Improving Forecasting of Pharmaceutical Spending - Insights from 23 OECD and EU Countries

Analytical Report

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Acknowledgements

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Note by Turkey: The information in this document with reference to "Cyprus" relates to the southern part of the Island. There is no single authority representing both Turkish and Greek Cypriot people on the Island. Turkey recognises the Turkish Republic of Northern Cyprus (TRNC). Until a lasting and equitable solution is found within the context of the United Nations, Turkey shall preserve its position concerning the "Cyprus issue".

Note by all the European Union Member States of the OECD and the European Union: The Republic of Cyprus is recognised by all members of the United Nations with the exception of Turkey. The information in this document relates to the area under the effective control of the Government of the Republic of Cyprus.

The analytical report and country notes will be launched in April 2019.

The report and relevant work under this project, part of the EU agenda for effective, accessible and resilient health systems, are available here:

https://ec.europa.eu/health/policies/costeffective_medicines_en

http://www.oecd.org/els/health-systems/routinely-collected-data-to-inform-pharmaceutical-policies.htm

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Executive Summary

Pharmaceutical expenditure accounts for a variable share of current health expenditure in OECD and EU countries, ranging from 7% (reported by Denmark and Norway) to 41% in Bulgaria. Recent trends in pharmaceutical markets have raised concerns about the sustainability of pharmaceutical spending, as well as interest for spending projections.

Short-term projections of pharmaceutical expenditures can be used to support the determination of needed resources, the setting of budgets, or in the context of a hard budget constraint, to estimate the available 'headroom' for the addition of new medicines to a national formulary. According to a survey of OECD and EU countries launched in spring 2018, 17 countries (out of 22 respondents) realise such projections. Almost all of them draw on past spending trends for these estimates. Ten countries reported taking into account potential new entrants; the budget impact of newly covered medicines; and/or changes in uptake of generics and biosimilars. Only seven reported taking into account 'demand- side factors', such as demographic trends, burden of disease and changes in prescribing or treatment patterns in their models.

Focusing on supply-side factors for short-term projections is consistent with what is observed in the literature. While long-term projections mainly focus on demand-side factors (e.g. demography and epidemiology), short-term ones tend to focus on supply. In addition, taking into account past trends (especially if utilisation data is available), is a good proxy for short-term changes in demand, except in therapeutic classes where innovative medicines address previously unmet medical needs.

Although spending projections are widely used, the literature review revealed (or confirmed) that short-term projections of pharmaceutical expenditures are not straightforward. Projections are not only difficult to undertake but may also be poorly predictive, even when using the most sophisticated models. Our analysis of the literature and of country practices, however, enabled us to draw the following conclusions for improved practices in forecasting.

Expenditure projections requires information on past trends, ideally at product level, in order to develop projections based on assumptions about market dynamics (new products, products going off patent, etc.). Yet, information on pharmaceutical expenditure and utilisation is available at product level in only half of the responding countries and most often only for reimbursed medicines dispensed by community pharmacies. In a few countries, however, expenditure tracking at product level extends to all medicines dispensed in hospitals and other settings (e.g. Belgium, Czech Republic, Italy, Portugal, and Sweden).

A foundational element of short-term projections is effective *horizon scanning*, to identify late stage products in global industry pipelines. In 2018, seven countries reported using the results of horizon scanning as an input to their expenditure projections: Australia, Belgium, Cyprus, Czech Republic, France, Netherlands and Sweden. Among respondents to the survey, the Netherlands, Norway and Sweden appear to have very well functioning horizon scanning systems, available to inform pharmaceutical expenditure projections.

Horizon scanning, however, is a resource intensive activity for which most countries cannot allocate substantial resources. The BeNeLuxA initiative aims to augment the effectiveness of individual country efforts through cooperation and collaboration, not only by aggregating sparse resources but also by reducing unnecessary duplication. Similarly, the EC's proposal on joint EU-wide HTA also includes a provision for cooperative horizon scanning (European Commission, 2018[1]). Yet, while industry pipelines are essentially global, the systematic monitoring and collation of country-specific data on *timing of market entry*, and in particular, the time from first marketing authorisation (MA) to market launch in country, and from MA to reimbursement or coverage, are important inputs. These parameters should be carefully monitored in individual countries.

Anticipating the (comparative) *therapeutic value* and likely *price* of a product yet to enter the market will remain very challenging. Using maximum willingness to pay (WTP) in countries that have determined fixed or even floating WTP thresholds could provide a nominal proxy for the upper bound of the expected price, depending on assumptions on the comparative therapeutic value of the product. However, this is a complex undertaking that would carry a number of inherent risks including that of overestimating unit costs, or indeed, including projected costs for products for which marketing approval or funding may not eventuate.

Perceived or anticipated therapeutic value of a new product is also likely to influence the various drivers of *uptake and diffusion*: the *place in therapy* (first or later line therapy); whether the therapy is likely to be additive or to displace older treatments, or whether it extends treatment to a previously untreated patient cohort. These are in turn influenced by the target population to be treated - requiring a knowledge of the underlying epidemiology and *burden of disease*; the indications likely to be approved for marketing, and those likely to be accepted for coverage or reimbursement. All these elements can be part of the horizon scanning process.

Determining the timing of *loss of exclusivity* (LoE) of a product, as a proxy for the timing of generic or biosimilar market entry, is essential to modelling the impact of generic and biosimilar competition. As noted previously, this requires not only access to multiple data sources, but also specialised skills in understanding the data obtained. Comprehensive public databases could improve access to the necessary intelligence both for generics/biosimilar manufacturers and analysts, but would not obviate the need for specific expertise in its interpretation. An alternative could be for countries/payers to require companies to provide comprehensive information on all forms of applicable IP protection as part of their applications for coverage/reimbursement. Although this information is a core element of companies' strategies, it is public in nature and sometimes disclosed in companies financial reports to investors, at least for the largest markets.

Data on past trends in *generic uptake* and their impact on markets (both volumes and prices) are useful to predict future effects of generic market entry, but less so for biosimilars. Biosimilar data remain sparse, not only because of the shorter history and smaller number of 'follow-on' products, but also because the uptake, acceptance and pricing effects of biosimilars appear to be more idiosyncratic, and seemingly dependent on drug class and location of use (i.e. hospital vs community), as well as on national/payer policies on pricing and substitution. Similarly, data on past trends in generic/biosimilar uptake may not be very informative in therapeutic classes where really innovative products are entering the market at a high pace.

The study did not investigate how countries cope with the development of confidential discounts and rebates, including product-specific ones. A few studies in the literature tried to address that challenges and to establish projections *net of rebates*. Country practices to take these rebates into account are now known. However, discounts and rebates should ideally be factored into the estimations, and therefore known by services in charges of expenditure forecasting.

For effective short-term projections, many model parameters that cannot be populated empirically must inevitably be driven by assumptions, thus highlighting the need for testing multiple scenarios and performing extensive multivariate sensitivity analyses. Although sophisticated sensitivity analyses have been used in the literature, their use by national institutions does not seem to be common.

Repeated comparison of actual trends to projected estimates is important for adjusting assumptions and improving both the confidence in, and the predictive value of these heavily parameter driven models, particularly if they are to be used to estimate the potential effects of proposals for policy reforms or to set budgets. This would also inform trade-offs between resource intensity and forecasting precision. According to our survey, only seven countries currently realise ex-post assessment of their predictions (Australia, Czech Republic, France, Ireland, Netherlands, Portugal, and Sweden).

The publication of available projections is also a good practice in terms of transparency, which is increasingly requested by a number of stakeholders. Among respondents to our survey, six countries publish their projections: Australia, Estonia, Ireland, Lithuania, the Netherlands and Sweden. The US administration also publishes its projections. Sweden distinguishes itself with a very transparent process for establishing projections, with formal consultations of stakeholders groups.

The survey also explored country practices in terms of setting budgets and pharmaceutical expenditure caps (beyond which pharmaceutical companies or other stakeholders are requested to pay rebates to public payers). Thirteen countries out of 22 respondents set pharmaceutical budgets and ten set expenditures caps at the macro-economic level. Past trends in pharmaceutical spending and overall budget constraints are the elements the most often taken into account when setting budgets and caps, followed by potential generic/biosimilar entry. Six countries reported considering potential entry of new products, their prices and recent market entry and coverage determinations of new medicines when setting budgets (Australia, Belgium, Cyprus, Ireland, Malta and Sweden). Although collected data does not allow establishing a direct link between projections and budget or cap setting, many elements are commonly required for both of them. It is reasonable to think that projections actually inform budgets or caps where they exist.

This might raise concerns that inaccurate projections could lead to restricted access to medicines. This, however, would only happen in systems where the budget is strict, rather than "notional", i.e. allowing the budget to be overspent in case of unanticipated demand.

Introduction

1.	Pharmaceutical expenditure accounts for a variable share of current health expenditures across OECD and EU countries, ranging from 7% (reported by Denmark of Norway) to 41% (in Bulgaria) in 2016.[1] The advent of the direct acting anti-virals (DAAs) for hepatitis C, the increasing use of high cost biologics, and the escalating launch prices of oncology medicines in particular, have raised concerns that pharmaceutical expenditure growth will become increasingly difficult to predict and may become challenging to sustain.

2.	In order to ensure adequate resource mobilisation, and to anticipate and manage the entry of major new therapies many countries see value in trying to anticipate changes in market dynamics and by doing so, attempt to forecast future expenditure.

3.	As part of the OECD's programme of work addressing the challenges of access to medicines, supported by the European Commission, the Secretariat undertook a study to explore potential approaches to projecting pharmaceutical expenditure and the feasibility of enhancing country capacity to better anticipate the impacts of changes in market dynamics, by:

- exploring countries' existing capacity for, and approaches to tracking pharmaceutical expenditure and anticipating changes in market dynamics, and understanding how these inform pharmaceutical and health policy decision-making;

- identifying those methods or approaches demonstrated to be most efficient and effective in anticipating changes in market dynamics;

- determining key drivers of pharmaceutical expenditure trends, both from the demand side (e.g. demography, epidemiology) and the supply side (e.g. entry of new products, patent expiries);

- identifying key information and data needed for effective monitoring of market dynamics and assessing the potential impact of different policy levers on these; and

- assessing potential gaps in information and data collection systems and proposing ways to address them at national or international level.

4.	The first section of this report maps OECD and EU member countries' current practices in tracking pharmaceutical expenditure and utilisation, and in setting budgets and spending caps, based on an online country survey conducted in April–May 2018. The second section presents a review of the literature on determinants of pharmaceutical spending and approaches to forecasting, and assesses the limitations of the studies. The last section of the report presents findings and recommended practices for countries currently undertaking, or planning to introduce pharmaceutical expenditure projections to inform future policy making.

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[1] Data extracted from OECD and Eurostat databases in April 2019, for 2016. In accordance with accounting principles set out in the System of health accounts, this share only includes pharmaceuticals dispensed by pharmacies to outpatients and does not include medicines administered in physician's settings or in hospitals.

1. Monitoring, budgeting, and forecasting pharmaceutical expenditure – Mapping of current practices in OECD and EU countries

5.   In the spring of 2018 the OECD launched a survey to collect information on country practices in pharmaceutical expenditure tracking and projections, and on budget and/or expenditure cap setting. Twenty-two of 40 countries[2] responded. The sections below build on responses to this survey to map countries' reported practices and describe country experiences with medicines expenditures and utilisation tracking (section 1.1; setting budgets or caps on pharmaceutical expenditure (section 1.2); and with modelling and forecasting future pharmaceutical spending (section 1.3).[3]

6.   This report does not purport to present the practices of all OECD and EU Member Countries, since only just over half of them responded to the survey. However, it does shed some light on existing approaches to the monitoring, control and prediction of pharmaceutical expenditures.

1.1. Country experiences with medicines expenditure and/or utilisation tracking

7.   As part of the System of Health Accounts (SHA) annual data collection, most OECD and EU countries provide information on 'retail pharmaceutical expenditures', and a few countries also report information on pharmaceutical expenditures in hospital settings.[4] This means that most countries are able to estimate, once a year, how much has been spent on medicines, by all stakeholders (governments, insurers, patients) for medicines dispensed in retail pharmacies or other outlets, prescribed or not. Fewer countries are able to estimate how much has been spent for medicines in hospitals, physicians settings or other institutional settings, mainly because payments for medicines are included in provider payments and bundled with other services provided.

8.   A survey launched in 2018 by the OECD sought to identify country practices in monitoring and disaggregating pharmaceutical expenditures. With the exception of Austria, all respondents reported tracking medicines expenditure at national or subnational level.

9.   Among the countries tracking expenditure, only Malta and Norway (for inpatient medicines) reported that they did not disaggregate these expenditures into sub-categories.[5] Countries mostly collect data through reimbursement claims (from patients

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[2]   The questionnaire was circulated to all Member Countries of the OECD and/or of the European Union, except Bulgaria, where no expert was identified. The list of respondents can be found in table 1.1.

[3]   Tables B1 to B5 Annex B present the information as originally reported by countries in the survey. Small inconsistencies between original responses and results summarised in these sections may be due to supplementary information provided by countries on request.

[4]   See (OECD, Eurostat and WHO, 2017[32]) for definitions of terms and guidelines for reporting.

[5]   In Norway, expenditure and utilisation for inpatient medicines are collected at a detailed level by hospital pharmacies and made available, for example, to hospitals and the Directorate

or pharmacies) and many are able to disaggregate expenditure by region (14 countries); by therapeutic area or ATC-level (15 countries); at the level of the active ingredient (ATC[6]-level 5 for 12 countries); or at the level of the individual product (12 countries). Data were reported as having been tracked for periods ranging from 5 years (Luxembourg) to 26 years (Australia), and are made publicly available in 14 countries.

10.   Most countries tracking expenditures and/or utilisation at product level do so only for medicines dispensed by retail/community pharmacies (See Table 1.1). Belgium, Italy, Norway, Portugal, and Sweden are able to track expenditure and/or utilisation for hospitalised patients. Nine countries reported making information derived from expenditure or utilisation tracking publicly available to researchers or the general public, either under the form of raw data (e.g. France) or in the form of analytical reports on trends (e.g. Belgium, Italy, Norway, Portugal).

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of Health. The Norwegian Ministry of Health does not track these expenditures for budgeting purposes (although the hospitals/Regional Health Authorities do).

[6]   The Anatomical Therapeutic Chemical (ATC) Classification System is used for the classification of active ingredients in medicines, according to the organ or system on which they act and their therapeutic, pharmacological and chemical properties. It is developed and maintained by World Health Organization Collaborating Centre for Drug Statistics Methodology (See https://www.whocc.no/atc/structure _and_principles/) and used by many (but not all) countries.

