Policy guidelines for risk-sharing agreements in South Africa

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Abstract

Background: Biological medicines are clinically effective but very expensive in South Africa. The business decisions of biological manufacturers and payers (medical schemes) impact the access patient's have to biological medicines. The paper is the second paper of a two part series that explore risk-sharing agreements for biologic medicines. In this paper, the events related to trastuzumab and Discovery Health are presented as a vehicle to explore the application of risk-sharing agreements in South Africa.

Methods: The paper critically reviews the current policy framework and assesses the implications for the manufacturer and the payer. The structural necessities for the outcomes-based reimbursement of biologic medicine are revisited and the paper extracts key lessons and presents these as policy guidelines covering the following four phases: pre-planning phase, planning, implementation and monitoring.

Results: There are numerous policy implications for the manufacturer of biological medicines and the payers (medical schemes). Each implication directly impacts the establishment of risk sharing agreements and inevitably determines the success or failure of such agreements. Two organisations comparable to the NCQA and NICE are required for the successful implementation of outcomes-based reimbursement. The precursors for the development of the such organisations already exist in South African legislation. Risk-sharing agreements have been narrowly conceptualized as a financial risk management tool devoid of clinical and QoL outcomes measurement.

Conclusions: A risk-sharing agreement is a useful tool to manage the risk of introducing clinically effective and very expensive medicines into the healthcare market. Clinical, QoL and financial outcome measures should be integrated into a risk-sharing agreement. A risk-sharing agreement is a tool that bridges the conflicting priorities of the manufacturer of biological medicine and the payer. Moreover, it is a mechanism that mitigates the ethical, social, and political consequences of denying care to patients often confronted with an all-or-nothing situation.

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Introduction

Biological medicines are clinically effective but very expensive in South Africa. These medicines are produced using a living organism, are complex protein structures typically much larger than traditional chemical medicines and are mostly administered by injection. Biological medicines are more advanced than conventional therapies and provide prescribers with enhanced tools for treating patients. Access to biological medicines is a contested terrain between the manufacturer of biological medicines and the payer (medical scheme), often to the detriment of the patient. A risk-sharing agreement is a tool for manufacturers of biological medicines and payers to manage the risk of introducing clinically effective and very expensive medicines into the healthcare market. As promising as the approach may seem, particularly for patients, risk-sharing agreements come with some challenges with regard to their implementation.

This paper is the second and final paper in a two-part series. In the first paper, risk-sharing agreements were discussed as a means of introducing biological medicines. The paper presented some prominent international experience with risk-sharing agreements and the nuances associated with such agreements. The paper also presented the outcomes-based reimbursement of biological medicines and structural necessities for its successful implementation. The first paper set the stage for a discussion of the application of risk-sharing agreements for biological medicines in South Africa.

The purpose of this paper is to discuss risk-sharing agreements in South Africa. Risk-sharing agreements will be discussed with reference to the events in 2006 related to trastuzumab (Herceptin®) and Discovery Health. This case carries within it many critical elements that merit further discussion. The paper explores the policy implications of entering into risk-sharing agreements for the manufacturer and payer. The paper also revisits structural necessities for risk-sharing agreements and concludes with policy guidelines aimed at enabling the successful implementation of risk-sharing agreements.

Trastuzumab and Discovery Health

The case of trastuzumab, produced by Roche Pharmaceuticals, and Discovery Health serves to illustrate the tension between the two players: manufacturers of biological medicines and payers. Trastuzumab is indicated for advanced stages of breast cancer and has received funding in the past for this indication. With evidence that appeared in the *New England Journal of Medicine*,[1] the use of trastuzumab in early-stage breast cancer was legitimised. It was during this time that nine women launched legal action against Discovery Health for turning down their respective doctor's motivation to provide access to trastuzumab.[2] Soon after the publication of the evidence in the *New England Journal of Medicine*, the South African Oncology Consortium released treatment guidelines that supported the use of trastuzumab in early-stage breast cancer for a nine-week period. Evidence suggests that the use of trastuzumab concurrently with other types of chemotherapy may be cost-effective when trastuzumab is used for shorter instead of longer treatment periods.[1] This highlighted the plight of breast cancer patients and encouraged them to mobilise themselves in order to obtain access to trastuzumab for early-stage breast cancer.

