4th PPRI Conference
Medicines access challenge -
The value of pricing and reimbursement policies

Wednesday, 23 October 2019 -
Thursday, 24 October 2019
Vienna, Austria

ABSTRACT
POSTER
BOOK
The abstract posters included in the abstract poster book were submitted to the conference organisers of the PPRI Conference 2019.

The printed version of the poster book (Version 17 October 2019) may not include all abstract posters that are displayed at the conference.

More information: https://ppri.goeg.at/ppriconference2019

The abstract poster book and the posters will be, upon approval of the authors, available for download after the conference at the conference website.
One third of the total budget went for Antibiotics while they are cheap

Analysing and controlling of Pharmaceutical Expenditures of National Health Insurance Fund, Sudan: Paying for value

Isam Eldin Ahmed
National Health Insurance Fund, Khartoum, Sudan

Background
Inaccessibility to medicines is a common problem worldwide
The Pharmaceutical Expenditures represent 45% out of the total health expenditures of the National Health Insurance Fund (NHIF).
In November 2016 the Central Bank of Sudan has Liberalized the exchange rate.

Objective
To analyze the total cost of pharmaceuticals of NHIF in Sudan to find opportunity for cost reduction

METHODS
A total cost analysis was performed using ABC, VEN and Therapeutic Categories tools. The Pharmaceuticals purchased by the NHIF, Sudan in 2016 were analysed. Outcome measure(s): the percentage of the cost of the ten costly medicines, the cost of the top 20% of the items.

Conclusions and lessons learned:
The main strategies to reduce the cost and improve the use of medicines would be implementation of antimicrobial policy and focus on local manufacturers.
**BIOSIMILAR UPTAKE IN DENMARK – A REVIEW OF SUCCESS**

**BACKGROUND AND OBJECTIVE**

Biosimilar uptake in Denmark has evolved significantly over the last decade. As a procurement body, Amgros has documented its learnings over time. The process has been evaluated and there has been focus on how to ensure successful biosimilar uptake, with each step in the process being considered equally important. The learning curve for the local Danish biosimilar task force has been steep, and the task force has made recommendations on where to focus in the process of introducing biosimilars.

The purpose is to describe the evolution and share recommendations based on the success of biosimilar uptake in Denmark.

**METHODOLOGY**

The abstract is based on a single case study using quantitative register data as well as qualitative data from evaluations in each phase of the process. The abstract focuses on one single therapeutic area with implementation of three biosimilars by looking at market shares on treatment days in different countries for Infliximab and Etanercept, Figure 1A & 1B, and Adalimumab, Figure 2.

**HOW IT WAS DONE**

The biosimilar implementation process (just below) in Denmark is led by a Biosimilar task force. Key learnings are that the partnerships (Figure 3) improve each step in the planning and execution phase throughout the whole process and make a successful set-up for implementation and uptake of biosimilars.

**CONCLUSIONS AND LESSONS LEARNED**

As a procurement body, Amgros developed a set-up where Planning, Dialogue and Involvement (Figure 3) are important to reach the target of successfully implementing a biosimilar in Denmark.

The learnings have involved several elements, both organisational structure and insight sharing as well as on the practical and logistical side after the procurement has been finalized.
Joint procurement pilot with partner countries in the Nordic countries resulted in efficient competition for older pharmaceuticals, with potential future benefits for constrained supply.

**CONCLUSIONS AND LESSONS LEARNED**

- Announced tender criteria were either price alone or price in combination with qualitative criteria.
- One of the tenders included a mandatory bid for all three markets, the rest of the tenders were mandatory for Denmark and Norway with optional submission for Iceland.
- The complexity of including more markets was countered by the supplier options of delivering to more markets.
- Evaluation of the submissions to the pilot showed that a majority of joint tenders had efficient competition with a representative amount of suppliers bidding.
- It took two years from start to announcement of the tender.
- A key conclusion is the need for collaboration with stakeholders and collection of proper insights from involved parties prior to announcement of tender.

**NEXT STEPS**

- A joint evaluation of supply compliance within the tender agreement period will be performed.
- Improvement in supply situation will be tracked during pilot evaluation period.
- Future logistic challenges and strategic solutions to these will be assessed in the early tender planning phase for any future joint procurements.

**QUESTIONS ON JOINT PROCUREMENT PILOT?**

Amgros@amgros.dk
BARRIERS TO ACCESS TO MEDICINES:

- **In rural area**: distance and travel cost to health facility/pharmacy, the amount paid and lack of willingness to pay for medicines; frequently lack of necessary medicines in nearest pharmacy; cost of medicines: quality of purchased medicines; forgetting the way of medicines administration; lack of therapeutic effect after medicine administration.

- **In urban area**: the amount paid and lack of willingness to pay for medicines; frequently lack of necessary medicines in nearest pharmacy; cost of medicines: quality of purchased medicines; lack of therapeutic effect after medicine administration.

ADDRESSING ACCESS BARRIERS TO MEDICINES IN REPUBLIC OF MOLDOVA

Elena CHITAN*, Mihail BRUMAREL*

* "Nicola Teodorescu" State University of Medicine and Pharmacy, Department of "Vasile Procopisin" Social Pharmacy, Chișinău, Republic of Moldova

elena.chitan@usmf.md; chitan.elena@gmail.com

BACKGROUND

- In middle- and low-income countries average availability of medicines is 35%, in public facilities and 66% in the private sector [1]. Cost of medicines and health services remain one of the most tangible households’ expenditures in Republic of Moldova.

OBJECTIVES

- To evaluate barriers in access to medicines in Republic of Moldova (RM) using four access dimensions: geographic accessibility, availability, affordability and acceptability [2].

METHODS

- A quantitative cross-sectional observational study was conducted through a sociological survey, regarding the access of the population of the Republic of Moldova to the medicines. The study was carried out at the national level, in the Republic of Moldova (2018 year), WHO EURO region.

- A sample of 400 people was selected. 302 answers were validated, 45% of respondents was from rural area and 55% from urban area, from which 64% was women and 36% men.

RESULTS

- Majority of population get medical facility by walking – 46% from which 22% are rural and 65% from urban area; going to pharmacy walking was mentioned by 58% of people (76%-urban and 35%- rural), at the same time in rural area is more characteristic use of private transport to arrive at pharmacy - 47% and health facility - 52%. The most expensive travel cost is for rural population, 35% of them spend 30-100 MDL (1€=19.8492 MDL), only 8% of urban population spend the same amount. Majority of people prefer to walk to health facility (58%) and to pharmacy (50%).

- **Affordability**: according to results of study 39% (30% urban and 41% rural) of population pay between 101-500 MDL for medicines and 41% (31% urban and 53% rural) for health services in the last month. People that pay 501-1000 MDL for medicines was 23% (14% urban and 34% rural), for health service only 8.6% (8% urban and 9% rural) mentioned that, to remark that 36.9% of urban population indicated that they don’t pay for health services. Willingness to pay was evaluated using Wilcoxon test, comparing 2 variables: influence of the monthly average amount paid by patient (AAPP) for medicines/health services and willingness to pay for them. AAPP for medicines was 426 MDL, willingness was 292 MDL; AAAP for health service was 327 MDL, willingness was 238 MDL. The value of the asymptotic significance was less than 5% for medicines, thus, have concluded that the amount paid has a strong influence on the amount available for payment, the last one being much smaller. The Wilcoxon test for medical services did not determine an influence - the value of asymptotic significance being >6% (figure 1).

- **Availability**: satisfaction through service in health facility was acceptable for 56% of population, unacceptable only for 7% for pharmacy service satisfaction was good for 34% of consumer; only 4% wasn’t satisfied. 70% of questioned mentioned that they have to wait 1-5 days, from the appointment to doctor’s visit. Majority of respondents, 57% remark about supplementary waiting time to get doctor’s visits from time scheduled, in pharmacy people wait 2-5 minutes to receive counselling by pharmacist, but pharmacist (58% of them) has a neutral attitude to the interests of patients. Only 53% of respondents said that they found needed medicines in pharmacy every time, 47% get them rare, very rare or never.

- **Acceptability**: The main cause that impede purchase of medicines was: cost of medicines in 85% rural and 80% urban population; quality of medicines was in 38% rural and 40% of urban population; remote pharmacy location in 25% rural and 15% urban cases. Weren’t selected as an obstacle to medicines use: quality of health/pharmacy service and health facility location. The answer ”I do not permanently purchase all the necessary medicines” was selected by 92% of rural and 89% of urban population. Factors that restrain medicines use was: forgetting the way of administration in 59% of rural and 30% of urban population; lack of money for a treatment cure 32% of rural and 39% of urban patients.

CONCLUSION

- To address barriers health system should ensure health equity, universal health coverage, provision of essential medicines and health care services, pay for performance and a good regulatory approaches using needs-based financing.

---

**Figure 1.** Four dimensions of the barriers for access to medicines

**Figure 2.** Willingness to pay was using Wilcoxon test for medicines and health services

**Figure 3.** Frequency of the presence of the necessary drugs in the pharmacy

**Figure 4.** Affordability test for medicines and health services
The rising costs of Orphan Drugs in Italy

Enrico Costa1,2, Paola Marini3, Massimo Riccaboni2, Claudio Jommi2
1 - Department of Pharmacy, University Hospital of Verona, Italy
2 - WHO Collaborating Centre for Pharmaceutical Policy and Regulation, Utrecht University, The Netherlands
3 - AXES research unit IMT School for Advanced Studies, Lucca, Italy
4 - Cergas, SDA Bocconi School of Management, Milan, Italy

Background
Orphan Medical Products (OMPs) are drugs intended for the treatment of serious conditions affecting less than 5 in 10,000 people in the EU.

OMPs have brought a huge contribution in many areas that had been orphan in therapeutics for a long time, improving the quality of life and the life-expectancy of patients.

On the other hand, although the ‘orphan’ designation allows applicants to benefit from incentives and conditional marketing authorization by the EMA to sustain their development, the prices of some these are very high and the increasing number of OMPs marketed every year has challenged the sustainability of the pharmaceutical expenditure.

Objectives
This paper aims to give some insights into the Italian Pricing & Reimbursement Policies on OMPs highlighting the strengths and weaknesses of the system.