Table 1.1. Pharmaceutical expenditure and utilisation tracking at product level in OECD/EU countries responding to the survey

	Medicines partially or totally funded by main coverage scheme				Medicines not funded by main coverage schemes		Info available*
	Medicines dispensed in retail pharmacies	Medicines dispensed by hospital pharmacies to outpatients	Medicines dispensed/administered in hospitals to inpatients paid for separately	Medicines dispensed/administered in hospitals to inpatients financed through hospital regular budget	Medicines prescribed	OTC medicines	
Australia							✓
Austria							
Belgium							✓
Cyprus[7]							
Czech Republic							
Estonia							
Finland							
France							✓
Ireland							✓
Italy							
Korea							
Japan							
Latvia							
Lithuania							✓
Luxembourg							✓
Malta							
Netherlands							✓
Norway			(a)				✓
Poland							
Portugal							✓
Sweden							✓
Switzerland							

Note: Dark blue: Expenditure **and** utilisation tracking at product level. Light blue: Expenditure tracking only at product level. *Information is publicly available, at product or ATC 5 level, either in the form of raw open data, on request, or in analytical reports. (a) This category does not exist in the country
Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

1.2. Country practices in budget setting and expenditure caps

11.   Countries may determine budgets for public health expenditures annually, especially where these expenditures are directly funded by national or sub-national governments. These budgets may be defined by sub-categories of care, which may or may not include a specific "budget line" for expenditures associated with medicines. Some countries do not define such budgets *per se*, but instead establish caps on pharmaceutical spending, beyond which companies are required to rebate part or all of the excess expenditure. The difference between "budgets" and "expenditure caps" is

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subtle, and the terms are often used interchangeably—though in reality they are quite different—and may even co-exist in some countries. Box 1.1 provides the definitions used in the survey to assist countries in preparing their responses.

Box 1.1. Budgets and expenditure caps - Definitions

A budget for pharmaceuticals refers to a <u>budget allocation</u> for the purpose of public expenditure on pharmaceuticals by a national or a sub-national government, as part of the budgetary process. This budget may include all public spending related to pharmaceuticals, or only some categories of medicines (e.g. only medicines purchased in community pharmacies, or only high-cost medicines). In fact, some countries may budget separately different categories of medicines. This budget may or may not be directly managed by the level of government setting it.

Example 1: In New Zealand, since 2000, the Ministry of Health set a "notional pharmaceutical budget" for the public funding of pharmaceuticals. The so-called 'Combined Pharmaceutical Budget (CPB)' included subsidies for medicines dispensed by community pharmacies and for some medical devices, vaccines, haemophilia treatments, nicotine replacement therapy and cancer medicines that are sometimes given in hospitals. It did not (at that time)[8] include hospital medicines and devices, which were funded from local authorities' (District Health Boards) hospital budgets. https://www.pharmac.govt.nz/about/your-guide-to-pharmac/factsheet-08-managing-combined-pharmaceutical-budget/

A cap on pharmaceutical spending refers to an <u>upper limit</u> on spending (or spending growth) beyond which, for example, pharmaceutical companies may be required to pay rebates to public payers. <u>Please note that this does not refer to the setting of a ceiling within a budgetary process.</u>

In the United Kingdom, the 2014 Pharmaceutical Price Regulation Scheme signed by pharmaceutical companies and the government set a limit on growth in the overall cost of branded medicines purchased by the NHS from companies that are members of the scheme. Beyond this limit, companies were required to pay a rebate to the NHS. The allowed growth rate for 2018 was 1.9% (Section 6 of the PPRS and Annex 3 from page 69) https://www.gov.uk/government/publications/pharmaceutical-price-regulation-scheme-2014

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12.   Thirteen of the 22 responding countries reported setting budgets for public pharmaceutical expenditure, most frequently with the objective of controlling spending (see Table 1.2). The factors the most often taken into account when setting budgets were: past trends in pharmaceutical spending (12 countries), overall fiscal constraints (11 countries), prices of new medicines (10 countries), potential entry and take up of generics and biosimilars (9 countries), and anticipated market entry of new medicines and recent market and coverage determination of new medicines (8 countries each). Demand-side factors (demographic changes, epidemiology and treatment guidelines) were only considered by 6 countries.

13.   Ten of the 22 responding countries reported defining a cap on total pharmaceutical expenditures (see Table 1.2). With the exception of Belgium, where the pharmaceutical industry is involved in cap negotiations, central budget authorities and Ministries are responsible for determining the spending cap in all countries. The criteria most often taken into account in defining the cap were overall fiscal constraints (8 countries) and past trends in pharmaceutical spending (7 countries), followed by potential generic/biosimilar entry and uptake (6 countries). Criteria related to

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[8] More recently the CPB has been expanded to include hospital medicines and medical devices.

demographic and epidemiologic trends (3 countries) were used less frequently to determine caps on pharmaceutical spending.

14.   Eight of the 22 responding countries, all European, reported setting *both* a budget for public pharmaceutical expenditures and a cap of pharmaceutical spending, beyond which companies may be required to pay ex-post rebates (see Table 1.2).

15.   The survey responses did not distinguish whether pharmaceutical budgets were strict or "notional", i.e. could be overspent in case of unanticipated demand.

Table 1.2. OECD and EU countries with a budget and/or a cap on pharmaceutical spending according to the 2018 Survey

Responding countries reporting setting a budget or a cap for
public pharmaceutical expenditures

	Cap	No Cap
Budget	Belgium, Cyprus, Estonia, Ireland, Italy, Latvia, Malta, Poland	Australia, Lithuania, Netherlands, Norway (outpatient), Sweden
No budget	Czech Republic, France	Austria, Finland, Korea, Japan, Luxembourg, Portugal, Switzerland,

Note: The table only reflects information on "macro-economic caps" on pharmaceutical expenditure. Countries may have caps on specific products or classes of products even when they have no macro-economic cap.
Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

1.3. Pharmaceutical expenditure forecasting in OECD and EU countries, survey results

16.   Seventeen countries reported forecasting pharmaceutical spending in the short term (1 to 5 years). Italy, Poland, Korea, and Switzerland do not forecast pharmaceutical expenditures. Poland and Korea indicated that such projections are not useful or needed. Italy is developing a methodology and performs ad hoc projections and Switzerland may develop forecasts in the near future. Nine countries reported that their pharmaceutical expenditure projections are developed as stand-alone exercises and eight that they are embedded in broader forecasting models (see Table 1.3). No respondent reported undertaking medium or long term forecasting (> 5 years), although Australia periodically undertakes long term modelling as part of a broader exercise assessing the long-term sustainability of Government policies and how changes to Australia's population size and age profile may impact economic growth, workforce and public finances over the succeeding 40 years (see Section 2.2 regarding *Mixed Models*).

Table 1.3. OECD and EU countries reporting projections of pharmaceutical expenditures in the 2018 survey, according to the type of projection

	Embedded in overall spending projections	Stand-alone exercise
Covering pharmaceutical spending as a whole	**Estonia,** France, **Ireland**, Japan, Latvia, Norway	Austria, Malta, **Netherlands**, Portugal, **Sweden**
Projections developed separately for sub-categories	Belgium, Luxembourg	**Australia**, Cyprus, Czech Republic, **Lithuania**

Note: Countries in bold publish their projections
Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

17.   Countries generally update their forecasts several times per year. When asked to indicate in a list which factors are taken into account in the projections, almost all countries reported drawing on past trends in pharmaceutical expenditures. Ten countries indicated that they take into account potential new entrants; the budget impact of newly covered medicines; and/or changes in uptake of generics and biosimilars. Only seven countries reported taking into account demand-side factors, such as demographic trends, burden of disease and changes in prescribing or treatment patterns in their models (See Table 1.4).

18.   Six countries publish their projections: Australia, Estonia, Ireland, Lithuania, the Netherlands and Sweden. A few respondents to the survey described their projection methods in the survey or provided links to reference. Details are provided in country notes. The example of Sweden is highlighted in the box below, because it displays a number of interesting features, including the transparency of the process and method (See Box 1.2).

Table 1.4. Frequency of updates and factors taken into account by countries developing projections or forecasts of total and/or public pharmaceutical expenditure

Country	Frequency of updates	Demographic trends	Population burden of disease	New medicines expected to receive marketing authorisation and/or be reimbursed	Budget impact as estimated in applications/ assessments for reimbursement or coverage	Past trends in pharmaceutic al spending	Changes in generic/ biosimilar uptake	Changes in medicine prices	Changes in prescribing or treatment patterns	Horizon scanning	Other
Australia	2x/year	1	1	1	1	1	1	1	1	1	(a)
Austria	4x/year	0	0	0	0	0	0	0	0	0	(b)
Belgium	2x/year	1	1	1	0	1	1	1	1	1	
Cyprus	Annually	0	1	0	0	1	1	1	1	1	
Czech Republic	Annually	1	1	1	1	1	0	0	0	1	
Estonia	Annually	0	0	0	1	1	1	1	1	0	
France	Several times per year	1	1	1	1	1	1	1	1	1	
Ireland	Monthly	1	1	1	1	1	1	1	0	0	
Japan	Annually	0	0	0	1	0	0	0	0	0	
Latvia	Annually	0	1	0	1	1	0	0	0	0	
Lithuania	2x/year	0	0	1	1	1	0	0	1	0	
Luxembourg	2x/year	1	0	1	1	1	1	1	0	0	
Malta	Monthly	0	0	0	0	1	0	0	0	0	
Netherlands	2x/year	0	0	1	0	1	0	1	0	1	
Norway	4x/year	0	0	0	1	1	1	1	0	0	
Portugal		0	0	0	0	1	0	0	0	0	
Sweden	2x/year	0	0	1	0	1	1	1	0	1	

Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

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Box 1.2. Method for forecasting pharmaceutical expenditures used by the National Board of Health and Welfare of Sweden

The forecast analyses are based on quarterly data from the Swedish e-Health Agency on drug sales at ATC level 5. Data are extracted at product or active ingredient level so that individual drugs can be distinguished. Historical statistics are then analysed to distinguish trends and deviations—for example, observing which drug groups and drugs are increasing or decreasing to a particularly large extent. Based on the analyses of historical developments, a first forecast of drug expenditure is made.

In the most recent forecast, six pharmaceutical groups were considered to be of particular importance to expenditure growth within 2 years: oncology, TNF-alfa inhibitors, novel oral anti-coagulants (NOACs), diabetes drugs (excl. insulin) and drugs for ADHD and multiple sclerosis. These areas, as well as the area of Hepatitis C were analysed in terms of anticipated market entry and prices of new medicines, estimated patient numbers for treatment, potential generic or biosimilar entry and uptake, clinical guidelines etc. The analyses were done in collaboration with clinical experts, epidemiologists and statisticians. The first forecast was adjusted based on these analyses, after which a draft forecast was sent out to external reference groups. Two external reference groups are consulted twice during the forecasting exercise to review the forecast and the assumptions made:

• One group with representatives from other government agencies and the regions (TLV, the e-Health Agency, the Medical Products Agency, Swedish Association of Local Authorities and Regions and five regions)

• One group with representatives from the pharmaceutical industry (Swedish Association of the Pharmaceutical Industry, The Association for Generic Pharmaceuticals and Biosimilars in Sweden and the Swedish Pharmacy Association).

Both groups have access to historical data and to the National Board of Health & Welfare's (NBHW) forecast. For the specially reviewed areas, NBHW describes in detail the assumptions made in the forecast. The discussion often involves assumptions about the development of the costs for specific products with high impact on expenditures. The NBHW receives important insights from the reference groups, which are then used to adjust the forecast, for example: estimates of future patient numbers expected to be treated with an expensive breast cancer drug; the effects of clinical recommendations; the distribution of inpatient and outpatient drugs. In several cases, the reference groups have also engaged with expert groups in the regions, such as the National Working Group on Cancer Drugs, which has helped with assumptions about specific drugs. In addition, the forecast group also discusses the development of pharmaceutical groups and individual products with the internal medical expertise of the NHBW, to further improve the validity of the assumptions.

This method has become more structured over time, evolving from individual meetings with experts to the formation or reference groups and gathering experts in the same room for joint discussion. The NBHW has also improved the structured use of internal experts. This has required more planning and coordination, but has led to an improved forecast, which is also more transparent and widely accepted.

Source: Personal communication with NBHW (Eriksson et al., 2017[2])(The National Board of Health and Welfare, 2018[18]).

Horizon scanning and spending projections

19. Horizon scanning consists of "the systematic identification of health technologies that are new, emerging or becoming obsolete and that have the potential to effect health, health services and/or society".[9] Horizon scanning is increasingly used in OECD and EU countries, mainly with the objective of anticipating the impact of *new* technologies on

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[9] http://htaglossary.net

health systems (OECD, 2017[3]). A number of countries are contemplating introducing formal horizon scanning systems and several reports have assessed existing systems to identify good practices according to predefined objectives (Eunethta, 2018[1]; Lepage-Nefkens et al., 2017[2]). In 2018, countries participating in BeNeLuxA launched an initiative for international cooperation on horizon scanning, inviting other countries to take part.[10]

20.   While informing projections of pharmaceutical expenditure is neither the sole, nor even the main objective of horizon scanning activities, seven countries reported using the results of horizon scanning as an input to their expenditure projections: Australia, Belgium, Cyprus, Czech Republic, France, Netherlands and Sweden. Some of them provided details (see country notes and Box 1.3).

Box 1.3. Examples of national horizon scanning systems

In the Netherlands, the current system of horizon scanning was established in 2016. The Dutch Healthcare Institute (ZIN) leads the work, supported by specialist groups. It then submits a report for validation to a board involving a wide range of stakeholders, such as associations of physicians, pharmacists, insurers, patients, hospitals, in charge of approving and validating ZIN's reports. A list of new indications or medicines recently approved or expected to be approved within 2 years is published and regularly updated, with available information on the added value of the product, the number of targeted patients, the annual cost per patient, the expected total annual cost, and the risk of off-label use.[11]

In Norway a national system of horizon scanning has been created, mainly as a practical starting point for identifying technologies for HTA, in a broader system aimed at managing the entry of new technologies (Norwegian Directorate of Health, 2018[4]). Horizon scanning for pharmaceuticals is performed by the Norwegian Medicines Agency (NOMA), in collaboration with the Norwegian Institute of Public Health. Its main purpose is to notify the arrival of new medicines with a predictable impact on the health system. HS reports are not assessments but they provide information on the product, indication, target population, etc. They are available online. Practically, NOMA prepares short reports (early alerts) for all new active substances and extended indications that result in new patients, between 6 and 12 months prior to the marketing authorization (MA). The scope is to ensure that new and important drugs are identified and prioritized for health technology assessment (HTA). Pipeline meetings with companies are organised as a supplement to the early alerts with a somewhat longer time perspective (24-36 months before MA). The Norwegian Hospital Procurement Trust and Division drug procurements (LIS) are represented at these meetings. Participants to these meetings are interested by many elements, among which: patient population that may be relevant for the medicine in Norway; price and expected budget impact or upcoming patent expiry.

