Supplementary Material

Exploring the feasibility of monitoring access to novel medicines: A pilot study in EU Member States

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This document presents material that supplements:
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Annex A. Glossary

Table A A.1. Glossary of terms used in the paper

Term	Definition
Advanced therapy medicinal products (ATMP)	A medicine for human use that is based on genes, cell, or tissue engineering.
Anatomical Therapeutic Code (ATC)	A unique code assigned to a medicine according to a hierarchy system based on therapeutic use or pharmacological class. The classification system is maintained by the World Health Organization. See https://www.whocc.no/atc/structure_and_principles/
Cost sharing arrangements	***Fixed co-payment:*** fixed sum paid by an insured individual for the consumption of itemised health care services (e.g. per prescription item). May also be referred to as a user fee, or prescription fee. ***Coinsurance:*** cost-sharing requirement whereby the insured person pays a fixed proportion of the cost of the medication. Sometimes referred to as *percentage co-payment*. ***Deductible:*** Threshold below which an insured person must pay out-of-pocket for medications before insurance coverage begins. Deductibles can be applied to a specific category of care (e.g. pharmaceutical spending) or to all health expenditures (general deductible). May be also referred to as an 'excess' in some countries. ***Extra-billing*:** refers to any difference between the price charged and the price used as the basis for determining the extent of reimbursement. An example is where a fixed reimbursement amount is determined for a product, but sellers remain free to charge higher prices. The patient pays out-of-pocket any difference between the price of the medicine and the reimbursement amount, in addition to any co-payment. ***Out-of-pocket (OOP) payments*:** include spending by people without coverage; spending on medicines that are not covered; and cost-sharing (deductibles, co-payments, coinsurance, extra-billing).
Defined daily dose (DDD)	Defined daily dose (DDD) is the assumed average maintenance dose per day for a medicine used in its main indication in adults. It is a unit of measurement and does not necessarily reflect the recommended or prescribed daily dose. Therapeutic doses for individual patients and patient groups will often differ from DDD as they will be based on individual characteristics. Only one DDD is assigned per ATC code and route of administration (e.g. oral formulation). See https://www.whocc.no/ddd/definition_and_general_considera/
Early access scheme (EAS)	Scheme or program that makes a product available to a limited number of patients at the initial stages in the lifecycle of a medicine, i.e. *prior* to marketing authorisation and/or the publicly funded coverage decision in a country. There are different types of early access schemes in Europe, for example: ***Population-based programmes*:** compassionate use programs initiated by pharmaceutical companies for a group of patients in a selected clinic or hospital. In some countries, population-based programs are broader and extend to the *entire* target population within the scope of an authorised indication. ***Named-patient programmes*:** granted in response to requests by physicians on behalf of specific (i.e. named) patients, on a case-by-case basis. Early access schemes may be funded by pharmaceutical companies (i.e. ***industry sponsored***) or third-party payers (i.e. ***government or insurer sponsored***). This report considers publicly funded population-based programmes that benefit the entire target population within an indication as "early access coverage schemes".
Health Technology Assessment (HTA):	A multidisciplinary process that determines the value of a health technology at different points in its lifecycle. HTA informs decision-making to promote an equitable, efficient, and high-quality health system. It is an evidence-informed process that determines whether the new therapy represents acceptable value for money in the context of its proposed use and based on an assessment of evidence of comparative clinical and cost-effectiveness.
Managed entry agreement (MEA)	Arrangements between firms (i.e. pharmaceutical company) and healthcare payers that allow for coverage of new medicines while managing uncertainty around their clinical performance, cost-effectiveness and/or budget impact (see taxonomy of MEA on p.14, Wenzl & Chapman, 2019 available at https://doi.org/10.1787/6e5e4c0f-en). Managed entry agreements do not include public procurement.

Term	Definition
Marketing authorisation (MA)	A license issued by a medicines' agency approving a medicine for market use based on a determination by authorities that the medicine meets the requirements of quality, safety, and efficacy for human use. It is a rigorous process, in which the balance of benefits and risks is assessed using data from pre-clinical and clinical studies. Also known as *marketing approval* or *regulatory approval*. In the European Union, additional terminology exists related to marketing authorisation: ***Centralised procedure:*** The European-wide procedure for the authorisation of medicines, where there is a single application, a single evaluation and single authorisation throughout the European Union. Only certain medicines are eligible for the centralised procedure. The evaluation takes a maximum of 210 days. ***Accelerated assessment:*** Rapid assessment of medicines in the centralised procedure that are of major interest for public health, especially those that are considered therapeutic innovations. Accelerated assessment usually takes 150 evaluation days, rather than 210. ***Conditional marketing authorisation:*** The approval of a medicine that addresses unmet medical needs on the basis of less comprehensive data than normally required. The available data must indicate that the medicine's benefits outweigh the risks and is contingent on the applicant providing the comprehensive clinical data in the future. ***Additional monitoring:*** The medicine needs to be monitored more closely than other medicines, generally because there is less information available on it (e.g. if new to market or limited long-term use data). ***Approval under exceptional circumstances:*** The applicant was unable to provide comprehensive efficacy and safety data of the medicine under normal conditions of use (e.g. if it treats a rare condition or collection of comprehensive information is not possible or is unethical).
Marketing Authorisation Holder (MAH):	The person or company who holds the authorisation to place a medicine on the market and is responsible for marketing it. They are licensed to distribute, sell, and commercialise a medicinal product.
Orphan medicine	A medicine for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition that is considered rare (not more than five cases in 10,000 people in the European Union) or where the medicine is unlikely to generate sufficient profit to justify research and development costs. ***Orphan designation:*** A specific status assigned to a medicine intended for use against a rare condition. The medicine must fulfil certain criteria for this designation so that it can benefit from incentives such as protection from competition once on the market.
Prescribed daily dose (PDD)	Prescribed daily dose (PDD) is the usual prescribed therapeutic dose for adult patients with normal renal function. It is not the same as DDD.
Prices	***Ex-factory price (list ex-factory price, manufacturer price, ex-manufacturer price, manufacturer's selling price, manufacturer's list price):*** The manufacturer's posted price of a pharmaceutical or other product. This generally excludes any confidential discounts or rebates to payers. ***Wholesale price (pharmacy purchase price):*** The price charged by wholesalers to the retailers (usually community pharmacies). It is based on the ex-factory price together with remuneration for the pharmaceutical wholesaler (e.g. in the form of a wholesale mark-up or margin). ***Pharmacy retail price (retail price, consumer price):*** The price charged by community pharmacies to the general public, usually based on the wholesale price with the addition of pharmacy remuneration in the form of a pharmacy mark-up or margin, and in many cases, a dispensing fee. Consumer prices can include or exclude value-added tax (net and gross retail prices, respectively). ***Reimbursement price (reimbursement list price):*** The maximum amount of reimbursement to a pharmacy paid by a third-party payer (e.g. a health system or insurer) excluding any adjustment for patient co-payment or coinsurance.

Term	Definition
Publicly funded coverage (or reimbursement)	Coverage of the costs of medicines which are eligible for reimbursement by a public payer (such as social health insurance / National Health Service) after a positive coverage decision. A decision or recommendation on coverage may be made at national, regional, or in some cases insurer/hospital level. Costs may be fully covered by third-party payers, or only partially.
Reimbursement list	A list that contains medicines regarding their reimbursement status. They may either include medicines eligible for reimbursement (**positive list**) or those explicitly excluded from reimbursement (**negative list**). Reimbursement lists may target either the out-patient sector (usually positive lists or negative lists) or the in-patient sector (typically called hospital pharmaceutical formulary), or both. **Positive list (i.e. formulary):** List of medicines that may be prescribed at the expense of a third-party payers (e.g. governments, insurers). **Negative list:** List of medicines that are not for reimbursement.

Source: Authors as cited. Some definitions come directly from the European Medicines Agency website (https://www.ema.europa.eu/en) as well as the glossary of the Pharmaceutical Pricing and Reimbursement Information (PPRI) Network (https://ppri.goeg.at/ppri-glossary), last accessed November 2022.

Annex B. Examples of existing initiatives

Table A B.1. Examples of existing initiatives measuring access to medicines

Initiative	Description	Frequency	Product/Disease Scope	Indicator(s)
Sustainable Development Goal Indicator (SDG) 3.b.3 by United Nations Statistical Division[1]	Indicator SDG 3.b.3 was developed to provide an index of access to medicines for a country and is defined as the "proportion of health facilities that have a core set of relevant essential medicines available and affordable on a sustainable basis". This indicator is obtained by surveying the presence and cost of a core basket of essential medicines in a sample of facilities in each country. It accounts for two dimensions: availability (i.e. a medicine that is found in a facility) and affordability (i.e. a medicine that requires no extra daily wage for the lowest unskilled government sector worker to purchase a daily dose of treatment of the medicine). When a medicine does not meet any of these requirements it is considered not accessible.	This indicator was intended to be obtained on a yearly basis; see Global SDG Indicators Database (https://unstats.un.org/sdgs/indicators/database/).	Each country must select from a basket of 32 medicines the ones that are relevant for the specific country. These medicines were identified to comprise acute and chronic conditions, communicable and non-communicable diseases in the primary health care setting, for example, asthma, diabetes, hypertension, cardiovascular diseases, depression, malaria, HIV, contraceptives, etc.	Steps to compute the indicator at the country level: (1) Select medicines relevant for the country. (2) Assign weights to each medicine, where the weights are given by the country's burden of disease of the condition treated/cured/controlled by the medicine. Specifically, the burden of disease is accounted for using disability adjusted life years (DALYs); then the weight of each medicine will be given by the proportion of the medicine-specific DALY with respect to the total sum of the DALYs of the medicines in the basket. (3) in each facility, to check if each medicine in the basket is both available and affordable; if both are satisfied, then the medicine is accessible; otherwise it is not accessible. (4) then, for each facility, aggregate (sum) the weights only for those medicines that are accessible. If a facility shows an aggregated sum of weights greater or equal than 80 percent, then that facility demonstrates access to medicine. (5) Finally, the access index for each country corresponds to the proportion of facilities that demonstrate access (i.e. aggregated sum of weights greater or equal to 80 percent). Data is collected through national and subnational surveys using methodology developed and implemented by WHO and Health Action International (HAI). Additionally, in 2016 WHO developed the Essential Medicines and Health Products Price and Availability Monitoring Mobile Application (WHO EMP MedMon), to monitor these medicines' prices and availability more cost-effectively. Prices collected correspond to retail prices, i.e. those paid in each facility. Data is obtained through facility visits by app data collectors, or by self-reporting sentinel facilities. Data on prices and affordability per medicine and country is publicly available online (See https://www.haiweb.org/MedPriceDatabase/).
Patients W.A.I.T. (Waiting to Access Innovative Therapies) Survey[2]	Survey conducted by the European Federation of Pharmaceutical Industries and Associations (EFPIA) and IQVIA. It aims at measuring availability of novel medicines approved centrally in the EU for 39 European countries, where availability is defined as "inclusion of a	Annually, since 2018. Running in evolving formats since 2004.	The latest version in 2021 analysed 160 new products approved centrally in the EU between January 2017 and December 2020, including 41 oncology products, 57 orphan drugs, 42 non-oncology orphan products,	Two core indictors that reflect availability and time-to-access for each country over a 4-year rolling cohort are computed: (1) rate of availability, measured as the total number of new medicines in each country that have obtained/achieved access to the reimbursement list; and (2) average time between marketing authorisation (MA) and patient

Initiative	Description	Frequency	Product/Disease Scope	Indicator(s)
	centrally-approved medicine on the public reimbursement list in a country" (i.e. patients can receive the medicine under a reimbursement scheme).		and 24 combination products.	access, computed by averaging the number of days elapsing from the date of the marketing authorisation (i.e. the date of central EU authorisation in most countries) to the day of completion of post-marketing authorisation administrative procedure , for each new medicine in each country. In 2021, a new indicator "rate of full availability" was added, measured as the proportion of medicines available to patients without any restrictions on patient population, or through named patient basis schemes. Results are presented per country and group of medicines, allowing for comparison between countries within groups of medicines (but not for differences between groups of medicines within countries or at the product-level).
Vintura Report 2020, Every day counts – presenting results from "Time to Patient Access" Initiative & Vintura Report 2021, Every day counts – Improving regulatory timelines to optimize patient access to innovative oncology therapies in Europe[3]	The 2020 report aimed to establish a common understanding of causes of delays in patient access to new oncology treatments, from the perspective of the pharmaceutical industry and relevant stakeholders. It also presented results of *the Patient Access Indicator*, which measures differences between EU member states in terms of use of oncology drugs. A follow-up report in 2021 "Every Day Counts – Improving Regulatory Timelines to Optimize Patient Access to Oncology Therapies in Europe" focused on the final stage of marketing authorisation. Both reports were initiated and financed by the Oncology Platform of EFPIA.	Two consecutive reports published in 2020 and 2021 respectively – not known if follow-up reports will be published.	Cancer medicines selected based on (1) positive opinion from the EMA between 2013-2017, (2) new active substances, (3) owned by one of members of EFPIA, and (4) manufacturer is willing and able to share uptake data. This results in 13 oncology therapies that cover: leukaemia, breast cancer, lung cancer, bladder cancer, multiple myeloma, melanoma, non-melanoma, skin cancer and ovarian cancer.	For each country a single patient access indicator was obtained by averaging the 'relative cumulative use' of each therapy during the first 12-months of the post-reimbursement period. The relative cumulative use for each therapy and country refers to the volume sold per month (or patients treated per month, based on volume sold), per capita, of each therapy with respect to the country with the highest use. Results for the indicator are presented at the country-level only. The benchmark illustrates differences rather than best practices. Data comes from routinely collected business information from pharmaceutical companies and private data providers.
Comparator Report on Cancer in Europe – Disease Burden, Costs and Access to Medicines[4]	The 2019 report describes the state of cancer in Europe (28 member states of EU and Iceland, Norway and Switzerland). It includes a section on access to and uptake of cancer medicines. The report was commissioned and funded by EFPIA and based on independent research by the Swedish Institute for Health Economics.	Previous versions published in 2005, 2007, 2009 and 2016.	Cancer medicines, covering a range of areas including, breast, colorectal, lung, prostate, and ovarian cancer, as well as malignant melanoma and multiple myeloma.	Access to cancer medicines is equated with market uptake, that is, usage measured as sales in volume (milligrams) or value (euros). In particular, usage is measured as 'sales relating to cancer mortality', i.e. volume or value per number of cancer deaths per country, to account for the disease burden of cancer in a country (need and demand for cancer care). Results are presented per country and per group of medicines, allowing for comparison between countries within groups of medicines (but not for differences between groups of medicines within countries). Data comes from MIDAS database maintained by IQVIA.
European Society for Medical Oncology (ESMO) study on	ESMO conducted a European and international study on the availability of anti-neoplastic medicines based on feedback from	Reports in 2016 & 2017.	Cancer medicines used in seven high-incidence cancers, including melanoma, renal cell cancer, lung	Whether it was permissible to prescribe the cancer medicine for this indication and if the medication is reimbursed for this indication (formulary availability); the proportion of the full retail price the average

Initiative	Description	Frequency	Product/Disease Scope	Indicator(s)
availability of oncology medicines[5]	ESMO members and oncology pharmacists. The study was based on results from a survey that consisted of three parts: part 1 consisted of six general questions regarding the country's healthcare system; part 2 surveyed the formulary of generic anticancer medication commonly used over a wide range of cancers, and part 3 surveyed the formulary of generic anticancer medication used in seven high-incidence cancers.		cancer, colorectal cancer, metastatic breast cancer and prostate cancer. The list of anticancer medications for each disease entity was derived from ESMO and National Comprehensive Cancer Network (NCCN) guidelines and 'UpToDate' subject reviews and included medications approved by either the US Food and Drug Administration (FDA) or the European Medicines Agency (EMA) as of March 2014.	patient pays for the medication (out-of-pocket costs); preauthorization requirements and delays of >4 weeks in the approval process (time-to-access); the actual availability of the medication for most patients in the country under question and, in cases where the medication is not always available, the reason(s) for lack of availability.
Reviews of HTA Outcomes Briefings, by Centre for Innovation in Regulatory Science (CIRS)[6]	CIRS publishes metrics aimed at monitoring regulatory and HTA performance. Specifically, it collects data on the appraisals of new active substances (NASs) made by 8 HTA agencies (Australia, Canada, England, France, Germany, Poland, Scotland, Sweden), to report on the synchronisation between the regulatory decision made in each country and the first HTA recommendations in terms of timing-decision and appraisal-outcomes. This report is part of the CIRS RD Briefing Series. The CIRS is a not-for-profit organisation, for industry, regulators, HTA agencies, and other healthcare stakeholders for the debate and advancing of regulatory, HTA and reimbursement policies.	Six reports have been published yearly since 2017: 2017 (studies 4 NASs for the 2014-2015 period); 2018 (studies 24 NASs for 2014-2017); 2019 (studies 38 NASs for 2014-2018); 2020 (studies 37 NASs for 2015-2019); 2021 (studies 26 NASs for 2016-20); 2022 (studies 37 NASs for 2017-20).	All NASs that received a recommendation by each of the 8 HTA agencies during a given period. For example, in the 2021 report 26 NASs were identified between 2016-2020 being appraised by these agencies. The NASs belong to major therapeutic classes, e.g. anti-cancer & immunomodulators, alimentary & metabolism, anti-infectives, blood and blood forming organs, and nervous system. Vaccines, applications with new clinical data, generics, applications for a new or additional name, among others, were excluded.	The reports present statistics related to the time elapsing between 'regulatory submission' and 'HTA recommendation', on one hand, and on the types of HTA recommendations, on the other, per jurisdiction (i.e. country-market of HTA agency), and per year (and in some cases per active substance), for the NASs analysed in each report. Time differences between regulatory approval and HTA decision is also broken-down into (1) regulatory authority review time, and (2) regulatory approval to HTA decision (at the national level). In the reports, the HTA decision for a NAS is categorised into four types: positive, positive with restrictions, and negative; and, when companies submit dossiers for another sub-indication with an approved regulator label, and the final HTA outcome for such sub-indication differs, the decision, for that NAS, is classified as multiple. Data on individual NASs appraised by HTA agencies for given periods is collected using public domain data retrieved from the agencies' official websites.
Access to medicines Index, a benchmark of pharmaceutical companies[7]	The Access to Medicine Index ranks 20 of the world's largest pharmaceutical companies according to three technical areas: governance of access, research and development, and product delivery.	Reports released every second year since 2008 (2008. 2010, 2012, 2014, 2016, 2018, 2021), with methodology every other year.	In 2021, the index covered 33 indicators, across 106 low and middle income countries, for 82 diseases, conditions and pathogens. Product scope includes medicines, microbicides, vaccines, vector control products, platform technologies, diagnostics, contraceptive methods and devices.	The methodology and indicators have developed over time. As per the 2022 methodology, the index consists of 3 technical areas, with 14 priority topics, and subsequent indicators per topic. A company's overall score is an aggregate of individual indicator scores, adjusted by the respective indicator, priority topic, and technical area weights. The weighting system is adjusted to reflect differences in companies' business models. The final scoring is the result of a multi-tiered analysis and quality assurance process. Among others, the index looks at how widely and rapidly companies filed to register their most recently launched products in LMIC countries; countries submitted a maximum of 10 recently launched products for analysis.