The public debate started with Discovery Health's refusing to fund trastuzumab and insisting that the price at which the medicine became cost-effective was R100 000 per annum, as opposed to Roche's R300 000 annual treatment cost.[4] Discovery Health claimed that in its pharmacoeconomic assessment of trastuzumab it was trying to find the appropriate price, thus bringing its price in line with the price in other countries. Soon after these claims Roche requested the Department of Health to verify that the South African price was on a par with that in other countries such as the United Kingdom, Australia, the United States of America, Mexico, France, Poland and Chile.[4] The media coverage of the case dwindled when Discovery Health announced its decision to fund trastuzumab for a nine-week treatment period and apologised for the process that had caused anxiety and uncertainty for breast cancer patients.[5] There was no public response from the Department of Health concerning the pricing of trastuzumab.

In this case, the majority of the debate focused on the price of trastuzumab – the claimed R100 000 cost-effective price of Discovery Health and Roche's R300 000 annual treatment cost. The gap between the two prices was dramatised as a battle of healthcare titans but little was conveyed about the clinical and quality of life (QoL) outcomes that breast cancer patients would achieve if provided with the medicine. The only objective was to reduce the price of the medicine with inadequate attention given to defining achievable clinical end points for patients and the measurement of a patient's QoL.

Striking that balance is the purpose of a well-crafted risk-sharing agreement. It is imperative that the local regulatory context be considered and that the lessons learnt from international experiences be applied. If risk-sharing agreements are being promoted as a tool to manage the risk of introducing biological medicines into the healthcare market, what are the policy implications for the payer and for the manufacturer of biological medicines? The current legislative framework contains some policy implications and these are extracted below and applied to the discussion in this paper. These include the Regulations related to the Transparent Pricing of Medicines of the Medicines and Related Substances Control Act[6] and the Medical Schemes Act[7] and its Regulations[9].

Policy implications for the manufacturer

A manufacturer may only sell a medicine at the published single exit price and may not discount the price of the medicine. The single exit price of a medicine is the price set by the manufacturer, inclusive of the distribution fee and value-added tax. A manufacturer is prohibited from supplying a medicine to the payer at a discount to the single exit price for members accessing healthcare services from a specific healthcare provider. A manufacturer may not enter into an agreement with a payer whereby with each additional patient put onto a medicine, the price of the medicine decreases incrementally as the number of patients increases. In addition, a manufacturer is not permitted to pay a payer for the listing of its medicine on a formulary or to provide free services for payers in return for the utilisation of its medicine.[6]

The Regulations related to the Transparent Pricing of Medicines of the Medicines and Related Substances Control Act do not permit a manufacturer to supply any medicine according to an incentive scheme.[6] A medicine may not be supplied to the payer in return for a preferential treatment of the manufacturer's product above other treatment alternatives. Other incentive schemes may include providing payers with subsidised access to journal subscriptions and other medical databases in return for the patronage of the manufacturer's products.[6]

These constraints placed on the manufacturer by legislation aim to avoid the differential pricing of medicine (different prices for different players on the market) and avoid perverse incentive schemes that strengthen the market power of large purchases of medicine. The legislation aims to establish a transparent pricing environment for the supply of medicines to the market.

Policy implications for the payer

There are numerous policy implications for payers wishing to enter into risk-sharing agreements. The Medical Schemes Act and its Regulations address risk sharing between the payer and healthcare providers, for example prescriber networks, hospital services and dispensing groups.[7,8] There is no specific reference to risk-sharing agreements between the manufacturer of biological medicines and the payer. However, if the same principles applicable to the above healthcare providers are adopted into the context of this discussion, the following scenario emerges:

Payers must produce a written protocol for inclusion in the risk-sharing agreement.[7] The written protocol must comprehensively address the processes used to evaluate the clinical need for a medicine and its appropriate use.[9] This includes the method used in the pharmacoeconomic assessment of the medicine. A detailed description of the data collection processes and data used in the pharmacoeconomic assessment is also required.