In Sweden, the four largest regions have been collaborating since 2009 to perform horizon scanning (HS). The HS working group first filters technologies retained for early assessment, according to a list of predefined criteria including for example the size of the population, the severity of the disease treated, the potential to clinically improve patient outcomes, potential high media or public interest, etc. Early assessment reports are communicated to county councils about 6 months before marketing

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10   See http://www.beneluxa.org/sites/beneluxa.org/files/201707/Horizon%20scanning_Scientific Report_full.pdf

11   https://www.horizonscangeneesmiddelen.nl/geneesmiddelen

authorisation. The medicines or indications selected for assessment are listed on the website for national managed introduction of new medicines[12] (Stockholm Regional Authority, 2018[4])

Eriksson and colleagues assessed the prioritisation system of the Swedish system of Early Awareness and Alert system for all new active substances approved by EMA between 2010 and 2015 (Eriksson et al., 2019[5]). Considering that medicines with drug sales valued at more than 0.1% of total pharmaceutical sales have a 'substantial impact on sales', the authors found that the system showed good sensitivity (proportion of prioritized medicines among all medicines exceeding the sales threshold), specificity (proportion of non-prioritized medicines among all medicines below the sales threshold), and negative predictive value (the proportion of medicines below the threshold among all non-prioritized medicines). The relatively low positive predictive value (proportion of medicines exceeding the sales threshold among all prioritized medicines) was explained by the fact that impact on sales is not the sole criterion taken into account in prioritisation.

21.   As short-term projections mainly take into account supply-side factors, outputs of horizon scanning should be an important input to these analyses. Although these results are by nature uncertain, they enable policy makers to anticipate changes in pharmaceutical markets and related expenditures, with sensitivity analyses where necessary

22.   This report does not focus on best practices in horizon scanning, which have been addressed elsewhere (Eunethta, 2018[1]; Lepage-Nefkens et al., 2017[2]). Instead, it highlights the potential role of horizon scanning on spending projections and budget or cap setting, where they exist.

23.   While international cooperation in horizon scanning will avoid unneccesary duplication of efforts to identify new products (which are global in nature), countries will need to adapt the outputs to their own contexts if using them for expenditure projections. Time to market, target population and expected budget impact are inherently country-specific.

1.4. Conclusions regarding country practices

24.   Almost all responding countries track pharmaceutical expenditures, most often for reimbursed medicines dispensed by community pharmacies, but only half of them track expenditures at the product level. This level of detail, however, would be needed to develop pharmaceutical spending projections based on assumptions about market dynamics (new products, products going off-patent, etc.).

25.   Thirteen out of 22 respondents set pharmaceutical budgets and ten set expenditures caps at the macro-economic level (beyond which pharmaceutical companies are requested to pay rebates). Past trends in pharmaceutical spending and overall budget constraints are elements the most often taken into account when setting budgets and caps, followed by potential generic/biosimilar entry. Six countries reported considering potential entry of new products, their prices and recent market entry and coverage determinations of new medicines when setting budgets (Australia, Belgium, Cyprus, Ireland, Malta and Sweden).

26.   Seventeen countries reported forecasting pharmaceutical spending in the short term (1 to 5 years). Almost all of them draw on past spending trends for these estimates. Ten countries reported also taking into account potential new entrants; the budget impact of newly covered medicines; and/or changes in uptake of generics and biosimilars. Only seven

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[12]   The list and assessment reports are presented here: https://www.janusinfo.se/nationelltordnatin forande/horizonscanning.4.728c0e316219da813569ab4.html

countries reported taking into account 'demand- side factors', such as demographic trends, burden of disease and changes in prescribing or treatment patterns in their models. Focusing on supply-side factors for short-term projections, however, is consistent with what was observed in the literature review; while long term projections mainly focus on demand-side factors, short-term ones tend to focus on supply. In addition, taking into account past trends (especially if utilisation data are available), is a good proxy for short-term changes in demand, except in therapeutic classes, where innovative medicines address previously unmet medical needs.

27.   Six countries publish their projections: Australia, Estonia, Ireland, Lithuania, the Netherlands and Sweden, which enable stakeholders to take position. Sweden distinguish itself for a very transparent process for these projections, with formal consultations of stakeholders groups.

28.   Seven countries reported using the results of horizon scanning as an input to their expenditure projections: Australia, Belgium, Cyprus, Czech Republic, France, Netherlands and Sweden. Sweden, the Netherlands and Norway appear to have very well-functioning horizon scanning systems, able to inform pharmaceutical expenditure projections.

2. Forecasting pharmaceutical expenditure – Overview of the literature

29.   This section presents a narrative review of the literature on forecasting pharmaceutical expenditure, presented in three parts. The first part presents two reviews of studies that attempt to identify the key determinants of pharmaceutical spending, while the second presents examples of studies exploring different approaches to projecting and forecasting, reflecting variations in methods, time horizons and perspectives. The strategies employed to identify and select papers for these literature reviews are presented in Annex C of this document. The third part draws lessons from this literature review and from current country's practices.

2.1. Studies of determinants of pharmaceutical spending

30.   Effective approaches to modelling projections of future pharmaceutical expenditure should ideally be informed by an understanding of the key drivers of expenditure. This section presents a brief overview of the literature addressing this issue. While the recent literature is sparse, in 2014, **Mousnad** and colleagues (Mousnad, Shafie and Ibrahim, 2014[6]) undertook a systematic review of English language studies that: (1) measured one or more of: total growth in pharmaceutical expenditures, price growth, or quantity growth; and (2) mentioned a clear method for analysing the impact of factors affecting the increases in drug expenditures, in an attempt to identify the main factors contributing to increases in pharmaceutical expenditure. Twenty five studies published between 1993 and 2010 met their inclusion criteria. The main determinant *categories* identified in the review were factors related to price, utilisation, therapeutic choice, demand, and the health care system. However, the authors found that the *major cost drivers* identified were in fact changes in utilisation and (mix of) therapies, as well as new drugs, with the least important factor being price changes for existing drugs. Notably, several studies included in the review reported that the ageing of the population had very little effect on pharmaceutical expenditure.

Table 2.1. Selected reviews of determinants of pharmaceutical spending

Study	Objective and scope	Data and methods	Summary of findings
Mousnad, 2014	English language studies that: (1) measured one or more of: total growth in pharmaceutical expenditures, price growth, or quantity growth; 1993-2010	Systematic review, 25 studies	Major cost drivers were changes in utilisation and therapies, as well as new drugs, with the least important factor being price changes for existing drugs; little effect noted from population ageing
Karampli, 2014	Review of studies addressing the impact of 'pharmaceutical innovation' on pharmaceutical expenditure growth, total health expenditure and population health outcomes.	Narrative review of research findings of seven selected studies, in six countries, over the period 1990-2010, spanning inpatient and outpatient expenditure	Most studies decomposed growth in spending into changes in three components: quantity of pharmaceuticals prescribed, prices, and prescribing choices/therapeutic mix. Prescribing choices and increases in volume of consumption were the main contributors to observed increases in real spending

31.   **Karampli** et al (Karampli, 2014[7]) reviewed studies addressing the impact of 'pharmaceutical innovation' on pharmaceutical expenditure growth, total health expenditure and population health outcomes. This review included some of the studies reviewed by Mousnad et al. Karampli and colleagues reported that the methodological approach adopted in most studies investigating determinants of pharmaceutical expenditure growth was the decomposition of growth in spending into changes in three components: *quantity of pharmaceuticals prescribed* (measured in Defined Daily Doses- DDDs), *prices* (using the pharmaceutical price index) and *prescribing choices/therapeutic mix.* The latter captures the change in average cost per DDD arising from changes in treatment patterns within therapeutic categories and classes of drug, with a negative change indicating a shift on average towards the prescribing of less expensive products. Despite significant variability in countries covered and study timeframes, Karampli found that prescribing choices and increases in volume of consumption were the main contributors to observed increases in real spending on pharmaceuticals.

2.2. Forecasting studies, models and factors taken into account

32.   Efforts to forecast or project pharmaceutical expenditure vary considerably internationally. Projection studies have been conducted by national agencies, market intelligence firms, and universities, using a wide variety of methods and data sources. Studies conducted by national agencies and universities have generally limited their projections to 1-3 years, while those undertaken by market intelligence firms often have longer time horizons, usually five years.

33.   The terms *projection a*nd *forecast* tend to be used interchangeably in the literature when referring to future pharmaceutical expenditure, but there is in fact an important distinction. Projections are estimates of the trajectory of future spending based on a range of assumptions underpinning the analysis. By contrast, forecasts are attempts to predict future values, based on current and past values and expectations of actual future events.

34.   One way to consider the factors that influence medicines use and expenditure is to categorise them as either demand side factors—such as demography (age and gender), epidemiology, drug coverage and clinical practice guidelines—while supply side factors refer to changes in the market due, for example, to new market entries, patent expiries, and price movements.

Short-term (1-5 years) projections, mainly based on past trends and predicted changes in supply

35.   Studies with short-term projections have focused to a greater degree on modelling the effects of supply side dynamics. The paragraphs below describe studies with projections for one to five year, for one country or for several countries.

36.   **Wettermark** et al. (2010) used linear regression analysis to forecast medicine utilisation and expenditure in the Stockholm region over the two year period 2010-2011 (Wettermark et al., 2010[8]). The analysis was based on observations over the four-year period 2006-2009, and adjusted for factors likely to increase or decrease future utilisation and expenditure, such as patent expiries, new drugs, new treatment guidelines, to produce crude predictions of expenditure over 2010-2011. The annual increase in total expenditure for prescription and hospital drugs was predicted to be 2.0% in 2010 and 4.0% in 2011, with increases in most therapeutic areas, but predominantly for antineoplastic and immune modulating agents as well as drugs for the nervous system, infectious diseases, and blood

and blood-forming organs. The authors noted that utilisation and expenditure were nevertheless difficult to forecast due to uncertainties regarding both the rate of adoption of new medicines and of the impact various activities in train intended to improve prescribing.

37.   In 2014, the **Office of Health Economics** published a research paper forecasting NHS expenditure on medicines from 2012 to 2015 for the United Kingdom (O'Neill et al., 2014[9]). This paper used a bottom-up approach, taking into account changes in the supply of pharmaceuticals (new entrants, patent expiries) by therapeutic class. "Core therapeutic areas" were identified as ATC level-1 therapeutic areas representing a large component of market value (>10%), or with a disproportionate impact on/contribution to growth (>10%). Pharmaceutical expenditure was disaggregated into four components:

- Products losing exclusivity between 2012 and 2018;

- New products launched between 2012 and 2018;

- Recent products launched in the previous five years (2007-2011); and

- Non-recent products (launched before 2007) not expected to lose exclusivity until after 2018.

38.   Information on the R&D pipeline and on attrition rates and development times was used to assess the number of new drugs to be launched by year and by therapeutic area. Uptake curves were estimated on past data to predict sales in years following launch. Historical analysis also enabled the prediction of the extent to which a new product was likely to replace older products (substitution effect) or to be used in addition to other products to determine the "net additive effect". As a result, 25% of sales of new products were considered to be additive, except in oncology where 75% of new products sales were considered additive, reflecting the extensive use of combination therapies. The impact of the loss of exclusivity (LOE) was also estimated through "erosion curves", derived from past data. For 'recent' and 'older' products, future trends were estimated using past trends according to a product's age.

39.   The **American Journal of Health System Pharmacy** publishes an annual one-year projection of pharmaceutical expenditure in the United States (see (Schumock et al., 2018[10]) for the last edition). Expenditure data are drawn from the IQVIA National Sales Perspectives (NSP) database, which tracks purchases of medications by hospitals, clinics, retail pharmacies, mail service pharmacies, home health facilities, long-term care outlets, and other healthcare entities in the United States. The estimates are generated through a combination of quantitative and qualitative analyses, considering all factors believed to influence future medicine expenditures, such as new products, price changes, patent expiries, volume changes.

- New products are identified;

- Medicines for which patent protection is expected to expire are identified by searching the internet for pharmaceutical and biotechnology business news articles.

40.   In addition, the list of potential patent expiries published in the preceding year is reviewed to determine any delays and identify those drugs expected to lose patent protection in the coming year. The list of potential patent expiries focuses primarily on medicines that represent substantial expenditure for the entire market, and those that are particularly important in the hospital or clinic setting.

41.   Projections from other sources are also examined and considered. These inputs are evaluated by the authors collectively, and a consensus view reached as to the anticipated

medicine expenditure growth for non-federal hospitals, clinics, and for all sectors combined.

42.   **EvaluatePharma**, a pharmaceutical market intelligence provider creates a consensus sales forecast using an unweighted average of up to six equity research forecasts (EvaluatePharma, 2017[11]). By taking an average, the impact of outlying forecasts is mitigated. Forecasts are updated regularly with the latest market events, with the current estimate to 2022. EvaluatePharma currently forecasts worldwide prescription drug sales to grow at 6.5% (CAGR) through 2022 to reach USD 1.06trn. The orphan drug market is expected to almost double between 2016 and 2022, to USD 217bn (and generate around one-third of expenditure growth globally). Sales of anti-diabetic and oncology drugs are projected to grow annually by 7% and 13% respectively, while overall growth will be partially offset by price erosion among top selling biologics.

43.   **IQVIA** (formerly Quintiles-IMS) publishes annual projections of market trends in the world, split by region and by country (United States, EU5, Japan, Canada, Australia, South Korea and some 'pharmemerging' markets), by market segment, by therapeutic class (11 categories) and by type of products (original brands, non-original brands, unbranded, and other products). The time horizon is 5 years. The latest projections for the whole market were published in 2018 and cover the period to 2022 (IQVIA et al., 2018[12]). The global market was projected to increase by 3-6% annually between 2018 and 2022 and expenditure projections are presented for individual countries. Public reports do not provide detail of the methodology used but describe new treatment options in the pipeline; discuss saving opportunities due to market entry of generics or biosimilars; and address the market impact of pharmaceutical and other health policies (e.g. coverage extensions in emerging markets). The projections are derived from various proprietary databases of national sales audits for individual products as well as information on drug pipelines, disease 'insights', national pharmaceutical policies, etc. (QuintilesIMS Institute, 2016[13]). The IQVIA Institute is now producing estimates of "invoice expenditure growth" and of "net expenditure growth" to take confidential off-invoice or ex-post rebates into account. Such estimates on past trends rely on the comparison of IQVIA sales data with sales revenues by product as reported in companies' financial reports. Projecting rebates in the future necessarily relies on expert opinions about trends in rebate practices.

44.   **Espin** and colleagues (Espin et al., 2018[8]) explored approaches to enhancing the accuracy of near term projections by taking into account the impact of discounts and rebates on overall expenditure in the EU5 to 2021. They did not develop *de novo* estimates, but instead drew on established forecasts of pharmaceutical expenditure for 5 EU countries, from 2017 to 2021, and then adjusted for discounts and rebates not previously considered. They found an increasing divergence between expenditure measured at list and net prices. When the forecasts for the five countries were aggregated, the EU5 (unweighted) average historical growth (2010–2016) rate fell from 3.4% compound annual growth rate at list prices to 2.5% at net. For the forecast, the net growth rate was estimated at 1.5% versus 2.9% at list, suggesting that future growth in pharmaceutical expenditure in Europe was not only likely to be lower than previously understood from forecasts based on list prices, but also below predicted healthcare expenditure growth in Europe, and more in line with long-term economic growth rates.