Initiative	Description	Frequency	Product/Disease Scope	Indicator(s)
European Medicines Agency's new IRIS system for reporting marketing status[8]	As of the end of July 2021, the European Medicines Agency (EMA) is using the IRIS database IT tool to better capture (i.e. monitor) marketing status information of centrally authorized products across EU member states. Marketing authorisation holders are asked to notify EMA of marketing status updates on these products (including withdrawn product notifications), to provide an overview of which products are marketed in which member states of the EU and EEA. The information is currently available to the European Commission and National Competent Authorities only. In the future, information on the availability of EU medicinal products might be made publicly available.	Ongoing from July 2021 – restricted access through an interactive dashboard.	Centrally authorized products in EU/EEA member states, at presentation level (i.e. pack size).	Marketing status information of centrally authorized products, by product presentation , for each Member State: 1) Marketing status (marketed / temporarily unavailable / not marketed) 2) Date of first marketing (i.e. initial placing on the market = the date of release into the distribution chain i.e. the date when the medicinal product comes out of the control of the MAH or importer) 3) Date of cessation 4) Estimated/actual/re-introduction date after temporary cessation 5) Reasons for marketing cessation
European Access Portal, operated by IQVIA on behalf of industry stakeholders (EFPIA)[9]	The European Access Portal provides a space where marketing authorisation holders can provide timely information on the timing and processing of pricing and reimbursement applications in EU countries, including reasons behind delays or non-submission. The portal is operated by IQVIA on behalf of industry stakeholders, including EFPIA, and released in April 2022. It aims to provide greater transparency relating to the availability of centrally approved products on EU markets. Submission of data into the portal is on a voluntary basis. Publication of information collected in the portal is under discussion – at this stage, disclosure of information at the aggregate or therapeutic level is likely appropriate, with greater transparency to be considered at a later stage.	Regular updating from January 2021 for a four year period. Report to be published every six months.	Innovative medicines or biosimilars that have been granted central marketing authorisation in their first indication in Europe from January 2021 for a four year period.	Ex-post data will be prefilled from public sources (e.g. published regulatory data, data submitted to IRIS and WAIT), and information submitted directly by pharmaceutical companies. Information collected in the portal includes: -Through which channels the product is available (full label reimbursed / partially reimbursed / early access programs (current only) / private market / compassionate use / other public (e.g. alternative public funding) / none) -Date of application to first step in local P&R process -Completion date of final step in P&R process -Whether or not the manufacturer has applied for reimbursement in the country -Reasons behind timing of the P&R process (process underway / delay due to misalignment on pricing and value / delay due to process involving associated infrastructure / negative outcome / delay due to misalignment on value assessment and request for additional evidence / delay due to bureaucratic process / application withdrawn / other) -Reasons behind non-application for reimbursement in the country (country filing requirements / lack of required health care infrastructure to support utilisation / evidence unlikely to meet country requirements / lack of healthcare funding to support utilisation / size of treatable population / insufficient local resources or presence to file P&R / impact

Initiative	Description	Frequency	Product/Disease Scope	Indicator(s)
				of external reference pricing on other EU countries / low value attributed to class competitors / other)
Pharmaceutical Pricing and Reimbursement Information (PPRI) Network's Online Indicators Tool[10]	The PPRI indicators online portal is under development and will be a sustainable reporting system for meta-indicators to describe, measure and analyse pricing and reimbursement systems / policies across countries. These meta-indicators will provide information and data on structural characteristics, processes and results of the design of pharmaceutical systems and policies; allow for benchmarking, and provide descriptive information on pharmaceutical systems to form the basis for evidence-based decisions and evaluations of policy measures. The tool will be available publicly online.	To be launched in 2022/3 – will be as up to date as possible.	This portal will focus on pharmaceutical pricing and reimbursement systems / policies in both the inpatient and outpatient sector. It does not focus specifically on product-level data. The initial phase will focus on European countries.	There will be 67 indicators in total, organised within subcategories, covering structural, process, and results indicators. Some indicators will include, for example: 1) Use of policies to regulate pharmaceutical prices (EPR, value-based pricing, regulation of distribution remuneration) 2) Information on reimbursement policies (reimbursement rates, use of reimbursement lists) 3) Generic shares etc. Data will be collected and validated by national experts. Indicators will be presented in the form of tables or maps, with downloadable data.
Defining Essential Innovative Medicines and Measuring their Use in Europe (IQVIA)[11]	This IQVIA report assessed the reimbursement status and per capita utilisation for selected groups of novel medicines across Europe. It is based on a systematic approach to identifying groups of new active substances (NAS) approved globally between 2011 and 2020 with which to measure utilisation and identify gaps in access. The rationale behind this grouping methodology was that while new medicines may not be uniformly available, there are those that have achieved consensus among stakeholders regarding their clinical value and have been widely reimbursed across countries. The presented analysis looks at share of medicine spending and growth (affordability), and volume use and rate and pace of adoption over time (accessibility) of those medicines.	Report in 2022, no routine monitoring announced	Of 504 NAS launched globally over the time period, 404 were available in Europe, from which 94 "innovative medicine groups" were identified (i.e. grouping molecules based on mechanism, indication, or use). Of these groups, 46 groups representing 309 NAS were classified as "essential innovative medicine groups" – EIM – (i.e. groups having one or more NAS reimbursed in more than half of the 26 European countries with available information on reimbursement status). Finally, 20 EIM groups representing 107 NAS across seven therapy clusters were selected for analysis as these groups were considered to represent significant advances in their disease areas with novel mechanisms. The seven therapy clusters included 8	The report covers several indicators related to the access dimensions of availability, affordability, and accessibility. For example, Availability: 1) Percent of 404 novel active substances reimbursed by country vs. percent of 94 innovative medicine groups with at least one novel active substance reimbursed 2) Reimbursement status for 20 analysed EIM groups. Affordability: 1) Per capita drug (hospital and retail) and health spending in real PPP 2) Spending on all medicines by segment including spending on EIM groups of widely reimbursed novel active substances & related products; other novel active substances with wide reimbursement; novel active substances without wide reimbursement at class level; all other medicines 3) Drivers of medicine spending growth by segment (see above) and country 4) Share of EIM spending by clusters (e.g. oncology, diabetes) 5) Share of spending on EIMs by therapy cluster Accessibility:

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			groups in oncology (e.g. PD-1/PD-L1 immuno-oncology checkpoint inhibitors, prostate cancer treatment); 3 in cardiology (e.g. direct factor Xa inhibitors, pulmonary arterial hypertension treatment); 3 in diabetes (e.g. DPP-4 inhibitors, SGLT-2 inhibitors and GLP-1 agonists); 2 in immunology (e.g. small molecules and biologic treatments that target a range of autoimmune disorders); 2 targeting the central nervous system (e.g. new oral treatments for multiple sclerosis, migraine); as well as hepatitis C antivirals; and new disease-modifying agents used in the treatment of cystic fibrosis.	1) Average utilisation (DDD per capita) of EIM across all groups 2) Index of volume use of 20 EIM groups compared to GDP per capita 3) Average DDD per capita index by EIM group and GDP per capita 4) DDD per capita indexed to European average for 35 countries and 7 EIM clusters 5) Index of country disease prevalence/incidence vs. index of volume use per capita 6) DDD per capita indexed to European average across 20 EIM groups 7) Adoption status of EIM groups by country (early adoption, later adoption) 8) DDD per capita indices for countries grouped by frequency of early adoption
Oslo Medicines Initiative (OMI)[12]	The Oslo Medicines Initiative (OMI) is a neutral platform for the public and private sectors to jointly discuss the issue of access to and affordability of effective, novel and high-priced medicines. Under the auspices of the OMI, technical and stakeholder engagement work was undertaken during 2021 and 2022, in which consultations were conducted with Member States, the pharmaceutical industry, civil society and patient groups. In addition, a series of 10 OMI technical reports was delivered by a range of academic experts, covering topics such as access to high-priced medicines in lower-income countries, and access to information in markets for medicines.	Ongoing initiative, commencing in 2021.	Novel and high-priced medicines.	Several proposals have been put forward to meet the challenges identified in the stakeholder consultations and technical reports. More specifically, stakeholders suggested the development of an independent public portal that includes different indicators for monitoring access in different countries. Metrics would have to be agreed upon by all stakeholders. Concretely, the OMI proposes that a multistakeholder working group – comprising governments, industry and civil society – develops a performance framework that is relevant, feasible and proportionate. The purpose of the framework will be to benchmark activity of companies and governments transparently and consistently against the social contract and across the major dimensions of access – availability, affordability, acceptability, and quality. This aims to facilitate corrective action to identify and address any access failures.
Measuring the delays to access of novel medicines (new	The chapter analyses access delays in four European countries – France, Italy, Spain, Germany - using a sample of 12 novel	First study published in 2022, follow-up analyses	The 2021 study measured access to 12 novel medicines (daratumab, pembrolizumab, nivolumab,	The report presented a new time-to-access indicator for consideration: - Time (in days) between marketing authorization and either the positive coverage decision OR the first reimbursed/publicly

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chapter in the yearly report of L'Assurance Maladie, France[13])	medicines, with a specific focus on early access mechanisms. In France, for example, population-wide early access may be possible while a product is undergoing the coverage and reimbursement process. Following the analysis, the report suggests the further consideration of population-wide early access schemes as complementary indicators of access (e.g. when considering times to patient access). The report also proposes to create an observatory to measure delays in access to novel medicines at the European level, with cooperation from country institutions in charge of medicines.	expected to be published in 2023.	palbociclib, osimertinib, lumacaftor/ivacaftor, dupilumab, emicizumab, glecaprevir/pibrentasvir, alirocumab, evolocumab, sacubitril/valsartan) across four countries: France, Spain, Italy and Germany.	financed early access on a population basis (i.e. for a group of patients) Early access schemes on a named-patient basis were not considered as they do not have the same impact on patient access as population-wide schemes.

Notes: CIRS Centre for Innovation in Regulatory Science; EFPIA European Federation of Pharmaceutical Industries and Associations; EMA European Medicines Agency; EPR external price referencing; HTA health technology assessment; MA marketing authorisation; NAS new active substance; SDG Sustainable Development Goal

1. See World Health Organization (2018[1]) https://www.who.int/data/gho/indicator-metadata-registry/imr-details/5559 2. See EFPIA (2022[2]) see: https://www.efpia.eu/media/676539/efpia-patient-wait-indicator_update-july-2022_final.pdf, and https://www.efpia.eu/media/636486/improving-regulatory-timelines-to-optimise-patient-access-to-innovative-oncology-therapies-in-europe.pdf. 3. See Ventura (2020[3]), available at https://www.ventura.com/wp-content/uploads/2020/08/White-paper-every-day-counts-improving-time-to-patient-access-to-innovative-oncology-therapies-in-europe_from-EFPIA_and_Ventura.pdf. 4. See latest report by Hofmarcher et al. (2019[4]), available at: https://ihe.se/wp-content/uploads/2020/10/IHE-Report-2019_7_.pdf. 5. (Cherny et al., 2016[5]; Cherny et al., 2017[6]). 6. See https://www.cirsci.org/?s=htadock. 7. See https://accesstomedicinefoundation.org/medialibrary/2022_access-to-medicine-index-1669982501.pdf . 8. See the Europeans Medicine Agency website and IRIS tool homepage https://www.ema.europa.eu/en/human-regulatory/post-authorisation/notifying-change-marketing-status, https://iris.ema.europa.eu/homenews/. 9. See EFPIA's media release at https://www.efpia.eu/media/636830/addressing-patient-access-inequalities-in-europe.pdf 10. Internal communication with PPRI network, 2021. 11. See https://www.iqvia.com/insights/the-iqvia-institute/reports/defining-essential-innovative-medicines-and-measuring-their-use-in-europe 12. See https://www.who.int/europe/initiatives/the-oslo-medicines-initiative/technical-reports. 13. See https://assurance-maladie.ameli.fr/etudes-et-donnees/2022-rapport-propositions-pour-2023-charges-produits.

Sources: Authors as cited, accessed May 2022 and updated November 2022.

Annex C. Possible product/indication-level indicators

Table A C.1. Example indicators to measure access across countries to novel products/indications across the different access dimensions

List of example indicators per dimension of access, according to the related stages of market entry, which could be computed for each novel product (specific to a particular indication) across countries, at a specific time period. Indicators could be presented at individual product/indication-level or aggregated across a defined (representative) sample – ***however, it is important to recognise that results are medicine-dependent***. Time-to-access indicators are presented in Table A C.2.

Dimension of access	Stage of market entry	Indicator name	Description	Purpose	Necessary data elements	Possible data source	Potential issues with data collection, analysis, or interpretation
Availability	*Early access scheme*	*Proportion of medicines by existence and type of early access scheme (e.g. Figure 2.3)*	*Existence of an early access scheme (yes / no)* *Type of funding arrangement (government or insurer-sponsored / industry-sponsored / unknown type)* *Type of scheme (named-patient / population-based)*	*To identify whether countries use early access schemes as a means to accelerate access for a small subset of the population and how those schemes are funded; the existence of an early access scheme may have an impact on the MA application or the application for HTA/coverage/pricing.*	*Early access scheme status* *Type of funding arrangement* *Type of scheme*	*Relevant authority (internal) database or website, provided by company, provided by expert through survey*	*Data generally not publicly available, unless published in list of active programmes.*
Availability	*Early access scheme*	*Proportion of medicines by timing of early access scheme*	*Timing of the early access scheme in relation to the MA or coverage decision*	*To identify when countries use early access schemes, in relation to MA and coverage decisions. The impact of whether the timing of the early access scheme could have an impact on the MA application or the application for HTA/coverage/pricing could also be explored.*	*Early access scheme in place: prior to central marketing authorisation AND/OR prior to a publicly funded coverage decision AND/OR continued after a publicly funded coverage decision* *OR collecting date of granting early access and date of coverage decision*	*Provided by company, provided by expert through survey*	*Data not generally publicly available.*
Availability	Marketing authorisation	Proportion of medicines by marketing authorisation status	Status of MA (approved / application in process / no application received / approval denied)	To identify the proportion of medicines approved across countries out of all novel medicines with a regulatory approval in any jurisdiction.	MA status	Publicly available via regulatory databases (e.g. EMA – centralised in Europe; FDA in the USA)	Not relevant for comparisons between EU countries due to centralised process– not explored further in this OECD pilot study.

Dimension of access	Stage of market entry	Indicator name	Description	Purpose	Necessary data elements	Possible data source	Potential issues with data collection, analysis, or interpretation
Availability	Launch	Proportion of medicines by launch status	Launch status (launched / not launched)	To identify the proportion of medicines launched across countries, out of all novel medicines with a regulatory approval in any jurisdiction.	Submission of HTA and/or coverage application OR Utilisation (sales) OR positive coverage decision OR inclusion in national pharmacy data base OR inclusion in national formulary	Depends on which data element is used: Relevant authority database or website, provided by company.	Differing definitions of "launch"; sales not disaggregated by indication and may not be able to distinguish reimbursed versus non-reimbursed sale; data may not be public.
Availability / acceptability	Coverage/ Pricing	Proportion of medicines covered (or with covered therapeutic alternatives) (e.g. Figure 2.1)	Coverage status of sample products (yes / no) If not covered, coverage status of therapeutic alternatives (yes / no)	To identify the proportion of medicines covered or reimbursed, in any form, by public funds. When looking at a defined sample, it is important to also consider the coverage of appropriate therapeutic alternatives – either within class or indication. Patients may not be disadvantaged without access to particular products in these cases.	Coverage status of sample products (or alternatives)	Relevant authority (internal) database, private data provider, provided by company, provided by expert through survey	Only relevant for countries with a positive reimbursement list; status likely only publicly available once a coverage decision has been made; in some countries, decisions on reimbursement are decentralised; does not take into account availability of appropriate alternative products.
Availability / affordability / accessibility	Coverage / Pricing; HTA; Utilisation	Proportion of medicines by breakdown of availability according to progress within the coverage process i.e. stage-in-process (e.g. Figure 2.2)	Stage-in-process (covered and sold / covered and not yet sold / covered and sales unknown / coverage denied or HTA evaluation and or coverage decision did not proceed / no HTA evaluation and or coverage application submitted / HTA evaluation and or coverage decision in process) Coverage status of sample products or therapeutic alternatives (yes / no)	To provide a more comprehensive overview of the availability of medicines at a particular point in time, which can provide insights into why medicines may not be covered. It is important to consider where a medicine is in terms of national HTA evaluation and coverage processes, in addition to resulting coverage decisions and sales, as well as the availability of appropriate alternatives. Some aspects are not relevant to some countries.	Coverage status of sample products (or alternatives) Status of HTA evaluation/assessment (completed / dossier under evaluation / HTA did not proceed / no dossier submitted / not applicable) Status of coverage/pricing decision (covered / denied / withdrawn or suspended / reimbursement determination or negotiation in progress / no application received / other). If denied (company withdrew its application or interest / insufficient evidence of comparative effectiveness and/or cost-effectiveness / incomplete application / product was not found to be cost-effective in this	Relevant authority (internal) database, private data provider, provided by company, provided by expert through survey	Overall: relies on data from several sources, which may not be publicly available. Status of HTA: Data generally not publicly available with this much detail unless evaluation complete and published; some countries have decentralised HTA evaluations; not applicable to those countries without an HTA process. Status of coverage/pricing decision: Only relevant for countries with a positive reimbursement list; status likely only publicly available once a coverage decision has been made; in some countries, decisions on reimbursement are decentralised. (Reimbursed) sale status: Sales may happen prior to positive reimbursement decision. Alternatives: Relevant for those products that are not covered; countries may consider different alternatives as appropriate, depending on their specific contexts.

Dimension of access	Stage of market entry	Indicator name	Description	Purpose	Necessary data elements	Possible data source	Potential issues with data collection, analysis, or interpretation
					indication) Product sold for use in this indication (yes/no)		
Availability/ Affordability	Coverage/ Pricing	Proportion of covered medicines by extent of coverage in comparison to the authorised indication (e.g. Figure 2.4)	Concordance between MA and coverage indication (full scope/limited scope/extent of scope unknown/not covered), by subcategories (e.g. patient population, duration or quantity of treatment, pre-requisite of failure to prior therapy)	To inquire about the extent of the coverage in relation to the original MA indication (and associated concordance) in each country. This can provide insight into any differences in coverage across countries. In the OECD pilot study, this was assessed by looking at whether the coverage was limited relative to the EU approved indication by (1) characteristics of the patient population or subgroup, (2) duration or quantity of treatment for individual patients), or (3) pre-requisite of failure of (or intolerance to) a prior therapy. Other categories with which to measure extent of coverage could be explored.	Original MA indication Coverage indication	Regulatory database e.g. EMA (centralised for EU); relevant authority (internal) database or website, provided by company, provided by expert through survey	Only relevant for covered products which may differ between countries; detailed data to answer these questions unlikely to be readily availability in a standardised format and reference to original documents may be required to clarify further details.
Availability/ Affordability	Coverage/ Pricing	Proportion of covered medicines by limitations or restrictions applied to coverage decisions (e.g. Figure 2.5)	Additional limits or restrictions applied to coverage decisions (no limits or restrictions/some limits or restrictions/limits or restrictions unknown/not covered), by subcategories (e.g. demonstrated treatment response / maximum number of patients per annum / prescriber type / other)	To identify any additional limits or restrictions on coverage that may limit access for patients in each country. In the OECD pilot study, this was assessed by looking at whether the following limits or restrictions were applied: (1) requirements to demonstrate a pre-determined response to treatment; (2) maximum number of patients eligible for treatment per annum; (3) limited to prescriber type; and (4) other.	Coverage indication Limits or restrictions on coverage	Relevant authority (internal) database or website, provided by company, provided by expert through survey	Only relevant for covered products which may differ between countries; detailed information on this is unlikely to be in a standardised format.
Affordability	Coverage/ Pricing	Cost of treatment relative to wealth (system-level) (e.g. Figure 3.1)	Cost of treatment for a defined time period (monthly, annually) relative to GDP per capita	To identify the relative affordability of medicines between countries, by adjusting individual drug prices or cost of treatment by a parameter of wealth. The use of GDP per capita as a denominator is a proxy for wealth in countries, although this does not provide information on affordability for individual patients. These estimates should not be aggregated across products without complex methods. Other estimates of country wealth could be used.	Cost of per person treatment for a defined time period (monthly, annually) – based on ex-factory list price GDP per capita Existence of confidential discounts	Relevant authority (internal) database or website, provided by expert, private data provider, national statistics	Not relevant to aggregate across medicines in the OECD pilot study; only relevant for covered products which may differ across countries; ex-factory list prices do not include confidential discounts or rebates, however their existence can be flagged; for individual drug prices to be standardised across countries generally need to select a particular presentation (strength/pharmaceutical form/ pack size) of the product to examine – which may not be available in all countries; pricing might not be

Dimension of access	Stage of market entry	Indicator name	Description	Purpose	Necessary data elements	Possible data source	Potential issues with data collection, analysis, or interpretation
							linear so choice of pack size might affect the analysis.
Affordability	Coverage/ Pricing	Out-of-pocket (OOP) cost of treatment relative to wage (patient-level) (e.g. Figure 3.2)	Out-of-pocket cost of treatment for a defined time period (monthly, annually) relative to average wage	To identify the affordability of medicines between countries, by adjusting out-of-pocket costs by wage. The use of average wage as a denominator is a proxy for patient wealth. In the OECD pilot study, the ratio of OOP for one month of treatment relative to average daily wage was calculated, to provide insight into the number of daily wages need to pay for one month of treatment across countries. These estimates should not be aggregated across products. Other measurements of patient wealth could be used.	Cost of per person treatment for a defined time period (monthly, annually) – based on patient OOP contribution Average population wage	Relevant authority (internal) database or website, provided by expert through survey, national statistics	See row above; OOP data may be difficult to obtain – not necessarily possible to calculate the OOP with only information on coinsurance levels.
Affordability	Coverage/ Pricing	Proportion of medicines by different types of cost-sharing (e.g. Figure 3.3)	Type and level of cost-sharing: free of charge, fixed co-payment, coinsurance, other (deductible, extra-billing). Cap (monthly, annually, per product)?	To identify the types and levels of cost-sharing arrangements applied to an adult patient who is not entitled to any special concession or exemption from cost sharing. Types and levels of cost-sharing are medicine-dependent.	Type of cost-sharing	Relevant authority (internal) database or website, provided by expert through survey	Only relevant for covered products which may differ between countries; any aggregate numbers do not take into account that these may be medicine-dependent (e.g. inpatient medicines free of charge)
Accessibility	Utilisation	Consumption or sales in the general population (e.g. Figure A G.1, Figure A G.2)	Consumption or sales in standardised units such as defined daily dose (DDD) per 1000 population per day. For products without a DDD, milligrams/1000/day or iu/1000/day could be computed	To identify usage of a medicinal product, measured in standard units such as DDD or milligrams or iu, per 1 000 population per day. Data on usage is unlikely to be indication-based. However, this indicator does not take prevalence nor presence of alternatives into account. Ideally, this indicator would be adjusted to account for the prevalence of the specific condition treated with the product, or for the proportion of the population that can benefit. Estimates should not be aggregated across products. Estimates should consider the most frequent setting of administration of a product.	Consumption or sales in DDD or milligrams or iu within defined time period Population	Private data provider, provided by expert through survey, national statistics	Not relevant to aggregate across medicines in the OECD pilot study ; DDD/1000 population/day best for chronic treatment, while DDD/inhabitant/year best for short course medicines; data not indication-based and does not take prevalence or burden of disease into account; countries have differences in clinical practice as well as other alternatives available; not always possible to disaggregate non-reimbursed and reimbursed sales; data may not be readily available for inpatient medicines.
Accessibility	Utilisation	Consumption or sales over time (e.g. Figures 4.8 & 4.9)	Time-series of consumption or sales in standardised units such as defined daily dose (DDD) or milligrams per 10,000 population since MA	To identify the uptake of a medicine since the MA or positive reimbursement decision. It can show delays in decision-making and patient access. Data on usage is unlikely to be indication-based. However, this indicator	Consumption or sales in DDD or milligrams or iu over time (e.g. monthly data over the span of a few years)	Private data provider, provided by expert through survey, national statistics	See row above; sales might start prior to positive reimbursement decision through early access or named-patient basis schemes and thus not reflect general population access.

