing to optimise the managed entry of new premium priced drugs including a critical appraisal of the growing number of risk sharing arrangements

- These arrangements are growing to enhance the ‘value’ of new drugs to aid their reimbursement/funding as well as limit their utilisation to agreed populations to help conserve resources
Risk sharing schemes are not new – an early example
As discussed, ‘risk sharing’ schemes are growing for pharmaceuticals particularly in Europe to help achieve two major aims. These include:
- Means by which payers can regulate their budgets especially where limited demand side measures to control utilisation, e.g. Price: Volume agreements
- Mechanisms by which pharmaceutical companies can enhance reimbursement/ funding for new drugs without cutting list prices, e.g. ‘free drug’ and ‘outcome guarantee’ schemes to improve the value proposition

However, there are concerns with definitions as many different terms have been and are still being used.

In addition, scarcity of published data regarding the impact and outcome of current schemes to provide future guidance.
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We recently defined risk sharing schemes from a payer perspective to reduce confusion

- Proposed definition
  ‘Risk sharing schemes for pharmaceuticals should be considered as agreements concluded by payers and pharmaceutical companies to diminish the impact on the payer’s budget of new and existing medicines brought about by either the uncertainty of the value of the medicine and/or the need to work within finite budgets’

- The agreements lie in setting the scope for such schemes and realising the mutual obligations by both parties – ‘the risk’. The ‘risk’ varies by the situation, e.g. expenditure higher than agreed or health gain from a new product lower in practice

- The various schemes can be subdivided into:
  - Financial/financial-based models
  - Outcome/performance-based models

Ref: Adamski, Godman et al 2010
Financial/financial-based models:
- Price: volume agreements (PVAs) for new and existing drugs – typically pay back/ rebate mechanisms if volumes and/or expenditure exceed agreed limits for the drug, class, or overall pharmaceutical expenditure
- Patient access schemes involving free/ discounted drugs
- Price cap schemes – whereby companies will cover the additional costs above agreed limits. This includes both patients and payers in the US

Performance based or outcome models (with growing role of HTA):
- ‘No cure, no pay’ schemes including rebates if drugs fail to produce desired outcomes
- Drugs provided free until their effectiveness is demonstrated
- Prices modulated if new drugs do not produce the desired patient benefits in practice

Ref: Adamski and Godman 2010
<table>
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<tr>
<th>Country</th>
<th>Examples of PVA financial based schemes across countries include</th>
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| **Australia** | - Two principal schemes exist - PVAs with price reductions if sales exclude pre-agreed volumes as well as rebate arrangements if costs exceed a subsidised cap or threshold  
- In addition, pricing arrangements for Section 100 drugs (specialist drugs for hospitals or other similar facilities) |
| **Estonia** | - Annual price: volume agreements are mandatory for all pharmaceuticals in the positive list  
- This includes the rationale supporting the figures  
- Rebates and/ or price reductions if expenditure exceeded |
| **France** | - Contracts are signed annually (some exceptions) taking account dosing and utilisation of single drugs as well as classes, with compensation if costs exceeded. Orphan drugs now included  
- Rebates in 2004 were €670mn – 3% of total pharmaceutical expenditure. €260mn in 2008 |
| **Italy** | - Payback schemes exist where pharmaceutical expenditure exceeds 14% in ambulatory care and 2.4% in hospitals  
- Rebates amounted to €773mn in 2005 |

Ref: Adamski and Godman 2010
### Country  |   Examples of patient access schemes
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#### Italy
- Costs of bevacizumab in approved cancers can not exceed €25,941 per patient per year
- This is in addition to other schemes to reduce costs for bevacizumab and other anti-cancer drugs in Italy

#### England and Wales
- Under the RANIBIZUMAB Reimbursement Scheme additional costs of injections above 14/ patient reimbursed by the company either as free drug or a credit note
- Discounts given for TARCEVA to ensure similar costs to docetaxel for patients with Non Small Cell lung cancer
- Sunitinib for patients with metastatic renal cell carcinoma - the first treatment cycle (6-weeks costing an average of GB£3139/ patient) is provided free via a patient access programme
- Sorafenib for metastatic renal cell carcinoma - the first pack (200mg x 112 tablets) provided free by the manufacturer equating to £2980.47p excluding VAT