Payers are required to ensure that risk-sharing agreements enable the sustainable provision of medicine benefits to their patients. The suspension of medicine benefits during the financial year places an undue burden on patients. It also results in the preferential treatment of patients before the suspension in contrast to those denied access to the medicine after the suspension. Instead, appropriate financial planning should be undertaken and included in risk-sharing agreements to avoid the *ad hoc* suspension of these benefits. Such planning may include steps that address each of the following: the measurement of clinical outcomes related to the specific patient population, financial indicators linked to budgetary considerations and indicators associated with the QoL of the patient and its fluctuation over time.

The decision-making processes of payers must enable the consistent and transparent application of their funding decisions.[9] All written

protocols, guidelines, clinical criteria and pharmacoeconomic assessment methods must be made available for inclusion in the risk-sharing agreements. Risk-sharing agreements must be made available to the patients of the payer.

Arriving at a risk-sharing agreement is not as simple as calculating and allocating the apportioned risk to each party. Nor is it about accounting for the direct cost of introducing a biological medicine and ensuring these costs are shared. It also includes the indirect benefits that accrue for the patient and the payer. Without concerted consideration of the policy implications discussed above, it is doubtful whether the manufacturer and payer could agree upon any form of meaningful risk sharing. However, this does not negate the need to forge a viable path for introducing biological medicines into the healthcare market.

This path is one that leads to the outcomes-based reimbursement of biological medicine. It involves a process of reimbursing the manufacturer of biological medicine for achieving defined clinical outcomes and improving the QoL of the patient within agreed financial parameters. It also requires the parties to measure the clinical and QoL outcomes of a patient utilising biological medicines.

Structural necessities

In the first paper it was discussed that two structures comparable to the National Committee for Quality Assurance (NCQA) in the United States of America and the National Institute of Clinical and Health Excellence (NICE) in the United Kingdom are required for the successful implementation of outcomes-based reimbursement.

The precursors for the development of the above two structures already exist in South African legislation. The Regulations related to the Transparent Pricing of Medicines state that the Department of Health may determine that the single exit price of a medicine is unreasonable.[6] In the process of reaching this decision several sources of information would be required, including the comparison of the South African price of the medicine to that in other countries. A robust pharmacoeconomic assessment of the medicine is also needed to determine whether the medicine price is unreasonable. In doing so, the method used for the evaluation would have a significant impact on the results. This necessitates that an accurate economic model be constructed for the assessment of a biological medicine. The sources of epidemiological data and price data files are also crucial in assessing the reasonableness of the medicine price. These principles embedded in legislation bring South Africa one step closer to enabling the establishment of a NICE-type structure.

The Regulations of the Medical Schemes Act provide further support to the above process. They stipulate that healthcare programmes must ensure that their processes document the criteria used in the decision making especially related to the funding of medicines. These must use the best available evidence and also take into consideration the cost-effectiveness of the medicine.[9]

The precursor for the development of an outcomes-measurement tool by payers is absent. There is no mention in the current legislation of an outcomes-measurement database that enables the comparison of one payer with another. There is insufficient incentive for the payer to establish a process of benchmarking with the use of a common set of outcomes-measurement criteria and making this information available to the public. If payers could be compared based on specific standards – quality, effectiveness of care, efficiency, customer satisfaction and others – the results could be used as a competitive tool for those who excel at keeping their patients healthy.

Policy guidelines for risk-sharing agreements

In this paper and the first, risk-sharing agreements have been discussed in the context of some prominent international experiences and the local application. The specific case of trastuzumab and Discovery Health was chosen as a vehicle to discuss risk-sharing agreements and the outcomes-based reimbursement of medicines. The policy guidelines offered herein are derived from each of these sources of information. They aim to be a set of recommendations to be used as a guide when embarking on a journey of exploring the merit of risk-sharing agreements. The guidelines are presented in four sections, each related to a phase of the risk-sharing management process.