Dimension of access	Stage of market entry	Indicator name	Description	Purpose	Necessary data elements	Possible data source	Potential issues with data collection, analysis, or interpretation
			or positive reimbursement decision	does not take prevalence nor presence of alternatives into account. Ideally, this indicator would be adjusted to account for the prevalence of the specific condition treated with the product, or for the proportion of the population that can benefit. Estimates should not be aggregated across products. Estimates should consider the most frequent setting of administration of a product.	Population		
Accessibility	Utilisation	Number of patients treated (e.g. Figure A G.3)	Number of patients treated per 1000 population in a defined period of time (e.g. annual).	Data on usage is unlikely to be indication-based. However, this indicator does not take prevalence into account. Ideally, this indicator would be adjusted to account for the prevalence of the specific condition treated with the product, or for the proportion of the population that can benefit. Estimates should not be aggregated across products. Estimates should consider the most frequent setting of administration of a product.	Approximate number of patients treated within defined time period	Private data provider, provided by expert through survey	Not relevant to aggregate across medicines; data not indication-based and does not take prevalence into account; countries have differences in clinical practice as well as other alternatives available; data on patient numbers not readily available; international comparisons hampered by differing data definition / specifications.
Accessibility	Utilisation	On-shelf availability at health facility or pharmacy	Availability of a product on the shelf at a health facility or pharmacy on a given day	To determine whether or not a patient can access a product in store when it is needed. Transport delays or shortages can have an impact.	Facility-level stock levels	Facility-based surveys	This indicator was not considered further in the OECD pilot study. It is dependent on individual facility stock levels.
Acceptability	Prescription	Proportion of medicines by consistency between covered indication and national guidelines or treatment protocols	Concordance between clinical guideline and coverage assessed using a binary indicator (yes / no / not applicable) or with multiple categories such as none/ partial /full concordance.	To inquire about the extent of inclusion in clinical guidelines or treatment protocols in relation to covered indications e.g. by place in therapy. For example, a medicine may be placed in the guideline as first line therapy, but only indicated as fourth line therapy as per the coverage conditions; this indicates a mismatch.	Covered indication (including place in therapy) Indication as per clinical guidelines or treatment protocols (including place in therapy)	Relevant authority (internal) database or guidelines, provided by expert through survey	Only relevant for covered products; various sources for clinical guidelines or no national sources; this type of data is often not regularly assessed or is challenging to assess.
Acceptability	HTA / Prescription	Added therapeutic value as determined by HTA	One method of evaluating therapeutic value is looking at the HTA report and whether or not the product was considered to offer added	To identify the priority products for access. Evaluating value is a complex task and the purview of specialists at HTA agencies in many EU and OECD countries. This process is inherently comparative, and greatly depends	HTA outcome (positive or negative) Considered added therapeutic value (none / added)	Relevant authority (internal) database or website e.g. published HTA reports, provided by	Only relevant for those products that have undergone an HTA evaluation; HTA reports often not published; this particular indicator only provides insight into a very basic determination of therapeutic value that can

Dimension of access	Stage of market entry	Indicator name	Description	Purpose	Necessary data elements	Possible data source	Potential issues with data collection, analysis, or interpretation
		(e.g. Table 5.1)	therapeutic value over alternatives, and if so, what level of therapeutic value was assigned.	on the standard of care available nationally as well as burden of disease. This is only one option of monitoring therapeutic value. More complex measures of the health benefits of medicines could, for example, combine evidence of efficacy, place of the medicines in the therapeutic class and the severity of disease.		expert through survey	be subjective; comparisons between the determined level of therapeutic value are difficult to make across countries due to differences in country classifications; "Positive" or "negative" recommendation not always given in some countries or is not sufficient to describe outcome of HTA evaluation.

Note: MA marketing authorisation; EMA European Medicines Agency; HTA Health Technology Assessment; OOP out-of-pocket, PPP purchasing power parity; iu international units
List includes examples of possible indicators and/or data elements that could be collected and reported to measure access. List is not exhaustive.
Source: Authors, compiled based on past experience and review of the existing evidence.

Table A C.2. Time-to-access product/indication-level indicators

Time to access indicators relate predominantly to the dimensions of availability and affordability. They could be presented at individual product/indication-level or aggregated across a defined (representative) sample – ***however, it is important to recognise that results may be medicine-dependent.***

Stage of market entry	Indicator name	What might these time differences reflect?	Necessary data elements	Potential source of data	Potential issues with data collection, analysis, or interpretation
Marketing authorisation	Time-to-MA since application of MA	Time differences in submission for review to regulatory agencies and the time for review procedure, including any clock-stops i.e. time taken by the company to provide additional requested information (N.B. this will be the same for all EU countries due to the centralised procedure).	Date of application for MA Date of granting of MA	Regulatory database e.g. EMA (centralised for EU)	Not relevant for comparisons between EU countries in the OECD pilot study.
Marketing authorisation - HTA	Time-to-first HTA submission since MA (e.g. Period 2 in OECD pilot study; Figure 2.8)	Launch strategy of the pharmaceutical companies (i.e. making decisions for sequential product launches and delayed application for coverage/pricing decisions – influenced by market size and the pharmaceutical policies in each country). This indicator is only relevant for countries with a national HTA procedure.	Date of MA Date of first submission of an HTA dossier by the company	Regulatory database e.g. EMA (centralised for EU); relevant authority (internal) database or website, provided by company, provided by expert through survey	Some countries have decentralised HTA evaluations; not applicable to those countries without an HTA process; some countries allow companies to apply for HTA prior to MA; date data difficult to obtain.
HTA	Time-to-first HTA completion since first submission	Nature and performance of HTA processes in each country. This indicator is only relevant for countries with a national HTA procedure.	Date of first HTA submission Date of first HTA completion	Relevant authority (internal) database or website, provided by company, provided by expert through survey	Some countries have decentralised HTA evaluations; not applicable to those countries without an HTA process; date data difficult to obtain.
HTA	Time-to-successful HTA submission since first HTA completion	Time taken to resubmit for an HTA assessment in the event that a previous attempt was unsuccessful. This indicator is only relevant for countries with a national HTA procedure	Date of first HTA completion (unsuccessful) Date of first successful HTA submission	Relevant authority (internal) database or website, provided by company, provided by expert through survey	Some countries have decentralised HTA evaluations; not applicable to those countries without an HTA process; date data difficult to obtain; there might be more than two attempts (one unsuccessful, one successful).
HTA	Time-to-successful HTA completion since successful HTA submission	Nature and performance of HTA processes in each country. This indicator is only relevant for countries with a national HTA procedure.	Date of first successful HTA submission Date of first successful HTA completion	Relevant authority (internal) database or website, provided by company, provided by expert through survey	Some countries have decentralised HTA evaluations; not applicable to those countries without an HTA process; date data difficult to obtain; could be compared with first submission - first completion.
HTA	Time-to-most recent HTA completion since first HTA submission	Nature and performance of HTA processes in each country, not taking into account that more than one HTA attempt may have been made. This indicator is only relevant for countries with a national HTA procedure.	Date of first (unsuccessful) HTA submission Date of first successful HTA completion	Relevant authority (internal) database or website, provided by company, provided by expert through survey	Some countries have decentralised HTA evaluations; not applicable to those countries without an HTA process; date data difficult to obtain; there might be multiple submission attempts.
Marketing authorisation - Coverage/ Pricing	Time-to-first-coverage application since MA (e.g. Period 2 in OECD	Launch strategies of companies (as described above). This indicator is relevant for countries where a coverage decision is made at the national level, and	Date of granting of MA Date of first coverage application	Regulatory database e.g. EMA (centralised for EU); Relevant authority (internal) database or	Some countries allow companies to submit applications for coverage before MA; in many cases the HTA and coverage applications are

Stage of market entry	Indicator name	What might these time differences reflect?	Necessary data elements	Potential source of data	Potential issues with data collection, analysis, or interpretation
	pilot study, Figure 2.8)	less so for others. Some countries allow companies to submit applications for coverage before MA.		website, provided by company, provided by expert through survey	simultaneous.
HTA - Coverage/ Pricing	Time-to-first application for coverage/reimbursement since HTA completion	Time differences between HTA completion and coverage application. This indicator is only relevant for countries with differentiated national HTA and coverage procedures.	Date of first successful HTA completion Date of first submission of application for coverage/reimbursement	Relevant authority (internal) database or website, provided by company, provided by expert through survey	In many cases the HTA and coverage applications are simultaneous.
Coverage/ Pricing	Time-to-first coverage decision since first application of coverage	Nature and performance of regulatory and coverage decision-making processes in each country, only if clock stops are considered. This indicator is relevant for countries where a coverage decision is made at the national level.	Date of first coverage application Date of first coverage decision	Relevant authority (internal) database or website, provided by company, provided by expert through survey	Clock stops must be considered if interpreting this indicator as a measure of the nature and performance of regulatory and coverage decision-making processes in each country.
Coverage/ Pricing	Time-to-first successful coverage application since first coverage decision	Time taken to resubmit a coverage application in the event that a previous attempt was unsuccessful. This indicator is relevant for countries where a coverage decision is made at the national level.	Date of first (unsuccessful) coverage decision Date of first successful coverage application	Relevant authority (internal) database or website, provided by company, provided by expert through survey	There might be more than two attempts (one unsuccessful, one successful) in between.
Coverage/ Pricing	Time-to-first successful coverage decision since first successful coverage application (e.g. Period 3a in OECD pilot study, Figure 2.10)	Nature and performance of regulatory and coverage decision-making processes in each country, only if clock stops are considered. This indicator is relevant for countries where a coverage decision is made at the national level.	Date of first successful coverage application Date of first successful coverage decision	Relevant authority (internal) database or website, provided by company, provided by expert through survey	Clock stops must be considered if interpreting this indicator as a measure of the nature and performance of regulatory and coverage decision-making processes in each country.
Marketing authorisation - Coverage/ Pricing	Time-to-first successful coverage decision since MA (e.g. Figure 2.7)	Proxy measure often used to compare differences in times to patient access across countries. Estimates may use positive coverage decision dates, or dates of inclusion in positive reimbursement lists.	Date of marketing authorisation Date of first successful coverage decision (e.g. positive coverage decision) or inclusion in positive reimbursement list	Regulatory database e.g. EMA (centralised for EU); Relevant authority (internal) database or website, provided by company, provided by expert through survey	Breakdown of time-to-access cannot be seen with this indicator; clock stops must be considered if interpreting it as a measure of the nature and performance of regulatory and coverage decision-making processes in each country; not as relevant for countries without a positive reimbursement list; also does not account for the use of population-wide early access coverage schemes.
Coverage/Pricing	Time-to-successful coverage decision since first coverage application (e.g. Period 3 in OECD pilot study, Figure 2.9)	Nature and performance of regulatory and coverage-decision making processes in each country, not taking into account that more than one coverage attempt may have been made. This indicator is only relevant for countries with a national coverage procedure.	Date of first (unsuccessful) coverage application Date of first successful coverage decision	Relevant authority (internal) database or website, provided by company, provided by expert through survey	There might be more than one (failed) attempts; clock stops must be considered if interpreting this indicator as a measure of the nature and performance of regulatory and coverage decision-making processes in each country.
Coverage/ Pricing -Utilisation	Time-to-launch since successful coverage decision (e.g. Period 4 in	Time difference between coverage decision and first sales, as a proxy for launch.	Date of coverage decision Date of actual launch OR	Relevant authority (internal) database or website, provided by company, provided by expert	Launch is not easy to identify, but first sales after a positive coverage decision can be used as a proxy. Sales are not indication-based. Sometimes a

Stage of market entry	Indicator name	What might these time differences reflect?	Necessary data elements	Potential source of data	Potential issues with data collection, analysis, or interpretation
	OECD pilot study, Figure 2.11)		Date of first utilisation in a given market as a proxy for actual launch date OR Date of coverage/pricing decision in a given market as a proxy for actual launch date (when data on utilisation not available)	through survey	product can be sold prior to the coverage decision.
Marketing authorisation - Utilisation	Time-to-launch since MA	Time differences here may reflect (1) the launch strategy of the pharmaceutical company (i.e. making decisions for sequential product launches and delayed application for coverage/pricing decisions influenced by market size and the pharmaceutical policies in each country) and (2) the nature and performance of the regulatory and coverage-decision making processes.	Date of granting of MA Date of actual launch OR Date of first utilisation in a given market as a proxy for actual launch date OR Date of coverage/pricing decision in a given market as a proxy for actual launch date (when data on utilisation not available)	Regulatory database e.g. EMA (centralised for EU); Relevant authority (internal) database or website, provided by company, provided by expert through survey	Breakdown of time-to-access cannot be seen with this indicator; clock stops must be considered if interpreting this indicator as a measure of the nature and performance of regulatory and coverage decision-making processes in each country; not as relevant for countries without a positive reimbursement list; also does not account for the use of population-wide early access coverage schemes; launch is not easy to identify, but first sales after a positive coverage decision can be used as a proxy; sales are not indication-based; sometimes a product can be sold prior to the coverage decision.

Note: MA marketing authorisation, EMA European Medicines Agency, HTA Health Technology Assessment.
HTA completion refers to when the HTA evaluation is finalised and transmitted to the competent authority on pricing and reimbursement. The final assessment may be published at the same time or a later date.
Source: Authors, compiled based on past experience and review of the existing evidence

Annex D. OECD data collection

The content of the paper is based on information available from public sources, including the peer-reviewed literature, grey literature and documents published by payers and government agencies, as well as survey data provided by country Experts to the OECD Expert Group on Pharmaceuticals and Medical Devices. OECD data collection methods are explained below, with further methodological details and additional analyses discussed by dimension in Annex E (availability) Annex F (affordability) and Annex G (accessibility).

OECD desk review

A desk review was performed in early 2021 to identify the different dimensions of access to medicines, examples of existing initiatives measuring access to medicines, as well as specific indicators with which to measure access to medicines at the product/indication level. This was done through searches of academic databases (e.g. PubMed, Scopus, Google Scholar) as well the grey literature. Key search terms, among others, included “indicator OR measure OR monitor”, “medicine OR drug OR pharmaceutical”, and “access OR availability OR affordability OR acceptability OR accessibility”.

A second desk review was undertaken in early 2022 to complement the initial research, particularly as it related to identifying indicators with which to measure access. This second review consisted of two phases, the literature search, and the literature review. In the first phase, search engines were used to retrieve and select academic and grey literature for further screening. Around 7 million items were retrieved using four different search engines (Google Scholar, PubMed, Scopus and Lancet), around thirteen thousand titles were briefly screened, and finally, less than fifty items were selected for further review. Keywords used for the search included “access indicators”, “access indicators pharmaceuticals”, “access indicators medicines”. Targeted searches of the grey literature were done by screening the websites of eleven institutions[1] working on access to medicines in some capacity using the Advanced Google search functionality as well as the keywords “availability”, “affordability”, “acceptability”, “accessibility”. Using this method, around eight hundred items were retrieved, the titles of all items were screened, and less than fifty were selected for further review. In the second phase of the review, all selected publications were ranked by relevancy and then screened for access indicators or initiatives relating to monitoring access, using an online qualitative analysis tool. Using the tool, relevant paragraphs were highlighted and attributed to keywords. The paragraphs were then extracted to Microsoft Excel where they could be filtered by publication, type of publication, keyword, and relevancy to inform the literature review.

Literature reviews were challenging due to the vast number of publications, particularly in the grey literature domain, as well as different perspectives taken when looking at the broad topic of access to medicines. As a result, the information in this paper should not be considered exhaustive nor definitive.

[1] World Health Organisation, European Commission, European Federation of Pharmaceutical Industries and Associations (EFPIA), Oslo Medicines Initiative (OMI), Pharmaceutical Pricing and Reimbursement Information (PPRI), European Observatory of Health Systems and Policies, European Integrated Price Information Database (EURIPID), Access to Medicine Foundation (A2M), Centre for Innovation in Regulatory Science (CIRS), Access Observatory, Health Action International (HAI).

OECD survey

In November 2021, the OECD Secretariat sent a survey to experts in a total of 29 countries, including OECD and EU member states, as well as non-OECD EU member states, and some countries in the European Economic Area[2]. The survey aimed to gather information on various dimensions of access to a sample set of novel medicines in EU member states, valid as at 01 October 2021. Country experts were asked to respond to a series of questions covering aspects of market entry for a sample of 15 centrally authorised product/indication pairs. The survey was delivered in the form of a conditionally formatted Microsoft Excel spreadsheet, to be completed, and a PDF document that provided an overall outline of the survey questions, but intended for reference only. Country experts included, for example, representatives from national insurance agencies, ministries of health, competent authorities for pricing and reimbursement, and HTA agencies.

Sample selection

A convenience sample of fifteen *index* product/indication pairs was chosen from among those new active substances (NAS) centrally authorised in the EU between 2015 to 2018 (allowing 3 years from the latest marketing authorisation of an active substance to 01 October 2021) – see Table A D.1. As this was a feasibility study, a number of factors were taken into account in selecting the product/indication pairs. These included therapeutic class; monotherapy; number of indications; route of administration; care setting; availability of alternatives; uniqueness of indication; orphan status; nature of marketing authorisation etc. Efforts were made to:

- reflect a broad representation of the distribution of NAS across therapeutic areas within the proposed timeframe;
- include a range of product archetypes (e.g. orphans, approval under exceptional circumstances, accelerated approvals, and one advanced therapy medicinal product [ATMP]);
- prioritise products with a single main indication, as utilisation data is unlikely to be available disaggregated by indication;
- favour products administered predominantly in an ambulatory care setting (i.e. via oral or subcutaneous injections), as data may be more readily available; and
- select products without mandatory co-administration of another product, as “access” would not depend on the availability of another product.

[2] Austria, Belgium, Bulgaria, Croatia, Cyprus*, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, Netherlands, Norway, Poland, Portugal, Romania, Slovak Republic, Slovenia, Spain, and Sweden.

*Note by Türkiye: 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. Türkiye recognises the Turkish Republic of Northern Cyprus (TRNC). Until a lasting and equitable solution is found within the context of the United Nations, Türkiye 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 Türkiye. The information in this document relates to the area under the effective control of the Government of the Republic of Cyprus.