Ref: Adamski and Godman 2010; NICE website
<table>
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<tr>
<th>Country</th>
<th>Examples of performance based/ outcome based schemes (continued)</th>
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<tr>
<td><strong>UK – England and Wales</strong></td>
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**Beta interferon for multiple sclerosis**
- NICE initially rejected funding for the β interferons in MS on clinical and cost-effectiveness grounds with a cost/ QALY of £42,000 to £98,000
- Under the proposed scheme, patients would be followed for over 10 years with prices reduced or refunds if the cost/ QALY was over £36,000/ QALY in reality
- Scheme heavily criticised as unscientific and impractical

**Bortezomib for multiple myeloma (VELCADE)**
- Scheme based on a 50% reduction in serum paraprotein levels by the fourth cycle. NHS will fund treatment in responders with the cost/ QALY reduced to £20,700/ QALY. J & J will refund drug costs if a 50% reduction in levels not achieved
- Prices remain at the launch price although discounts given
- Concerns though whether M-protein reliable surrogate and 10-15% of patients have no measurable levels

**Omalizumab for severe persistent allergic asthma**
- Manufacturer agreed to refund the cost of additional drug, as free drug, in patients who fail to respond by 16 weeks

*Ref:* Adamski and Godman 2010; NICE website
Additional risk sharing schemes in the UK include certolizumab pegol for RA patients

- Additional examples of patient access (cost sharing) schemes in the UK include the scheme for certolizumab pegol (a ‘recombinant, humanised antibody Fab’ fragment against tumour necrosis factor alpha conjugated to polyethylene glycol).

- Certolizumab pegol is approved in combination with methotrexate for the treatment of moderate to severe rheumatoid arthritis in adult patients with inadequate response to DMARDs.

- It can also be given as monotherapy when patients are intolerant to methotrexate or when continued treatment with methotrexate is inappropriate.

- A scheme was developed with NICE in the UK to enhance its ‘value’ as well as enhance its market share for new patients.

Ref: NICE 2010
The NICE Patient Access Scheme was designed to facilitate the access of certolizumab pegol to all eligible NHS patients.

Under the scheme, the Company covered the costs of the first 12 weeks of treatment for all eligible patients, i.e. equating to 10 free doses/patient. This is managed by a third party to help reduce the administrative burden.

The decision point was driven by the clinical evidence suggesting that the majority of RA patients respond in the first 12 weeks. Consequently, clinicians have the option to investigate alternative treatments if needed without initially incurring NHS expenditure.

The Company had difficulties though explaining that the drug was ‘free’ for the first 12 weeks.

Risk sharing schemes in the UK include certolizumab pegol for RA patients (continued)
There are both benefits and concerns with financial based schemes that need addressing

**Benefits with financial-based schemes**
- Enhances the opportunities for reimbursement as well as for payers to work within defined budgets
- Shifts cost/ usage considerations to pharmaceutical companies – essential where concerns of excessive utilisation and limited demand side measures in practice in pertinent countries
- Limits ‘off label’ usage/ indication creep in practice – especially important for expensive biological drugs and new orphan drugs

**Concerns with financial-based schemes**
- The ‘first’ patients in PVA schemes may not always be the most appropriate, and schemes may not always factor in issues such as compliance
- Pharmaceutical companies may benefit from early access of ‘unproven’ technologies
- Can be complex to administer limiting savings in practice
- Potentially issues of patient confidentiality and follow up, e.g. dose capping schemes

Ref: Adamski and Godman 2010; Godman CEESTAHC 2011, Godman Belgrade 2012
Concerns with patient access schemes include logistics and administration costs