Methodology:

Data source: Pharmaceutical Expenditure Pricing & Reimbursement policies and procedures Legal framework

Region covered: Italy
Time period: 2017

Conclusion
In Italy the policies on OMPs are largely inclusive: the National Healthcare System allows the access to these drugs even before standard marketing authorization through special pathways.

Orphan drugs are allowed flexibility in the grade of assessment to get the innovative status: a) specific early access programs, b) they were not affected to payback by the pharmaceutical companies, should the drugs budget be overrun.

Incentives provided at EU level, along with the status of innovative granted by the AIFA – even in presence of moderate or low level of evidence - were set up to sustain the survival of OMPs, not to make some of them the new blockbusters.

References

Keywords: Orphan Drugs, rare diseases, affordability

Contact: enrico.costa@aovr.veneto.it

Results
In 2010 the expenditure for OMPs increased from €652 millions in 2010 (3.5% of the whole public Pharmaceutical Expenditure) to €1,599 millions in 2017 (7.2%).

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>OMP expenditure</td>
<td>357</td>
<td>386</td>
<td>371</td>
<td>387</td>
<td>380</td>
<td>383</td>
<td>393</td>
<td>599</td>
</tr>
<tr>
<td>OMPs/DD</td>
<td>6.6</td>
<td>7.5</td>
<td>5.9</td>
<td>7.5</td>
<td>8.5</td>
<td>51.2</td>
<td>11.4</td>
<td>13.7</td>
</tr>
<tr>
<td>OMP</td>
<td>0.53</td>
<td>0.53</td>
<td>0.22</td>
<td>0.22</td>
<td>0.31</td>
<td>0.31</td>
<td>0.04</td>
<td>0.04</td>
</tr>
<tr>
<td>Expenditure in €M</td>
<td>0</td>
<td>100</td>
<td>200</td>
<td>300</td>
<td>400</td>
<td>500</td>
<td>600</td>
<td>700</td>
</tr>
</tbody>
</table>

Some OMPs are ranking within the first 30 top-selling. Drugs for cancer and haematological malignances account for an important proportion of pharmaceutical expenditure for OMPs.

Out of the 99 OMPs authorized by the EMA, 85 were reimbursed by the AIFA. The remainders were either marketed though temporary not-reimbursed or accessible through law 326/2003 (AIFA 5% Fund), which provides the reimbursement of not-yet-marketed OMPs through a fund financed by the 5% of annual expenses for the promotion activities of the pharmaceutical companies. In 2017 the AIFA fund supported the access to 13 OMPs for 40 patients (€13.465.742).

AIFA may grant a medicine the status of innovative drug according to 3 criteria: unmet medical needs, clinical added value and quality of evidence. This allows access to special funds, exemption from payback mechanisms and the immediate availability at local/regional level.

Unlike non-orphan drugs - where high-quality of evidences are required - OMPs may be granted the status of innovative also when the level of evidence is moderate or low.
Price Realignments and Commercial Agreements have resulted in **significant savings to the HSE**, albeit at the expense of transparency of prices.

An Examination and Assessment of the Processes Involved in Setting Reimbursement Prices for Medicines in Ireland

BRADLEY, Declan
Senior Pharmaceutical, Corporate Pharmaceutical Unit, Primary Care Reimbursement Service, Health Services Executive

**Background**

The Irish Health Service faces significant future challenges with growing costs of new medicines, combined with a pipeline of highly expensive medicines. Non-transparent commercial arrangements have helped manage the adoption and funding of expensive medicines. The 2016 IPHA Framework Agreement between the State and Irish Pharmaceutical Healthcare Association (IPHA), was anticipated to achieve significant savings, in part through Schedule 5 which ensures that list prices of all medicines will be realigned, downwards only.

**Objective**

This study examined processes involved in setting reimbursement prices for new medicines whilst determining the financial benefits from having an assessment and commercial negotiation process. The extent to which price realignments over time improved transparency of commercial arrangements and the long-term commercial impact of commercial negotiations at application stage were assessed. This study sought to assess whether or not there are more appropriate or efficient means of setting reimbursement prices for medicines in Ireland, determining if and what the financial benefits of the overall processes are and addressing the benefit of offsetting the transparency of pricing in favour of achieving savings.

**Methods**

A literature review was completed for between 2010, when the first medicine approved for reimbursement with a commercially confidential agreement was applied for and 2018, to collect information on reimbursement processes and the use of commercially confidential agreements across the EU. 25 medicines with commercially confidential agreements were examined. Letters of approval, commercially confidential agreements, price and application forms, rapid review documents, health technology assessment reports and summaries and communications between manufacturers and the CPU were analysed. Annual realignments as per Clause 5.2 of the IPHA Framework Agreement were analysed.

**Results**

From a sample of 25 commercially confidential agreements, commercial discounts ranged between 5% and 60%. Agreements consisted of budget caps, discounts off list prices and tiered discounts. Most agreements included discounts off the list price collected through rebates. Forecasts estimated commercial agreements to last from less than 500 days to almost 3500 days. The majority (72%) of medicines realigned downward in price annually, in 2016, 2017 and 2018. 12% (n=3) of medicines have realigned below their non-transparent commercially agreed price. The average time taken to reimbursement decreased year on year. For the 25 medicines examined, savings resulting from non-transparent commercially confidential arrangements total approximately €50 million to date.

**Conclusions**

Annual realignments and commercial arrangements have proven beneficial to the Irish State with significant savings made. CPU has played an integral role in negotiating confidential agreements with pharmaceutical companies. Transparent pricing would be preferable but is challenging given international reference pricing constraints. The process for setting reimbursement prices in Ireland is robust and this study goes some way to support that. Nevertheless, with significantly greater challenges expected in future, additional measures are required.

---

Contact: Declan Bradley, e-mail: declan.bradley@hse.ie
CPU website: https://www.hse.ie/eng/about/who/cpu/
Innovative policies to achieve sustainable drug prices – a literature review

The objective of this study was to facilitate an evidence-based discourse on innovative policy options to reduce drug prices at market launch. We reviewed the literature to make an inventory of options, analyzed the underlying evidence, and selected promising policies.

Background: Access to medicines is essential to secure people’s right to health. High expenditure on novel anticancer drugs threatens this right and, considering finite resources, the financial sustainability of care. Innovative solutions are needed and highly discussed.

Methodology: We performed a systematic scoping review to identify policy options to reduce drug prices at market launch that are relevant to oncology and high-income countries. We inventoried policy options, categorized publications based on evidence, and analyzed quantitative articles. To select promising options, we identified main price mechanisms, rated policies based on their system disruption and potential price impact. Finally, we asked European experts in the field of oncology and health regulation to rate proposals and challenge our selection of promising policies.

Region covered: We screened globally and selected for the EURO region. Time period: 2001-2019

Results: We screened 4775 articles and selected 80 articles that we used to produce an inventory of policy options in the intellectual property, pricing, and the research & development environment. 22 articles used a quantitative approach but, overall, there was low available evidence. We identified promising options of which experts prioritized transparency and combined purchasing. Two-part-pricing and de-linkage were the most controversial policies.

Conclusions and lessons learned: Although it is important to reform pharmaceutical regulation to secure access to medicines, a coordinated approach to structurally evaluate proposals is lacking. Quantitative methods are rarely used, and current evidence is insufficient to structurally evaluate proposals. We advise testing proposals with small-scale experiments, dynamic simulations, and pilots.

Classification of included articles according to their evidence

<table>
<thead>
<tr>
<th>Evidence</th>
<th>Study type</th>
<th>Intellectual property</th>
<th>Pricing</th>
<th>R&amp;D</th>
</tr>
</thead>
<tbody>
<tr>
<td>Empirical</td>
<td>Policy evaluation</td>
<td>Increase competition</td>
<td>Low</td>
<td>Medium</td>
</tr>
<tr>
<td></td>
<td>Market dynamics evaluation</td>
<td>Earlier generic entry</td>
<td>Low</td>
<td>Medium</td>
</tr>
<tr>
<td></td>
<td>Dynamic, numerical</td>
<td>Increase competition</td>
<td>Medium</td>
<td>High</td>
</tr>
<tr>
<td></td>
<td>Static, numerical</td>
<td>Control prices</td>
<td>Medium</td>
<td>Medium</td>
</tr>
<tr>
<td></td>
<td>Static, abstract</td>
<td>Increase information</td>
<td>Medium</td>
<td>Medium</td>
</tr>
<tr>
<td>Subtotal</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Qualitative</td>
<td>Framework to score policy options</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Systematic review</td>
<td>Reduce prices for subsets of population or drugs</td>
<td>Low</td>
<td>Medium</td>
</tr>
<tr>
<td></td>
<td>Theoretical model</td>
<td>Control prices</td>
<td>Medium</td>
<td>Medium</td>
</tr>
<tr>
<td></td>
<td>Perspective</td>
<td>Increase information to improve competition</td>
<td>Medium</td>
<td>Medium</td>
</tr>
<tr>
<td>Opinon</td>
<td></td>
<td>Reduce R&amp;D costs</td>
<td>Medium</td>
<td>Medium</td>
</tr>
<tr>
<td>Grand total</td>
<td></td>
<td>Increase competition</td>
<td>High</td>
<td>High</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Recoup investments</td>
<td>Medium</td>
<td>Medium</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Reduce granted benefits</td>
<td>High</td>
<td>High</td>
</tr>
</tbody>
</table>

Promising policies to reduce drug prices are: transparency, de-linkage, two-part-pricing, public research, orphan drug reform, and public clinical trials.

There is limited quantitative evidence available. We advise structurally testing policy options with pilots and simulation models.

Questions? N.Franzen@nki.nl
Ludwig Boltzmann Institute for Health Technology Assessment, Vienna, Austria

Monitoring evidence on overall survival benefits of anticancer drugs approved by the EMA

N. Grössmann1,3, M. Robausch1,2, K. Rosian1, C. Wild1, J. Simon3,4

1 Ludwig Boltzmann Institute for Health Technology Assessment, Vienna, Austria
2 Austria and Lower Austrian Sickness Fund, St. Pölten, Austria
3 Department of Health Economics, Center for Public Health, Medical University of Vienna, Vienna, Austria
4 Ludwig Boltzmann Institute for Applied Diagnostics, Vienna, Austria

Background and project aim

The introduction of fast-track licensing strategies increases the approval of anti-cancer drugs with ambiguous benefit-risk profiles [1]. Thus, in many instances there is lacking evidence about overall survival (OS) at the time of marketing authorisation [2-4]. Our objective was to monitor and characterise therapies with ambiguous benefit-risk profiles and identify any post-approval updates on median OS after at least three years of approval by the European Medicines Agency (EMA).