Table A D.1. Convenience sample of 15 product/indicator pairs

ID	INN	Brand Name	ATC Code	EU Marketing Authorisation Date	Therapeutic Indication	European Public Assessment Report (EPAR) link
1	edoxaban	Lixiana®	B01AF03	19 June, 2015	Prevention of stroke; embolism and treatment of venous thromboembolism	https://www.ema.europa.eu/en/documents/assessment-report/lixiana-epar-public-assessment-report_en.pdf
2	asfotase alfa	Strensiq®	A16AB13	28 August, 2015	Treatment of paediatric-onset hypophosphatasia	https://www.ema.europa.eu/en/documents/assessment-report/strensiq-epar-public-assessment-report_en.pdf
3	alirocumab	Praluent®	C10AX14	23 September, 2015	To reduce LDL-C and increase HDL-C	https://www.ema.europa.eu/en/documents/assessment-report/praluent-epar-public-assessment-report_en.pdf
4	sacubitril / valsartan	Entresto®	C09DX04	19 November, 2015	Treatment of heart failure (NYHA class II-IV)	https://www.ema.europa.eu/en/documents/assessment-report/entresto-epar-public-assessment-report_en.pdf
5	mepolizumab	Nucala®	R03DX09	01 December, 2015	Treatment of asthma	https://www.ema.europa.eu/en/documents/assessment-report/nucala-epar-public-assessment-report_en.pdf
6	sofosbuvir / velpatasvir	Epclusa®	J05AP55	06 July, 2016	Treatment of chronic hepatitis C virus (pan-genotype)	https://www.ema.europa.eu/en/documents/assessment-report/epclusa-epar-public-assessment-report_en.pdf
7	palbociclib	Ibrance®	L01XE33 (NEW L01EF01)	09 November, 2016	Treatment of breast cancer	https://www.ema.europa.eu/en/documents/assessment-report/ibrance-epar-public-assessment-report_en.pdf
8	baricitinib	Olumiant®	L04AA37	13 February, 2017	Treatment of moderate to severe active rheumatoid arthritis (RA) in adult patients who have responded inadequately to, or who are intolerant to one or more disease-modifying anti-rheumatic drugs.	https://www.ema.europa.eu/en/documents/assessment-report/olumiant-epar-public-assessment-report_en.pdf
9	nusinersen	Spinraza®	M09AX07	30 May, 2017	The treatment of Spinal Muscular Atrophy (SMA)	https://www.ema.europa.eu/en/documents/assessment-report/spinraza-epar-public-assessment-report_en.pdf
10	tivozanib	Fotivda®	L01XE34 (NEW L01EK03)	24 August, 2017	Treatment of adult patients with advanced renal cell carcinoma (RCC)	https://www.ema.europa.eu/en/documents/assessment-report/fotivda-epar-public-assessment-report_en.pdf
11	dupilumab	Dupixent®	D11AH05	27 September, 2017	Treatment of moderate to severe atopic dermatitis in adults who are candidates for systemic therapy	https://www.ema.europa.eu/en/documents/assessment-report/dupixent-epar-public-assessment-report_en.pdf
12	niraparib	Zejula®	L01XX54 (NEW L01XK02)	16 November, 2017	Treatment of epithelial ovarian, fallopian tube, or primary peritoneal cancer	https://www.ema.europa.eu/en/documents/assessment-report/zejula-epar-public-assessment-report_en.pdf
13	ocrelizumab	Ocrevus®	L04AA36	08 January, 2018	Treatment of multiple sclerosis treatment of multiple sclerosis	https://www.ema.europa.eu/en/documents/assessment-report/ocrevus-epar-public-assessment-report_en.pdf
14	semaglutide	Ozempic®	A10BJ06	08 February, 2018	Treatment to improve glycaemic control in adults with type 2 diabetes and to prevent cardiovascular events	https://www.ema.europa.eu/en/documents/assessment-report/ozempic-epar-public-assessment-report_en.pdf
15	erenumab	Aimovig®	N02CD01	26 July, 2018	Prophylaxis of migraine	https://www.ema.europa.eu/en/documents/assessment-report/aimovig-epar-public-assessment-report_en.pdf

Note: INN international non-proprietary name; ATC Anatomical Therapeutic Chemical classification
Source: European Medicines Agency.

Survey content

As mentioned above, the survey aimed to gather information on various dimensions of access to a sample set of novel medicines in EU member states. As such, the survey was organised into sections around seven relevant topic areas A to F: (A) early access schemes, (B) Health Technology Assessment (HTA), (C) coverage and pricing, (D) treatment costs, (E) prescription, and (F) utilisation. Table A D.2 gives a high level indication of the questions asked for each index product/indication pair. Experts were requested to answer the questions with information valid as at 01 October 2021, and to the extent possible, ensure that responses reflected the specific indication specified in the initial marketing authorisation of the product in the European Union. Experts were also asked to answer a number of questions related to the sources and methods of data collection, in order to inform country comparisons and explore the feasibility of future work.

Table A D.2. Example survey questions

Section	Example questions
A.Early access scheme	Existence, either as at 01 October 2021 or in the past (yes / no)
	Timing (in relation to marketing authorisation or coverage decision)
	Type of funding arrangement (government or insurer-sponsored / industry-sponsored
B.Health Technology Assessment	Submission status (yes / no / not applicable) If no submission, indication about submission from company (intends to submit / does not intend to submit / not indicated its intention to submit)
	Completion status (yes / no) If not completed, indication about reason (HTA could not proceed as dossier was incomplete / dossier under evaluation / other)
	Recommendation in most recent report (positive / negative)
	Product considered to offer added therapeutic value over alternatives in most recent report (yes / no) If yes added therapeutic value over alternatives, what level?
	Date of first submission
	Date of most recent submission
	Date that most recent report finalised or transmitted to a pricing and reimbursement authority
	Date that most recent report made publicly available
C.Coverage and pricing	Coverage status (yes / no) If not covered, indication about reason (coverage denied / coverage withdrawn or suspended / no application for coverage or reimbursement submitted / other) If coverage denied, indication about reason (company withdrew its application or interest / insufficient evidence of comparative effectiveness and or cost-effectiveness / application incomplete in other ways / product not found to be cost-effective / other)
	If not covered, is there another covered product, either within the same, or from another therapeutic class that is considered to be a satisfactory alternative? (yes / no) If yes, name of alternative
	Date of first submission of application for coverage or reimbursement
	Date of first coverage decision (favourable or denial)
	Date of first successful submission of application for coverage or reimbursement
	Date of first favourable coverage decision
	Clock stops between first successful application and first favourable coverage decision
	Is the reimbursed or covered indication narrower than the EMA-authorised indication by patient population or subgroup / duration or quantity of treatment / prerequisite of failure of (or intolerance to) a prior therapy
	Limitations or restrictions applied to coverage decision by demonstrated treatment response / maximum number of patients per annum / prescriber type / other

Section	Example questions
D.Treatment costs	Average cost per pack, in local currency, based on ex-factory price
	Average cost per pack, in local currency, based on hospital or retail price
	Average cost per pack, in local currency, based on patient contribution (i.e. out of pocket costs for an adult patient who is not entitled to any special concession or exemption from cost-sharing)
	Existence of any confidential discounts or rebates from ex-factory price (yes / no)
	Type of cost-sharing arrangements applied to an adult who is not entitled to any special concession or exemption from cost-sharing (none / fixed co-payment / coinsurance / other)
E.Prescription	Concordance of covered indication with national clinical or treatment protocols (yes / no / not applicable) If not, indication of the reason
F.Utilisation	Sales status in this indication as at 01 October 2021 (yes / no / not yet available)
	Date of first sale
	Number of patients treated in the 12 months prior to 01 October 2021
	Number of units sold in the 12 months prior to 01 October 2021
	Most frequent setting of administration (hospital [inpatient] / primary and ambulatory care [outpatient])

Source: OECD survey on access to novel medicines 2021.

Survey responses

As of May 2022, a total of 21 countries responded to the survey (Table A D.3), with most countries able to provide answers to questions in each of the sections A to F for at least one product/indication pair.

Table A D.3. Responses to OECD access survey

As of May 2022

Country (abbreviation)	Sections of the survey[1]					
	A	B	C	D	E	F
Austria (AUT)	√	√	√	√	√	√
Belgium (BEL)	√	√	√	√	√	√
Bulgaria (BGR)	√	√	√	√	√	√
Czech Republic (CZE)	√	√	√	√	√	√
Cyprus (CYP)	√	√	√	√	√	√
Estonia (EST)	√	√	√	√	√	√
Finland (FIN)		NA	√	√	√	√
France (FRA)	√	√	√	√	√	√
Germany (DEU)	√	√	NA	√	√	√
Greece (GRC)	√	√	√	√	√	√
Hungary (HUN)	√	√	√	√	√	√
Iceland (ISL)	√	NA	√	√	√	√
Italy (ITA)	√	√	√	√	√	√
Lithuania (LTU)	√	NA	√	√		
Luxembourg (LUX)	√	NA	√	√	√	√
Malta (MLT)	√	√	√	√	√	√
Norway (NOR)	√	√	√	√		√
Portugal (POR)	√	√	√	√	√	√
Slovenia (SVN)	√	√	√	√	√	√
Spain (ESP)	√	√	√	√	√	√
Sweden (SWE)	√	√	√	√		√
Count of countries (N=21)	*20*	*17*	*20*	*21*	*18*	*20*

Note: √=able to provide some data. NA not applicable. 1. (A) early access schemes, (B) Health Technology Assessment (HTA), (C) coverage and pricing, (D) treatment costs, (E) prescription, and (F) utilisation.
Source: OECD survey on access to novel medicines 2021.

Survey sources and methods of data collection

To inform country comparisons and explore the feasibility of future work, country Experts were also asked several questions related to the sources and methods of data collection they used in answering the survey. These included whether responding countries already measure or monitor access to medicines at national level, and if so, how; what data sources or websites were used to complete the various sections of the question; whether the data provided were nationally representative; whether the data provided were valid as at 01 October 2021; and frequency of update of data sources. Table A D.4 and Table A D.5 summarise the responses to the sources and methods section of the survey.

EURIPID as a validation tool and alternative data source

Medicine price data for 26 EU countries is already collected and maintained in the European price database EURIPID (European Integrated Price Information Database)[3]. The EURIPID collaboration is a voluntary cooperation between national competent authorities for pricing and reimbursement of medicines, whereby national prices of medicines are shared in a standardised format in the EURIPID database. The database contains data on official prices of publicly reimbursed medicines, predominantly in the outpatient setting, that are published by public authorities in line with the EC Transparency Directive (Council Directive 89/105/EEC 89\105\EC).

EURIPID data were used to support analysis of the survey domains (C) coverage and pricing, (D) treatment costs, and (F) utilisation. The database contains product-level information on coverage status of (mostly outpatient) medicines at a defined period; package costs, based on ex-factory or retail prices; the existence of managed entry agreements; as well as consumption data. To the extent possible, data from the OECD survey were compared to that available in EURIPID.

[3] See https://euripid.eu/ , last accessed 16 April 2022.

Table A D.4. Summary of survey sources and methods

Country	1. Access monitoring at national level?	2. Data provided nationally representative?	3. Data provided as at 01 October 2021?	5. How frequently are the databases / data sources updated?
Austria	X (outpatient expenditure and utilisation)	√ (for outpatient drugs; hospital drugs represent 4 regions)	√	utilisation, pricing and sales data: monthly submissions and timelines for evaluations: daily
Belgium	√	√	√ (with the exception of utilisation data; delay)	public databases: monthly Internal databases: continuously
Bulgaria		√	√	most of the registers: monthly HTA reports: when available
Czech Republic	X	√ (with the exception of niche products)	√	utilisation: quarterly other databases: monthly
Cyprus				
Estonia	X	√	√ (except Praluent®)	quarterly
Finland		√	√	continuously
France		√	√	updated continuously
Germany	√	√	√	every 2 weeks or as required
Greece	X	√ (covers 95% of the Hellenic population)	√	continuously
Hungary	X	√	√	pricing, reimbursement, sales: monthly prescription guideline: occasionally
Iceland	X	√	√	pricing, reimbursement: twice per month
Italy	√ (consumption & expenditure)	√	√	continuously
Lithuania				
Luxembourg				
Malta		X (Government Health Services only)	√ (utilisation data not available)	continuously
Norway	X (financing decisions and sales; not linked)	√	√ (utilisation data yearly)	some daily, others yearly
Portugal	√	√	√	daily, except prescribing or dispensing data
Slovenia	X	√	√	
Spain		√	√	pricing, reimbursement: monthly others: continuously
Sweden	X	√	√ (except utilisation data)	TLV: daily, sales: monthly other patient data: monthly or yearly.

Note: √=yes; X=no; green=no response provided. HTA health technology assessment; TLV Dental and Pharmaceutical Benefits Agency, Sweden.
Source: OECD survey on access to novel medicines 2021.

Table A D.5. Data sources used to complete each aspect of the survey, including data accessibility status

Country	A. Early access scheme	B. HTA	C. Coverage & pricing	D. Treatment costs	E. Prescription	F. Utilisation	Other sources?
Austria	Compassionate use programs (list) – public [BASG]	Social Insurance Database – not public [DVSV]	Social Insurance Database – not public [DVSV]	Social Insurance Database – not public [DVSV]		Social Insurance Database – not public [DVSV]	
Belgium	Compassionate use programs(list) – public [FAMHP]	Internal database – not public [NIHDI]	Internal database – not public [NIHDI] NIHDI website – public [NIHDI]	NIHDI website – public [NIHDI]	Internal database – not public [NIHDI] NIHDI website – public [NIHDI]	Several different follow-up system exist, all based on the individual delivered/reimbursed units: Farmanet (for public pharmacies) – not public (data on request) [NIHDI] Inter Mutualistic Agency (additional information on hospitals) – not public [IMA]	
Bulgaria		NCPR website – public (HTA reports themselves not available) [NCPRMP] - positive decisions - negative decisions	NCPR portal– public [NCPRMP]	NHIF quarterly reports – public [NHIF]	Regulations for adoption of pharmaco-therapeutic guidelines – public [NCPRMP]		
Czech Republic	MoH website (public bulletins)– public – [MoH] MoH internal sources - not public [MoH]	SUKL website (administrative info and public files on price and reimbursement procedures) – public [SUKL]	SUKL website (administrative info and public files on price and reimbursement procedures) – public [SUKL]	SUKL website (medicinal products database with medicines info including prices and reimbursement) – public [SUKL]	Various scientific websites and published clinical guidelines (knowledge of clinical assessors)	SUKL website (distribution and wholesale reports) – public [SUKL]	
Cyprus							
Estonia	The State Agency of Medicines (SAM)	Estonian Health Insurance Fund	Estonian Health Insurance Fund – public – prices – health care services	Estonian Health Insurance Fund – public – prices – health care services	Estonian Health Insurance Fund	The State Agency of Medicines (SAM)	
Finland	Not public	Finland does not have one common HTA evaluation process for all medicines (see Table A E.6) - Hospitals: Council for Choices in Health Care (PALKO) makes recommendation, FIMEA gathers HTA dossier - Outpatient: elements of HTA dossier evaluated in the Pharmaceuticals Pricing Board (HILA)	Pharmaceuticals Pricing Board (HILA) website – public [Ministry of Social Affairs and Health] Finish Medicines Agency (FIMEA) medicines database – public [FIMEA]	Association of Finnish pharmacies	Not public	Finish Medicines Agency (FIMEA) medicines database – public [FIMEA]	Pharmaceuticals Pricing Board database – not public [Ministry of Social Affairs and Health]

Country	A. Early access scheme	B. HTA	C. Coverage & pricing	D. Treatment costs	E. Prescription	F. Utilisation	Other sources?
France	Early access framework (including list of products) – public [Ministry of Solidarity and Health]	HAS website– public [HAS]	Official Journal (list of reimbursable medicines by national health insurance) – public [Ministry of Solidarity and Health]		Various links – public HAS French cardiology society Cancer protocols Dermatology info		
Germany		Federal Joint Committee (G-BA) website	Coverage not applicable – there is no positive list; all medicines entering the market are reimbursed by sickness funds (with some exceptions) - prices - not public [CGM]				
Greece	Extracted from the Prior Authorization System – not public	Not public No formal HTA procedure prior to August 2018. From 2018, final outcome for HTA and Negotiation is published on MoH website - public	Up to August 2018 there was a Positive List Committee in the Ministry of Health and no formal HTA procedure were followed. Therefore as day of HTA application has been considered the day of the publication of the Price in the Price Bulletin. Limited publicly available data for the Positive List before September 2018; data for the Price Bulletins on MoH website.	Ministry of Health website – public. Patient contribution is based on the statutory co-payment percentage.	Data for therapeutic protocols is available on the MoH website – public. Additionally there is a specific department in the Ministry of Health responsible for protocols and registries.	MoH website. Data on utilisation is not publicly available. Data for the number of patients has been extracted a) from the Prior Authorization System (for high cost medicinal products) and b) from the BI for prescriptions for patients in private pharmacies. Data on number of patients and volume of sales is considered as commercially sensitive	Data has been provided from the inclusion of a product in the Positive List.
Hungary	National Institute of Health Insurance Fund Management – not public	Division for Health Technology Assessment at the National Institute of Pharmacy and Nutrition of Hungary – not public. The summary of HTA opinions is published regularly in the National Institute of Pharmacy and Nutrition's database, under each entry of medicinal product	Data of the National Institute of Health Insurance Fund Administration available in EURIPID and published on the NEAK website – public.	Calculated by using data of the National Institute of Health Insurance Fund Administration available in EURIPID and published on the NEAK website – public.	Professional guidelines by the National Directorate-General for Hospitals– public.	Data of the National Institute of Health Insurance Fund Administration available in EURIPID and published on the NEAK website – public.	
Iceland	There is no list of early access schemes. The source used is Icelandic Drug Market (IDM), e.g. sales of products before Marketing Authorisation combined with information	Not applicable	Excel files, price catalogue, are published twice per month on the Icelandic Medicines Agency website – public. Guidelines on individual reimbursement on the	Excel files, price catalogue, are published twice per month on the Icelandic Medicines Agency website – public Information from the Pricing and reimbursement division	Clinical guidelines at the National University Hospital.		

Country	A. Early access scheme	B. HTA	C. Coverage & pricing	D. Treatment costs	E. Prescription	F. Utilisation	Other sources?
	provided by the National University Hospital and information on prescription of medicines via Exemption prescription.		Icelandic Health Insurance (IHI) website – public. Information from the Pricing and reimbursement division at the Icelandic Medicine Agency – not public	at the Icelandic Medicine Agency – not public			
Italy	Internal sources – not public	Internal sources – not public	Internal sources – not public	Internal sources – not public – and Official Journal	Internal Sources – not public – and Official Journal	Internal sources – not public	Internal sources (available also in the AIFA website)
Lithuania		Information on applications for HTA – public [State Medicines Control Agency] Reimbursement of medicines and medical aids – public [Ministry of Health]	Legislation regarding approval of the description of the basic prices for outpatient medicinal products for calculating patient premiums– public [Ministry of Health]		Legislation regarding the rules for writing prescriptions – public [Ministry of Health]		
Luxembourg		Not applicable					
Malta	Compassionate use data – not public [requested from the Pharmaceutical Unit, Superintendent of Public Health] Named Patient - Exceptional Medicinal Treatment Database – not public [Directorate for Pharmaceutical Affairs]	HTA internal database – not public [Directorate for Pharmaceutical Affairs]	Government Formulary List - public [Directorate for Pharmaceutical Affairs] Internal HTA and Exceptional databases – not public [Directorate for Pharmaceutical Affairs]	Procurement Price List (Q3 2020 period) and Central Procurement and Supplies Unit website - public		Exceptional Medicinal Treatment Database – not public [Directorate for Pharmaceutical Affairs]	
Norway		Internal database – not public - and information from https://nyemetoder.no/	Internal database – not public - and information from https://nyemetoder.no/, legemiddelksok.no and https://legemiddelverket.no/	Information from legemiddelso.no.		Database from pharmacy association and hospital pharmacies, https://reseptregisteret.no/	
Portugal	Internal data – not public. Active early access programs are available at INFARMED website	Internal data – not public. HTA assessments are available at INFARMED website	For outpatient medicines data about pricing and coverage is available at INFARMED website	Not available	Not available publicly for all medicines.	These data are partially publicly available on the website of INFARMED.	In INFARMED have a database that supports the whole process, and was used to provided the data needed to fill the survey. However this information is not publicly available
Slovenia	Not public	These data are only partially publicly available in the form of Reimbursement Committee reports.	These data are only partially publicly available at Central Drug Database	These data are only partially publicly available in the Central Drug Database and on the website of the Public Agency for medicinal products and medical devices.	These data are publicly available and published on various websites.	These data are only partially publicly available on the website of the Health Insurance institute of Slovenia	Data that are not publicly available are kept at the Health Insurance Institute of Slovenia or at the Public Agency for medicinal products and medical devices.

Country	A. Early access scheme	B. HTA	C. Coverage & pricing	D. Treatment costs	E. Prescription	F. Utilisation	Other sources?
Spain	Online application database – not public	Internal database – not public Published national HTA reports – public – [AEMPS]	Information on products dispensable through pharmacies – public [Ministry of Health] Information on financing of medicines – public [Ministry of Health]				
Sweden	Data from MPA upon request – not public. Current compassionate use programs	Data from decisions – not public If published, HTA reports on TLV website - public	TLV website – public Internal programs used for handling applications – not public	TLV website public Data on agreements - public		Data for sales are available to TLV through a service from the Swedish eHealth Agency. Data for patients are from a public database provided by the National Board of Health and Welfare	

Note: Grey boxes = data requested not publicly accessible (or only partially publicly accessible). Note that some data are missing.
BASG Austrian Medicines and Medical Device Agency (Austria); DSVS (Austria); FAMPHP Federal Agency for Medicines and Health Products (Belgium); NIHDI National Institute for Health and Disability Insurance (Belgium); IMA Inter Mutualistic Agency (Belgium); MoH Ministry of Health; SUKL State Institute for Drug Control (Czech Republic); SAM The State Agency of Medicines (Estonia); HTA Health Technology Assessment; PALKO Council for Choices in Health Care (Finland); FIMEA Finnish Medicines Agency (Finland); HILA Pharmaceuticals Pricing Board (Finland); HAS Haute Autorité de Santé (France); G-BA The Federal Joint Committee (Germany); NEAK National Health Insurance Fund (Hungary); AIFA Italian Medicines Agency (Italy); AEMPS The Spanish Agency of Medicines and Medical Devices (Spain); MPA Swedish Medical Products Agency (Sweden); TLV Swedish Dental and Pharmaceutical Benefits Agency (Sweden).
Source: OECD survey on access to novel medicines 2021. Links last accessed November 2022.