45.   The **EU Pharmaceutical Expenditure Forecast Project** (Vataire et al., 2014[9]) (Toumi, Rémuzat and Creativ-Ceutical, 2012[10]) was intended to assess the overall net effect of two countervailing expenditure drivers on seven EU member states' pharmaceutical spending from 2012 through 2016: on one hand the impact of market entry

of new patented medicines and, on the other hand, patent expiries and the advent of generic/biosimilar competition. The model also aimed to estimate the uncertainty surrounding predictions of pharmaceutical expenditures. The model used IMS sales values for 2011 as its starting point. It then followed distinct strategies to assess the impact of new entrants and the impact of patent expiries. The impact of new entrants was assessed via the following method:

a) Potential new entrants were identified through information on pipelines collected through private sources and information published by the EMA, taking into account: the development phase of the new drug and the risk of failure; the potential marketing authorization dates; the status of the disease (rare or not); 2011 sales for each of the main therapeutic areas and for main competitors; and the potential impact of the new product on sales.

b) For the latter, the model took into account: the time to market (time elapsed between marketing authorisation and market access, assumed to be one year in all countries except Germany, where market access is immediate); the time to peak sales (assumed to be one year after market entry for orphan drugs and 3 years for other products). The study board of experts also assessed the impact of new medicines with expected added benefits (assuming that drugs without added value would not have a net positive impact on spending), using information on target populations and "expected prices".

46.   The impact of generics and biosimilars was assessed in two steps.

a) The direct impact of generics took into account: sales value of the original product in 2011; the date of marketing authorization (assumed to be the date of patent expiry); the time to market after marketing authorization (assumed to be 1 year for generics in the retail market; longer for biosimilars because of time to uptake for biosimilars13; and assumed to be null for direct sales to hospitals); the price reduction of the generic/biosimilar versus the original branded/reference drug; generic/biosimilar penetration versus original off-patent branded drug by volume; the time to peak generic/biosimilar sales; and the impact of generic/biosimilar entry on the originator brand/reference product price.

b) The indirect impact took into account: the market share of the molecule within its therapeutic class; the price reduction within the same therapeutic class; life cycle management strategies; and increases in volumes in some therapeutic areas due to ageing.

47.   Budget impact was assessed from several perspectives (total expenditure at retail prices, public expenditure and sales revenues for the industry). One remarkable aspect of this study is the publication of sensitivity analyses for the estimates (Vataire et al., 2014[9]). Deterministic one-way sensitivity analyses, whereby input parameters are given values ranging from -30% to +30% of the value chosen for the main model, allowed the main determinants of growth in each country to be identified and presented in tornado diagrams (see Figure 2.1 below with results for Portugal). A probabilistic sensitivity analysis making all parameters vary within the [-30%; +30%] range under the assumption of uniform

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13   At the time of the study, biosimilar substitution was not permitted in any countries studied and switching patients was not common. The uptake of biosimilars therefore relied heavily on the initial treatment of new patients.

distribution for all of them, allows the prediction of the probability of having a net budget impact of a given value (see Figure 2.2 for Portugal).

Figure 2.1. Change in pharmaceutical budget impact from the healthcare public payer perspective (millions EUR)

Tornado diagram (with all parameters increased and decreased by 30%) for Portugal
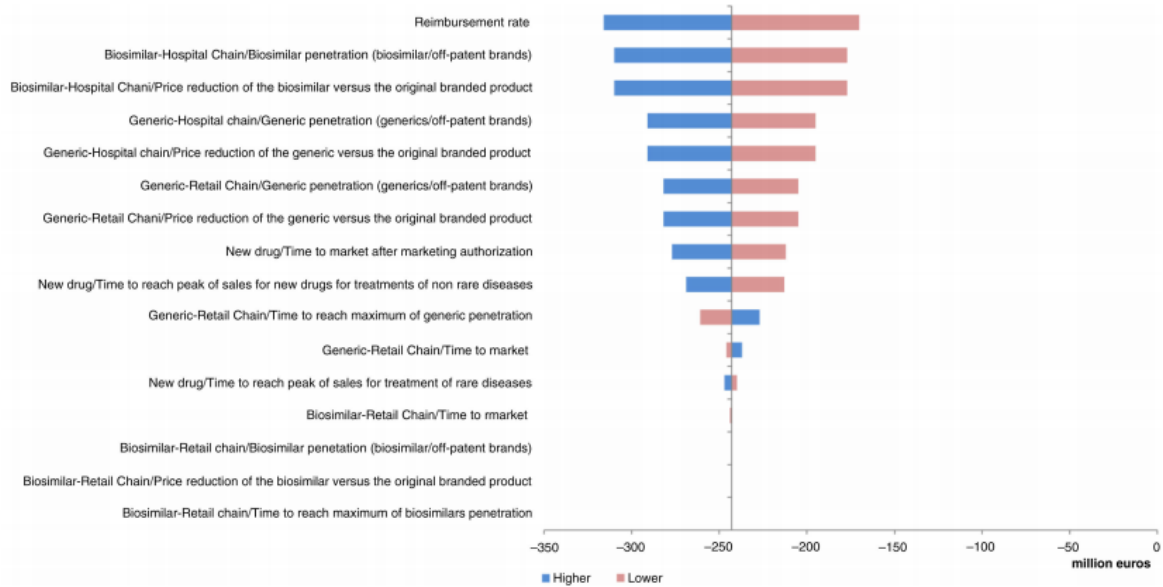
Source: (Vataire et al., 2014[9])

Figure 2.2. Probabilistic sensitivity analysis: budget impact probability curve for Portugal
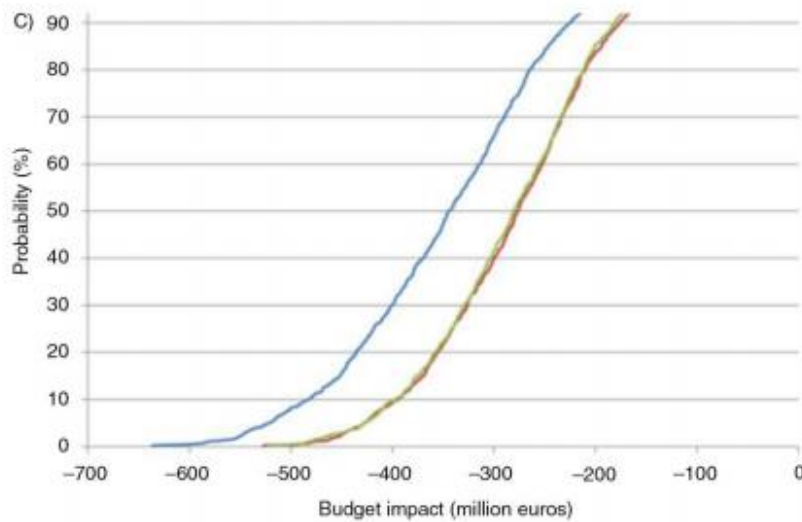
Note: Individual curves represent the probability of occurrence of a certain budget impact from different perspectives. The red line represents the manufacturer's perspective (impact on ex-factory sales); the green line represents the perspective of the health care payer (the Portuguese NHS); the blue line represents the public/society's perspective.
Source: (Vataire et al., 2014[9])

Table 2.2. Selected published studies with short term (1-5 years) projections, based mainly on past trends and predicted changes in supply

Study	Location and Horizon	Scope and Data	Methods	Outputs
National studies				
(Wettermark et al., 2010[8])	Sweden 2010-2011	Stockholm region. All therapeutic areas. Aggregate data on hospital sales and drugs dispensed in ambulatory care	Linear regression analysis, based on observations over 2006-2009, adjusted for factors likely to increase or decrease future utilization and expenditure, e.g. patent expiries, new drugs, new treatment guidelines, and crude predictions to estimate spending over 2010-2011	Annual increases in total expenditures for Rx and hospital drugs of 2.0% in 2010, 4.0% in 2011. Increases in most therapeutic areas, but predominantly for antineoplastic and immune-modulating agents, nervous system, infectious diseases, and blood / blood-forming organs
(O'Neill et al., 2014[9])	United Kingdom 2012-2018	NHS expenditure on outpatient and inpatient medicines. Source: IMS information on past sales, R&D pipeline, expert opinions	Bottom-up approach, using past data to model future sales	
(Schumock et al., 2018[10])	United States 2018	U.S. pharmaceutical spending, by care setting	Past prescription drug expenditure data obtained from the IQVIA Health National Sales Perspectives database and analysed descriptively. Expenditure projections are based on a combination of quantitative and qualitative analyses and expert opinion	In 2018, pharmaceutical expenditures is predicted to increase by of 3.0–5.0% overall (all sectors combined), compared with 2017. Drug spending in clinics and non-federal hospitals will increase by 11.0–13.0%
Multi-country and global studies				
IQVIA (formerly Quintiles-IMS) – Annual reports (IQVIA Institute, 2018[18])	Global, regional and by country (US, EU5, Japan, Canada, Australia, South Korea and some 'pharmemerging') markets 2018 - 2022	Pharmaceutical market (retail and hospital). The data come from proprietary databases covering national sales audits for individual products as well as information on drug pipelines etc.	Annual projections of market trends split by region, by market segments, by therapeutic class 11 categories) and by type of products (original brands, non-original brands, unbranded, and other products). Little detail on methodology	The global pharmaceutical market is projected to increase by 3–6% annually at constant prices between 2018 and 2022. Projected growth for developed countries is 2-5%. It is higher in the United States 4-7% than in other countries.
EvaluatePharma 2017 (EvaluatePharma, 2017[11])	Global 2018-2022	Global prescription drug sales data, with a split for generics, orphan and other Rx. 15 therapeutic areas, incl. oncology, diabetes, rheumatology, vaccines, antivirals. Top 50 products worldwide and in US	Integrated multiple consensus forecasts by equity analysts with historic results, as reported by companies	Global prescription drug sales forecasted to grow at 6.5% (CAGR) through 2022 to USD 1.06trn. 32% of the 2022 increase in sales to come from orphan drugs (+USD95bn)
EU Pharmaceutical Expenditure Forecast Project (Toumi, Rémuzat and Creativ-Ceutical, 2012[10])	United Kingdom Germany, France Poland, Greece, Portugal, Hungary 2012-2016	To assess the net effect of two countervailing expenditure drivers – the impact of market entry of new patented medicines vs patent expiries and the advent of generic/biosimilar competition – on seven EU member states' pharmaceutical spending from 2012 through 2016.	Estimated savings from drugs losing patent protection in 2010-11 or with patent expiry expected in 2012-16; additional spending due to new entries in 2010-11 and likely entries from 2012-16. Computed budget impact of generics and new medicines using 2011 IMS sales values and patent expiry date, time to launch, price discount, penetration rate, time to peak sales, impact on price. Six experts assessed impact of new drugs with expected added benefits using data on target populations and "expected prices".	The overall assessment of budget impact from 2012 to 2016 was that with the exception of Poland, all countries would experience reductions in expenditure. Savings were forecast to be largest for the United Kingdom, followed by France, with Greece and Germany well behind. Comparison with actual expenditure trends over that period showed an underestimate of the budget impact of hepatitis C medicines
(Espin et al., 2018[7])	EU 5 (France, Italy, Germany, Spain, United Kingdom) 2017-2021	Pharmaceutical sales at retail prices (outpatient and inpatient)	Used IMS forecast for 2017-2021 by country (at list prices) and adjusted with historical discount rates	Projected AAGR for 2017-2021 at resp. list and let price: France: 1.8% and 0.6% Germany: 3.2% and 2.0% Italy: 3.2% and 1.1% Spain: 2.5% and 1.1% United Kingdom: 3.8% and 2.3%

Note: AAGR= Average Annual Growth Rate. CAGR=Compound Annual Growth Rate
Source: As indicated in the table.

Medium to long-term projections, focusing on demand

48.   Some studies have focused mainly on modelling future expenditure based on past expenditure trends and factors influencing demand such as demographic growth, but without directly considering influences on supply such as the advent of new drug classes, launch prices, or patent expiries (Table 2.3).

49.   **Lenihan** et al (2015) used Monte Carlo simulation to model the cost of General Medical Services (GMS) scheme prescriptions in Ireland from 2016 to 2026. The GMS is the largest community drug scheme in Ireland with approximately 40% of the population eligible for free drugs and appliances in 2012. Central Statistics Office (CSO) population projections (2013) and GMS population prescription data produced by the Primary Care Reimbursement Service (PCRS) of the Health Service Executive (HSE) (2012) were used to populate four variables: population, GMS coverage, average cost per claimant, and claims rate. The model simulated the effect of these four variables on GMS costs, by health board region, age cohort and sex. Age is a key driver of GMS expenditure, specifically those aged under 11 and over 70. The Irish population is projected to grow by approximately 10% between 2012 and 2026 and the over 70s population is estimated to grow by 64%. The model estimated that GMS expenditure would increase by 64%, from EUR 1.1 billion in 2016 to EUR 1.8 billion by 2026 (Lenihan and Woods, 2015[20]).

50.   **Thiébaut** et al (2013) used a Markov microsimulation model to forecast national drug expenditure in France to 2029 under a range of epidemiological scenarios of chronic morbidity (Thiébaut, Barnay and Ventelou, 2013[11]). The data were drawn from the public health insurance database, the *Échantillon permanent d'assurés sociaux* (EPAS), and a French household survey *Enquête sur la santé et la protection sociale* (ESPS). The EPAS comprises a panel of public health insurance recipients and contains exhaustive information on reimbursement claims, albeit excluding over-the-counter medications and drugs administered in hospitals. The ESPS uses a sample of 22 000 individuals who are representative of over 96% of the French population, and the database captures individual and household data, demographic and epidemiological characteristics (including several summary indicators of health status), socioeconomic data, and information about individuals' insurance coverage. For the population aged 25+, the results predicted an increase in reimbursable drug expenditure of between 1.1% and 1.8% annually, attributable solely to the ageing population and changes in health status.

51.   A study by **Boecking** et al (Boecking et al., 2012[22]) estimated the "pure" impact of demographic changes on the increase in pharmaceutical expenditures up to 2050. Projections were based on observed expenditures by gender and age cluster for a reference year (1997 for France and 2004 for Germany, a difference considered to be "negligible") and only took into account demographic changes, based on national demographic projections, with expenditure by age cluster held constant. The model thus ignored possible changes in utilisation patterns or pharmaceutical prices.