Methods

In our cross-sectional analysis, we included all originator anti-cancer drugs with initially ambiguous benefit-risk profiles that received marketing authorisation by the EMA between Jan 1, 2009 and May 31, 2015. Our monitoring timeframe for the identification of OS updates was at least three years after EMA-approval. To identify study updates, the following three sources were included: clinicaltrials.gov, EPARs, and PubMed, whereby the terms for the systematic literature search were “(name of the active substance) AND (NCT number OR trial name)” with no further restrictions.

Results

In total, we identified 102 eligible approval studies. Out of these, a negative difference in median OS or no information was available in 43 (42.2%) instances. During monitoring, 11 updates with accessible information on median OS could be identified. Including monitoring results, there are still 32 remaining therapies (31.4%) where no or negative information (n=27 [26.5%] and n=5 [4.9%], respectively) regarding median OS is present at least three years after EMA approval.

Conclusion

One-third of oncology drugs with ambiguous benefit-risk profiles fail to demonstrate a survival benefit even after several years of marketing authorisation. Systematic and transparent post-approval monitoring mechanisms will be of high relevance to assure a clinically relevant patient benefit, since the trend towards faster access to medicine with uncertain benefit is increasing rather than declining.

References


nicole.grossmann@hta.lbg.ac.at
LBI-HTA Garmischgasse 7/20, A-1090 Wien http://hta.lbg.ac.at/
The experience of the Tuscan Region in managing biosimilar penetration

Elisa Guidotti *, Bruna Vinci 1-2, Francesco Attanasio 1, Federico Vola 1

1Laboratorio Management e Sanità, Institute of Management and Department EMbeDS, Scuola Superiore Sant’Anna, Pisa, Italy
2SFRO, Scuola Specializzazione Farmacia Ospedaliera, Università di Pisa, Italy
3Drugs and appropriateness policy sector, Tuscan Regional Authority, Florence, Italy

Background

Italy is a leading country in the uptake of biosimilars, with their use been constantly growing; nevertheless, their diffusion is not uniform across Regions. Most Regions have implemented specific policies concerning biosimilar governance to guarantee equity and financial sustainability.

Objective

Some Italian Regions established policies to promote the entry of biosimilars into the therapeutic plans (i.e. Tuscany); others have drawn up late and unfocused policies having a low penetration of biosimilars (i.e. Lazio).

The purpose of this paper is to investigate which governance tools support a high penetration of biosimilars ensuring equity and financial sustainability.

The case of the Tuscany Region has been developed.

Methodology

Regional pharmaceutical administrative flows were analyzed to identify the penetration rate of biosimilars in Tuscany.

Molecules with low penetration and high potential for economic savings were selected and a catalogue of indicators for these molecules realized. An engagement process with managers and specialists of Tuscan Local Health Authorities was started to discuss the indicators and define shared targets of increasing the uptake. The engagement process was soon transformed into regular meetings to monitor the achievements, benchmark against each other and revise objectives.

Results

The panel of indicators on biosimilars, the definition and continuous revision of shared targets and the constant and systematic benchmarking fostered biosimilars penetration over the period 2017-2018 in Tuscany. The percentage of biosimilar molecule Etanercept, for instance, grew from 21,05% to 68,70%, the % Biosimilar Rituximab from 7,1% to 74,64%.

The increase was either better or in line with that of the other Italian regions. The greater usage of biosimilars contributed to the reduction of the pharmaceutical expenditure of the Tuscan Region from € 1.157.044.094 in 2017 to €1.118.523.838 in 2018. However, both an intra and inter-regional avoidable unwarranted geographic variation was observed.

For further information on biosimilar penetration indicators please visit http://performance.sssup.it/netval/start.php or scan the QR code

Please contact me at: elisa.guidotti@santannapisa.it
Medicines prices in Morocco on average decreased since implementation of the 2014 decree on medicines prices

Taking a closer look at the price set-up and evolution of margins: regulating prices should be closely linked to reviewing mark-up schemes and mechanisms

Selected findings

**Current medicines prices in Morocco**

- currently 3,937 medicines commercialised/exported
- more than 600 medicines in price category of 10 to 20 dirham (0.94 to 1.87 Euro)
- price composition differs between price categories and status

**Evolution of medicines prices in Morocco since implementation of Decree**

- price data available before and after implementation of decree for 4,917 medicines (including non-marketed)
- for (still) commercialised medicines prices and margins decreased, on average, for all price levels after implementation of the Decree, wholesale margins decreased, pharmacy margins increased on average

**Table: Evolution of price and margin for commercialised medicines**

| Source: DMP, prepared by authors |

- pharmacy margins reduced rather for high prices medicines; for lower priced medicines pharmacy margins may have increased, as illustrated in following example

**Figure: Paracetamol 500 mg, example evolution of price set-up after implementation of decree (Source: DMP, prepared by authors)**

Full study will also contain qualitative analysis on stakeholders’ views
How the Euripid Collaboration contributes to the affordability of medicines in Europe

Claudia Habl, Gergely Nemeth and Peter Schneider on behalf of the Executive Committee of the Euripid Board of Participants

- EURIPID (www.euripid.eu) is a voluntary collaboration between European countries to run a database with information on national prices of pharmaceuticals in a standardised format.
- Prices of publicly funded medicines are made more transparent via a reliable 24-hour–online database.
- Access only for registered users, currently from 26 countries + EC.

Guiding Principles for External Price Referencing (ERP) of Medicinal Products

1. ERP is an important policy tool that should be used in a mix with other instruments and not as stand-alone policy tool.
2. ERP should take place on single product basis rather than by indices.
3. The aim of the national pharma policy should determine the selection of reference countries.
4. Evidence has shown that ERP is most effective when applied to medicines without generic or therapeutic competition.
5. Comparison of prices of medicines should be done on the 1st price (type) in the distribution chain.
6. Authorities should apply clear and transparent procedures to determine which medicines are considered as comparable.
7. The pricing formula applied should reflect the national objective of ERP.
8. ERP procedures should be performed with the highest accuracy and completeness of data sources.
9. If price information is adjusted to national requirements, it should be done in a transparent and sustainable manner.
10. ERP activities need careful planning → consider it as a policy tool for price revisions and monitoring.
11. The procedures and price inputs to ERP should be as transparent as possible, to ensure predictability and effectiveness.
12. Policy-makers should consider strengthening their cooperation, in particular through the contribution and benefits of existing policies.

Conclusions and Lessons Learned

- The twelve principles are an important step towards a more balanced use of ERP policy and thus a higher acceptance in Europe.
- The Euripid database is aiding countries to perform price comparisons for ERP or price monitoring in a standardised format.
- A stakeholder dialogue platform was founded in April 2019 to allow a continuous information exchange in the area of pricing of medicines in Europe.
- Efforts are made to improve affordability of medicines by better price transparency (e.g., adding information on MEA + volumes → see the recent WHO Transparency Resolution that urges countries to take appropriate measures.

Source: https://jasmin.goeg.at/432/1/EURIPID_GuidanceDocument_V8.1_310718.pdf
National reimbursement policies need to reflect public preferences by engaging the public through deliberative processes.

Integrating public preferences into national reimbursement decisions: A descriptive comparison of approaches in Belgium and New Zealand

Christine Leopold1, Christine Y. Lu1, Anita K. Wagner1

1 Department of Population Medicine, Harvard Medical School and Harvard Pilgrim Health Care Institute, Boston, MA, USA.

INTRODUCTION

1. Increasing pressures to make transparent and sustainable coverage decisions.
2. Need for public engagement in coverage decisions.

OBJECTIVES

Aim is to explore how Belgium and New Zealand used deliberative processes to engage the public to change their public reimbursement system and to identify lessons learned from these countries’ approaches.

METHODS

1. Review of key country documents
2. Semi-structured interviews of 5 key stakeholders
3. Qualitative content analysis

LESSONS LEARNED

1. Need for political commitment to initiate change.
2. Need for broad involvement of all stakeholders.
3. Need for commitment of all to engage in a long-term process.
Predictability and transparency were key for ensuring medicine price stability: based on a cross-government structure, governance and enforcement mechanisms, a technical body to support decision making, contributing to economic growth and access.

18 years of economic regulation of medicines in Brazil: outcomes, challenges and lessons learnt

Adriana M Ivama-Brummell1, Daniella Pingret 1, Rosiene R de Andrade1, J Ricardo Santana1

1 Medicines’ Market Regulation Chamber Executive Secretariat (SCMED)/Brazilian Health Regulatory Agency (Anvisa), Brasilia, Brazil; E-mail: adriana.ivama@anvisa.gov.br

INTRO

- The current health and economic medicines regulatory framework emerged from a deep crisis in the Brazilian pharmaceutical sector with falsified and substandard medicines, shortages, very high prices, among other practices.

OBJECTIVES

To review the implementation of the economic regulatory framework for medicines in Brazil and the adopted regulatory policy options based on WHO recommendations, describing its outcomes, challenges and perspectives

METHODS

- Policy analysis combining descriptive with qualitative analysis;
- Data review from official databanks: such as the Medicines’ Market Monitoring System (SAMMED) and the national public procurement system (Compras-net).
- Region covered: Brazil (PAHO/WHO region).

RESULTS

- There was an increase of new medicines in the Brazilian market: from 2011 to 2017: 230 new medicines entered the Brazilian market. 201 (87%) of them from transnational and 29 (13%) from national companies, with 25 different therapeutic classes.
- In 2017, the revenue of the Brazilian pharmaceutical market was USD 21 billion with 4.4 billion units commercialised (1.4 billion units of generic medicines, 32.4%).
- In 2018, the mandatory discount for public procurement was 20.16% of the maximum prices, leading to important savings.