Annex E. Availability dimension

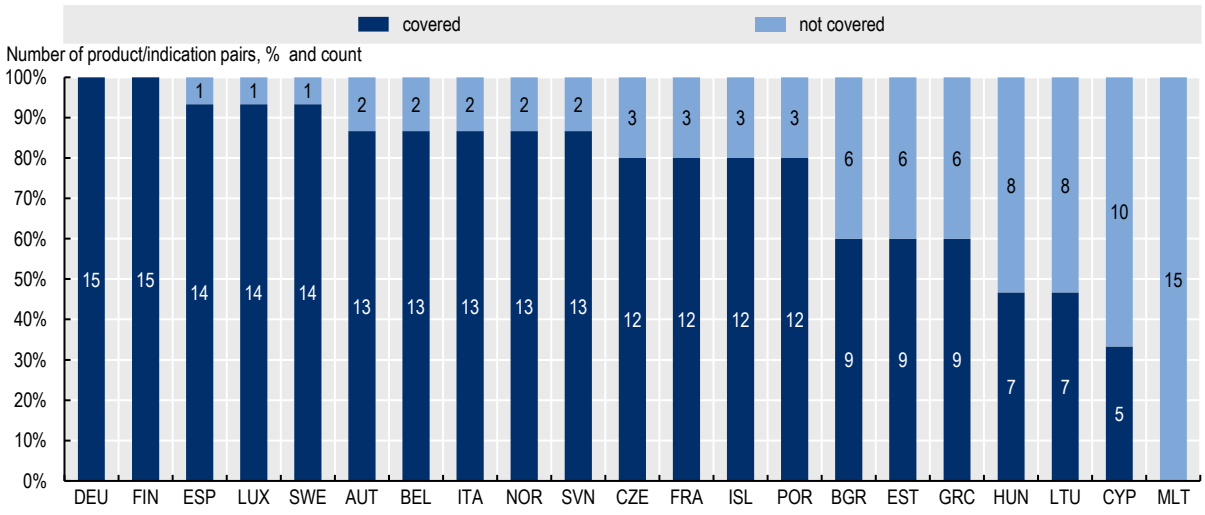
Assessments of availability (see Section 2.1 of the paper)

Table A E.1. Therapeutic alternatives to the sample of 15 index product/indication pairs

Index product (INN)	Broad indication	Possible Alternatives (INN)
alirocumab	reduce LDL- and increase HDL-cholesterol	evocolumab
asfotase alfa	paediatric-onset hypophosphatasia	none
baricitinib	moderate to severe rheumatoid arthritis	tofactinib
dupilumab	severe atopic dermatitis	none
edoxaban	prevention of stroke; treatment of embolism	apixaban rivaroaban dabigatran
erenumab[1]	migraine prophylaxis	galcanezumab fremanzeumab
mepolizumab	asthma	none
niraparib	ovarian, fallopian tube, or primary peritoneal cancer	olaparib rucaparib
nusinersen	spinal muscular atrophy	onasemnogene abeparvovec risdiplam
ocrelizumab	multiple sclerosis	alemtuzumab natalizumab
palbociclib	breast cancer	ribociclib abemaciclib
sacubitril / valsartan	heart failure	SGLT2-inhibitors (e.g. dapagliflozin)
semaglutide	type 2 diabetes	exenatide liraglutide lixisenatide albiglutide dulaglutide
sofosbuvir / velpatasvir	chronic hepatitis c virus (pan-genotype)	glecaprevir/pibrentasvir (Maviret®)
tivozanib	renal cell carcinoma	sunitinib pazopanib sorafenib axitinib

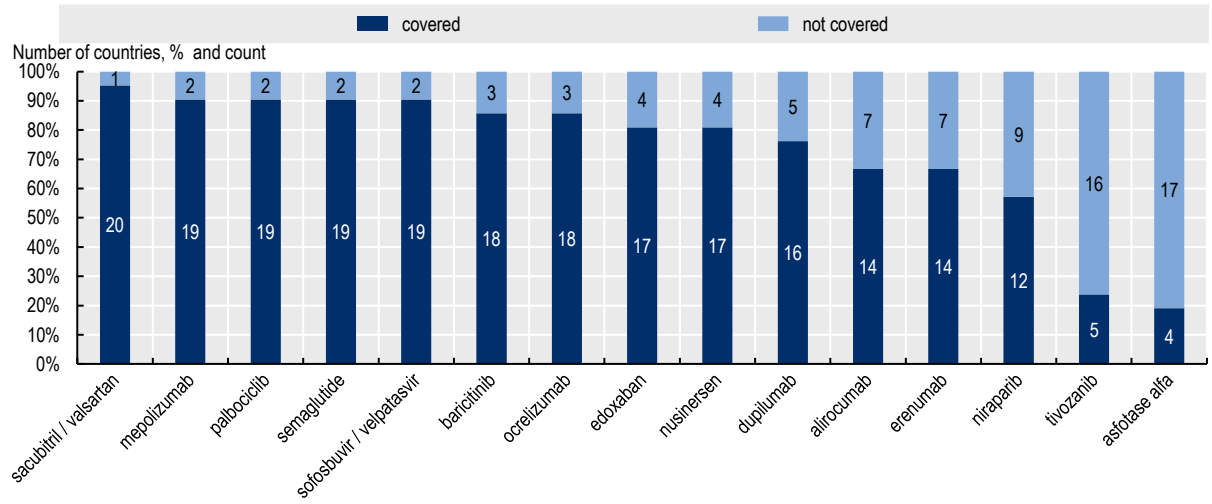
Note: INN international non-proprietary name.
1. Only those products in the same class were included here, however other products in different classes may be considered as appropriate alternatives in this case.
Source: Authors, based on information available in European Public Assessment Reports, comparisons in select health technology assessment reports, and provided by responding countries.

Figure A E.1. Proportion of index product/indication pairs by coverage status across countries



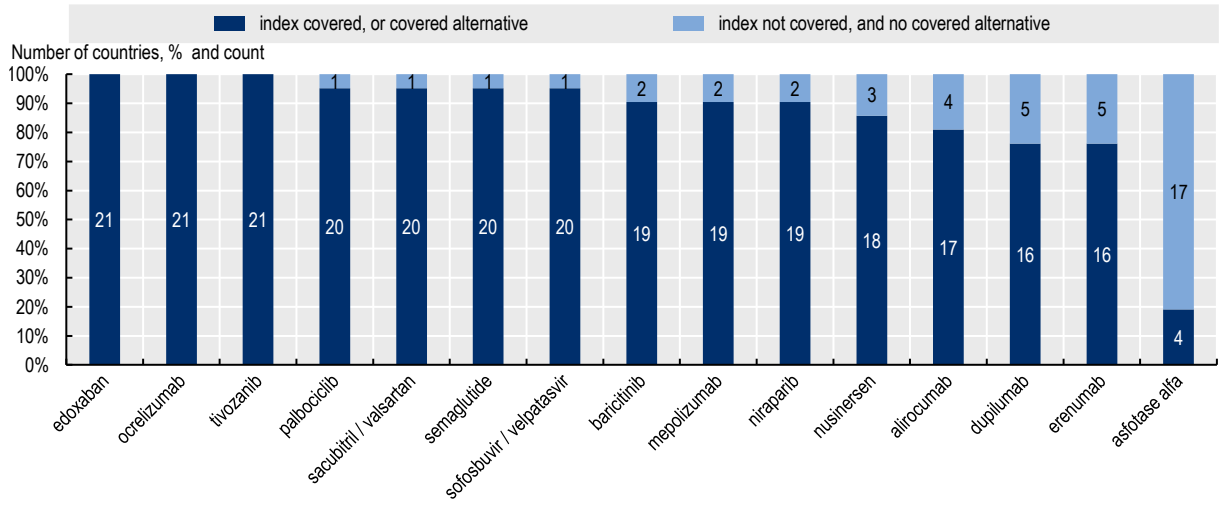
Note: Proportions based on coverage status of a sample of 15 index product/indication pairs, in 21 responding countries, as at 01 October 2021.
Data labels show counts of product/indication in each category, per country.
Source: OECD survey on access to novel medicines 2021.

Figure A E.2. Proportion of countries by coverage status across index product/indication pairs



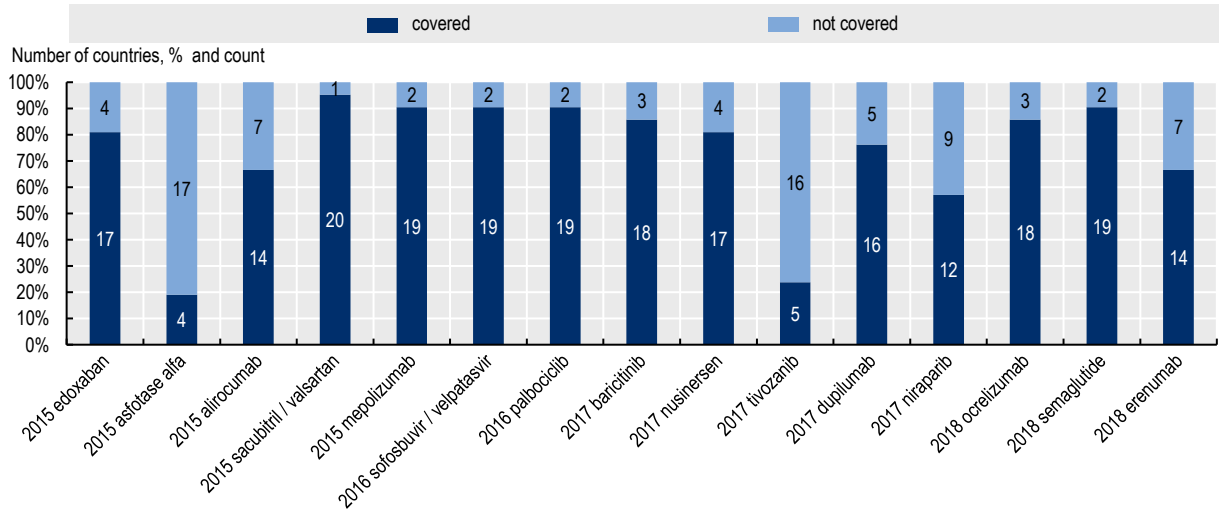
Note: Proportions based on coverage status of a sample of 15 index product/indication pairs, in 21 responding countries, as at 01 October 2021.
Data labels show counts of countries in each category, per product/indication pair.
Source: OECD survey on access to novel medicines 2021.

Figure A E.3. Proportion of countries by coverage status across index product/indication pairs (or alternatives)



Note: Proportions based on coverage status of a sample of 15 index product/indication pairs (or appropriate therapeutic alternatives) in 21 responding countries, as at 01 October 2021. Data labels show counts of countries in each category, per product/indication pair (or alternative).
Source: OECD survey on access to novel medicines 2021.

Figure A E.4. Proportion of countries by coverage status across index product/indication pairs, ordered by year of EU MA



Note: Based on coverage status of a sample of 15 index product/indication pairs in 21 responding countries, as at 01 October 2021. Data labels show counts of countries in each category, per product/indication pair. Year refers to EU central marketing authorisation.
Source: OECD survey on access to novel medicines 2021.

Table A E.2. Overall availability of convenience sample of 15 index product/indication pairs across countries

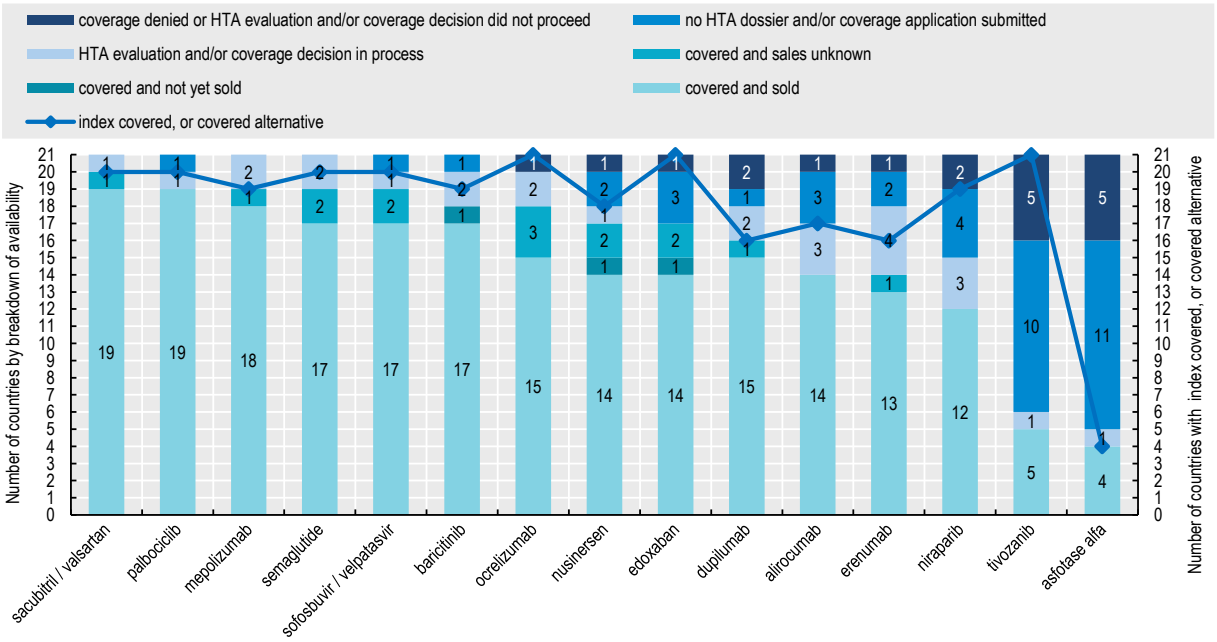
Reflects coverage status of index products and availability of alternatives within the indicated broad indication, valid as at 01 October 2021

INN	Broad indication	AUT	BEL	BGR	CYP	CZE	DEU	ESP	EST	FIN	FRA	GRC	HUN	ISL	ITA	LTU	LUX	MLT	NOR	POR	SVN	SWE
alirocumab[1,2]	*reduce LDL- and increase HDL- cholesterol*			+	–				+				–			–		–		+		
asfotase alfa[3]	*paediatric-onset hypophosphatasia*	–	–	–	–	–		–	–			–	–	–	–	–		–	–	–	–	–
baricitinib	*moderate to severe rheumatoid arthritis*				+											–		–				
dupilumab	*severe atopic dermatitis*			–	–							–	–					–				
edoxaban	*prevention of stroke; treatment of embolism*				+						+	+						+				
erenumab[4, 5]	*migraine prophylaxis*			–	–				–		+	+	–					–				
mepolizumab[1]	*asthma*				–													–				
niraparib	*ovarian, fallopian tube, or primary peritoneal cancer*			+	+	+			+			+	–	+		+		–				
nusinersen[6]	*spinal muscular atrophy*								+				–			–		–				
ocrelizumab[1, 7]	*multiple sclerosis*												+					+	+			
palbociclib	*breast cancer*															+		–				
sacubitril / valsartan	*heart failure*																	–				
semaglutide[8]	*type 2 diabetes*				+													–				
sofosbuvir / velpatasvir[9]	*chronic hepatitis c virus (pan-genotype)*															+		–				
tivozanib[6]	*renal cell carcinoma*	+	+	+	+	+			+		+	+	+	+	+	+	+	+		+	+	

Note: covered and sold; covered and sales unknown; covered and not yet sold; HTA evaluation and/or coverage decision in process; no HTA dossier and/or coverage application submitted; coverage denied or HTA evaluation and/or coverage decision did not proceed. For those products that are not yet covered in the indicated indication: (+) means there is another covered (i.e. reimbursed) product, either within the same, or from another therapeutic class, that is considered a satisfactory alternative (–) means there are no covered alternatives.

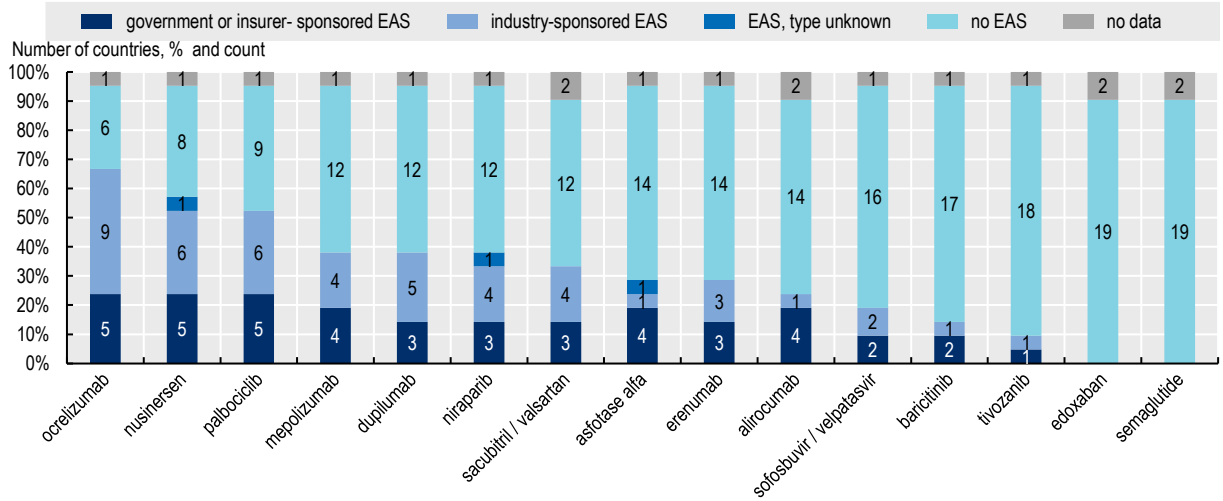
1. In Malta, process for official introduction on the government formulary list was in process. 2. In Estonia, alirocumab has been covered since 01/01/2022. 3. In Bulgaria, negotiation process completed on 23/12/2021, and product reimbursed from 01/01/2022. 4. For the purposes of this analysis, only galcanezumab and fremanezumab were considered as alternatives (see Table A E.1), however other products in different classes may be considered as appropriate alternatives in this case (such as botox). In France, fremanezumab and galcanezumab are covered in the inpatient sector. In Greece, the recommendation for coverage is publicly available, but inclusion in the positive list is pending. 5. In Malta, migraine is not one of the diseases and conditions for which medicines are reimbursed. 6. In Lithuania, nusinersen and tivozanib are intended for the treatment of very rare diseases and decisions are only made on an individual basis. 7. In Austria, reflects data in the primary and ambulatory care setting; financing in the hospital setting preceeded that in the private practice sector. 8. In Cyprus, the government procures GLP-1 inhibitors based on competitive tenders; the reimbursement of the entire category is under assessment. 9. In Finland, this is reimbursed 100% by the municipalities; in Iceland, there was a nationwide elimination campaign from January 2016 to end of 2021. Source: OECD survey on access to novel medicines 2021.

Figure A E.5. Proportion of countries by availability breakdown across index product/indication pairs



Note: Left-hand vertical axis: bars represent breakdown of availability of a sample of 15 index product/indication pairs, in 21 responding countries. Right-hand vertical access: blue line shows number of covered products (either index or alternative). As at 01 October 2021.
The number of countries with covered products – either index (shown) or alternative – on the right-hand vertical axis has no relation to the breakdown of availability categories shown in the columns on the left-hand axis. It is included to show that the overall availability of a medicine alone is not reflective of access to treatment if alternatives are available.
Example of how to read the graph: Tivozanib is covered in five countries. However, 21 countries cover either tivozanib or appropriate alternatives.
Source: OECD survey on access to novel medicines 2021.

Figure A E.6. Proportion of countries by early access scheme (current or in the past) across index product/indication pairs



Note: EAS = early access scheme, either named-patient or population-based. Proportions based on a sample of 15 product/indication pairs, in 21 responding countries, as at 01 October 2021. Data labels show counts of countries in each category, per product/indication pair.
Source: OECD survey on access to novel medicines 2021.