**Logistic concerns with PAS impacting on the value proposition**
- Whether the system can cope with the time scales for refunds, e.g. time between monitoring disease progression and the next physician visit to stop therapy
- Whether the national health system is set up to receive free goods/ rebates from pharmaceutical companies
- Whether refunds to hospitals/ hospital trusts are passed back to the ‘payers’ in practice to fully realise the resource benefits (was happening in approximately 50% of UK hospitals)

**Administrative concerns with PAS schemes**
- Capacity to manage PAS schemes without funding additional staff – can prove difficult in practice
- Time taken to administer schemes
- Communication between physicians and pharmacists to ensure refunds/ rebates, e.g. every missed claim for bortezomib looses the hospital GB£12000

Ref: Adamski, Godman et al 2010; Williamson 2010; Williamson and Thomson 2010; Godman Belgrade 2012
Benefits of performance based/ outcome based schemes enhancing value

- Payers only fund treatments that produce desired health gain and treatments can be targeted where health gain is greatest
- Payers can monitor usage in practice against agreements and monitor safety especially given the selective nature of Phase III clinical trials and possible safety concerns with some new drugs
- Enhances the chances of successful reimbursement and funding

Concerns with performance based/ outcome based schemes ↓ value

- Whether the objective is fully explicit and transparent, and the level of evidence sufficient to make robust decisions
- Who will end up funding registries/ databases, and whether these can be introduced with current regulations/ staff
- Length of follow-up and general administrative burden including refund mechanisms especially for rebates
- Potentially accelerating the uptake of new medicines in practice

Ref: Adamski and Godman 2010; Godman CEESTAHC 2011; Belgrade 2012
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We have identified a number of key issues that need to be considered for the future

Issues that need to be considered for the future include:

- Appropriateness of the schemes, e.g. help contain utilisation in practice where currently limited demand side measures
- Whether the objective(s) of the scheme and scope are explicit and transparent and ‘openness’ (similar to contracting)
- Whether the new drug is novel in a high priority disease area backed up by good transitional science
- The economics and outcomes, e.g. whether the new drug could have a substantial beneficial impact on service delivery and/ or safety but difficult to prove this in Phase III trials
- Time scales – overall and for specific situations
- The likely administration costs/ burden versus the potential savings (certolizumab pegol vs. bortezomib in the UK)
- Whether health services can monitor outcomes in practice via patient registries, who funds these and who owns the data

Ref: Adamski and Godman 2010; Godman CEESTAHC 2011
Issues include situations where payers should be critical of proposed schemes

Overall, Health Authorities and Health Insurance Agencies should be critical of risk sharing schemes where:

- Effective and low cost standards already exist (led to the disbanding of the atorvastatin scheme in the UK)
- Health authorities will end up funding a substantial proportion of a new drug’s development costs without payment
- Patient compliance is important but not been fully addressed
- There will be a high administrative burden – but this has not been considered/ factored in
- Ethical considerations have not been fully addressed
- Insufficient competent staff as well as IT support
- Provisional reimbursement schemes are being proposed which could encourage the ‘over’ prescribing of new expensive drugs to accelerate their assessment

Ref: Adamski and Godman 2010; Godman CEESTAHC 2011
Key factors to enhance acceptance and ‘value’ of risk sharing schemes includes targeting

- Key criteria to enhance the chance of successful ‘risk sharing’ arrangements from a ‘payer’ perspective could include:
  - The objectives and scope are explicit and transparent
  - The new drug is:
    - A novel treatment with envisaged health gain with few if any treatments currently available
    - Ability to target treatment to defined populations
    - With/ without long term safety concerns
  - Translational science suggests good effectiveness and delaying treatment may not be in key stakeholders’ interest
  - The likely health gain can be determined within a relatively short time frame with proven biomarkers
  - Patient access schemes can appreciably lower the cost of new drugs having factored in all administrative costs
  - New drug not competing against low cost standard drugs
  - Patient compliance is not a major issue
  - Price: volume schemes enable access to new cost-effective treatments whilst controlling usage in practice

Ref: Adamski and Godman 2010; Godman CEESTAHHC 2011; Belgrade 2012
Thank You

Any Questions!

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