CONCLUSIONS AND LESSONS LEARNT

- The intersectoral governance mechanism of CMED and the regulatory system allowed for it be consolidated as a State policy, being able to continue through different governments.
- The pharmaceutical sector continued growing, even during austerity periods.
- The challenges include the need of improving the regulatory framework, appraisal process and transparency and find alternatives for high priced medicines with preliminary or poor-quality evidence and without ERP.

<table>
<thead>
<tr>
<th>WHO principles</th>
<th>Implemented policy intervention/outcome in Brazil*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Use a combination of different pharmaceutical pricing policies</td>
<td>Different legal instruments for pharmaceutical pricing policies and regulation, medicines production and innovation, incorporation and procurement established by different laws and policies.</td>
</tr>
<tr>
<td>Transparent pricing policies, processes, and decisions</td>
<td>Rules, criteria for pricing and the decisions taken are standardised and transparent, including authorised prices and public procurement prices public available (electronic systems for price application; procurement prices).</td>
</tr>
<tr>
<td>Appropriate legislative framework, governance and administrative structures, supported by technical capacity, and regularly reviewed, monitored and evaluated</td>
<td>The Law 10.742/2003 set the basis for medicine price regulation and established a governance and administrative structure – the Medicines’ Market Regulatory Chamber (CMED) with representatives from the Ministry of Health (President), the Presidency’s Office (Casa Civil), the Ministry of Economy and Ministry of Justice and Public Security. The decision-making levels are: • the Ministerial Council, the Executive; • Technical Committee (CTE) and • Executive Secretariat (SCMED), at the Brazilian Health Regulatory Agency (Anvisa), a technical body for supporting the decision making, implementing its decisions and monitoring the pharmaceutical market.</td>
</tr>
<tr>
<td>Combination of pharmaceutical policies</td>
<td>The national medicines policy (1998), pharmaceutical services policy (2004) and science and technology in health policy (2005), among others, address both supply and demand issues. These policies were formally approved and implemented.</td>
</tr>
<tr>
<td>Effective implementation of regulation of pharmaceutical prices and ensure compliance</td>
<td>Enforcement mechanisms and monitoring system with enforcement power in place to ensure compliance of the price regulation. Annual adjustment are authorised (not mandatory) based on productivity factor, intra-sectoral factor and inter-sectoral factor (with calculating parameters defined and publicly available, including the Broad Consumer’s Prices National Index (IPCA) (Law 10.742/2003 and additional regulations). Price not reviewed.</td>
</tr>
<tr>
<td>Policies to promote the use of quality assured generic medicines to increase access and affordability.</td>
<td>Generic medicines' policy and legal framework (Law 9.787/1999) with regulations from Anvisa setting requirements for quality, safety, efficacy, prescribing by the international non proprietary name (INN) and generic substitution and pricing rules (Res 02/2004) fully implemented.</td>
</tr>
<tr>
<td>Countries’ collaboration/exchange of information</td>
<td>Brazil is a member of networks of the Americas’ Regional Initiative of competent authorities related to price policies and regulation and the network of Health Technology Assessment of Americas (Redetsa), both supported by PAHO/WHO.</td>
</tr>
</tbody>
</table>
Price regulation, the mandatory minimum discount and a maximum government procurement price (PMVG) have led to great savings, helping to increase the access to medicines. They allowed the procurement of more than twice the volume for 2,3 times the number of patients in 2018, compared to the previous year.

Case study of the judicialisation of eculizumab (Soliris®): challenges in the price regulation and the impact of establishment of the maximum government price in Brazil

Adriana M Ivama-Brummell, Daniella Pingret

INTRO
- In Brazil, access to health, including the access to medicines is a Constitutional right;
- Due to limited budgets, there is "judicialisation" (court cases) to ensure access to medicines in the Unified Health System (SUS);

OBJECTIVES
The objective of this study was to describe and review how the economic regulation has contributed to promote access to medicines for very high-priced medicines in Brazil.

METHODS
- Policy analysis combining a descriptive study with data review from the Medicines’ Market Monitoring System (SAMMED) and the national public procurement system (Compras-net) regarding the procurement of eculizumab (Soliris) from 2010 to 2018, reviewing key results.
- Region covered: National study in Brazil (PAHO/WHO region).

RESULTS
- In 2016, eculizumab (Soliris®), for treatment of paroxysmal nocturnal haemoglobinuria (PNH), a rare disease, costed USD 187 million (R$ 620 million) to the SUS (average unit price: USD 8,347,82, R$ 27,614.60), purchased due to court cases, before marketing authorisation and its incorporation to the health system.
- In 2017, when the eculizumab became regulated by CMED, it had a CAP discount of 19,28% and CMED established the PMVG of USD 3,710,00 (R$ 12,274.83).
- Due to this price difference, in 2018, MoH purchased more than twice the volume (31,056 units for 431 patients) compared to 2017 (13,721 units for 190 patients), based on the recommended daily doses for adults in the main indication (figure 1).

CONCLUSIONS AND LESSONS LEARNED
- External Reference Pricing (ERP) is still a very useful tool for pricing. Therefore, price transparency and cooperation with information sharing among countries is important.
- Despite great savings and increased access to medicines, there are still challenges for the health system in providing very high-priced medicines, with few or no external reference prices.
- The legal provision for setting a provisional maximum price and PMVG "ex oficio" with administrative process and penalties for commercialisation before approval can contribute to tackling very high prices.

Price Regulation
- The Law 10.742/2003 sets the basis for medicines prices regulation and established a governance and administrative structure – the Medicines’ Market Regulatory Chamber (CMED) with representatives from the Ministry of Health (President), the Presidency’s Office (Casa Civil), the Ministry of Economy and Ministry of Justice and Public Security.
- The Medicines’ Market Regulatory Chamber (CMED) regulates medicines’ prices (price cap) since 2003, based on Health Technology Assessment, External Reference Pricing (ERP) and Internal Reference Pricing (IRP).
- In 2006, CMED established the Price Acquisition Coefficient (CAP), a mandatory minimum discount with a maximum government procurement price (PMVG) to a positive list of medicines.
- The Resolution CMED no. 2/2018 established that it is an infringement to offer a medicine without an authorised price by CMED and that CMED will provisionally set the maximum price allowed (ex oficio).
High-cost specialty therapies dominate the new medicine landscape

Insight into the market for new medicines

With rising numbers of new drug market approvals and increasingly specialized therapies in the pipeline, new medicines represent a growing source of cost pressure for payers in Canadian and global markets. In 2018, nearly 8,000 new medicines were in clinical evaluation and pre-registration with the US Food and Drug Administration (FDA), representing 87% of the total pipeline. Over 700 new medicines were in the late stages of development, and a total of 51 medicines were approved internationally in 2018. Of these 51, more than half had an orphan designation, with almost a third being for the treatment of cancer.

Using data from the IQVIA MIDAS® Database and the GlobalData Healthcare Database, this study features pipeline candidates in Phase III clinical trials or pre-registration with the FDA, and analyzes the market entry dynamics of new medicines approved in Canada and internationally in 2018. Pipeline medicines are considered for their potential impact on future clinical development and/or drug spending. Newly approved medicines are identified based on the date of first-time market approval by the FDA, the European Medicines Agency (EMA), and/or Health Canada.

1. A dominant share of new medicines in development are indicated to treat cancer

Many new oncology medicines in the pipeline and on the market have come with orphan designations. This may be the result of the introduction of precision technology, such as biomarkers, which has allowed cancer therapies to become more targeted and disease-specific.

- Oncology treatments dominated the 2018 pipeline, accounting for roughly one third of medicines in all phases of clinical evaluation.
- More than 300 orphan-designated cancer treatments are currently in research and development with over 300 companies.
- Other prominent therapeutic areas included treatments for infectious diseases such as HIV and pneumonia (12% of medicines) and medicines for serious-system diseases such as Alzheimer’s disease and depression (11% of medicines).

2. Medicines for rare diseases continue to represent a significant share of the pipeline

Recent innovation in pharmaceutical technology has shifted the new medicine landscape toward specialty therapies, such as medicines for rare diseases and cancer treatments that target specific genetic deficiencies.

- Orphan medicines accounted for 16% of the total pipeline, and 29% of Phase III clinical trials in 2018.
- This share increased to 40% for the oncology medicines undergoing Phase III clinical trials and pre-registration.
- Of the medicines that reached approval in Canada by Q4-2018, more than half (59%) had received an orphan designation from either the FDA or the EMA.

3. A greater than average number of new medicines were approved in 2017 and 2018, including an increasing share of specialty therapies

In 2018, 51 medicines received first-time approval through the FDA, the EMA, and/or Health Canada. By comparison, 31 new medicines were approved in 2016, and the annual average between 2009 and 2015 was 36.

- Over half (50) of the 2018 new medicines received an orphan designation from the FDA or EMA, explaining the rising share observed over recent years.
- Almost one third (15) of new medicines were approved for cancer indications, seven of which were orphan-designated oncology treatments.

4. Sales of 2018 new medicines were concentrated in four therapeutic areas, with one HIV treatment making up over half of the total revenues

Although new medicines launched in Canada and the PMPRB® in 2018 covered a wide range of therapeutic areas, their sales were highly concentrated.

- 10 of the 51 medicines, representing the top four therapeutic classes, accounted for over 95% of all 2018 new medicine sales and the PMPRB® by Q4-2018.
- One new treatment achieved blockbuster status within less than a year of market entry: Biktarvy, an orphan-designated medicine indicated to treat HIV, was approved in the US in February 2018, and Europe and Canada the following July.
- Despite demonstrating slight or no improvement over existing therapies, as assessed by the PMPRB’s Human Drug Advisory Panel, Biktarvy accounted for 32.5% of the total sales for all new medicines in Canada and the PMPRB® by Q4-2018.

Limitation: Canadian and international sales and list prices available in the IQVIA MIDAS® Database are estimated manufacturer-factory-gate list prices and do not reflect off-invoice price rebates and allowances, managed entry agreements, or patient access schemes.

Disclaimer: Although this information is based on part data obtained under license from IQVIA MIDAS® Database and the GlobalData Healthcare database, the statements, findings, conclusions, views, and opinions expressed in this study are exclusively those of the PMPRB and are not attributable to IQVIA or GlobalData.