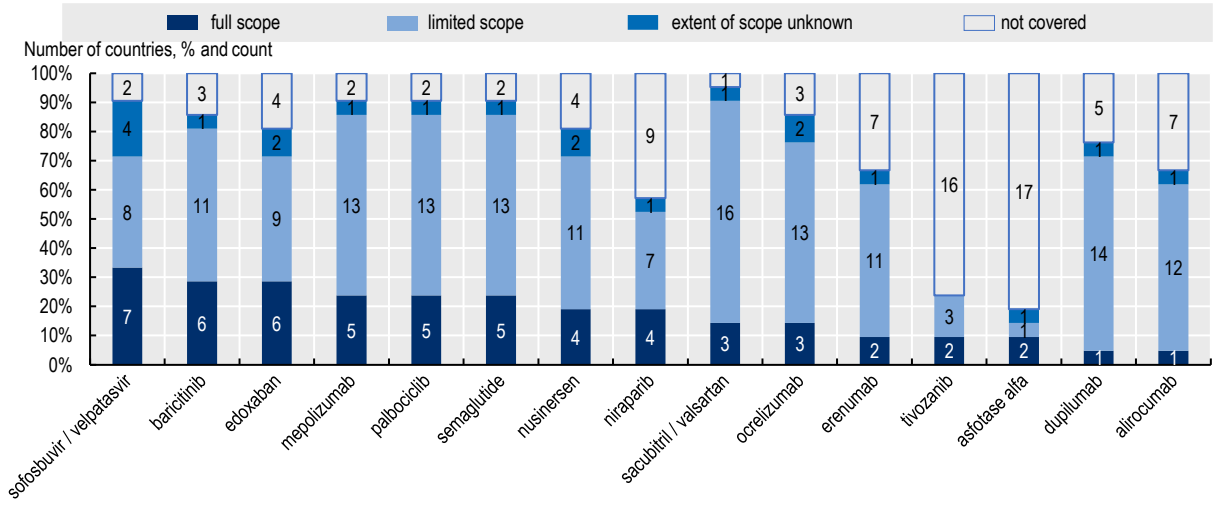
Table A E.3. Existence of early access scheme for convenience sample of 15 index product/indication pairs across countries

Based on a sample of 15 product/indication pairs, in 21 responding countries, at 01 October 2021

INN	Broad indication	AUT	BEL	BGR	CYP	CZE	DEU	ESP	EST	FIN	FRA	GRC	HUN	ISL	ITA	LTU	LUX	MLT	NOR	POR	SVN	SWE
alirocumab	*reduce LDL- and increase HDL- cholesterol*				†							†	†				*†	†				
asfotase alfa	*paediatric-onset hypophosphatasia*		†	†				†				†	†		†							
baricitinib	*moderate to severe rheumatoid arthritis*		†									†	†									
dupilumab	*severe atopic dermatitis*		†			†		*†	†		*†		†							†		
edoxaban	*prevention of stroke; treatment of embolism*																					
erenumab	*migraine prophylaxis*		†		†	†		†				†	†									
mepolizumab	*asthma*		*†		†	*†		*†				†	†		*†			†				
niraparib	*ovarian, fallopian tube, or primary peritoneal cancer*	*†	†					*†			*†	†	†		*†					†		
nusinersen	*spinal muscular atrophy*	*†	*†	†	†	†		*†			*†		†		*†					†	*†	
ocrelizumab	*multiple sclerosis*		*†		†			*†	*†		*†	†	†	*†	*†		*†	†		†	*†	*†
palbociclib	*breast cancer*		*†					*†	*†		*†	†	†				*†			†	*†	*†
sacubitril / valsartan	*heart failure*		†		†			*†			*†		†				*†	*				
semaglutide	*type 2 diabetes*																					
sofosbuvir / velpatasvir	*chronic hepatitis c virus (pan-genotype)*												†	†	*†					†		
tivozanib	*renal cell carcinoma*											†			†							

Note: government or insurer-sponsored EAS; industry-sponsored EAS; EAS, type unknown.; no EAS; no data. * EAS in place prior to EU-wide marketing authorisation; † EAS in place prior to publicly funded coverage decision.
EAS = early access scheme, either named-patient or population-based. Survey responses in answer to the question: *"Is there (or was there in the past) an early access scheme, as at 01 October 2021?"*;
Source: OECD survey on access to novel medicines 2021.

Figure A E.7. Proportion of countries by extent of coverage in comparison to the EU marketing authorisation indication across index product/indication pairs



Note: Proportions based on a sample of 15 product/indication pairs, in 21 responding countries, as at 01 October 2021. Data labels show counts of countries in each category, per product/indication pair. Full scope = covered indication not narrower than the EU authorised indication by (1) patient population or subgroup, (2) duration or quantity of treatment for individual patients, or (3) prerequisite of failure of (or intolerance to) a prior therapy. Limited scope = covered indication narrower than the EU authorised indication by at least one of categories (1), (2) or (3).
Source: OECD survey on access to novel medicines 2021.

Table A E.4. Extent of coverage in comparison to MA for convenience sample of 15 index product/indication pairs across countries

Based on a sample of 15 product/indication pairs, in 21 responding countries, at 01 October 2021

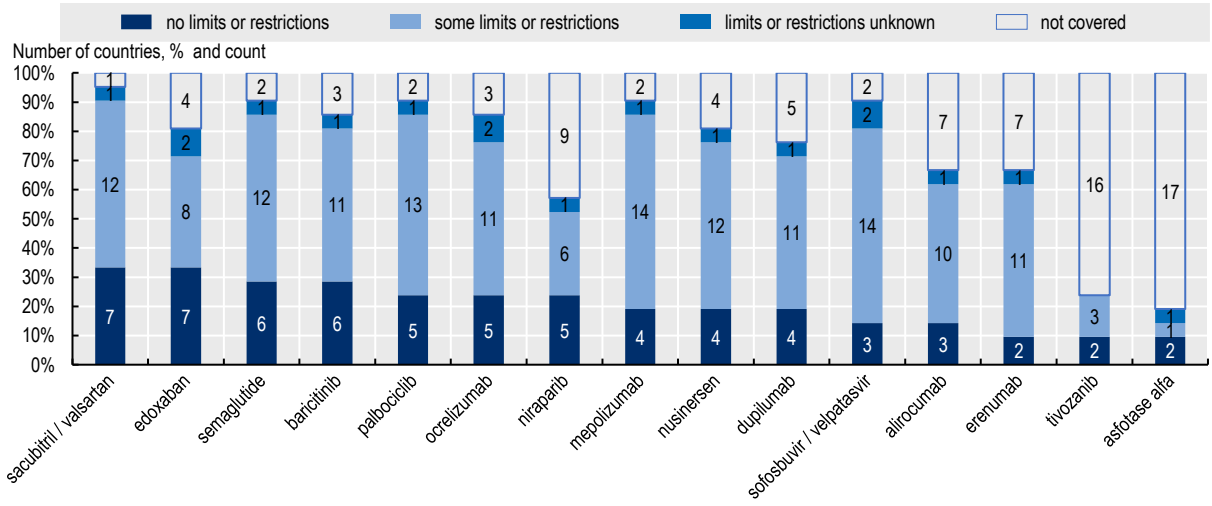
INN	Broad indication	AUT	BEL	BGR	CYP	CZE	DEU	ESP	EST	FIN	FRA	GRC	HUN	ISL	ITA	LTU	LUX	MLT	NOR	POR	SVN	SWE
alirocumab	*reduce LDL- and increase HDL- cholesterol*																					
asfotase alfa	*paediatric-onset hypophosphatasia*																					
baricitinib	*moderate to severe rheumatoid arthritis*																					
dupilumab	*severe atopic dermatitis*																					
edoxaban	*prevention of stroke; treatment of embolism*																					
erenumab	*migraine prophylaxis*																					
mepolizumab	*asthma*																					
niraparib	*ovarian, fallopian tube, or primary peritoneal cancer*																					
nusinersen	*spinal muscular atrophy*																					
ocrelizumab	*multiple sclerosis*																					
palbociclib	*breast cancer*																					
sacubitril / valsartan	*heart failure*																					
semaglutide	*type 2 diabetes*																					
sofosbuvir / velpatasvir	*chronic hepatitis c virus (pan-genotype)*																					
tivozanib	*renal cell carcinoma*																					

Note: full scope = covered indication not narrower than the EU authorised indication by (1) patient population or subgroup, (2) duration or quantity of treatment for individual patients, or (3) prerequisite of failure of (or intolerance to) a prior therapy; limited scope = covered indication narrower than the EU authorised indication by at least one of categories (1), (2) or (3); extent of scope unknown; not covered.

Malta was excluded as no product/indication pairs were covered. In Finland, nusinersen, ocrelizumab and sofosbuvir/velpatasvir are available through hospitals.

Source: OECD survey on access to novel medicines 2021.

Figure A E.8. Proportion of countries by additional limits or restrictions applied to the coverage decision across index product/indication pairs



Note: Proportions based on a sample of 15 product/indication pairs, in 21 responding countries, as at 01 October 2021. Data labels show counts of countries in each category, per product/indication pair. Full coverage = covered indication not narrower than the EU authorised indication by (1) patient population or subgroup, (2) duration or quantity of treatment for individual patients, or (3) prerequisite of failure of (or intolerance to) a prior therapy. Limited coverage = covered indication narrower than the EU authorised indication by at least one of categories (1), (2) or (3).
Source: OECD survey on access to novel medicines 2021.

Table A E.5. Additional limitations and restrictions on coverage for convenience sample of 15 index product/indication pairs across countries

Based on a sample of 15 product/indication pairs, in 21 responding countries, as at 01 October 2021

INN	Broad indication	AUT	BEL	BGR	CYP	CZE	DEU	ESP	EST	FIN	FRA	GRC	HUN	ISL	ITA	LTU	LUX	MLT	NOR	POR	SVN	SWE
alirocumab	*reduce LDL- and increase HDL- cholesterol*																					
asfotase alfa	*paediatric-onset hypophosphatasia*																					
baricitinib	*moderate to severe rheumatoid arthritis*																					
dupilumab	*severe atopic dermatitis*																					
edoxaban	*prevention of stroke; treatment of embolism*																					
erenumab	*migraine prophylaxis*																					
mepolizumab	*asthma*																					
niraparib	*ovarian, fallopian tube, or primary peritoneal cancer*																					
nusinersen	*spinal muscular atrophy*																					
ocrelizumab	*multiple sclerosis*																					
palbociclib	*breast cancer*																					
sacubitril / valsartan	*heart failure*																					
semaglutide	*type 2 diabetes*																					
sofosbuvir / velpatasvir	*chronic hepatitis c virus (pan-genotype)*																					
tivozanib	*renal cell carcinoma*																					

Note: no limits or restrictions = coverage not restricted by (1) requirement for demonstrated response to treatment; (2) maximum number of patients per annum; (3) prescriber type; or (4) other; some limits or restrictions = coverage restricted by at least one of categories (1), (2), (3) of (4); limits or restrictions unknown; not covered.
Source: OECD survey on access to novel medicines 2021.

Table A E.6. HTA and coverage are part of a joint process in most countries

COU	Notes on health technology assessment (HTA) and coverage processes
AUT	The Federation of Social Insurances (DVSV) is an HTA and P&R body (for outpatient medicines). Evaluations are not public.
BEL	In Belgium the HTA, appraisal, MEA systems and decision are part of one national procedure.
BGR	HTA and coverage applications are connected and start at the same time.
CYP	Health Insurance Organisation (HIO) is the reimbursement authority for the NHS in Cyprus. Cyprus does not perform its own HTA assessments but rather uses data from larger agencies. Even though there is no original HTA work done, the decisions of the institutions used as references are evaluated in an HTA sense by the Drug Assessment Committee.
CZE	There is only one process in the Czech Republic in place. Once the coverage application is submitted to the State Institute for Drug Control (SUKL), together with the HTA documentation, the HTA appraisal process is initiated automatically.
EST	Applications for HTA dossier and reimbursement are together in Estonia and are evaluated together by the Estonian Health Insurance Fund.
FIN	Finland does not have one common HTA evaluation process for all medicines; inpatient and outpatient medicines are evaluated through different HTA evaluation systems under different decisive bodies. In hospital-used medicines the Council for Choices in Health Care (PALKO) makes the recommendations and the Finnish Medicine Agency (FIMEA) gathers together the HTA dossier. In outpatient care the elements of HTA dossier are evaluated when the price and reimbursement decisions are made in the Pharmaceuticals Pricing Board (HILA).
FRA	The manufacturer submits an application to the Haute Autorité de Santé (HAS), which performs HTA to determine whether or not a medicine should be included in the positive list. Appraisal is undertaken by the Transparency Committee (TC). The final opinion is sent to the National Health Insurnace Funds (Uncam) to determine the level of co-payment, and the Economic Committee of Healthcare Products (CEPS) to determine the price. The final decision for inclusion in the positive list is taken by the Ministry of Health.
DEU	The law reforming the pharmaceutical market (Arzneimittelmarkt-Neuordnungsgesetz – AMNOG), which took effect in January 2011, has kept the principle of free pricing at launch but imposes a systematic and formal assessment of the "added therapeutic benefit" of new medicines in order to negotiate the price according to the therapeutic value of the drug within twelve months after market launch (this has since been changed to six months in the new legislation in 2022). If a new drug has some added therapeutic benefit over existing standards of care, a reimbursement price is negotiated based on the prices of appropriate comparators (the current standard of care) between the national association of statutory health insurance funds (Spitzenverband Bund der Krankenkassen – GKV-SV) and the pharmaceutical company. Since 2022, price negotiations for new pharmaceuticals with no additional benefit result in a price at least 10% lower than appropriate patent-protected comparative therapy. For new pharmaceuticals with minor additional benefit or non-quantifiable benefit a price is negotiated that should not be higher than the price of the appropriate patent-protected comparator.
GRC	HTA procedure only introduced in 2018, when the HTA and Reimbursement Committee was established by law. There is an HTA Committee which at first creates an HTA assessment (data on submission and outcome of the HTA is not publicly available to this stage) and then the report is send to the Negotiation Committee. After successful negotiation, the HTA gives the final positive recommendation for the inclusion of a product in the Positive List.
HUN	A reimbursement application is made to the National Health Insurance Fund (NHIF), which invites the National Institute of Pharmacy and Nutrition (NIPN) to review the submission. The Health Technology Assessment (HTA) committee makes a final conclusion on the professional submission aspects.
ISL	HTA not applicable.
ITA	HTA and coverage/reimbursement processes are centralised in Italy, managed by AIFA.
LTU	HTA process is new in Lithuania and started only in 01/01/2020. Before HTA, procedure for medicinal products inclusion consisted of: 1. Evaluation of therapeutic value (State Medicine Control Agency 2. Evaluation of pharmacoeconomic value (Department of Pharmacy of MoH) 3. Evaluation of the budget impact (National Health Insurance Fund)
LUX	HTA not applicable.
MLT	Within the National Health System, the Directorate for Pharmaceutical Affairs (DPA) is responsible for developing and implementing equitable and sustainable Government pharmaceutical policies. The Health Technology Assessment (HTA) Unit within the DPA assists in the decision making process regarding inclusion of new medicines on the Government Formulary List (GFL). The HTA Unit provides technical support to two committees (the technical committee GFLAC (Government Formulary List Advisory Committee) and the financial committee ACHCB (Advisory Committee for Health Care Benefits). The HTA Unit is involved in the pre and post committee procedures, and in creating technical reviews of the drugs being assessed.
NOR	HTA and coverage application occur at the same time. For products included under the National Insurance Scheme (i.e. outpatient products), the Norwegian Medicines Agency (NoMA) is responsible for both HTA for medicines, as well as reimbursement decisions under the National Insurance Scheme. For hospital products (including oncology treatments), the four regions are responsible for reimbursement. However, the four regions participate in a Decision Forum, which reaches a common decision for all regions that is de facto national. NoMA is also responsible for HTA for these medicines.
POR	The reimbursement in Portugal is conditional to an HTA assessment for all new medicines and new indications, so HTA and coverage are filed at the same time. The company requests reimbursement in an electronic platform, SIATS, where the company also upload the therapeutic value dossier and in a subsequent phase the economic study, if required.
SVN	In Slovenia, there is no HTA agency. The economic part (HTA) dossier of the company is evaluated at the Health Insurance Institute together with the clinical part of the application. The Reimbursement Committee, the independent body under the Health Insurance Institute, consisted of external experts, assess the applicable and prepare the reports, that are publicly available.
ESP	In Spain, the pharmaceutical company may communicate its intention to commercialise the product prior to the EU marketing authorisation date, only for informative purposes and in order to get the therapeutic positioning report elaboration process started. However, an HTA dossier will not be submitted to the P&R authority by the company before the EU marketing authorisation date, since the P&R process is only initiated once the

COU	Notes on health technology assessment (HTA) and coverage processes
	marketing authorisation has been granted and communicated to the AEMPS (Spanish Agency for Medicines and Medical Devices). The manufacture submits the dossier to the Interministerial Committee for pricing and reimbursement, then a drug appraisal report is made by AEMPS, followed by a decision by the Interministerial Committee for Pricing and Reimbursement.
SWE	The Swedish Dental and Pharmaceutical Benefits Agency (TLV) is responsible for both HTA assessments and reimbursement decisions for outpatient pharmaceuticals. For pharmaceuticals that are for inpatient care, the regions make the decisions. They have a joint New Therapies (NT) Council that make recommendations. These recommendations are not mandatory, and a region can choose to use a pharmaceutical even though the NT Council has recommended that it should not be used. This means that there are no definite decisions made regarding coverage and pricing.

Note: HTA health technology assessment; P&R Pricing and reimbursement; MEA managed entry agreement; NHS National Health Service
Source: OECD survey on access to novel medicines 2021, supplemented by information available in IMPACT HTA's country vignettes, available at https://www.impact-hta.eu/country-vignettes, last accessed November 2022.

Time-to-access was decomposed into different time periods (see Section 2.2 of the paper)

For comparisons among countries within Europe, the most pertinent measures of time-to-access (TTA) begin from the date of the EU-wide marketing authorisation. All product/indication pairs within the sample were authorised via the centralised procedure and thus the marketing authorisation date is the same across countries. Indeed, while marketing authorisation is the first step in market access, in many countries, companies may choose to launch a medicine only once it has coverage (often at a regulated price) or delay it because of extensive use of external price referencing. There are some exceptions: in Germany for example, every authorised medicine is covered by default, and a company generally introduces a product to market at the same time as the first HTA application. In some countries, a product may also be supplied prior to a coverage decision, generally on a case-by-case basis but sometimes wider population-access is also possible.

In this study, the total time (in days) between EU-wide marketing authorisation and a national positive coverage decision[4] was first anlaysed, and then further decomposed into several time periods. Figure A E.9 shows the decomposition into various periods (see Table A C.2 for additional time periods that could be analysed). Given the differences across country systems, Figure A E.9 also displays the various sequence-of-event scenarios for the decomposed time periods[5]:

- **Period 1** was defined as the time between the EU-wide marketing authorisation and either the first submission of an HTA application or first coverage application. Differences between countries in this indicator may reflect the launch strategies of pharmaceutical companies (i.e. sequential product launches and delayed application for coverage/pricing decisions – influenced by market size and national pharmaceutical policies). In most cases within the sample both the first HTA and coverage applications were filed at the same time. In some cases the coverage application was made after the HTA application, in which case this period reflects time between the marketing

[4] It is recognised that the national coverage decision date is not necessarily the same date as actual coverage i.e. when a product is included in a positive reimbursement list or available to be prescribed in a national pharmacy database. This date was chosen for practical reasons and can be considered as a proxy of coverage date. It is also important to recognise that time-to-access as measured in this study does not measure time limit delays as defined in the Transparency Directive (directive 89/105/EC). This time period also does not account for population-wide early access schemes that may be used to accelerate access in some countries, meaning the time difference displayed could be shorter for some countries.

[5] Note that the sequence of events (e.g. when application for HTA and/or coverage are made) may differ among countries but also within countries, across product/indication pairs. The product/indication pairs for which these time periods are computed may also differ among countries. This needs to be a consideration of any further analyses of time-to-access indicators.

authorisation and first HTA application. In others, there was no HTA process at all, and this period represents the time between marketing authorisation and first coverage application. In order to accelerate access, some countries allow companies to submit HTA and/or coverage applications before a product receives marketing authorisation. However, there were only a few cases in this sample. This time difference is most relevant for countries where a coverage decision is made at a national level, and less so for others.