**Table 2.3. Medium to long term projections
based mainly on past trends and changes in demand**

Study	Location and Horizon	Scope and Data	Methods	Outputs
(Lenihan, 2015[23])	Ireland 2016 to 2026	General Medical Services (GMS) scheme prescriptions. The GMS is the largest community drug scheme in Ireland with approximately 40% of the population eligible for free drugs in 2012.	Central Statistics Office (CSO) population projections (2013) and HSE-PCRS GMS population prescription data (2012) used to develop four variables: population, GMS coverage, average cost per claim, and claims rate. Monte Carlo simulation of effect of changes on GMS costs	Estimated GMS expenditure would increase by 64%, from EUR 1.1 billion in 2016 to EUR 1.8 billion by 2026.
(Thiébaut, Barnay and Ventelou, 2013[10])	France To 2029	Sample from the French household survey ESPS. Excluded OTCs and hospital drugs	Markov micro-simulation model to forecast future national drug expenditure, under different epidemiological scenarios of chronic morbidity:	For the population aged 25+, increases in reimbursable drug expenditure of 1.1% - 1.8% (annual growth rate), attributable to ageing, and changes in health status.
(Boecking et al., 2012[22])	France, Germany To 2050	Pharmaceutical covered by social insurance Sources: national data from health insurance	Estimate of pharmaceutical expenditure for 9 age groups in reference year and of impact of "pure" demographic changes.	Pharmaceutical expenditure per capita expected to grow by 0.5% per year due to demographic change, or about 25% for the whole period.

Mixed models

52. The Office of the Actuary (OACT) in the **Centers for Medicare & Medicaid Services** (CMS) produces annual, short-term (10-year) projections of health care spending for categories in the National Health Expenditure Accounts (NHEA), including drug spending (Office of the Actuary (OACT), 2018[25]). The OACT uses an econometric model to produce projections of future health care spending by private payers (insurers, households and others) that are consistent with exogenous projections for Medicare[14], Medicaid, the Children's Health Insurance Program (CHIP), and key macroeconomic variables. The model uses historical data on spending (by type of service, source of funding and sponsor of health care), on prices[15] and on insurance enrolments, as well as 'exogenous inputs', i.e. the most recent available macroeconomic and demographic assumptions from the Social Security Administration (SSA), such as GDP, economy-wide inflation, labour market indicators, demographic projections by age and gender; and actuarial projections for Medicare, Medicaid, and CHIP spending and enrolment. The future impact of provisions under current law is also considered in the model (either as part of actuarial

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[14] Published in the annual report of (The Boards of Trustees of the Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds, 2018[31])

[15] A composite price index is computed as an average of Producer Price Indexes (PPIs) and Consumer Price Indexes (CPIs), weighted by the relative shares of personal health care spending categories. The CPI is used for drug spending.

projections realised for Medicare and Medicaid, or as projections of enrolment via the ACA Marketplaces and through CHIP). The NHE econometric model can be characterized as top-down, in the sense that spending is projected by sector individually, but all projections are constrained to the aggregate personal health care projection. <u>As a result, the model only implicitly takes</u> into account shifts between sectors (e.g. from hospital to ambulatory care).

53.   Every five years, the **Australian Government** (Australian Government, The Treasury, 2015[12])produces an *Intergenerational Report* (IGR) assessing the long-term sustainability of current Government policies and how changes to Australia's population size and age profile may impact economic growth, workforce and public finances over the succeeding 40 years. Each report provides projections adopting a 'point-in-time' format— that is, using the assumption that current government policies will continue over that period, without change. The 2015 Intergenerational Report is the 4th such IGR. The projections use the 2014-15 *Mid-Year Economic & Fiscal Outlook* (MYEFO)[16] as the base for the first four years of the projections. Over the rest of the report's 40 year projections, the IGR initially uses component models for public hospitals, pharmaceutical benefits, medical benefits and private health insurance rebates to project Health expenditure until 2027-28. The use of component models for this period seeks to balance the desire for more detailed projections against the uncertainty as to whether recent trends in individual components of government health expenditure will be representative of longer term trends. As the uncertainty around the distribution of health expenditure between the components of health spending increases with the length of the projection, from 2027-28 onwards, an aggregate model is used to project total Australian Government health spending, and assumes non-demographic growth trends towards the historical non-demographic growth rate for health spending by all levels of government over the longer term. The pharmaceutical benefits model covers spending under the Pharmaceutical Benefits Scheme. Projections are derived by applying non-demographic growth to current spending on pharmaceutical benefits per person for each age group in each gender. Population and CPI projections are then applied to derive nominal projections of spending. The non-demographic growth rates are derived from trends in historical data on pharmaceutical benefits expenditure. This is done by adjusting historical spending by age group for CPI growth to derive real spending per person for each age group in each gender. Non-demographic growth is projected forward as a constant real dollar increase in spending each year for each age group in each gender (Australian Government - The Treasury, 2015[13]).

2.3. Observations from current practices and existing studies

Limitations of studies

54.   In the pharmaceutical market, both supply side factors – such as the entry of new products, competition from generics and biosimilars, pricing and reimbursement mechanisms – and demand side factors – changes in demand or patterns of use due to price, clinical guidelines, prescriber behaviour and burden of disease – all influence

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[16]   The Mid-Year Economic and Fiscal Outlook (MYEFO) is a report released by the middle of each financial year comparing estimated expenditure to actual expenditure to allow assessment of the Government's fiscal performance against the strategy set out in preceding national Budget. MYEFO estimates include any government decisions made since the preceding Budget that affect expenses, revenues and capital estimates. MYEFO also updates the budgetary position, including Budget aggregates, by incorporating any changes to economic parameters.

pharmaceutical expenditure, albeit to different degrees. The studies reviewed here attempted to take some or all of these factors into account in their projections. However pharmaceutical spending is likely affected by several other factors; arguably among the most important are changes in the economy, political dynamics, policy settings, and health system reforms. In addition, all OECD countries are experiencing ageing of their populations, as well increases in both demand and consumer expectations (a concomitant of GDP growth). With the exception of population ageing, none of the studies assessed these broader factors explicitly.

55.   Yet the influence of other factors is arguably in evidence when looking at actual retail and total pharmaceutical expenditure across selected OECD countries (Figure 2.1) and observing variations in both the magnitude and pattern of growth over the period 2011-2015. This highlights some of the challenges inherent in projecting future expenditure effectively.

Figure 2.3. Growth in retail and total current pharmaceutical expenditure in OECD countries, 2011-2015
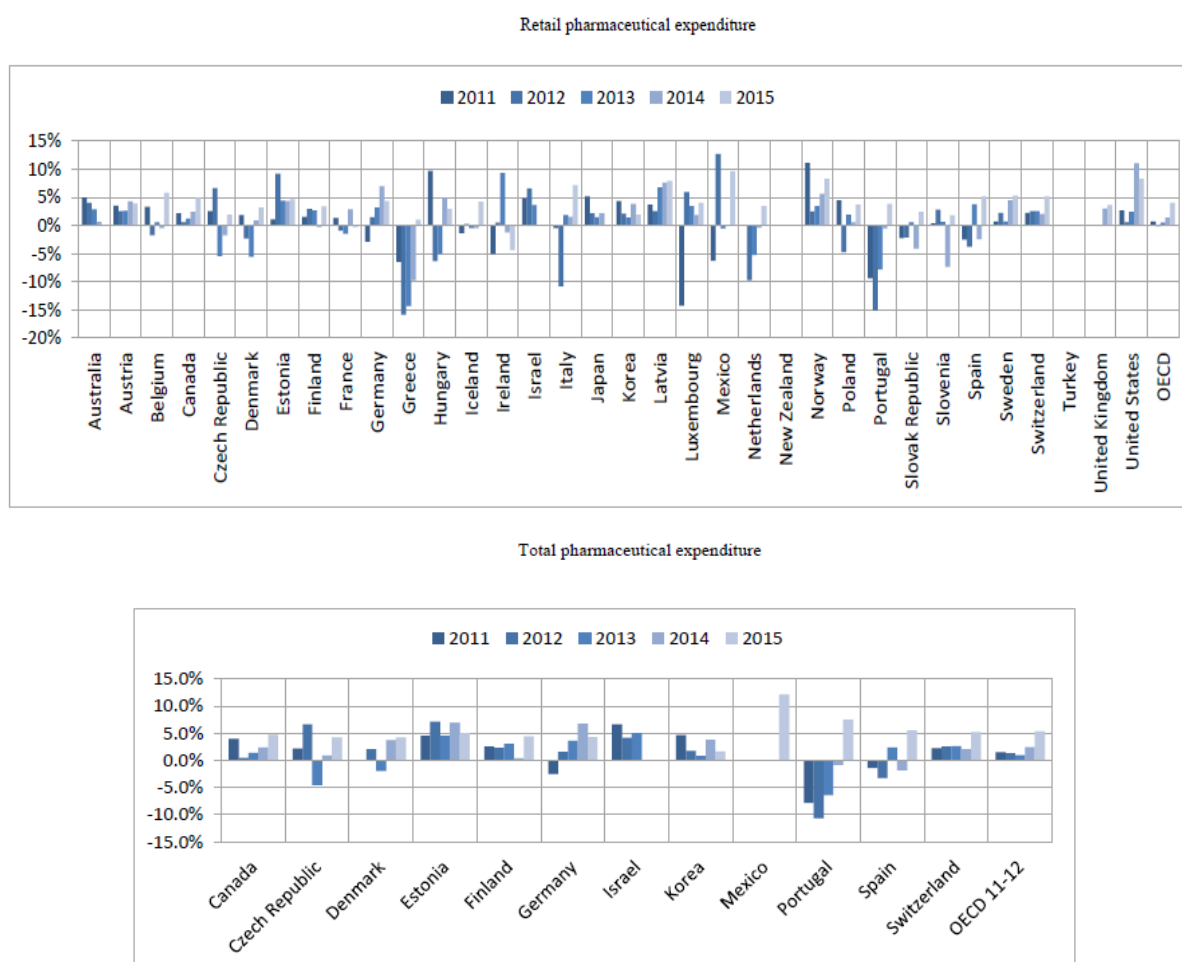
Note: Total pharmaceutical expenditure includes expenditure for medicines administered to patients in hospitals and physicians settings.
Breaks in series: in 2011 for Denmark, Iceland and Luxembourg; in 2012 for Italy; in 2013 for the Czech Republic and Latvia; and in 2014 for Slovenia. *Source*: Data extracted from OECD.Stat

56.   Importantly, several of the studies relied heavily on consensus among small groups of experts to identify key assumptions, such as timing of market entry and launch prices of new medicines; penetration rates; price drops due to generic/biosimilar market entry, and market behaviour under different scenarios. In addition, all of the studies examining supply side factors expressed uncertainty surrounding the identification of the timing of patent expiries and in horizon scanning for new products.

The accuracy of projections

57.   The accuracy of projections is challenging to assess because actual values of projected 'aggregates' are not always publicly available. Comparisons are possible when projections are published on a regular basis, together with prior data. For example, a comparison of the predicted and actual values for 2016 presented in two reports by Quintiles-IMS showed that the actual market values for the 5 big EU markets, Canada and Korea were within the predicted ranges, while the actual value for the US market was almost 18% higher than the upper predicted value, and for Japan was 14% lower than the lowest value range (authors' calculations based on (IMS Institute, 2012[26]; Quintiles IMS Institute, 2016[27]).

58.   **Hartke** and colleagues (Hartke et al., 2015[28]) evaluated the accuracy of AJHP annual forecasts of drug expenditures in non-federal hospitals and clinics with those produced by the Centers for Medicare and Medicaid Services (CMS). AJHP-published forecasts of drug expenditure growth for non-federal hospitals (for the years 2003 through 2013) and clinics (for the years 2004 through 2013) were compared with data on actual growth. Actual spending growth was found to be within the range of the forecast published in AJHP for only 2 of 11 years for non-federal hospitals and for only 3 of 10 years for clinics; the forecasts for non-federal hospitals and clinics were directionally accurate 27.3% and 60.0% of the time, respectively. The mean absolute errors of the AJHP-published drug expenditure forecasts for the non-federal hospital and clinic sectors were 2.0 and 4.7 percentage points, respectively. The CMS forecasts of overall drug spending were in range 4 times in 10 years, directionally accurate 70% of the time, and the mean absolute error (2.2 percentage points) was not statistically different from that of either sector forecast published in AJHP.

59.   In the United States, CMS also assessed the accuracy of projections by comparing the projected growth rates from each iteration of the 10-year NHE Projections since 1997 (19 sets) to the corresponding current historical NHE estimates for 2016 (CMS, 2018[29]). Projections of prescription drug spending growth have, on average, overestimated actual spending growth by 0.6 percentage point in the first year, 0.8 percentage point in the second year, and 1.0 percentage point in the third year. The mean absolute difference[17] was 2.4 percentage points in the first year, 3.0 percentage points in the second year, and 4.1 percentage points in the third year. The accuracy of the direction (increase/decrease) was nearly 85% in the first year, 78% in the second year, and 71%, 63%, 53% respectively for the third, fourth and fifth years.

60.   Despite sophisticated modelling and sensitivity analyses, Creativ Ceutical predictions for 7 EU countries for the period 2012-2016 proved to be inaccurate. For Portugal for example, the probability of reaching the actual value of net budget impact during the period for public pharmaceutical expenditure (all distribution channels) was

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[17]   Projections can under- or –overestimate actual values. The "absolute difference" does not consider the direction of the difference.

assessed to be null (Vataire et al., 2014[9]). While net budget impact was found to be negative in all predictions in this study, in reality it was slightly positive (+94 million Euros). This result, however, was due in part to the launch of the direct-acting antivirals for hepatitis C, which most experts did not anticipate.

3. Lessons for OECD and EU countries introducing, or seeking to improve expenditure projections

61.   This section draws on previous sections to identify good practices for projections of pharmaceutical expenditures. Section 3.1 discusses the importance of defining clear objectives for the exercise as a pre-requisite. Section 3.2 describes information needs and possible sources to improve short-term projections, which seem to represent the most pressing need for policy makers.

3.1. Defining clear objectives

62.   Methods and inputs used for projections clearly depend on the time horizon of the modelling exercise. Defining clear and context-relevant objectives, taking into account local capabilities, is thus an important first step in determining the approach to be used, and the inputs needed.

63.   While several international organisations (Eurostat, OECD) and national institutions run long-term projections for health expenditures as a whole, they generally do not perform projections by sector of care. From the available literature and from the OECD survey on country practices (see Annex B, Table B.5), short-term projections are more common for pharmaceutical expenditure.

64.   Short-term projections most often take market dynamics into account, notably the entry of new products, as well as LoE and anticipated market entry/availability of generics/biosimilars. They also frequently take into account past expenditure and utilisation trends in order to model diffusion rates for new products, as well as the impact of market entry and uptake of generics and biosimilars on utilisation and prices. Only a few models – used by national agencies – have attempted to assess the impact of current or future pharmaceutical policy settings through scenario analyses.

65.   Countries with fixed or target budgets for public pharmaceutical expenditure may use short-term projections to inform budgeting processes. Short-term projections can inform financing needs, taking account of the net effects of new entrants and patent expiries. They can also be used to model the impact of policies intended to maintain spending within budget limits. Ideally, and especially when the use of new medicines is expected to have an impact on expenditures on other types of care (whether savings or additional costs), projections should take these into account. However, spillovers of this kind were not considered in any of the reviewed projection studies.