This analysis was undertaken by the Institut de recherche sur les politiques de la santé (IRPS), which operates independently of the regulatory activities of the PMPRB.
The PMPRB now has the tools and information needed to meaningfully protect Canadian consumers from excessive prices today and into the future.

Patented Medicine Prices Review Board Framework Modernization

Canada has recently amended its Patented Medicines Regulations, bringing significant enhancements to its regulatory price regime, and providing the Patented Medicine Prices Review Board (PMPRB) with the tools and information it needs to protect Canadians from excessive medicine prices today and into the future.

As the regulator responsible for giving effect to the amendments on July 1, 2020, the PMPRB is consulting its stakeholders and the public on new pricing Guidelines, to ensure they are fair, functionally sound, and rationally connected to the nature and scope of the regulatory changes.

The current regulatory pricing framework provides the PMPRB with dated tools to fulfill its consumer protection mandate. In essence, it simply relies on internal and external price referencing that includes premium priced comparator countries, most notably the United States. The framework also regulates prices at list-price level, which are not reflective of confidential rebates negotiated by manufacturers and payers. In today’s environment, medicines are often priced for value, a factor not in the PMPRB toolbox, and some may create affordability challenges for consumers given their large market size. The existing framework poses a very real threat to the sustainability of the pharmaceutical system in Canada, which was the impetus for the regulatory changes.

The regulatory amendments update the PMPRB’s framework to a risk-based approach that includes (1) a new schedule of countries, (2) additional price regulatory factors, and (3) patentee information reporting requirements, as described below.

1. An updated schedule of comparator countries

The new framework includes countries with similar consumer protection priorities, economic wealth, and marketed medicines as Canada. The basket of comparator countries now also includes Australia, Belgium, Japan, Netherlands, Norway, and Spain, while the premium priced countries, such as the United States and Switzerland, were removed from the list.

<table>
<thead>
<tr>
<th>Previous comparator countries: PMPRB7</th>
<th>Foreign-to-Canadian price ratio*</th>
<th>New comparator countries: PMPRB11</th>
</tr>
</thead>
<tbody>
<tr>
<td>France</td>
<td>0.76</td>
<td>France</td>
</tr>
<tr>
<td>Germany</td>
<td>0.97</td>
<td>Germany</td>
</tr>
<tr>
<td>Italy</td>
<td>0.85</td>
<td>Italy</td>
</tr>
<tr>
<td>United Kingdom</td>
<td>0.83</td>
<td>United Kingdom</td>
</tr>
<tr>
<td>Sweden</td>
<td>0.86</td>
<td>Sweden</td>
</tr>
<tr>
<td>Switzerland</td>
<td>1.25</td>
<td>Australia</td>
</tr>
<tr>
<td></td>
<td>0.79</td>
<td>Belgium</td>
</tr>
<tr>
<td></td>
<td>0.92</td>
<td>Japan</td>
</tr>
<tr>
<td></td>
<td>0.89</td>
<td>Netherlands</td>
</tr>
<tr>
<td>United States</td>
<td>3.21</td>
<td>Norway</td>
</tr>
<tr>
<td></td>
<td>0.80</td>
<td>Spain</td>
</tr>
</tbody>
</table>

* Average foreign-to-Canadian price ratios are calculated based on patented medicines prices reported in IQVIA’s MIDAS database. Note that Canadian and international prices available in MIDAS are estimated manufacturer list gate list prices, and do not reflect off-invoice price rebates or managed entry agreements.


2. Additional price regulatory factors

The new regulatory framework adds new factors that the PMPRB must consider when determining whether the price of a patented medicine is excessive, and now includes the medicine’s value to and financial impact on consumers in the health system. The addition of the new factors allows the PMPRB to assess the economic impact of a patented medicine’s price, enabling it to develop screening criteria for medicines that are likely to pose affordability challenges. The amendments bring Canada in line with other countries that are adopting newer methods of evaluating prices by looking at the cost of the medicine relative to its health benefits and the impact reimbursement has on overall health system expenditure.

Pharmacoeconomic value
A measure of how much a medicine costs for the health benefit it provides

Market size
Takes into account the impact of paying for the medicine for everyone who needs it

GDP and GDP per capita
These are indicators of the overall societal and individual wealth in Canada

3. Changes in reporting requirements

The new framework requires the actual price obtained by the patentee to be reported to the PMPRB, taking into account any adjustments. This includes reporting the confidential rebates and discounts that manufacturers negotiate in confidence with payers and do not disclose publicly. Requiring patentees to provide this information will facilitate compliance with the new, lower price ceilings that are expected to result from the new guidelines.
When less means more: Insight into spending on Expensive Drugs for Rare Diseases

An increasing number of expensive drugs (EDRDs) have emerged in recent years, bringing hope to patients suffering from life-threatening or debilitating conditions. Using sales data from IQVIA’s MIDAS® Database, this analysis provides insight into Canadian and international EDRD markets, with information on pricing, sales, and market shares.

EDRDs are the fastest growing market segment in Canada, with 30% average annual increases in sales and a steady influx of specialty medicines. In 2014 and 2017 alone, 21 oncology and non-oncology EDRDs were approved in Canada. Based on the profile of the drug pipeline, this trend is expected to continue, with EDRDs becoming an increasingly significant driver of future pharmaceutical spending.

For the purpose of this study, EDRDs are defined as medicines with at least one orphan designation and estimated treatment costs exceeding $100,000 per year and $7,500 per 28 days for oncology drugs.

### 1. An increasing number of EDRDs are being introduced, fueling this fast-growing market segment

Over the past several years there has been a substantial increase in the number of EDRDs and the spending on them. The top growth in EDRD prescriptions is six times higher than the national growth in prescriptions for all prescription medicines in Canada — highlighting the growing importance of these drugs. The Canadian experience mirrors a wider global trend, with the 30% compound annual growth rate (CAGR) for EDRDs in Canada almost identical to the median OECD rate over the past seven years.

- Canadian sales of EDRDs reached $1.8B in 2018, representing close to a 5-fold increase from $0.4B in 2012.
- Oncology drugs represented a substantial portion of these medicines, accounting for close to 80% of EDRD sales over the past seven years.
- In 2018, the Canadian EDRD market share of total pharmaceutical sales reached 7%, 3.7 times greater than in 2012.

### 2. Affordability of drugs and sustainability of healthcare budgets are global issues

Payers globally are struggling to fund the increasing numbers of exorbitantly high-priced treatments. The cost of specialty drugs for cancer and rare diseases is threatening the sustainability of publicly funded healthcare systems.

The variation in pricing among countries suggests that international price referencing alone cannot address budget sustainability for all prescription medicines in Canada – highlighting the growing importance of these drugs. The Canadian experience mirrors a wider global trend, with the 30% compound annual growth rate (CAGR) for EDRDs in Canada almost identical to the median OECD rate over the past seven years.

- In 2018, Canadians spent $50 per capita on EDRDs, marginally higher than the OECD median of $44.
- Prices of EDRDs in Canada were among the highest in the OECD, ranking 88th place; however, median foreign-to-Canadian price levels were only 6%, lower than Canadian levels and 12 countries had prices that were within 10% of Canadian levels.

### 3. EDRDs have the potential to generate the same revenues as lower-cost high-volume drugs

Manufacturers argue that the relatively small market for EDRDs necessitates higher prices to recoup R&D costs and fund new developments. However, this analysis reveals that the revenue generating potential of EDRDs is comparable to that of high-volume medications.

The analysis focuses on EDRDs and non-EDRD medicines launched in Canada since 2005. The results are based on the highest annual sales attained for each medicine within three years of their launch date. Figure 3 illustrates the median sales of medicines with annual sales over $10M, by increasing sales bands, as well as the corresponding share of sales revenue.

- 54% of EDRDs have more than $10M in annual sales within the first three years after introduction, compared to 40% of non-EDRDs, which indicates that EDRDs have a higher probability of reaching larger sales revenues.
- Medicines with annual sales greater than $10M account for 94% of the total sales for both EDRD and non-EDRD medicines.
- While a greater percentage of EDRD medicines have sales over $10M, non-EDRDs have higher average annual sales, $34M compared to $28M.
- The median sales values for both EDRDs and non-EDRDs are approximately $10M, with EDRDs having slightly higher median sales.
- Non-EDRDs can be examined in more detail by breaking them down into groups of higher-cost medicines (with annual treatment costs at $10,000) and lower-cost medicines (with annual treatment costs < $10,000). The results show that in the first three years, only 4% of the lower-cost medicines have annual revenues exceeding $10M — 10% less than for EDRDs — while 61% of the higher-cost medicines have annual revenues exceeding $10M — 7% less than for EDRDs.

A 2016 study by Hughes and Poletti-Hughes found that EDRDs are five times more profitable than non-orphan drugs; in addition, according to our analysis, they can also achieve a comparable level of sales by the third year after launch.

### The Canadian experience echoes the message highlighted by the 2018 World Health Organization Technical Report on the Pricing of Cancer Medicines and its Impacts:

**Figure 1: Canadian sales of EDRDs, 2012–2018**

![Chart showing Canadian sales of EDRDs from 2012 to 2018](image1)

**Figure 2: EDRD sales share, sales per capita, and price comparison, OECD countries**

![Table showing sales share, sales per capita, and price comparison for OECD countries](image2)

**Figure 3: EDRD and non-EDRD distribution of medicines and of sales, by the highest annual sales in the first three years after launch in Canada**

![Chart showing EDRD and non-EDRD distribution of medicines and sales](image3)

Note: This analysis was undertaken by the NPHRI’s research initiative, which operates independently of the regulatory activities of the PMPRB.


Note: Results are based on list prices and do not capture off-invoice price rebates, managed entry agreements, or patient access schemes.

Data sources: MIDAS® Database, IQVIA. All rights reserved.

For the Canadian market, MIDAS® data was supplemented with data captured by the PMPRB and IQVIA Private Pay Direct Drug Plan databases to ensure that all EDRDs authorized for sale in Canada were accurately identified.

Disclaimer: Although this information is based in part on data obtained under license from IQVIA MIDAS® Database and Private Pay Direct Drug Plan databases, the statements, findings, conclusions, views, and opinions expressed in this study are exclusively those of the PMPRB and are not attributable to IQVIA.