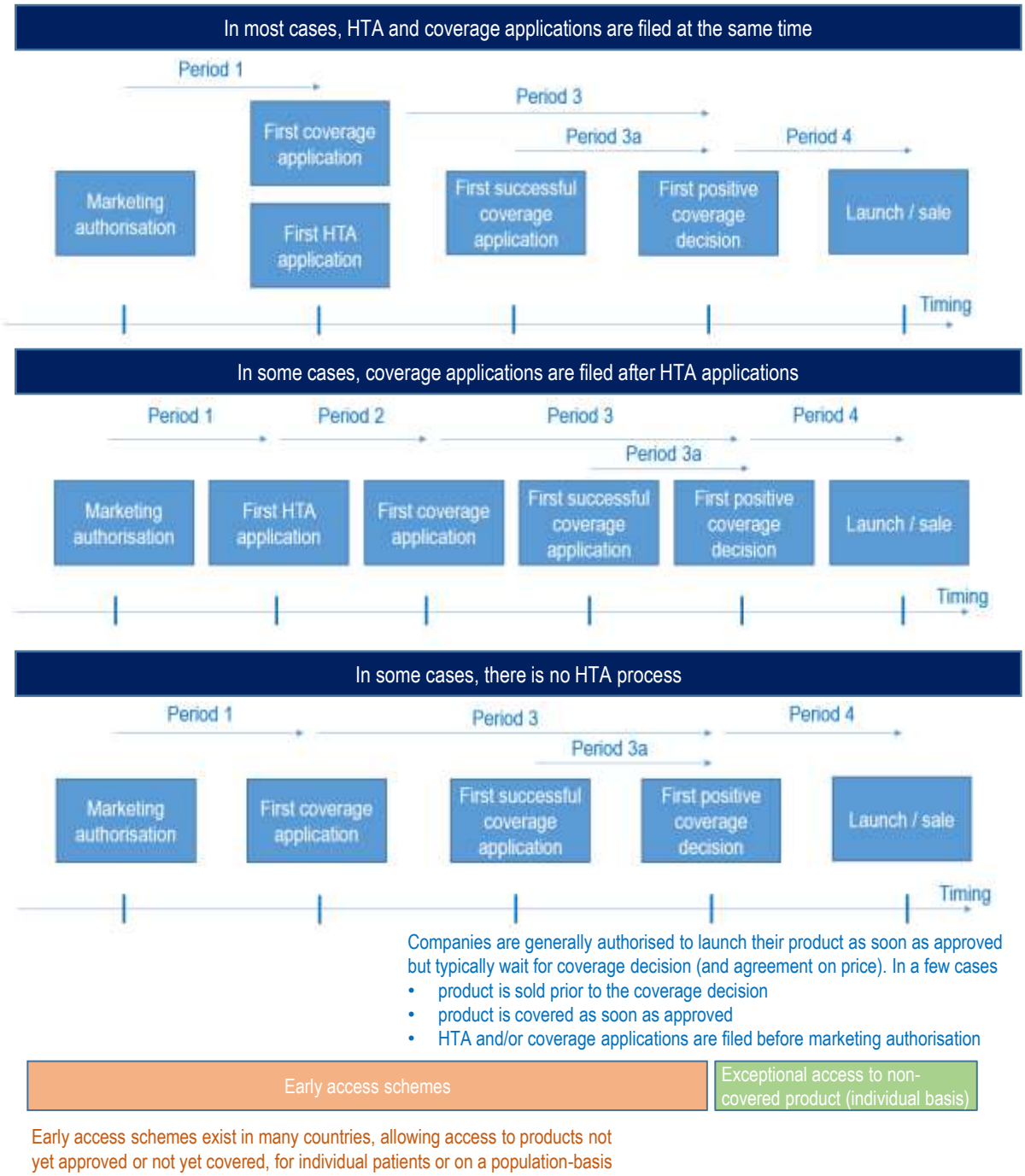
- **Period 2** was defined as the time between first HTA application and first coverage application. This time difference is only relevant for those cases in which applications for HTA occur prior to coverage applications.
- **Period 3** was defined as the time from first coverage application to first positive coverage decision. For countries without automatic coverage, this time difference may reflect several factors; for those product/indication pairs with a successful coverage decision on the first attempt, this includes the nature and performance of pricing and coverage-decision making processes in the country. However, clock stops have not been taken into account here. In other cases, this time difference may also reflect one or multiple unsuccessful submissions. This type of indicator is most relevant for countries where coverage decisions are made at national level.
    - **Period 3a** was defined as the time from first *successful* coverage application to subsequent first positive coverage decision, without considering clock stops. For a subset of countries, clock stops were able to be accounted for (**Period 3b**).
- **Period 4** was defined as the time between the first positive coverage decision and the date of first sale (i.e. date of first utilisation in a country market). The date of first sale can be used as a proxy for "launch" of a product, though in some cases sales data may only be available monthly. Several countries provided first sales dates prior to granting of coverage, likely reflecting access on an individual patient basis such as through named-patient early access schemes. However, in many cases this would be considered as "pseudo" access as the product may only be accessible for a limited number of patients and not necessarily widely accessible to all patients through publicly funded coverage. In these cases, the sales dates were adjusted to the positive coverage decision dates.

While these are the only time periods discussed in this paper, others could be further explored. For example, the impact of early access coverage schemes, as well as a breakdown of the health technology process, or the role of pricing decisions. The latter is particularly pertinent when reimbursement and pricing decisions are interlinked. Although the impact of early access schemes was not explored in this analysis, each of the time difference graphs are supplemented by counts of products (from those with available dates) granted some form of early access.

As with the other indicators, presented data on time-to-access cannot be considered as representative of the access situation in individual countries. This was a feasibility study, looking at time differences using a small convenience sample of 15 product/indication pairs, and subsequently testing metrics and presentations of data. As not all 15 index product/indication pairs were covered in all countries, data on time differences are limited. The share of each time period as a proportion of the total time between marketing authorisation and positive coverage decision may also differ substantially according to the medicine.

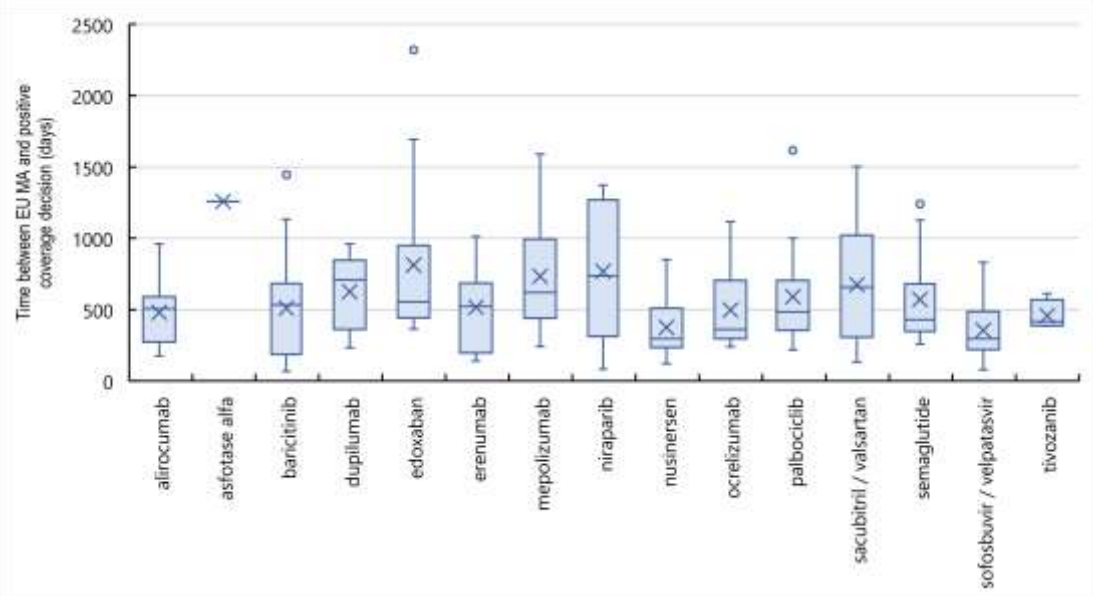
Due to lack of data availability, it was not possible to undertake an in-depth sub analysis on time differences for those products for which coverage was denied, the decision did not proceed, or the HTA and/or coverage decision was in process. However, it would be of interest to further explore times between marketing authorisation and first application of either HTA or coverage for these products, as well as the time to denial.

Figure A E.9. Time-to-access periods, as measured in this study



Note: The time difference calculations in this study do not account for population-wide early access schemes that may be used to accelerate access in some countries.
Source: Authors, adapted from (Chapman, Paris and Lopert, 2020[7]).

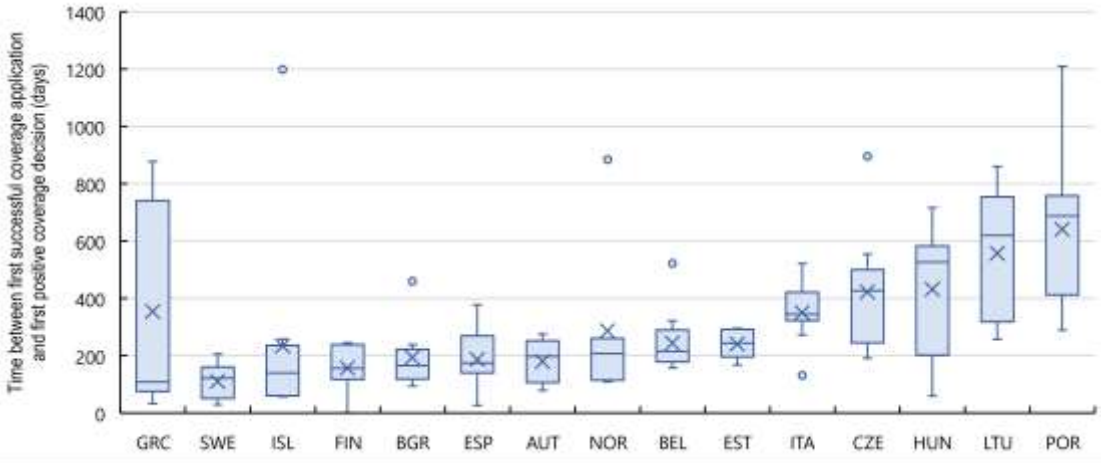
Figure A E.10. Time between EU marketing authorisation and positive coverage decision across index product/indication pairs, in days (excluding early access coverage schemes)



No. covered	*14*	*4*	*18*	*16*	*17*	*14*	*19*	*12*	*17*	*18*	*19*	*20*	*19*	*19*	*5*
Available dates	*10*	*1*	*15*	*13*	*14*	*11*	*16*	*9*	*10*	*12*	*15*	*15*	*16*	*13*	*4*
- No. with EAS	*1*	*0*	*3*	*5*	*0*	*3*	*6*	*5*	*7*	*8*	*8*	*3*	*0*	*3*	*0*

Note: X = average time in days per product/indication pairs, across the count of countries; EAS = early access scheme, either named-patient or population-based (see Table A E.3). Based on *covered* products with available date information, from a non-representative convenience sample of 15 product/indication pairs in 21 responding countries. The countries for which these time periods are computed differs among product/indication pairs. The time difference calculations do not account for population-wide early access schemes that may be used to accelerate access in some countries.
Source: OECD survey on access to novel medicines 2021.

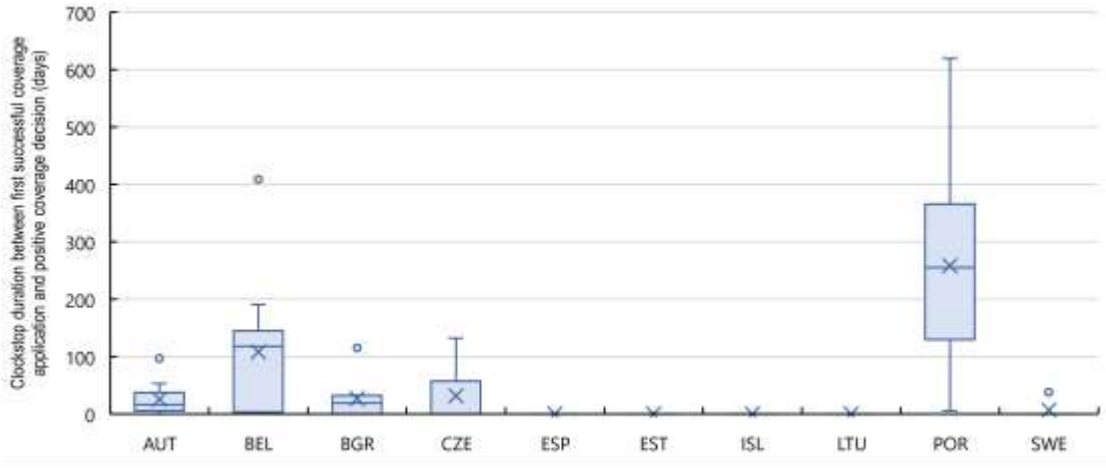
Figure A E.11. Time between first successful coverage application to first positive coverage decision, without considering clock stops, in days (Period 3a)



No. covered	*9*	*14*	*12*	*15*	*9*	*14*	*13*	*13*	*13*	*9*	*13*	*12*	*7*	*7*	*12*
Available dates	*9*	*12*	*11*	*12*	*8*	*11*	*12*	*7*	*13*	*8*	*11*	*11*	*7*	*7*	*12*
- No. with EAS	*5*	*1*	*1*	*0*	*1*	*6*	*1*	*0*	*9*	*3*	*5*	*3*	*5*	*0*	*6*

Note: X = average time in days per country, across the count of product/indication pairs. EAS = early access scheme, either named-patient or population-based (see Table A E.3). Based on *covered* products with available date information, from a non-representative convenience sample of 15 product/indication pairs in 21 responding countries. The product/indication pairs for which these time periods are computed differs among countries. Source: OECD survey on access to novel medicines 2021.

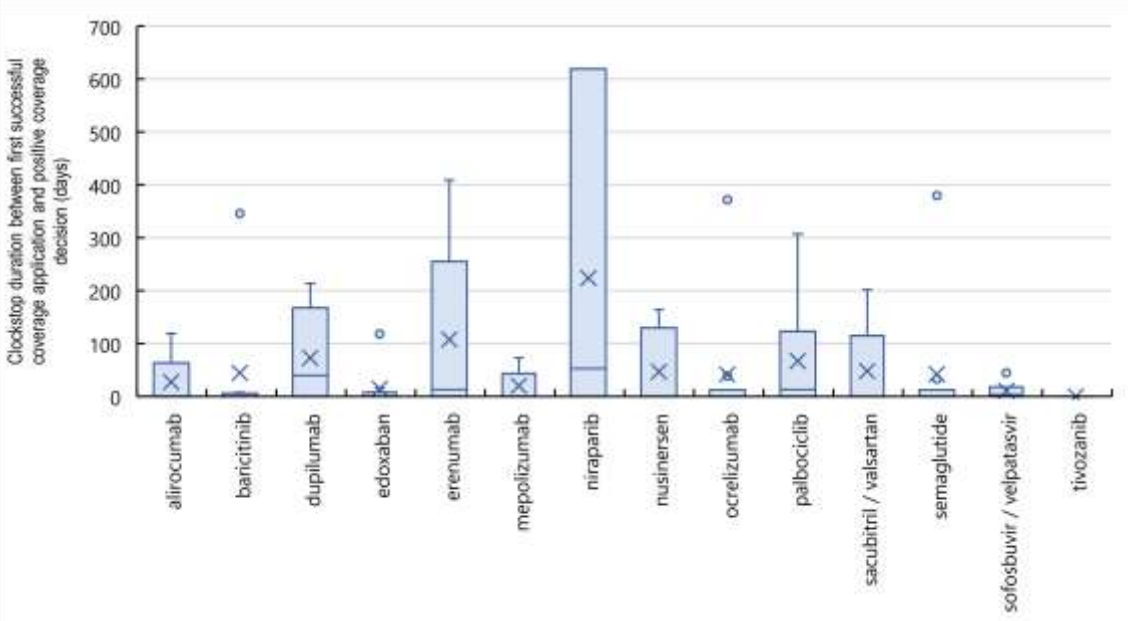
Figure A E.12. Clock stops between first successful coverage application and subsequent positive coverage decision across countries, in days



No. covered	*13*	*13*	*9*	*12*	*14*	*9*	*12*	*7*	*12*	*14*
Available dates	*12*	*11*	*8*	*11*	*11*	*7*	*11*	*7*	*12*	*12*
- No. with EAS	*1*	*8*	*1*	*3*	*6*	*2*	*1*	*0*	*6*	*1*

Note: X = average time in days per country, across the count of product/indication pairs; EAS = early access scheme, either named-patient or population-based (see Table A E.3). Hungary and Italy were removed as data were only available for one and three product/indication pairs, respectively. Based on *covered* products with available clock stop information, from a non-representative convenience sample of 15 product/indication pairs in 21 responding countries. The product/indication pairs for which this time is computed differs among countries.
Source: OECD survey on access to novel medicines 2021.

Figure A E.13. Clock stops between first successful coverage application and subsequent positive coverage decision across index product/indication pairs, in days



No. covered	*14*	*18*	*16*	*17*	*14*	*19*	*12*	*17*	*18*	*19*	*20*	*19*	*19*	*5*
Available dates	*7*	*9*	*9*	*9*	*8*	*11*	*4*	*7*	*11*	*9*	*12*	*11*	*9*	*1*
- No. with EAS	*0*	*1*	*5*	*0*	*3*	*3*	*3*	*6*	*7*	*5*	*4*	*0*	*1*	*0*

Note: X = average time in days per product/indication pairs, across the count of countries; EAS = early access scheme, either named-patient or population-based (see Table A E.3). Based on *covered* products with available clock stop information, from a non-representative convenience sample of 15 product/indication pairs in 21 responding countries. The countries for which this time is computed differs among product/indication pairs. Source: OECD survey on access to novel medicines 2021.

Table A E.7. Summary of decomposed date data able to be provided by survey respondents

COU	Date of first HTA application	Date of first coverage application	Date of first coverage decision (favourable or denial)	Date of first successful application for coverage	Date of first favourable coverage decision (i.e. positive coverage decision)	Date of first sales	Clock stop amount between first successful application and positive decision
AUT	√	√	√	√	√	√	√
BEL	√	√	√	√	√	√	√
BGR	√	√	√	√	√	√	√
CYP							
CZE	√	√	√	√	√	√	√
DEU	√	n/a	n/a	n/a	n/a	√	n/a
ESP	√	√	√	√	√	√	√
EST	√	√	√	√	√	√	√
FIN	n/a	√	√	√	√		
GRC	n/a	√	√	√	√	√	
HUN	√	√	√	√	√	√	√
ISL	n/a	√	√	√	√	√	√
ITA	√	√	√	√	√	√	√
LTU	n/a	√	√	√	√		√
LUX	n/a						
NOR	√	√	√	√	√	√	
POR	√	√	√	√	√	√	√
SVN	√	√			√	√	n/a
SWE	√	√	√	√	√	√	√

Note: Grey = no answer provided. n/a = not applicable due to organisation of country health system or because another date was not provided. Not applicable to Malta as none of the sample pairs were covered. Measuring time-to-access in France without taking into account early access coverage schemes can be misleading.
Source: OECD survey on access to novel medicines 2021.

Annex F. Affordability dimension

Affordability estimates were made on individual medicine prices, following a “like for like” methodology i.e. between products with identical characteristics (active substance, strength, pharmaceutical form, and pack size). It is not recommended to aggregate medicine prices to an average across a sample, as prices vary significantly by medicine. In general, price comparison analyses should only be made for individual medicines, with more complex methods required for any aggregate analyses (World Health Organization and Health Action International, 2008[8]).

Several sources were consulted to inform the affordability methodology used in this paper, including (Vogler et al., 2021[9]; World Health Organization and Health Action International, 2008[8]; Habl et al., 2018[10]; The Dental and Pharmaceutical Benefits Agency (Sweden), 2020[11]; Iyengar et al., 2016[12]).

Affordability to the system

A combination of survey response and EURIPID data were used to calculate the ratio of the cost of a year of treatment with each medicine in a country and GDP per capita (indicator of affordability for the system), using ex-factory prices in local currency. Priority was given to EURIPID data given the high quality and comparability of its data set, and survey data points were only used for medicine country combinations missing from EURIPID. Prices were transformed to reflect the pack size set by the questionnaire (prices for packs bigger than 1.5 times the pack size set by the questionnaire were not taken into consideration). Annual treatment cost was then calculated by adjusting for an estimated years’ worth of treatment (see treatment regimen – Table A F.1[6]). The ratio of estimated annual treatment cost to GDP per capita was calculated using 2021 GDP per capita (current LCU) from the World Bank database. Asfotase alfa and tivozanib were excluded due to lack of data availability.

Affordability to the patient

The ratio of the monthly out-of-pocket cost and average daily wage (indicator of affordability for patients) was calculated using survey data. Many countries provided the out-of-pocket price as a percentage of the retail or reimbursement price, so the nominal out-of-pocket contribution needed to be calculated first. As for ex-factory price data, out-of-pocket costs were transformed to reflect the pack size set by the questionnaire (prices for packs bigger than 1.5 the pack size set by the questionnaire were not taken into consideration). Monthly out-of-pocket costs were calculated by adjusting for a months’ worth of treatment (see treatment regimen – Table A F.1). The ratio of out-of-pocket costs to the average daily wage was calculated using 2021 average wage data in current prices in NCU from the OECD data base (annual wage was divided by 240 working days). Affordability was expressed as the number of days of average wages needed to pay for one month of treatment.