3.2. Identifying core data elements and their sources

66.   Short-term projections require considerable data on market dynamics, including information on potential new entrants (expected date of launch, prices and diffusion rates) and on potential competition from generics and biosimilars (date of LoE, entry and uptake of competitors, and impact on prices and volumes).

New and upcoming entrants

67.   Identifying potential new entrants and modelling their potential impact requires considerable information as well as several assumptions. Starting from medicines present

in the pipeline, analysts have to predict the timing and likelihood of entry, as well their therapeutic value and expected rate of diffusion.

68.   The identification of medicines in the pipeline is, in and of itself, a significant undertaking, especially if commencing from a zero base. For example, Vataire et al. list many diverse sources consulted for this step: the European Medicines Agency (EMA) public information, Datamonitor reports, the PharmaVitae database, the Medtrack database, pharmaceutical company websites and press releases, investors' reports, an in-house proprietary drug information database, and study registries such as *clinicaltrials.gov* (Vataire et al., 2014[9]). This could be simplified with the development of regular and systematic horizon scanning in countries, a part of which could be undertaken collectively to reduce duplication of efforts.

69.   Once medicines in the pipeline have been identified, analysts can apply average rates for the probability of success and average development times. Estimates of these parameters are published on a regular basis, for different categories of products, such as orphan medicines, oncology medicines (see for example (Thomas et al., 2016[14]). Estimates used for projections should be as recent as possible, in order to take into account both the development of very new therapies and the proliferation of accelerated approval pathways. What remains highly challenging to predict, however, is the comparative or incremental therapeutic value of new products, which in most countries will influence both the price and the target population. Data on the incidence and prevalence of disease are insufficient to determine population targets, as in many countries the latter will also depend on the registered indication(s), evidence of comparative therapeutic value and comparative cost-effectiveness, all of which will influence placement within a treatment protocol or clinical guideline.

70.   Moreover, while information on medicines in the pipeline, the probability of successful entry to market, and to some extent, therapeutic value[18] are generally global in nature, many aspects of horizon scanning are necessarily country specific, such as the time from marketing authorisation to effective launch, and the likely diffusion rate. Another important aspect with respect to diffusion rate is whether a new medicine will be used as a substitute for existing therapies or whether it will be additive. In the baseline scenario of their model, O'Neill et al. (O'Neill et al., 2014[9]) assumed that 25% of sales of future launches would be additive, with the exception of oncology for which the baseline assumption was 75%. They reasoned that when first introduced, oncology medicines tended to be used in combination with existing therapies, or as third or fourth line treatments thus, by definition, replacing existing treatments far less frequently. However there is no empirical data from which to determine that this assumption is reasonable across jurisdictions with differing levels of resourcing. In general, multiple products within a given therapeutic class are typically in development simultaneously for a given indication. As the patient population for a particular indication will in many cases remain the same, each new indication will not necessarily add to the volume of patients treated, but rather compete for the same patients.

71.   Given these challenges, it is not surprising that horizon scanning undertaken at individual country level tends to focus on medicines likely to reach the market within 1 to

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[18]   Comparative clinical value will also vary by country if standards of care, and therefore comparator therapies, differ.

3 years, limiting the need for long-term assumptions that introduce increasing degrees of uncertainty.

72. More systematic data collection on time from first marketing authorisation to marketing authorisation in country, and on time from marketing authorisation to effective launch would be useful for modelling.

73. Early scientific dialogues involving manufacturers, regulators and HTA entities, as well as clinical experts and patient representatives, can provide a valuable opportunity not only to improve the quality of evidence generated to support both regulatory approval and assessment for coverage and reimbursement, but can also inform an understanding of likely therapeutic value, price, target patient population and potential uptake. Nevertheless, predicting therapeutic value, price and rates of diffusion with accuracy will remain challenging, and modelling of a range of scenarios may be necessary. Some studies have relied on past diffusion rates, the applicability of which will depend on the characteristics of the medicine in question as well as on national policies and clinical guidelines

74. Despite these challenges it is notable that the Swedish Early Awareness and Alert System has proven to be reliable in identifying all new medicines that would go on to have substantial potential impact on sales (Eriksson et al., 2019[5]).

Demographic data and burden of disease

75. Demographic and burden of disease data are necessary for the estimation of potential utilisation. Vataire (Vataire et al., 2014[9]) drew on a variety of sources including Datamonitor epidemiology reports, public databases such as Medline and Embase, and web resources such as Orphanet for rare diseases.

76. In countries with well-established HTA processes, sources of demographic and burden of disease data should already be identifiable, with the possible exception of data on rare diseases or previously untreated conditions, for which patient numbers are likely to be small. For conditions for which therapies already exist, a key issue will be to determine whether an anticipated treatment will expand the current treatment population, or as noted above, be used in addition to, or as an alternative to current therapies.

Loss of market exclusivity (LoE) and generic/biosimilar uptake

77. In this report, 'loss of market exclusivity (LoE)' refers to the expiry of all forms of legal protection from generic/biosimilar competition conferred by virtue of patents or other forms of intellectual property protection (such as data exclusivity provisions).

78. LoE is country specific. It depends on the intellectual property protections applicable in a given country (albeit with minimum standards for all OECD and EU countries set out in the TRIPS Agreement[19]); on the date at which a patent application was filed; on any 'supplementary protection' conferred under European and some national laws; and on the date of launch of the product (Copenhagen Economics, 2018[30]).

79. Supplementary protection mechanisms exist in the European Union, the United States, Australia, Canada, Japan, Israel and Korea[20], and can provide extensions to selected

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[19] The Agreement on Trade-Related Aspects of Intellectual Property Rights, Annex 1C of the Marrakesh Agreement Establishing the World Trade Organization, signed in Marrakesh on 15 April 1994.

[20] In Europe, the patent term may be extended in certain circumstances by the granting of a *Supplementary Protection Certificate;* in Australia this is referred to as *Patent Term Extension*; in the US, *Patent Term*

patent terms in certain circumstances. In addition, other forms of effective market exclusivity[21] are available in most OECD countries and can extend the LoE beyond patent expiry. However a recent study of 558 products marketed in Europe showed that for about half of them, the patent was the last protection to expire (ibid.).

80.   Both the Copenhagen Economics (Copenhagen Economics, 2018[30]) and Vataire (Vataire et al., 2014[9]) studies showed that data on LoE are not readily available, and require both considerable effort, resources and skills to be located and interpreted. This not only creates an effective impediment to generic entry but is also an important issue in pharmaceutical expenditure modelling. Some private companies maintain patent and exclusivity databases and are able to provide the information to national authorities for individual products. In the two analyses mentioned earlier, researchers had to access several databases in order to obtain reliable information on LoE dates (see table 3.1). Vataire also noted that patent expiration dates are often more difficult to find for biologics and mentioned the Generics and Biosimilars Initiative (GABI)[22] as a source.

81.   This highlights the need for a publicly accessible data warehouse for all relevant information, so that generic or biosimilar entry can be more readily predicted by all stakeholders, as well as generic/biosimilar manufacturers having ready access to information from which to determine whether a launch is potentially 'at-risk'.

82.   However, generic entry does not always follow the end of market exclusivity. For instance, in Mexico, a report by *La Comisión Federal de Competencia Económica* (COFECE) showed that on average more than two years elapse between the expiration of a patent and the launching of the first generic (Cofece, 2017[15]). This raises the question of whether delays may be due – in some cases – to uncertainty around dates of LoE of originator/reference products.

83.   Furthermore, in some countries there may be additional uncertainty in the timing of generic or biosimilar entry arising from other factors such as patent litigation and 'pay for delay' agreements.[23] These challenges may increase the uncertainty of expenditure forecasting.

Restoration. Following accession to, and implementation of the provisions of the *Comprehensive and Progressive Agreement for Trans-Pacific Partnership*, supplementary protections will also become available in New Zealand.

[21]   Refers to data and market exclusivity periods conferred by regulatory agencies, and which are not mandated by TRIPS.

[22]   www.gabionline.net

[23]   See https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm607495.htm for a discussion of these issues.

Table 3.1. Sources of information on IPRs and loss of market exclusivity (LoE)

	Comment	Sources
(Copenhagen Economics, 2018[30])	To find information on loss of market exclusivity (patent, SPC and other incentives), the authors had to access six databases, including databases from the US to identify relevant patents (for details, see Appendix of the report)	EMA for: - European Marketing Authorisations (centralised) - European public assessment reports EMA - European Exclusivity Extensions Paediatrics, - Rare disease Designations Heads of Medicine Agencies (HMA MRI Product index) for Marketing Authorisations obtained through mutual recognition in Europe European Patent Office "PATSTAT" database for the identification of EU patents and Supplementary Protection Certificates (SPCs) through patent families and US patents Patent Watch (private)Private for: - US Drug Approvals - US Patents
Vataire et al. (Vataire et al., 2014[9])	To find information on pipelines and data on loss of market exclusivity (patent, SPC and other incentives), the authors had to access multiple public, private and proprietary resources	A number of private sources Datamonitor reports, the PharmaVitae database, the Medtrack database, Patent databases (free access patent databases such as the World Intellectual Property Organization, the European Patent Office, and Espacenet; and commercial online databases, such as STN International/CAPADOC, Questel Orbit, and GenericsWeb - Pharmaceutical company websites, press releases, investors' reports, - IMS database, - In-house proprietary drug information database For biosimilars: Generics and Biosimilars Initiative (GaBi)

Impact of generic/biosimilar entry on prices and expenditures

84.   The impact of generic and biosimilars in national markets (uptake of generic and biosimilars, changes in volumes and prices of originators and follow-on products), depends on the policies and incentives in place. Studies taking this impact into account most often rely on past trends observed in country to predict future uptake. This requires historical data, which are limited for biosimilars. These data can also aid in predicting the impact of policy measures aimed at improving uptake of, or enhancing competition between generics/biosimilars and originator/reference products.

85.   Prices may of course be affected by factors beyond LoE and market entry of generics and biosimilars. On-patent competition does occur in some therapeutic areas (an example being direct acting anti-virals for hepatitis C), although it is far from systematic.

Information on rebates

86.   The proliferation of discounts and rebates adds another challenging dimension to the complexity of expenditure projections. These may be product-specific (and may not always be known by the institutions developing projections) and may differentially affect classes of products.

87.   Two approaches to taking rebates into account have been reported in the literature. Espin et al. applied an adjustment to IMS projections for 5 EU countries, realised at 'list prices' for the average rates of rebates applied in each country in the recent period (Espin et al., 2018[8]). The IQVIA Institute prepares projections using 'list' and 'net' prices,

comparing its own sales data (at invoice prices) with companies' reports for individual products, to impute discounts and rebates. While the first method may be useful at a macro-economic level, it is less so where analysts seek to model different scenarios for classes of products. The approach taken by IQVIA Institute, by contrast, requires access to relevant and proprietary commercial information. To be able to conduct similar analyses at individual country level, analysts would need access to data on companies' sales of single products, in addition to reported expenditures.

88.   Moreover, where analysts responsible for expenditures projections have access to information on confidential rebates, they can build more accurate models but in all likelihood may not publish details of the projections.

3.3. Conclusions

89.   Short-term projections of pharmaceutical expenditures can be used to support the determination of needed resources, the setting of budgets, or in the context of a hard budget constraint, to estimate the available 'headroom' for the addition of new medicines to a national formulary. While-long term projections appear to draw mainly on demand side parameters and historical expenditure patterns, short-term projections are more volatile and more readily influenced by supply-side movements and market dynamics. Even with sophisticated modelling techniques and detailed market intelligence, such projections are not straightforward.

90.   A foundational element is effective *horizon scanning*, to identify late stage products in global industry pipelines. This is a resource intensive activity for which most countries cannot allocate substantial resources. The BeNeLuxA initiative aims to augment the effectiveness of individual country efforts through cooperation and collaboration, not only by aggregating sparse resources but also reducing unnecessary duplication. Similarly, the EC's proposal on joint EU-wide HTA also includes a provision for cooperative horizon scanning (European Commission, 2018[1]). While industry pipelines are essentially global, the systematic monitoring and collation of country-specific data on *timing of market entry*, and in particular, the time from first marketing authorisation (MA) to market launch, and from MA to reimbursement or coverage in country, are important inputs. These parameters should be carefully monitored in individual countries.

91.   Anticipating the (comparative) *therapeutic value* and likely *price* of a product yet to enter the market will remain very challenging. Using maximum willingness to pay (WTP) in countries that have determined fixed or even floating WTP thresholds could provide a nominal proxy for the upper bound of the expected price, depending on assumptions on the comparative therapeutic value of the product. However, this is a complex undertaking that would carry a number of inherent risks including that of overestimating unit costs, or indeed, including projected costs for products for which funding (or indeed marketing approval) may not eventuate. In addition, as noted above, the proliferation of confidential *discounts and rebates* must also be factored into the estimations.

92.   Perceived or anticipated therapeutic value of a new product is also likely to influence the various drivers of *uptake and diffusion*: the *place in therapy* (first or later line therapy); whether the therapy is likely to be additive (i.e. used in combination with existing treatment options) or to displace older treatments, or whether it extends treatment to a previously untreated patient cohort. These are in turn influenced by the target population to be treated - requiring a knowledge of the underlying epidemiology and *burden of disease*;

the indications likely to be approved for marketing, and those likely to be accepted for coverage or reimbursement.

93.   Determining the nature and timing of *loss of exclusivity* (LoE) of a product, as a proxy for the timing of generic or biosimilar market entry, is essential to modelling the impact of generic and biosimilar competition. As noted previously, this requires not only access to multiple data sources, but also specialised skills in understanding the data obtained. Comprehensive public databases could improve access to the necessary intelligence both for generics/biosimilar manufacturers and analysts, but would not obviate the need for specific expertise in its interpretation. An alternative could be for countries/payers to require companies to provide comprehensive information on all forms of applicable IP protection as part of their applications for coverage/reimbursement.

94.   Data on past trends in *generic uptake* and their impact on markets (both volumes and prices) are useful to predict future effects of generic market entry, but less so for biosimilars. Biosimilar data remain sparse, not only because of the shorter history and smaller number of 'follow-on' products, but also because the uptake, acceptance and pricing effects of biosimilars appear to be more idiosyncratic, and seemingly dependent on drug class and location of use (i.e. hospital vs community), as well as on national/payer policies on pricing and substitution (QuintilesIMS, 2017[16]). Similarly, data on past trends in generic/biosimilar uptake may not be very informative in therapeutic classes where really innovative products are entering the market at a high pace.

95.   For effective short term projections, many model parameters that cannot be populated empirically must inevitably be driven by assumptions, thus highlighting the need for testing multiple scenarios and performing extensive multivariate sensitivity analyses.

96.   Above all, repeated comparison of actual trends to projected estimates is important for adjusting assumptions and improving both the confidence in, and the predictive value of these heavily parameter driven models, particularly if they are to be used to estimate the potential effects of proposals for policy reforms. This will also inform an assessment of the need for trade-offs between resource intensity and forecasting precision.

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Annex A. Pharmaceutical Expenditure and Budgeting Survey

In this survey, the OECD Secretariat aims to map current practices in tracking, budgeting and projecting pharmaceutical expenditure.