This analysis was undertaken by the NPHRI’s research initiative, which operates independently of the regulatory activities of the PMPRB.

**Medicines for orphan diseases, despite smaller patient populations, have the commercial potential to generate revenue for the original companies at least as great as for non-orphan medicines**

---

**Canadian health care system faces challenges in funding rare disease therapies**

- The Affordable Care Act of 2010 included provisions to address the challenges of funding rare disease therapies.
- The 21st Century Cures Act, signed into law in 2016, established a new pathway for the approval of drugs for rare diseases, including orphan drugs.
- The National Institutes of Health (NIH) Rare Diseases Clinical Research Network (RDCRN) was established to facilitate research on rare diseases and to improve patient care.
- The Orphan Drug Act of 1983, which provides incentives for the development of orphan drugs, has been amended several times to expand the definition of rare diseases and to increase the incentives available to companies.

Despite small patient populations, EDRDs are a rapidly growing market, gaining sizable sales through high prices.
Well known processes from other industries have improved drug supply for hospital pharmacies

**Good practice to improve supply of hospital drugs and prevent backorders (tax funded, public sector)**

**Background and objective:**
After years with an increasing number of backorders and many unplanned drug changes implemented under time pressure in the hospitals, we decided in 2017 to replace working in “firefighting mode” with being proactive through better supply chain transparency between hospital pharmacies and suppliers.

- The aim was to improve the supply of drugs to hospitalised patients in Denmark and to reduce the increasing number of backorder from suppliers

**Methodology**
We established a national Sales & Operations Planning (S&OP) unit to develop and implement a national S&OP process for drugs on national tenders:

- All hospital pharmacies estimate their expected purchase volume (number of packages) on each item-number (Arrow 1 – on figure below)
- National estimates for each item-number are shared on a national supplier web portal. Suppliers are also advised about new estimates by e-mail (Arrow 2)
- On the national supplier web portal the suppliers confirm drug supply capability (Arrow 3a) or report potential supply problems (Arrow 3b)
- The national S&OP unit initiates and identifies proactive solutions for potential supply problems (Arrow 3b)
- Suppliers share production lead-time to give better understanding of their capabilities, e.g. to support planning of drug changes and decision making
- Monthly review process in place: Hospital pharmacies/clinical pharmacy update their estimates for changes in drug use (Arrows 4, 5 and 6)
- Suppliers re-confirm their supply capabilities, and national solutions are made for any identified supply problems (Arrows 1, 2, 3a and 3b)

**The transparent Supply Chain:**

1. Estimates from each hospital pharmacy
2. National estimate shared with suppliers
3a. Confirmation of supply capability according to national estimate OR 3b. Early info on potential supply problems -> solutions identified
4. Significant deviations between estimate and actual purchases
5. Questions to Clinical Pharmacy about changes in drug consumption
6. Input to updated estimates

**How it was done**
I. **Involving** hospital pharmacies and suppliers in the step-by-step development of the S&OP process. **Helping** hospital pharmacies with estimation procedure
II. **Help** to identify estimates that needed revision through quantitative and qualitative models, to improve estimate accuracy and supply security
III. **Active communication** of estimates and revised estimates to suppliers
IV. **Rebuilding suppliers’ trust** in our estimates as accuracy improved
V. **Asking suppliers to confirm supply capability and report potential supply problems proactively**
VI. **Open and cross-functional communication** about possible solutions to potential supply problems

**Conclusions and lessons learned**
- It’s hard work to implement a new focus area with many stakeholders, but be patient, maintain focus and results will show
- Positive feedback from both hospital pharmacies and suppliers on the benefits of participating in the national S&OP process. **A win-win situation.**
- Number of backorders have stabilised during 2017-2018 whilst other countries have experienced a sharp increase
- Transparency across the supply chain has generated trust and enabled more value adding and cross-functional dialogue e.g. sharing causes for estimate changes, supporting suppliers to get a reliable volume allocated to Denmark and early sharing of knowledge about potential supply problems
- Proactive solutions for potential supply problems have improved the overall supply situation, and have improved patient safety as fewer unplanned drug changes are implemented under time pressure

**Next steps**
- Continue to improve the S&OP process and tools by involvement, support, active communication, trust and cross-functional communication
- Increased focus on education of clinical pharmacy staff and physicians, for them to give early warnings to hospital pharmacies prior to changes in drug use
- Help hospital pharmacies to predict changes in drug use, based on impact from national decisions about drug selection in the therapeutic areas
The Italian Region **Emilia-Romagna** implements an **evidence-based drug governance policy** involving multi-stakeholder workgroups to promote equitable and sustainable access to drugs.

**Drug Governance in the Emilia-Romagna Region, Italy**

Francesco Nonino, Maria Chiara Silvani, Roberta Gioldini, Elisabetta Pasi, Lucia Magnano, Giulio Formoso1, Anna Maria Marata

Direzione Generale Cura della Persona Salute e Welfare, Servizio Assistenza Territoriale, Regione Emilia-Romagna, Bologna (Italy )

WHO Collaborating Centre in Evidence-Based Research Synthesis and Guideline Development.

1 AUSL di Reggio Emilia, Reggio Emilia (Italy)

**Background**

- The Italian National Health Service provides assessment, pricing and reimbursement of medicines through the Italian Medicines Agency (AIFA).
- However, each Italian Region can implement its own tailored drug governance policy within the national reimbursement regulation.

**Methods**

1. In the region Emilia-Romagna (RER) evidence-based recommendations on the use of medicines are issued by multi-stakeholder workgroups (MSWG), informing the decisions of a Regional Drug and Therapeutic Committee (DTC) that monthly updates the Regional Drug Formulary (RDF).
2. Recommendations produced by means of the GRADE method are monitored through quantitative indicators expressing the expected prescription rates. **Yearly reports** are produced for conditions with high impact on resources.
3. Drugs are purchased through centralized procurement procedures by a public independent regional agency.
4. Cost-opportunity evaluations to foster competition among pharmaceutical companies are part of RER’s drug governance policy.

**Results**

- **1,242** drugs included in the Regional Drug Formulary
- **255** documents on drugs issued by the DTC since 2006
- **79** with evidence-based recommendations and quantitative expected prescription rates
- **62** produced with the GRADE methodology
- **12** active workgroups

**Discussion**

- RER implements a drug governance policy based on evidence-informed, structured, explicit and flexible guidance process involving MSWGs.
- Differences between observed and expected prescription rates help understanding the determinants of variability among prescribers and can inform decisions about resource allocation.
- Appropriate use of drugs is key for the sustainability of a reimbursement-based system, warranting equitable access to treatments.

A full list of guidance documents in Italian is available at: [http://salute.regione.emilia-romagna.it/documentazione/1/index-guide-e-recomandazioni-etr](http://salute.regione.emilia-romagna.it/documentazione/1/index-guide-e-recomandazioni-etr)
A drug governance policy incorporating cost-opportunity in evidence-based recommendations produced with the GRADE method

Is cost-opportunity an effective strategy for drug expenditure governance? The experience on oncology drugs of the Emilia-Romagna Region, Italy

Lucia Magnano, Francesco Nonino, Roberta Giroldini, Elisabetta Pasi, Maria Chiara Silvani, Anna Maria Marata

Direzione Generale Cura della Persona Salute e Welfare, Servizio Assistenza Territoriale - Area Farmaco e Dispositivi Medici, Regione Emilia Romagna, Bologna, Italy - WHO Collaborating Centre in Evidence-Based Research Synthesis and Guideline Development

Background
- High cost oncology drugs challenge the sustainability of healthcare systems.
- The Emilia-Romagna Region (RER) implements a drug governance policy by producing evidence-based recommendations and monitoring them through quantitative indicators.

Methods
- The GReFO (Gruppo Regionale Farmaci Oncologici) is a RER multi-stakeholder oncology workgroup producing guidance by means of the GRADE method [1].
- Although formal cost-effectiveness analysis is not performed, if drugs of the same class show no difference in terms of efficacy and safety, cost-opportunity (prescribing the least expensive drug) is recommended and prescription rates are formally monitored.
- The aim is to optimize the use of financial resources while warranting appropriate and equitable use of medicines, and to foster competition among drug companies.
- We describe the financial impact of implementing such policy to the first-line treatment of advanced stage melanoma (ASM).
- Expected melanoma cases and expected prescription figures were based on the Italian Association of Cancer Registries (AIRTUM) data and extrapolated from epidemiological studies.

Results
- In 2017, licensed monotherapies for wild-type patients with ASM were nivolumab (Nivo), pembrolizumab (Pembro) and ipilimumab.
- Patients with the BRAF-V600 mutation (BRAF+) were eligible also to anti-BRAF/anti-MEK associations (BMAs).
- Recommendations with the same strength and direction were issued by GReFO for Nivo and Pembro in wild-type (strong positive) and in BRAF+ (weak positive) patients.
- According to cost-opportunity issues, GReFO recommended, within the immunotherapy class, the least expensive drug (Nivo) in BRAF+ patients.
- Considered for analysis: a sample of 154 ASM patients (70% of the total) undergoing immunotherapy in 2018.
- 76% and 24% of ASM patients were treated with Nivo and Pembro, respectively. The overall expenditure was €5,826,509 (rough figure, without considering the median duration of treatment).
- Compared with a hypothetical treatment of 50% of patients with each drug, adherence to cost-opportunity recommendation produced an estimated saving of 5% on the observed overall expenditure.
- Considering an adjusted cost/patient/year estimate, the savings my have been up to 11%.

Conclusions
- An evidence-based drug governance policy involving multiple stakeholders and sharing context-specific issues is feasible in a public healthcare system.
- Incorporating cost-opportunity issues in the production of evidence-based recommendations may result in substantial savings
Consider ways to enable ex-post analysis and evaluation of effective prices when Managed Entry Agreements terminate

Ex-post analysis of medicines subject to Managed-Entry-Agreements (MEAs) – a feasible approach for monitoring and price analyses

Research in the field of medicine prices requires decisions on the methods applied in the study.

The decisions on certain approaches are often determined by the study purpose, objectives and perspective, but the main goal is to make meaningful comparisons.

Aim: To assess which information competent authorities, researchers and stakeholder in the field of pharmaceutical pricing need when they conduct price analyses.