Preplanning phase

- A legal framework that supports the enforcement of the risk-sharing agreements must be provided.
- Any legislation that prevents the implementation of risk-sharing agreements should be revised. This may include an audit of pharmaceutical and health-specific legislation detailed in this paper.

Planning phase

- Risk-sharing agreements are not easily transferred from one country to another, particularly if the healthcare structure, the costs of providing healthcare and the epidemiological profile vary from country to country. Critically assess international risk-sharing agreements for local application purposes.
- A written protocol must accompany each risk-sharing agreement, detailing processes used to evaluate the clinical need for and the appropriate use of the medicine. This includes methods and data used to complete pharmacoeconomic assessments.
- All stakeholders must agree to the outcomes measures to be used for monitoring the risk-sharing agreements. Measures must include clinical, QoL and financial parameters and must not be ambiguous.
- All stakeholders must agree as to how failure to comply with the outcomes measures should be calculated. This may serve as the basis for further co-operation and perhaps a refund by the manufacturer for under-performance.
- Appropriate financial planning must be undertaken for each risk-sharing agreement with the intention of ensuring the sustainable provision of treatment.
- Patient groups should be involved from the start. Their role particularly with reference to improving compliance must be explored and clearly stated if outcomes-based reimbursement is to be successful.
- Robust criteria for including and excluding patients in a risk-sharing agreement must be developed.

Implementation phase

- The practical considerations of implementing risk-sharing agreements include establishing a co-ordinating committee with a schedule of regular meetings, allocating additional resources for implementing and monitoring the risk-sharing agreements and drawing up a communication plan that targets prescribers and patients.
- Payers should increase available resources to collect data for postmarketing studies of biological medicines. The responsibility for coordinating and funding these studies is that of the manufacturer. The aim is to continuously monitor the efficacy of the biological medicine after its launch.

Monitoring phase

- A thorough process of monitoring the progress of a patient must be developed. This must include the monitoring of clinical, QoL and financial measures.

- The scientific rigour of measuring outcomes must be thoroughly considered. These measures include comparison of randomised cohorts, power calculations, blinded assessment of outcomes and explicit assumptions associated with all calculations. Here universities and the academic community could play a mediating role to ensure credible results.
- Payers must be encouraged to use data management software that monitors the clinical progress, QoL status and costs incurred for each patient.

Conclusion

Managing the risk of introducing biological medicines into the market presents many challenges for both the manufacturer and the payer. The current legislative environment should be considered for a robust risk-sharing agreement to emerge. This may necessitate some amendments to the current legal environment.

Risk-sharing agreements have been narrowly conceptualised as a financial risk management tool devoid of clinical and QoL outcomes measurement. In this two-part series, both papers have advocated for the outcomes-based reimbursement of biological medicine. Clinical, QoL and financial outcomes measures should be integrated into a risk-sharing agreement. There are numerous approaches, each with peculiar differences that emphasise particular elements. A single and universally applicable model for a risk-sharing agreement is unlikely and will most probably be ineffective. Each risk-sharing agreement must be customised and as far as possible adhere to the policy guidelines offered herein.

A risk-sharing agreement that steers toward the outcomes-based reimbursement of biological medicine is a tool that balances the conflicting interests of the manufacturer and payer. It is also a tool that provides patients with some hope of gaining access to the newest therapies available for their condition. For them it is often an all-or-nothing situation. With risk-sharing agreements in place the situation could change to some access instead of no access. Denying care carries ethical, social and political consequences as evidenced in the case of trastuzumab and Discovery Health. These consequences could be mitigated with risk-sharing agreements.

Declaration

The author is the owner of PharmaLogica, a consulting company active in South Africa and the United States. Its aim is to respond with effective and innovative insight to the needs of the pharmaceutical sector in developing countries. PharmaLogica has worked for non-profit institutions and government agencies and has also consulted on a project basis for pharmaceutical companies. This research was self-initiated by the author and funded by PharmaLogica. The author's views are his own.

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