Please complete the survey using the *Checkbox* survey tool.

If you encounter any problems completing the survey please contact ruth.lopert@oecd.org or valerie.paris@oecd.org

Your response by May 25th 2018 would be appreciated.

Before proceeding with the questionnaire, please provide the contact information of the person primarily responsible for its completion.

Country*
Name*
Position
Organisation
Email*
Telephone

1. Pharmaceutical expenditure tracking

1a). In your country, does the government track pharmaceutical *expenditure* at national or sub-national level?

○ Yes (go to 1a)

○ No (go to 1e)

1b) If YES, is the expenditure disaggregated?

Select all that apply

○ By region

○ By type of payer

○ By therapeutic area (eg cardiovascular disease)

○ By ATC code (please specify level)

○ Expenditure is not disaggregated

1c) If you answered YES to question 1, please describe the mechanism(s) used to track expenditure

1d) For how many years have expenditure data been tracked?

1e) If you answered NO to question 1, is *any* expenditure tracking undertaken?

○ Yes

○ No

If YES, please describe the scope of expenditure tracking undertaken

2. Pharmaceutical Utilisation Tracking

2a. In your country, does the government track public pharmaceutical *utilisation* at national or sub-national level?

○   Yes (go to 2b)

○   No (go to 2i)

2b) If YES, is the utilisation tracked ..?

Select all that apply

○   By therapeutic area (eg cardiovascular disease)

○   By setting of use (eg hospital, outpatient or ambulatory care)

○   By ATC level 3

○   By ATC level 4

○   By ATC level 5

○   By individual product

○   Other (*please specify*)

2c) If you answered YES to question 2, please describe the mechanism(s) used to track utilisation and/or provide a link to any documents describing the processes.

2d) For how many years have utilisation data been tracked?

2e) Are the data publicly available?

○   Yes

○   No

If YES, please provide a link

2f) Do you compare actual utilisation against projected or anticipated utilisation?

○   Yes

○   No

2g) If YES, is the comparison undertaken (tick all that apply)

○   By ATC level 2

○   By ATC level 3

○   By ATC level 4

○   By ATC level 5

○   Other (*please specify*)

2h) Are comparison (predicted vs actual) data publicly available?

○   Yes

○   No

If YES, please provide a link

2i) If you answered NO to question 2, is *any* tracking of utilisation undertaken?

○   Yes

○   No

If YES, please describe the nature and scope of utilisation tracking undertaken

3.   Pharmaceutical Budget Setting

Please read the definition below carefully before continuing

Definition

A budget for pharmaceuticals refers to a budget allocation for the purpose of public expenditure on pharmaceuticals by a national or a sub-national government, as part of the budgetary process. This budget may include all public spending related to pharmaceuticals, or only some categories of medicines (e.g. only medicines purchased in community pharmacies, or only high-cost medicines). In fact, some countries may budget separately different categories of medicines. This budget may or may not be directly managed by the level of government setting it.

Example 1: In New Zealand, since 2000, the Ministry of Health sets a "notional pharmaceutical budget" for the public funding of pharmaceuticals. The so-called "Combined Pharmaceutical Budget (CPB)" includes subsidies for medicines dispensed by community pharmacies and some medical devices, vaccines, haemophilia treatments, nicotine replacement therapy and cancer medicines which are sometimes given in hospitals. It does not include hospital medicines and devices, which are funded from local authorities' (District Health Boards) hospital budgets.

https://www.pharmac.govt.nz/about/your-guide-to-pharmac/factsheet-08-managing-combined-pharmaceutical-budget/

3a) In your country, does the government set a _budget_ for public pharmaceutical spending at national or sub-national level as part of the budgetary process?*

○   Yes (go to 3b)

○   No (go to 4)

3b) What are your country's policy objectives in setting a pharmaceutical budget? *Please select all that apply*

○   To control spending

○   To prioritise expenditures according to health priorities

○   To allocate a fixed share of health expenditure to pharmaceuticals

○   Other, *please specify*

3c) What does this budget cover?

Please select all that apply. If different budgets for pharmaceutical spending are defined, please only respond about the budget accounting for the largest share of public expenditure.

- ○ Spending for medicines dispensed in community pharmacies
- ○ Spending for medicines administered in outpatient settings (e.g. primary care practices, outpatient specialist clinics etc)
- ○ Spending for medicines dispensed or administered in hospitals (e.g. as part of an outpatient or inpatient treatment) and included in global budgets or DRG-payments
- ○ Spending for medicines used in treatment for specific conditions (e.g. high-cost cancer medicines prescribed and administered in inpatient settings) and paid "on top" of other hospital payments
- ○ Other, *please specify:*

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3d) Are budgets for pharmaceutical spending defined (please select all that apply):

- ○ At the central level
- ○ At subnational levels (regions, States, provinces)
- ○ At the financing system level (e.g. for the Health Insurance Fund)
- ○ Other, *please specify*

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3e) Which of the following issues are taken into account in setting this budget?

Please select all that apply.

- ○ Macro-economic factors (eg. economic growth)
- ○ Overall fiscal/government budget constraints
- ○ Demographic trends
- ○ Epidemiologic trends
- ○ Clinical guidelines involving pharmaceutical treatments
- ○ Past growth trends in pharmaceutical spending
- ○ Anticipated patent expiry dates for on-patent products
- ○ Potential generic or biosimilar entry and uptake
- ○ Anticipated market entry of new medicines
- ○ Prices of new medicines
- ○ Recent market entry and coverage determinations of new medicines
- ○ Other, *please specify*

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3f) Who is involved in the preparation of this budget (that is, directly involved or consulted during the preparation)?

Please select all that apply.

- ○ Ministry of Health
- ○ Ministry of Finance/ Treasury
- ○ Health care payers (e.g. compulsory health insurance funds, regional authorities, etc.)
- ○ Other, *please specify:*

3g) Who makes the final decision?

Please select all that apply

- ○ Ministry of Health
- ○ Central Budget Authority (e.g. Ministry of Finance)
- ○ Executive Cabinet or Agency, *please specify* _____
- ○ National Parliament
- ○ Regional/Local authority, *please specify* _____
- ○ Independent body, *please specify* _____
- ○ Other, *please specify*

3h) If possible, please indicate the budgeted amounts for pharmaceuticals for 2018 and provide links to any official documents where these budget items appear.

If different budgets for pharmaceutical spending are defined, please respond only for the budget item that accounts for the largest share of public expenditure. Please indicate the budgeted amount and provide any links.

3i) If budgets are set at the sub-national level, please provide one example of such a budget and provide a link to an official document where this budget item appears

Please indicate budgeted amount and provide any links.

4. Defining caps on pharmaceutical spending

Please read the definition below before continuing.

Definition

A cap on pharmaceutical spending refers to an upper limit on spending or spending growth beyond which, for example, pharmaceutical companies may be required to pay rebates to public payers. Please note that this does not refer to the setting of a ceiling within a budgetary process.

- *In the United Kingdom, the 2014 Pharmaceutical Price Regulation Scheme signed by pharmaceutical companies and the government set a limit on growth in the overall cost of branded medicines purchased by the NHS from companies that are members of the scheme. Beyond this limit, companies are requires to pay a rebate to the NHS. The allowed growth rate for 2018 is 1.9% (Section 6 of the PPRS and Annex 3 from page 69).*

  https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/675465/The_pharmaceutical_price_regulation_scheme_2014.pdf

- *In France, the Parliament votes annually on a cap on growth in the overall turnover of pharmaceutical companies on reimbursable medicines, beyond which companies must pay rebates to social insurance funds. In 2018, two separate caps were set: 0% for pharmaceuticals dispensed to outpatients and 3% for those pharmaceuticals dispensed in hospitals for which prices are not included in DRG tariffs.*

4a) Does your country set a <u>cap</u> for total and/or public pharmaceutical expenditure?*

○   Yes (please answer 4b and 4c)

○   No

Comments/clarifications (if any):

4b) If YES, who determines the cap for total and/or public pharmaceutical expenditure?

Please select all that apply.

If different caps are set by different entities, please respond only with respect to the cap reflecting the largest share of public expenditure

○   Ministry of Health

○   Central Budget Authority (e.g. Ministry of Finance)

○   Executive Cabinet or Agency, *please specify* _____

○   National Parliament_

○   Negotiation between government and pharmaceutical industry

○   Regional/Local authority, *please specify* _____

○   Independent body, *please specify* _____

○   Other, *please specify* _____

4c) What criteria are taken into account in defining the cap? *Please select all that apply.*

○ Macro-economic factors

○ Overall fiscal/government budget constraints

○ Demographic trends

○ Epidemiologic trends

○ Clinical guidelines involving pharmaceutical treatments

○ Past trends in pharmaceutical spending

○ Patent expiry dates for on-patent products

○ Potential generic or biosimilar entry and uptake

○ Medicines in development (pipeline)

○ Predictable changes in medicines distribution costs

○ Other, *please specify*

4d) If possible, please indicate the spending cap(s) set for 2018 (in absolute value or growth rate, or both) and provide links to any official documents in which these caps appear.

Please indicate cap(s) and provide a link

5. Projecting pharmaceutical spending

5a) Are any projections or forecasts of total and/or public pharmaceutical spending currently prepared in your country?

○ Yes

○ No (please go to question 5k)

5b) Which entity is responsible for preparing these projections or forecasts?

Please select all that apply.

○ Ministry of Health

○ Ministry of Finance

○ Office for budget responsibility

○ Other, *please specify*

5c) Do these projections cover the pharmaceutical budget as a whole?

○   Yes

○   No, projections are carried out for different sub-budgets separately (please specify)

○   Other, *please specify*

5d) How frequently are these projections updated?   _____

5e) Are these projections/forecasts undertaken as a stand-alone exercise or embedded in a model of overall health spending?

○   Stand-alone

○   Embedded in a broader forecasting model

○   Other*, please specify:*

5f) Which of the following inputs are included in the projections?

Please select all that apply.

○   Demographic trends

○   Population burden of disease

○   Horizon scanning

○   New medicines expected to receive marketing authorisation and/or be reimbursed by health coverage schemes

○   Budget impact as estimated in applications/assessments for reimbursement or coverage

○   Past trends in pharmaceutical spending

○   Changes in generic/biosimilar uptake

○   Changes in medicine prices

○   Changes in prescribing or treatment patterns

○   Horizon scanning

○   Other, pl*ease specify*:

5g) Please describe the type of modelling undertaken, or provide links to any documents describing the approach used.

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5h) What period do the projections cover?

Please select all that apply.

○ Between 0-5 years
○ Between 6-10 years
○ Between 10-25 years
○ Other or over 25 years, *please specify* _____

5i) Are pharmaceutical spending projections publicly available?

○ No, the projections are for internal purposes only
○ Yes, they are published and available to the public

If YES, please provide a link to where projections may be found

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5j) Is any *ex-post* assessment of the predictive value of the projections undertaken?

○ No
○ Yes

 If YES, please describe the results to date or provide a link to any relevant document(s)

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5k) If pharmaceutical spending projections are not used, please indicate the reasons why.

Please select all that apply.

○ Not useful in the context of our health system
○ Lack of data
○ Lack of resources and/or necessary skills
○ Not needed for budget setting
○ Other, *please specify*

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6. Horizon scanning

If you undertake any form of horizon scanning please describe the scope, approach used and data sources used.

Please provide links to any relevant documents

Annex B. Pharmaceutical Expenditure and Budgeting Survey- Additional Data Tables

Table B.1. Pharmaceutical expenditure tracking at national or subnational level in 22 OECD and EU countries in 2018

		Expenditure tracking at national or subnational level								Available period	Coverage (1)	Public availability
		By region	By payer	By care setting	By therapeutic area	By ATC Level	Which ATC level	At product level	Not disaggregated			
Australia	Yes	1	1	1	1	1	1 to 2	0	0	Since 1992	C/X	Yes
Austria	No											
Belgium	Yes	0	0	0	0	1	5	1	0	Since 1996	C/ O,H,S*,I	
Cyprus	Yes	0	0	0	0	0		0	0	10 years		Yes
Czech Republic	Yes	1	0	1	1	1		1	0	2015-2017	C/ O,H*,S,I*	
Estonia	Yes	1	0	0	1	1	5	1	0	15	C/ O,H,S	
Finland	n.a.								0			
France	Yes	1	1	1	1	1	5	1	0	> 10 years	C/O,H*	Yes
Ireland	Yes	0	0	0	0	1	5	1	0	21: 1998-2018		
Italy	Yes	1	0	1	1	1	5	1	0	>15 years		Yes
Japan	Yes	0	0	0	0	0		0	1	8 years		
Latvia	Yes	1	0	0	1	0		1	0	Since 2004	C/O,H,S,I	Yes
Lithuania	Yes	1	1	0	1	1	5	1	0	8	C/O	
Luxembourg	Yes	0	0	0	0	1	5	1	0	5		No
Malta	Yes	0	0	0	0	0		0	1	6		No
Netherlands	Yes	1	1	1	1	1	5	1	0	15-20		
Norway (ambulatory)	Yes	1	1	0	0	1	5	1	0	2004	C/O	Yes
Norway (hospital)	Yes	1	1	0	1	0		1	0	2010	O/H	Yes
Poland	Yes	1	0	0	0	0		0	0			No
Portugal	Yes	1	0	1	1	1	5	1	0	20		Yes
Republic of Korea	Yes	1	0	1	1	1	5	1	0	Since 2001		Yes
Sweden	Yes	0	1	0	1	1	5	0	0	Since 2003	C,O,H,S,I	No
Switzerland	Yes	1	0	0	0	0		0	0	23 years		Yes

Note: (1) "Coverage" indicates whether expenditure tracking covers all medicines (A), or only medicines covered by the main health coverage scheme (C) and whether it covers medicines dispensed to patients in outpatient care (O) and/or dispensed or administered to inpatient in hospitals (H), or administered to patients in physician settings (S) or other institutions (I). Partial coverage is marked *.

1= Yes, 0= No, In Austria, the main Association of Austrian Social Security Institutions: reimbursed medicines in the outpatient sector. Statutory sickness funds: reimbursed medicines Statistics Austria: System of Health Accounts.

Norway provided two responses to distinguish responses dispensed by pharmacies (including both medicines financed by the hospitals and medicines financed by the National Health Insurance scheme) and medicines financed by hospitals for inpatient use.

Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

Table B.2. Pharmaceutical utilisation tracking at national or sub-national level

	Utilisation tracking at national or sub-national level	By setting of use (e.g. hospital, outpatient or ambulatory care)	By therapeutic area (e.g. cardiovascular disease)	By ATC level 3	By ATC level 4	By ATC level 5	By individual product	Availability period	Publicity
Australia	Yes	1	1	1	1	1	1	Since 1992	Yes
Austria	No								
Belgium	Yes	1	0	1	1	1	1	Since 1996	Yes
Cyprus	Yes	1	1	0	1	1	0	2014	No
Czech Republic	Yes	1	1	1	0	0	1		No
Estonia	Yes	0	1	1	1	1	1	15 years	Yes
Finland	n.a.	0	0	0	0	0	0		
France	Yes	1	1	0	0	1	1		
Ireland	Yes	0	0	1	1	1	1	21 years	No
Italy	Yes	1	1	1	1	1	1	> 15 years	Yes
Japan	Yes	0	0	0	0	0	0	8 years	No
Latvia	Yes	1	1	0	0	0	1	Since 2004	Yes
Lithuania	Yes	0	1	1	1	1	1		Yes
Luxembourg	Yes	0	0	0	0	1	1	10 years	Yes
Malta	Yes	1	0	0	0	0	0	Not known	Yes
Netherlands	Yes	1	1	1	1	1	1	15-20 years	Yes
Norway (ambulatory)	Yes	0	0	1	1	1	1	Since 2004	Yes
Norway (hospital)	Yes	1	1	1	1	1	1	Since 2010	No
Poland	Yes	0	0	0	0	0	0		No
Portugal	Yes	1	1	1	1	1	1	20 years	Yes
Republic of Korea	Yes	1	1	1	1	1	1	Since 2001	Yes
Sweden	Yes	1	1	1	1	1	0	Since 2006	Yes
Switzerland	No								

Note: 1= Yes, 0= No, Norway provided two responses to distinguish medicines dispensed by pharmacies (including both medicines financed by the hospitals and medicines financed by the National Health Insurance scheme) and medicines financed by hospitals for inpatient use. .
Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

Table B.3. Countries where governments sets budgets for public pharmaceutical spending at national or sub-national level as part of the budgetary process

		Australia	Belgium	Cyprus	Estonia	Ireland	Italy	Latvia	Lithuania	Malta	Norway	Poland	Sweden	Netherlands
Objectives	Control spending	1	1	1	1	1	1	1	1	0	1	1	0	1
	Prioritise expenditures according to health priorities	0	0	1	1	1	0	0	0	0	0	0	1	0
	Allocate a fixed share of health expenditure to pharmaceuticals	0	0	1	0	1	1	1	0	1	0	1	1	0
Budget scope	Medicines dispensed in community pharmacies	1	1	0	1	1	1	0	1	0	1(a)	1	0	1
	Medicines administered in outpatient settings	1	1	1	1	1	1	1	0	0	0	0	1	1
	Medicines dispensed or administered in hospitals and included in global budgets or DRG-payments	1	1	1	1	1	1	0	0	0	0	0	0	0
	Medicines used in treatment for specific conditions and paid on top of other hospital payments	1	1	1	1	1	1	0	0	0	0	0	0	0
	Other	(b)					(c)			(d)		(e)	(f)	
Budget scope	At the central level	1	1	1	1	1	1	1	1	1	1	1	1	1
	At subnational levels (regions, States, provinces)	0	1	0	0	0	1	0	0	0	0	0	1	0
	At the financing system level (e.g. for the Health Insurance Fund)	0	0	0	0	0	0	1	1	0	0	0	0	0
	Other							(g)						

		Australia	Belgium	Cyprus	Estonia	Ireland	Italy	Latvia	Lithuania	Malta	Norway	Poland	Sweden	Netherlands
Aspects taken into account in budget setting	Macro-economic factors (e.g. economic growth)	0	1	0	0	1	1	0	1	1	0	0	1	0
	Overall fiscal/government budget constraints	1	1	1	1	1	1	1	1	1	0	0	1	1
	Demographic trends	1	1	0	0	1	1	0	0	1	0	0	1	0
	Epidemiologic trends	1	1	0	0	1	1	0	0	1	0	0	1	0
	Clinical guidelines involving pharmaceutical treatments	1	1	1	0	1	0	0	0	1	0	0	1	0
	Past growth trends in pharmaceutical spending	1	1	1	1	1	1	1	1	1	1	0	1	1
	Patent expiry dates for on-patent products	0	1	1	1	1	1	0	0	1	0	0	1	0
	Potential generic or biosimilar entry and uptake	0	1	1	1	1	1	0	1	1	1	0	1	0
	Anticipated market entry of new medicines	1	1	1	1	1	0	0	0	1	0	0	1	1
	Prices of new medicines	1	1	1	1	1	1	1	0	1	1	0	1	0
	Recent market entry and coverage determination of new medicines	1	1	1	0	1	1	0	0	1	1	0	1	0
	Other	(h)										(i)		
Entities involved in budget preparation	Ministry of Health	0	1	1	1	1	1	1	1	1	1	1	1	1
	Ministry of Finance/Treasury	0	1	1	1	1	1	1	1	1	1	1	1	1
	Health care payers (e.g. compulsory health insurance funds, regional authorities, etc.)	0	1	0	1	1	1	1	1	0	0	1	0	0
	Pharmaceutical industry	0	1	0	0	0	0	0	0	0	0	1	0	0
	Other	(j)									(k)			

		Australia	Belgium	Cyprus	Estonia	Ireland	Italy	Latvia	Lithuania	Malta	Norway	Poland	Sweden	Netherlands
Final decision maker	Ministry of Health	0	0	0	0	0	1	0	0	0	0	0	0	1
	Central Budget Authority (e.g. Ministry of Finance)	0	0	1	0	1	1	0	0	0	0	1	1	0
	Executive Cabinet or Agency	0	0	0	0	0	0	0	0	0	0	0	0	0
	National Parliament	1	0	0	0	0	0	1	1	1	1	0	0	0
	Regional/Local authority	0	0	0	0	0	0	0	0	0	0	0	0	0
	Independent body	0	0	0	0	0	0	0	0	0	0	0	0	0
	Other	(l)	(m)		(n)									

Note: 1= Yes, 0= No. Austria, Czech Republic, France, Japan, Luxembourg, Norway, Portugal, Korea and Switzerland do not set budgets for public expenditure on medicines.

(a) The budget is set for medicines financed by the National Insurance scheme. The medicines may be dispensed in any pharmacy, community or hospital. (b) Distribution costs. (c) Innovative medicines can have access to dedicated funds (Fund for innovative oncologic products; Innovative non oncologic products). (d) All medicines supplied free to the community pharmacies and the NHS. The private sector sells other medicines not funded by government. (e) Fixed pharmaceutical budget covers pharmacy reimbursement, drug programmes and medicines used in chemotherapy. (f) Medicines that are assessed as cost-effective by TLV and therefore subsidised by the government. (g) At prescribers (physicians) level. (h) Anticipated patent expiry dates for on-patent products, Potential generic or biosimilar entry and uptake, Anticipated market entry of new medicines, Prices of new and amended medicine listings, Recent market entry and coverage determinations of new medicines, Legislated price amendments, Macro-economic factors (e.g. changes in the Australian Consumer Price Index). (i) Budget for pharmaceuticals is fixed. Yearly limit of pharmaceutical expenditure is 17% of total budget for health care services financed from public funds. (j) Australian Government Department of Health, Department of Veterans' Affairs, Department of the Treasury, Department of Finance, Department of the Prime Minister and Cabinet, Department of Human Services, Department of Social Services. (k) Norwegian Directorate of Health. (l) Executive Cabinet of the government. (m) The King, after deliberation by the Council of Ministers (=federal government) (cf. Law of 14 July 1994 - art. 69 Â§ 5). (n) Board of Health Insurance Fund

Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

Table B.4. Countries with a cap on total and/or public pharmaceutical expenditures

Country	Who determines the cap?	Macro-economic factors	Overall fiscal/government budget constraints	Demographic trends	Epidemiologic trends	Clinical guidelines involving pharmaceutical treatments	Past trends in pharmaceutical spending	Patent expiry dates for on-patent products	Potential generic or biosimilar entry and uptake	Medicines in development (pipeline)	Predictable changes in medicines distribution costs
Belgium	Negotiation between government and pharmaceutical industry	1	1	1	1	1	1	1	1	1	1
Cyprus	Central Budget Authority (e.g. Ministry of Finance)	0	1	0	0	1	1	1	1	1	1
Czech Republic	Proposal by health insurance funds; validated by MoH and MoF; approved by Chamber of Deputies of the Parliament	1	1	0	1	0	1	0	1	1	1
Estonia	National Parliament	0	1	0	0	0	0	0	0	0	0
France	Ministry of Health	0	1	1	1	1	1	1	1	1	1
Ireland	Central Budget Authority (e.g. Ministry of Finance)	1	1	1	0	1	0	1	1	1	1
Italy	Executive Cabinet or Agency (please specify below)	0	0	0	0	0	1	1	1	0	0
Latvia	Cabinet of Ministers (regulation)	0	1	0	0	0	1	0	0	0	0
Malta	Central Budget Authority (e.g. Ministry of Finance)	1	1	0	0	0	1	0	0	0	0
Poland	Ministry of Health	0	0	0	0	0	0	0	0	0	0

Note: Australia, Austria, Finland, Japan, Korea, Latvia, Lithuania, Netherlands, Norway, Portugal, Sweden, Switzerland reported they do not define a cap on total and/public pharmaceutical spending.
Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

Table B.5. Countries developing projections or forecasts of total and/or public pharmaceutical expenditure

Country / Availability of projections	Responsible institution(s)	Do projections cover the pharmaceutical budget as a whole?	Frequency of updates	Stand-alone exercise or embedded in a model of overall	Inputs included in projections									
					Demographic trends	Population burden of disease	New medicines expected to receive	Budget impact as estimated in	Past trends in pharmaceutical	Changes in generic/biosimilar	Changes in medicine prices	Changes in prescribing or	Horizon scanning	Other
Australia (published)	Australian Government - Department of Health, Department of Finance, Department of Veterans Affairs.	No, projections are carried out for different sub- budgets separately	2x/year	Stand-alone	1	1	1	1	1	1	1	1	1	(a)
Austria	Statutory sickness funds and Main Association of Austrian Social Security Institutions	Yes	4x/year	Stand-alone	0	0	0	0	0	0	0	0	0	(b)
Belgium	NIHDI, industry, Minister of Social Affairs	No, projections are carried out for different sub- budgets separately (c)	2x/year	Embedded	1	1	1	0	1	1	1	1	1	
Cyprus	Ministry of Health	No, projections are carried out for different sub- budgets separately	Annually	Stand-alone	0	1	0	0	1	1	1	1	1	
Czech Republic	Ministry of Health in cooperation with the Institute of Health Information and Statistics of the Czech Republic	No, projections are carried out for different sub- budgets separately (d)	Annually	Stand-alone	1	1	1	1	1	0	0	0	1	
Estonia (published)	Health Insurance Fund	Yes	Annually	Embedded	0	0	0	1	1	1	1	1	0	

Country / Availability of projections	Responsible institution(s)	Do projections cover the pharmaceutical budget as a whole?	Frequency of updates	Stand-alone exercise or embedded in a model of overall...	Inputs included in projections									
					Demographic trends	Population burden of disease	New medicines expected to receive	Budget impact as estimated in	Past trends in pharmaceutical	Changes in generic/biosimilar	Changes in medicine prices	Changes in prescribing or	Horizon scanning	Other
France	Ministry of Health; Ministry of Finance	Yes	Several times per year	Embedded	1	1	1	1	1	1	1	1	1	
Ireland (published)	Ministry of Health, Ministry of Finance, Health Service Executive (HSE)	Yes	Monthly	Embedded	1	1	1	1	1	1	1	0	0	
Japan	Ministry of Health		Annually	Embedded	0	0	0	1	0	0	0	0	0	
Latvia	Ministry of Health	Yes	Annually	Embedded	0	1	0	1	1	0	0	0	0	
Lithuania (published)	NHIF	No, projections are carried out for different sub-budgets separately (e)	2x/year	Stand-alone	0	0	1	1	1	0	0	1	0	
Luxembourg	National health insurance (CNS)	No, projections are carried out for different sub-budgets separately (f)	2x/year	Embedded	1	0	1	1	1	1	1	0	0	
Malta	Ministry of Health	Yes	Monthly	Stand-alone	0	0	0	0	1	0	0	0	0	
Netherlands (published)	Ministry of Health, Zorginstituut Nederland	Yes	2x/year	Stand-alone	0	0	1	0	1	0	1	0	1	

Country / Availability of projections	Responsible institution(s)	Do projections cover the pharmaceutical budget as a whole?	Frequency of updates	Stand-alone exercise or embedded in a model of overall ...	Inputs included in projections									
					Demographic trends	Population burden of disease	New medicines expected to receive	Budget impact as estimated in	Past trends in pharmaceutical	Changes in generic/biosimilar	Changes in medicine prices	Changes in prescribing or	Horizon scanning	Other
Norway	Norwegian Directorate of Health	Yes	4x/year	Embedded	0	0	0	1	1	1	1	0	0	
Portugal	INFARMED/ Ministry of Health	Yes		Stand-alone	0	0	0	0	1	0	0	0	0	
Sweden (published)	The National Board of Health and Welfare	Yes	2x/year	Stand-alone	0	0	1	0	1	1	1	0	1	

Note: Italy, Poland, Korea, Switzerland and Finland do not forecast pharmaceutical expenditures
(a) Past trends in pharmaceutical utilisation; current medicine price; legislated changes in prices; ad hoc price changes or new price offers; recent market entry of new medicines; changes to the contribution amounts paid by patients; fees determined under the Community Pharmacy Agreement; economic indexation; new or amended medicine listings on the PBS, RPBS and LSDP. (b) Expert opinion. (c) Community pharmacy, outpatient setting, in patient setting. (d) The projections only include health insurance companies' expenditure on special group of innovative medicines prescribed by special centres. (e) CHIF. (f) Projections exclude vaccines and is limited on reimbursed medicines
Source: 2018 OECD Survey on Pharmaceutical Expenditure and Budgeting

Annex C. Bibliographic search

Selection of papers on pharmaceutical expenditure projections

An initial search was conducted using Scopus and the following keywords: (projection or forecast) and ("pharmaceutical expenditure" or "pharmaceutical spending" or "drug expenditure" or "drug spending") for the period starting in 1990. This search yielded 61 results, of which 34 cover the United States. Papers were selected for review with the following criteria:

- French or English language;
- Focus on pharmaceutical expenditures;
- Coverage of one or several OECD or EU countries at national level;
- Not focusing on specific age groups (e.g. children, seniors) or circumstances (proximity to death);
- Not focusing on a specific disease or therapeutic area.
- Most recent version when the projection was updated annually.

The original list of publications is below, with references reviewed in bold. Several other publications, mainly from governments or statistical institutes were brought to our attention by countries. They have been considered to the extent of possible. Projections from two widely-known private companies have also been considered.

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Erratum: National trends in prescription drug expenditures and projections for 2016 (Special Feature (2016) (1069))(2016) American Journal of Health-System Pharmacy, 73 (17), p. 1294.
DOI: 10.2146/cor160008

Aitken, M., Berndt, E.R., Cutler, D., Kleinrock, M., Maini, L.(2016), Has the era of slow growth for prescription drug spending ended? , *Health Affairs*, 35 (9), pp. 1595-1603. DOI: 10.1377/hlthaff.2015.1636

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