Survey on information needs to conduct meaningful analyses/comparisons

A needs assessment survey has been conducted among competent authorities and stakeholders in the field of pharmaceutical policy.

The questionnaires contained 30 items and was structured in five overall topics.

The questionnaire was distributed to 90 persons from 56 national and European institutions and associations.

The survey was completed by 24 institutions (15 competent authorities for pricing and reimbursement, 9 international organisations, European associations of affected stakeholders and experts on pricing and reimbursement).

MEA hamper meaningful analyses and comparison at any point in time

Respondents emphasised the importance of making meaningful comparisons/evaluation of medicine prices.

The more information available, the more meaningful is the analysis.

Respondents identified information about the existence of Managed Entry Agreements (MEAs) and the type of MEAs as a supportive piece of information.

The practice of MEA has disrupted the informational value of prices and and shattered established methods into pieces.

Current legal requirements do not allow monitoring or evaluation by third parties at any point in time.

The Valletta group (Cyprus, Greece, Ireland, Italy, Malta, Romania, Portugal, Slovenia & Croatia) also pressed in 2019 for more transparency of prices of pharmaceuticals.


Gesundheit Österreich GmbH, Stubenring 6, 1010 Wien www.goeg.at
Price developments of biological medicines do not correspond to estimated price levels

Estimating price developments of biological medicines during market exclusivity
Peter SCHNEIDER, Lena Lepuschütz, Nina Zimmermann, Sabine Vogler

- A medicine passes through several different stages which is known as a ‘product life cycle’
- Each stage is embedded in a regulatory and policy environment, which determines price dynamics
- **Aim:** To estimate price developments of biological medicines during the stage of market exclusivity and compare these results with list prices of biosimilars prior to the entry of the first biosimilar

Survey results were used as inputs for a statistical model

- Primary data collection on the use and practice of EPR, including detailed methodological information – among 30 European countries
- Results were used as inputs to model price developments through a discrete-event-simulation (DES)
- The model ran over a 10 year time horizon
- Pharma Price Information (PPI) service provided list prices of two biological medicines (Adalimumab and Rituximab) in the months before the first biosimilar entered the market

The estimated average price was higher than the average of actual list prices

- The model predicted that after ten years, the average price level over the 30 countries was 80.2% of the starting price
- In the model, the highest price countries were Austria, Belgium, Luxembourg and Switzerland, while lower prices were predicted in Spain, Romania and Croatia
- In comparison to the model’s estimations, the average price level of list prices was 66.8%
- The countries with the highest price level were Germany, Switzerland and Poland, while lowest prices were observed in UK, France and Greece

**Source:** Vogler et al (2015) Study on enhanced cross-country coordination in the area of pharmaceutical product pricing

**Source:** https://amgros.dk/media/2165/udbudsslangen_web_2_uk_hvid_bakgrund.jpg

**Source:** https://ppri.goeg.at
Substantial steps have been taken to improve the mechanism of developing the List of Reimbursement Outpatient Medicines, but further efforts will be need to be undertaken to achieve long-lasting changes in the area of transparency, relevance of decisions, revisability and implementation.

Mechanism for introduction of outpatient medicines in the reimbursement list in the Republic of Moldova: development and challenges

CONTEXT
In the context of global commitments to ensure extensive access to safe, effective, quality and affordable medicines, the assessment identifies barriers and factors that facilitate access to reimbursed medicines in the Republic of Moldova.

OBJECTIVES
Objectives: The operational research of the national regulatory framework on developing the list of reimbursed outpatient medicines (LROM) by the mandatory health insurance funds aimed at identifying deficiencies and designing solutions for ensuring a transparent, holistic and feasible mechanism.

METHODS
1. Analysis of the regulatory framework for outpatients medicines to be included in the list of reimbursed outpatient medicines (LROM).
2. Qualitative research of the opinions and perceptions of the beneficiaries of medicines and actors of the system.

RESULTS
Mandatory health insurance implemented in the Republic of Moldova has shown to be an effective tool for improving the population’s access to medicines. The LROM has evolved from 5 INN in 2005 to 148 INN in 2019. Public expenditures for LROM increased from 7403.5 thousand Moldavian lei (MDL) in 2005 to 523 859.3 thousand MDL in 2017. At the same time, the LROM did not significantly change compare to the national list of essential medicines. The first regulation on mechanism for introduction of outpatient medicines in the LROM was approved in 2010 and was revised fundamentally two times, with the most recent revision being done in 2015. The regulation was improved evidently, however, it is in need of further revision to: (1) improve transparency in establishing priorities for reimbursement; (2) re-introduce mandatory the cost–effectiveness criteria and budget impact analysis; (3) develop guidelines to enhance coherence and justifications of the process; (4) involve multidisciplinary expert teams. Qualitative research highlighted that access to LROM is perceived differently by different categories of population and actors of the system.

Pathway of the application for approval of medicines for reimbursement by the Mandatory Health Insurance in Republic of Moldova

Application of the company
Annually, until the July 1st

Evaluation stage: Secretariat of the Health Insurance Company evaluates the dossier, including evidences on pharmaco-therapeutical & pharmacoeconomic, assesses the clinic benefit. Develops the technical evaluation report and presents to the Consilium.

Evaluation in 180 days (+32 days)

Appraisal Stage: Consilium of the MoHLSP assesses the dossier, technical evaluation report, evaluation score provided by the Secretariat and experts’ opinion to take decision on inclusion/exclusion medicine from reimbursement

Collaboration with WG of experts, MDMD, USMF etc.

Approval stage: Consilium issues a Decision on inclusion/exclusion on introduction medicine in the reimbursement system, signed by the Minister.

The deadline – until the end of the fiscal year.

MoHLSP and HMIC signs the order on inclusion of medicines in the reimbursement list.

Take a picture to download the full paper
EML in Moldova is outdated. Public procurements show low share of EML out of LMCP. This is a lost opportunity to ensure access and value for money and compliance with WHO EML.

Assessing access to essential medicines list (EML) in the Republic of Moldova

Rita Seicas*, Ghenadie Turcanu, Stela Bivol, Angela Carp

CONTEXT
While Moldova has adopted policies on essential medicine list (EML), implementation has never been systematically reviewed. The PAS Center conducted a study on access to essential medicines.

METHODS
1. Analysis of national legislative and regulatory framework on essential medicines against international practices.
2. Analysis of alignment of the national EML (NEML) to WHO EML and reflection in the list of medicines of centralized public procurement (LMCPP).

RESULTS
The first NEML approved in 1996 was revised four times, last one in 2011, which reveals that the number of medicines has expanded considerably. Comparative analysis of the NEML (635 molecules) with 2017 WHO EML reveals that 337 molecules are common to both lists, 152 molecules of WHO EML missing in 2011 NEML and 263 molecules of NEML not part of WHO EML.

<table>
<thead>
<tr>
<th>Year of NEML approval</th>
<th>Total number of molecules (excluding duplicates)</th>
<th>Total number of molecules (including duplicates)</th>
<th>Total number of pharmaceutical forms</th>
<th>Rate of pharmaceutical form per molecule</th>
<th>Total number of therapeutic categories</th>
</tr>
</thead>
<tbody>
<tr>
<td>1996</td>
<td>106</td>
<td>108</td>
<td>147</td>
<td>1.36</td>
<td>0</td>
</tr>
<tr>
<td>2007</td>
<td>475</td>
<td>504</td>
<td>718</td>
<td>1.42</td>
<td>29</td>
</tr>
<tr>
<td>2009</td>
<td>519</td>
<td>578</td>
<td>819</td>
<td>1.41</td>
<td>27</td>
</tr>
<tr>
<td>2011</td>
<td>576</td>
<td>635</td>
<td>856</td>
<td>1.34</td>
<td>29</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Year</th>
<th>2015</th>
<th>2016</th>
<th>2017</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>47.01%</td>
<td>49.58%</td>
<td>51.61%</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Year</th>
<th>2015</th>
<th>2016</th>
<th>2017</th>
</tr>
</thead>
<tbody>
<tr>
<td>a. Total number of INNs in the procurement list:</td>
<td>n/date</td>
<td>723 (100%)</td>
<td>603 (100%)</td>
</tr>
<tr>
<td>b. The total proportion of EMs/INNs in the procurement list:</td>
<td>n/date</td>
<td>43.43% (314)</td>
<td>49.92% (301)</td>
</tr>
<tr>
<td>c. Total procured INNs/EMs without offers</td>
<td>n/date</td>
<td>5.10% (16)</td>
<td>3.99% (12)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Year</th>
<th>2015</th>
<th>2016</th>
<th>2017</th>
</tr>
</thead>
<tbody>
<tr>
<td>585</td>
<td>83.13% (601)</td>
<td>92.87% (560)</td>
<td></td>
</tr>
<tr>
<td>585</td>
<td>16.87% (122)</td>
<td>7.13% (43)</td>
<td></td>
</tr>
<tr>
<td>275</td>
<td>94.90% (298)</td>
<td>96.01% (289)</td>
<td></td>
</tr>
<tr>
<td>275</td>
<td>5.10% (16)</td>
<td>3.99% (12)</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Year</th>
<th>2015</th>
<th>2016</th>
<th>2017</th>
</tr>
</thead>
<tbody>
<tr>
<td>585</td>
<td>601</td>
<td>560</td>
<td></td>
</tr>
<tr>
<td>275</td>
<td>298</td>
<td>289</td>
<td></td>
</tr>
<tr>
<td>47.01%</td>
<td>49.58%</td>
<td>51.61%</td>
<td></td>
</tr>
</tbody>
</table>
A comprehensive policy framework conceived as a practical instrument to analyse and evaluate pharmaceutical systems, identify functional gaps, and choose reform interventions fitting the specific local needs and capacities.

**CONCLUSION**

The framework proposes a general approach that to be applied across low, middle and high-income settings. It helps decision-makers and technical staff analyse and envisage how the pharmaceutical system could be improved given the local context data availability and human capacity.

---

**THE FRAMEWORK – A STEP BY STEP APPROACH:**

I. Evaluate if the six mandatory functions of the system are present

> **Optimal**: all functions should be present, even if multiple institutions fulfil them

II. When all functions are present, a very well defined sequence between the functions is needed to ensure optimal decision efficiency, starting from regulatory and ending with the monitoring and feedback function

III. To ensure optimal system operations, each function should have a specific set of data and tools used, with the output generated by one function used as input by the next one

*The flow of information should be organised as a continuous process
Feedback should be continuously available on volumes used, epidemiology, mortality and morbidity drivers, uptake of the new treatment, efficacy in real world settings, costs etc.*

---

**METHODS**

- Multi-year, mixed methods work, across public & private sectors encompassing:
  - a) Desk review of policies, HTA assessments and qualitative interviews in 72 countries
  - b) Identified commonalities of high income country systems
  - c) Results adjusted for middle-income settings (Eastern Europe)
  - d) Framework validated in Sub-Saharan Africa and South East Asia

**COUNTRY EXPERIENCES**

- The framework has been recently used in Indonesia, Philippines and Togo
  - In Indonesia, it identified the main drivers behind the persistent out of pocket spending despite the newly introduced social health insurance
  - In Philippines, the framework was used to create and integrate the HTA unit within Department of Health and develop the Primary Care Benefit package
  - In Togo, the framework helped develop a sustainable formulary and adjusted pricing method for the public health insurance

---

**REFERENCE**

Ioana Ursu, Viktoria Rabovskaia

1) Mapping Health, London, UK
2) GIZ GmbH, Eichborn, Germany

---

Contact: i.ursu@mappinghealth.org
Different methodological approaches of external price referencing lead to different medicine prices.

**Background**
- External price referencing (EPR) is a frequently used medicine pricing policy.
- It aims to lead to more affordable (lower) prices.
- Different dimensions are to be taken into consideration when designing an EPR system, e.g.
  - Basket of reference countries
  - Calculation of the benchmark price
  - Exchange rate
  - Weighting price data of reference countries

**Objective**
- To investigate the impact of changes in the EPR methodology on medicine prices (list prices)

**Methods**

<table>
<thead>
<tr>
<th>Parameter</th>
<th>Methodological approaches</th>
</tr>
</thead>
<tbody>
<tr>
<td>Model</td>
<td>Discrete-event simulation (DES)</td>
</tr>
<tr>
<td>Price data</td>
<td>Fictitious prices</td>
</tr>
<tr>
<td>Countries included</td>
<td>All 28 EU Member States except Denmark, Sweden and UK; plus Iceland, Norway and Switzerland</td>
</tr>
<tr>
<td>Time period</td>
<td>Period of 120 months, starting Q1 / 2015</td>
</tr>
<tr>
<td>Base case</td>
<td>&quot;Real-life setting&quot;; Dimensions of EPR in the countries as in place in 2015, as surveyed</td>
</tr>
<tr>
<td>Simulations</td>
<td>Different scenarios were simulated</td>
</tr>
<tr>
<td>Assumptions</td>
<td>Prices were held constant until a re-evaluation was due according to legislation</td>
</tr>
</tbody>
</table>

**Findings**

**Base case** (continuation of 2015 methodology):
- -21.9% after 10 years

**Simulations with highest impacts**:
- Consideration of discounts (assumed 20% discount in 6 large economies and mandatory discounts in DE, EL & IE); -47.2%
- Calculation based on lowest price in ref. countries; -34.2%

**Simulations with mixed impacts**:
- Adjusting price data to PPP; -16%

**Simulations with further impacts**:
- Regular price revisions
- Changes in the basket of reference countries
- Shorter intervals of the average exchange rates

**Conclusions**
- The methodological design of EPR can result in (partially substantial) changes of the price
- Savings for payers through strategic choices

**Acknowledgements go to**
- Lena Lepuschütz, GÖG for her collaboration in the study & PPRI network members for providing data for the base case.
- This analysis was part of a larger study funded by the Health Programme of the European Union (see QR code), we acknowledge the funding of the European Commission.

Contact details: sabine.vogler@goeg.at
For on-patent medicines one presentation per active ingredient can be sufficient for a price comparison.

P18: Choosing the right medicines for price comparisons
Analysis of prices of pharmaceutical presentations of the same active ingredient

Background
- Selecting medicines for international price comparison is a major challenge
- Is it sufficient to select one single pharmaceutical presentation to represent the active ingredient or should all presentations of an active ingredient be included?

Objective
- To analyse the prices of different pharmaceutical presentations of the same active ingredient in European countries
- With a view to assessing possible differences between them

Methods

<table>
<thead>
<tr>
<th>Parameter</th>
<th>Methodological approaches</th>
</tr>
</thead>
<tbody>
<tr>
<td>Medicines selected</td>
<td>22 active ingredients (at least 1 presentation per active ingredient among high-cost medicines for Austrian public payers in Q2/2017); at least 2 presentations per active ingredient studied</td>
</tr>
<tr>
<td>Countries</td>
<td>27 countries (all 28 EU Member States except Malta)</td>
</tr>
<tr>
<td>Data source</td>
<td>Pharma Price Information (PPPI) service of GÖG</td>
</tr>
<tr>
<td>Survey date</td>
<td>September 2017</td>
</tr>
<tr>
<td>Analysis</td>
<td>ex-factory prices (list prices, before discounts) per unit (e.g. tablet, vial)</td>
</tr>
</tbody>
</table>

Findings

Same prices of different presentations of an active ingredient
- For 18 of the 22 studied active ingredients, the per unit ex-factory prices were the same for the surveyed pairs of the pharmaceutical presentations in several countries
- The relative ranking of unit prices across the European countries did not differ considerably between presentations of the same active ingredient

A different pattern was found in cases
- Of the marketing of different presentations for different indications (denosumab) and
- Of emerging generic competition, which also impacted originator prices (rosuvastatin)

Conclusions and lessons learned

- The findings suggest that for on-patent medicines the inclusion of a single presentation per active ingredient in a price comparison can be sufficient, since prices or ranking of those do not differ substantially.
- As soon as generic competition starts, however, price dynamics will likely occur, and it is recommended including further pharmaceutical presentations of an active ingredient in a price study.

Funding
- This is a follow-up analysis of a medicine price study performed for the Austrian Federal Ministry of Labour, Social Affairs, Health and Consumer Protection (BMASGK), see QR code.
- The Pharma Price Information (PPPI) service, from which medicine price data were sourced, is financially supported by BMASGK.

Sabine Vogler, Peter Schneider
Gesundheit Österreich GmbH (GÖG), Stubenring 6, 1010 Vienna, www.goeg.at & https://ppri.goeg.at
The design of pricing and reimbursement policies varies greatly across PPRI member countries. For conclusions on access to medicines further research is needed.

Comparative analysis of Pharmaceutical pricing and reimbursement policies in 47 PPRI member countries

Nina Zimmermann, Sabine Vogler, Margit Gombocz

WHO Collaborating Centre for Pharmaceutical Pricing and Reimbursement Policies, Pharmacoeconomics Department, Gesundheit Österreich GmbH (GOG / Austrian National Public Health Institute), Vienna, Austria

Background
- Knowledge of appropriate measures, including methodological aspects and practice experience, in other countries as well as evidence of their impacts are of major importance for policy-makers.

Objective
- To offer a comprehensive, concise and up-to-date comparative analysis of pharmaceutical pricing and reimbursement policies implemented in the outpatient and inpatient sectors in the 47 member countries of the PPRI network.

Methodology
- Primarily through primary surveys of competent authorities in 47 PPRI member countries.
- Additional sources were used (e.g. WHO, OECD, etc.)

Price regulation in almost all PPRI member countries

- Price control at the ex-factory (or sometimes wholesale) price level in almost all PPRI member countries.
- Mostly targeting reimbursable medicines or prescription-only medicines.
- External price referencing is applied in 41 countries using different methodologies:
  - Country baskets ranges from 1 (Luxemburg) to 39 countries (Kazakhstan).
  - 18 of the EPR-applying PPRI countries employ an average or a median benchmark price, whereas 9 countries relate to the lowest price of the reference countries.
- 32 PPRI countries apply a so-called generic price link (i.e. the generic price is set at a defined percentage of the originator price), and 23 countries use this policy for biosimilar medicines.
- Only one country with a full-fledged value-based pricing system: Sweden.
- 37 apply HTA, or elements of HTA including pharmacoeconomic instruments in their pricing and/or reimbursement decisions, thereof 18 countries (e.g. Germany, France, Norway, UK) in a systematic way (with assessment and appraisal processes having been implemented).
- 32 countries with regulated wholesale remuneration and 43 countries with regulated pharmacy remuneration.
- 23 countries apply tendering as the predominant procurement method for medicines in hospitals.
- Managed-entry agreements (MEA) were reported from 33 PPRI network member countries. Financially-based MEA are more commonly used than performance-based MEA.

In the majority of the PPRI countries a large share of pharmaceutical expenditure is covered by public payers

- 46 PPRI member countries have one or more reimbursement lists for outpatient medicines in place; of those 41 apply solely positive lists (i.e. explicitly indicating those medicines that are included in reimbursement).
- Key criteria for inclusion in coverage scheme: The (added) therapeutic benefit of a medicine, medical need, financial considerations such as budget impact and the cost-effectiveness.
- 32 PPRI countries apply a reference price system (RPS), which defines the same reimbursement amount for similar or identical medicines in a cluster which are established at ATC level.
- 43 PPRI countries have prescribing by international non-proprietary name in place and 43 countries implemented generic substitution. Biosimilar substitution, however, is only in place in 15 countries.
- At least 43 PPRI countries apply co-payments for outpatient reimbursable medicines, in the form of a prescription fee (20 countries), a percentage co-payment of the price of the medicine (30 countries) or a deductible (9 countries; some countries have more than one co-payment in place).
- All of these PPRI countries apply exemptions from or reductions of co-payments for defined population groups.

Further research on impact on access to medicines needed

Since the implementation of pricing and reimbursement policies is in the national competence of governments, policies used vary greatly with regard to their aims, design and enforcement. For identifying best-practice policies with regard to facilitating affordable and equitable access to essential and cost-effective medicines further research is needed. This policy review offers descriptive information as basis for further research.

Nina Zimmermann (nina.zimmermann@goeg.at), Gesundheit Österreich GmbH / Austrian National Public Health Institute, Stubenring 6, 1010 Vienna, Austria

https://ppri.goeg.at