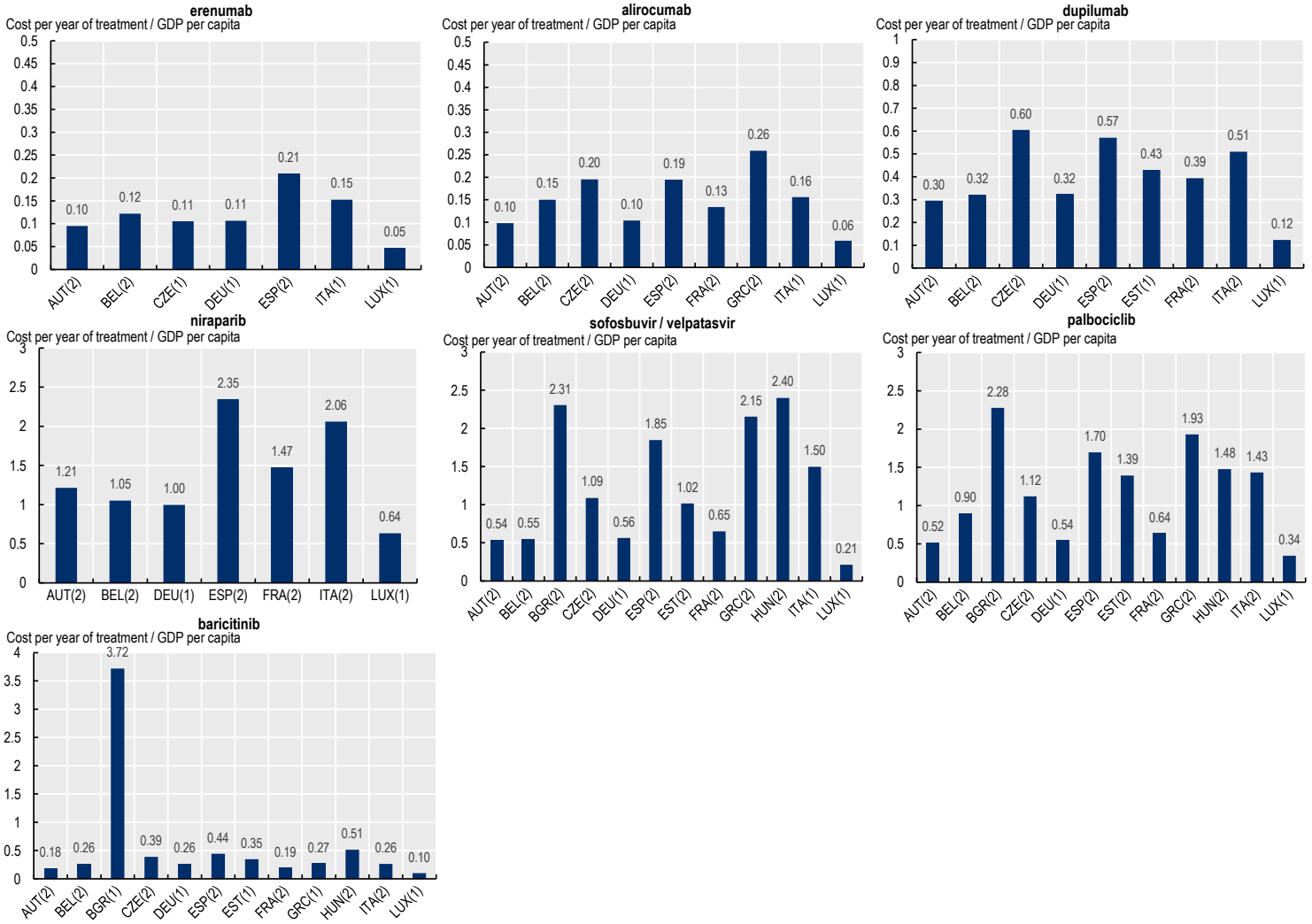
[6] Annual cost for sofosbuvir/velpatasvir was calculated using a 12-week treatment course.

Table A F.1. Treatment regimens for sample of 15 index product/indication pairs

INN	Treatment Regimen			WHO DDD	Prescribed daily dose (PDD)
	Dose	Form	Frequency		
edoxaban	60mg	Orally	Once daily	60mg oral	60mg
asfotase alfa	2mg/kg If weight='20kg,' then 40mg per dose	Subcutaneously	3 times per week	None	40mg x 12 / 28 days = 17.14mg
alirocumab	75mg (Starting dose)	Subcutaneously	Once every 2 weeks	5.4mg parenteral, based on dosing every 2nd week	75mg / 14 days = 5.4mg
sacubitril/ valsartan	97mg/ 103mg	Orally	Twice daily	2 UD (tablet/capsule)	2 tablets
mepolizumab	100mg	Subcutaneously	Once every 4 weeks	3.6mg parenteral	100mg / 28 days = 3.6mg
sofosbuvir / velpatasvir	400mg/100mg	Orally	Once daily for 12 weeks	1 UD (tablet/capsule)	1 tablet
palbociclib	125mg	Orally	Once daily for 21 days, then 7 days off	94mg oral	125mg x 21 days / 28 days = 94mg
bariticinib	4mg	Orally	Once daily	4mg oral	4mg
nusinersen	12mg	Intrathecally	Once every 4 months (after loading doses)	0.1mg parenteral	12mg / 112 days = 0.1mg
tivozanib	1340microgram	Orally	Once daily for 21 days, followed by 7 days off	1mg oral	1340mcg x 21 days / 28 days = 1005 mcg = 1mg
dupilumab	300mg	Subcutaneously	Once every 2 weeks	21.4mg parenteral	300mg / 14 days = 21.4mg
niraparib	200mg	Orally	Once daily	None	200mg
ocrelizumab	600mg	IV infusion	Once every 6 months	3.29mg parenteral	600mg / 182.5 days = 3.29mg
semaglutide	0.5 to 1mg	Subcutaneously	Once weekly	0.11mg parenteral	0.5mg / 7 days = 0.07mg 1mg / 7 days = 0.14 0.75mg / 7 days = 0.11 mg
erenumab	70mg	Subcutaneously	Once every 4 weeks	2.5mg parenteral	70mg / 28 days = 2.5mg

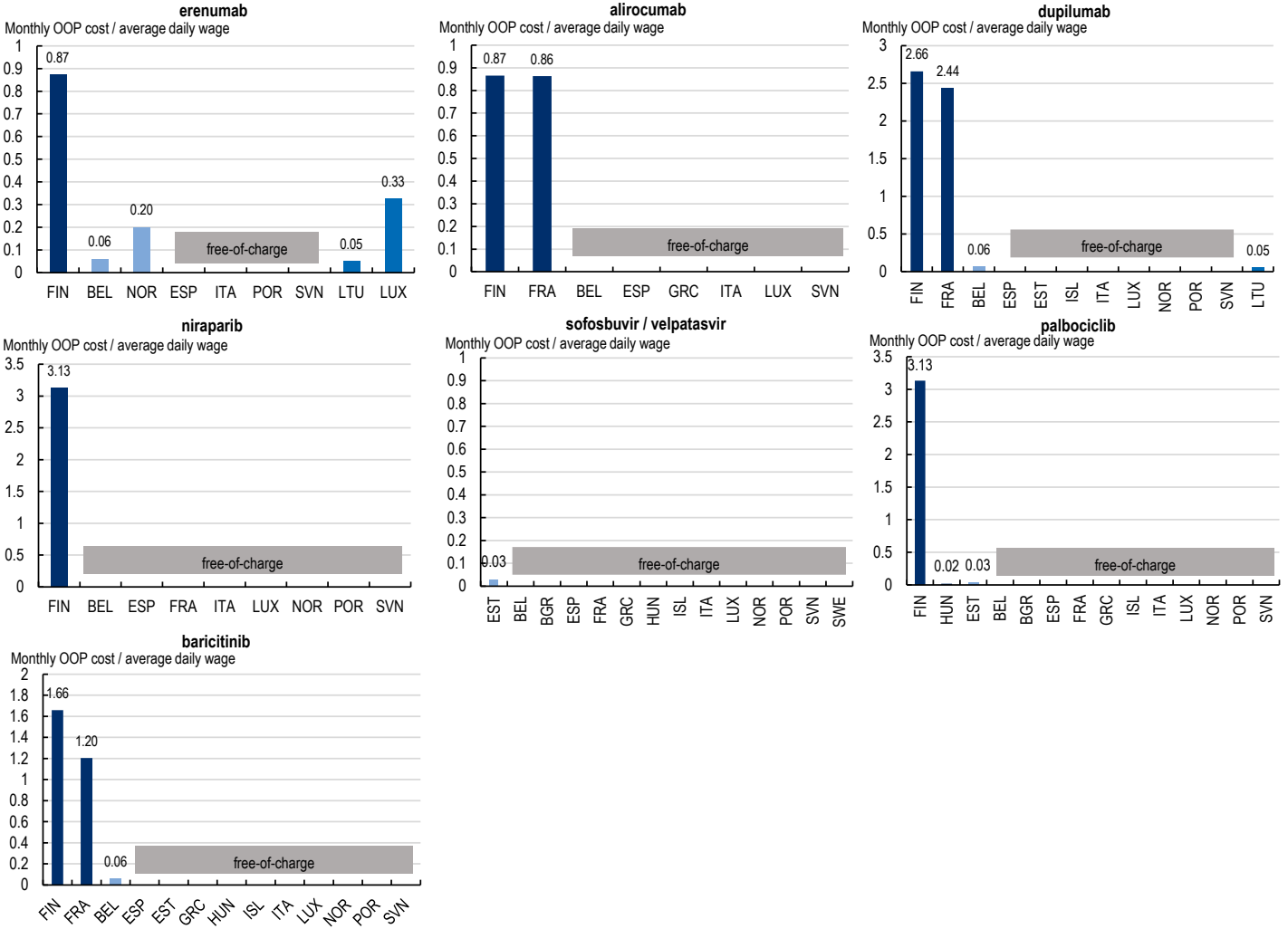
Note: INN international non-proprietary name; WHO DDD World Health Organization's Defined Daily Dose.
Source: Authors, based on review of 2021 WHO DDD, see https://www.whocc.no/ddd/definition_and_general_considera/ and the European Medicines Agency Summary of Product Characteristics dosing, see links in Table A D.1.

Figure A F.1. Estimated cost per year of treatment relative to GDP per capita, using ex-factory prices, at 01 October 2021



Note: Example of how to read the graph: an annual treatment with baricitinib would be an estimated 3.72 times the GDP per capita in Bulgaria. Based on *covered* products with available ex-factory price information, from a non-representative convenience sample of 15 product/indication pairs in 21 responding countries. Asfotase alfa and tivozanib excluded due to lack of data availability. Scales differ by medicine.
Source: (1) OECD survey on access to novel medicines 2021; (2) EURIPID database, 2021.

Figure A F.2. Estimated out-of-pocket costs for one month of treatment relative to average daily wage, at 01 October 2021



Note: OOP out-of-pocket. Columns are color-coded by type of cost-sharing: coinsurance, fixed co-payment, other (e.g. deductible, extra-billing).
Example of how to read the graph: purchasing one month of treatment with baricitinib would cost around 1.66 day's wages in Finland, whereby patient contributions are structured as co-insurance. Based on *covered* products with available out-of-pocket cost information, from a non-representative convenience sample of 15 product/indication pairs in 21 responding countries. Asfotase alfa and tivozanib excluded due to lack of data availability. Finnish estimates were adjusted based on the annual cap amount, where a patient would reach this cap with one month's treatment. Scales differ by medicine. Source: OECD survey on access to novel medicines 2021.

Annex G. Accessibility dimension

Accessibility estimates were made for individual medicines – at the level of the active substance. It was not relevant to aggregate utilisation across the heterogenous sample given country differences in disease prevalence and clinical practice. The possibility of accounting for disease prevalence for individual medicines was explored by reviewing the specific indications in which each sample pair was approved and comparing it to categories used in the Global Burden of Disease Study 2019[7]. However, notwithstanding the issues of considering uptake of alternative products, categories were deemed too broad to be able to estimate prevalence for each product/indication pair.

Measures of accessibility explored in this pilot study included

- Consumption in milligrams or DDD per 1000 population per day (see further explanation of methodology below)
- Approximate patient numbers over a 12-month period
- Time series consumption data since marketing authorisation or positive coverage decision, in milligrams.

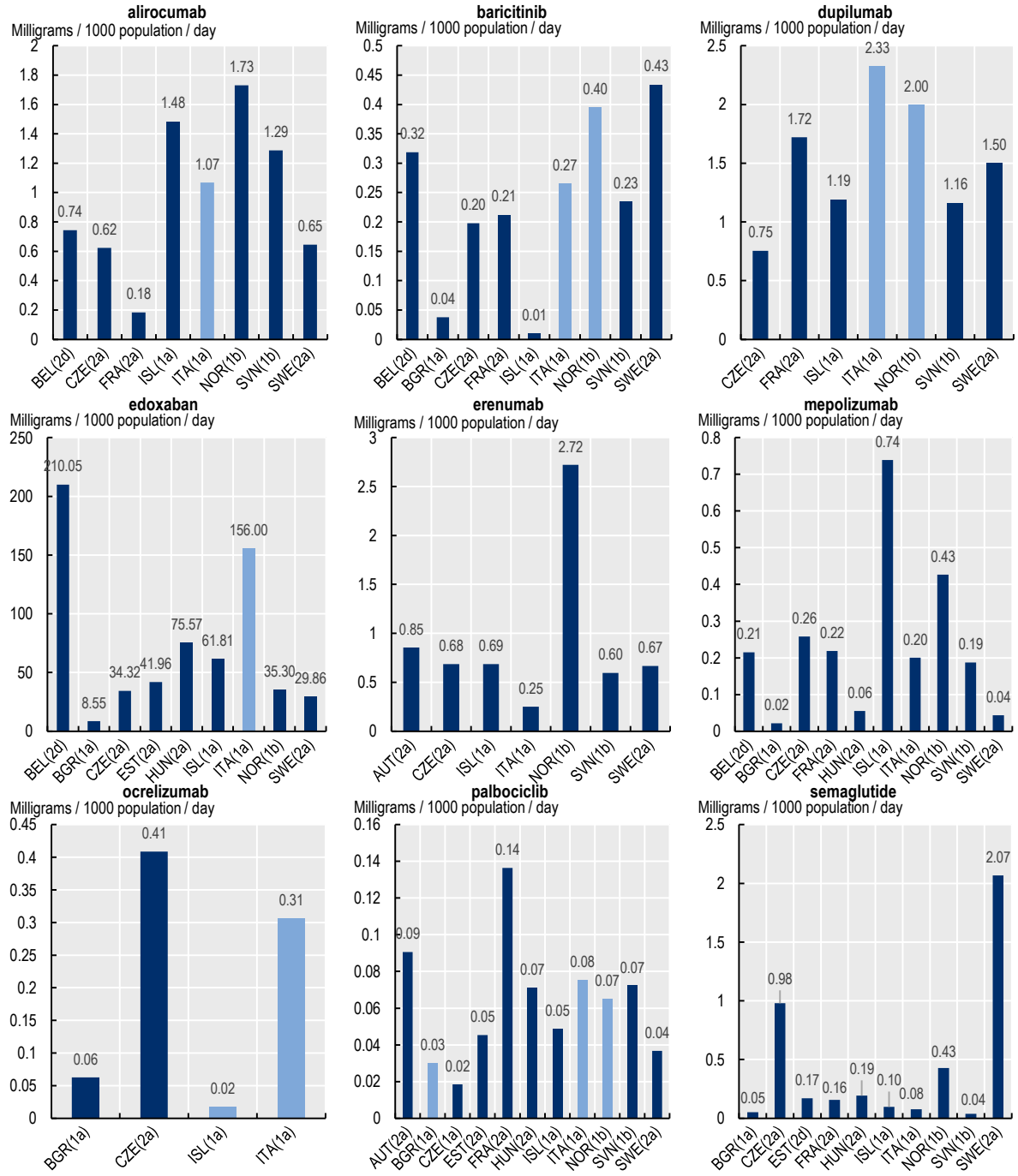
Consumption in milligrams or DDD per 1000 population per day

A combination of survey response and EURIPID data were used to calculate the average consumption of each sample medicine in each country (in milligrams and/or defined daily dose [DDD]) per 1000 population per day), estimated using the total consumption over the 12-month period prior to October 2021. Estimates included consumption across all product presentations, regardless of pharmaceutical strength / form / pack size. For those countries with available data, priority was given to consumption data extracted from EURIPID. EURIPID provides total monthly consumption data, so annual figures were calculated by summing the data from the twelve months prior to October 2021 (as specified in the survey). Milligrams were converted to DDDs by dividing the annual consumption in milligrams by the WHODDD; for those products without a WHODDD the approximate prescribed daily dose was used (see Table A F.1) Survey data alone were used to calculate the consumption in DDD for combination medicines sacubitril/valsartan and sofosbuvir/velpatasvir, as the WHODDD for those medicines only allows for a transformation from units (e.g. tablets, vials) to DDD, not milligrams to DDD. Survey data provided in total number of packages sold could not be used since units sold (i.e. packages) are only useful to calculate DDDs or milligrams if disaggregated data are available (i.e. number of packs sold per pharmaceutical strength/form/pack size). To overcome this issue, some countries provided additional survey data upon request, which consisted of the number of packages disaggregated by pharmaceutical strength/form/pack size, or total consumption in either milligrams or DDDs. These additional data were used where EURIPID data were unavailable. After collating data from the different sources, the consumption data were standardised (per 1000 population per day) to allow for cross-country comparisons. Population data from 2021 were extracted from Eurostat. Some countries could not provide data for the 12-month time period prior to 01 October 2021, as they collect data on an annual basis. Data sources and time periods are indicated in the relevant graphs.

[7] See https://ghdx.healthdata.org/gbd-2019, accessed September 2022.

Figure A G.1. Consumption, in milligrams, for the index product/indication pairs across countries

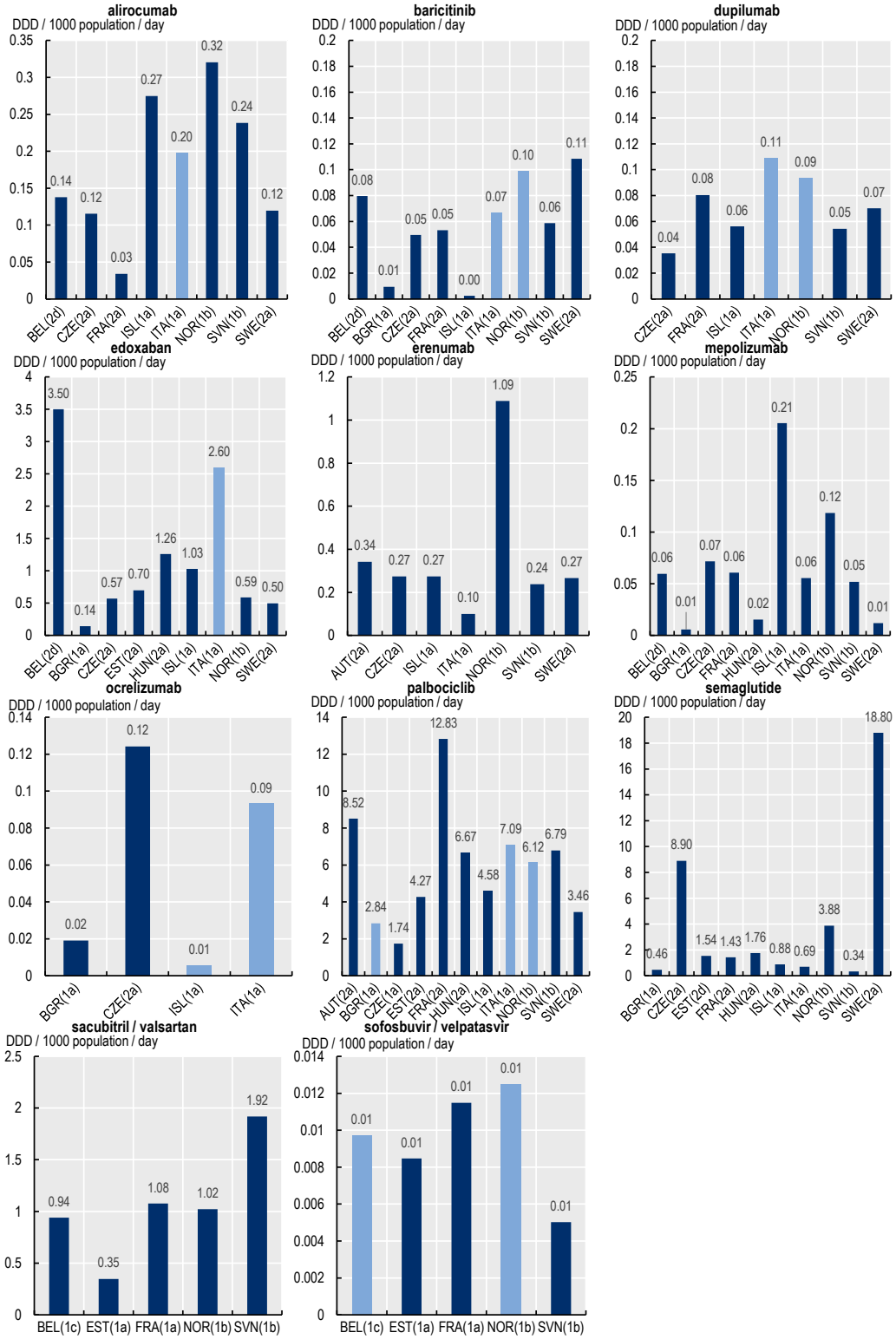
Consumption in milligrams per 1000 population per day



Note: most frequently administered in primary or ambulatory care; most frequently administered in the inpatient setting.
Asfotase alfa, nusinersen, niraparib, tivozanib, and two combination products were excluded due to data availability. Scales differ by medicine.
Estimated using total consumption over a yearly period: (a) 12 months prior to 01/10/2021; (b) 2021; (c) 30/07/2020 to 30/06/2021; (d) 2020.
Source: (1) OECD survey on access to novel medicines; (2) EURIPID database.

Figure A G.2. Consumption, in DDD, for the index product/indication pairs across countries

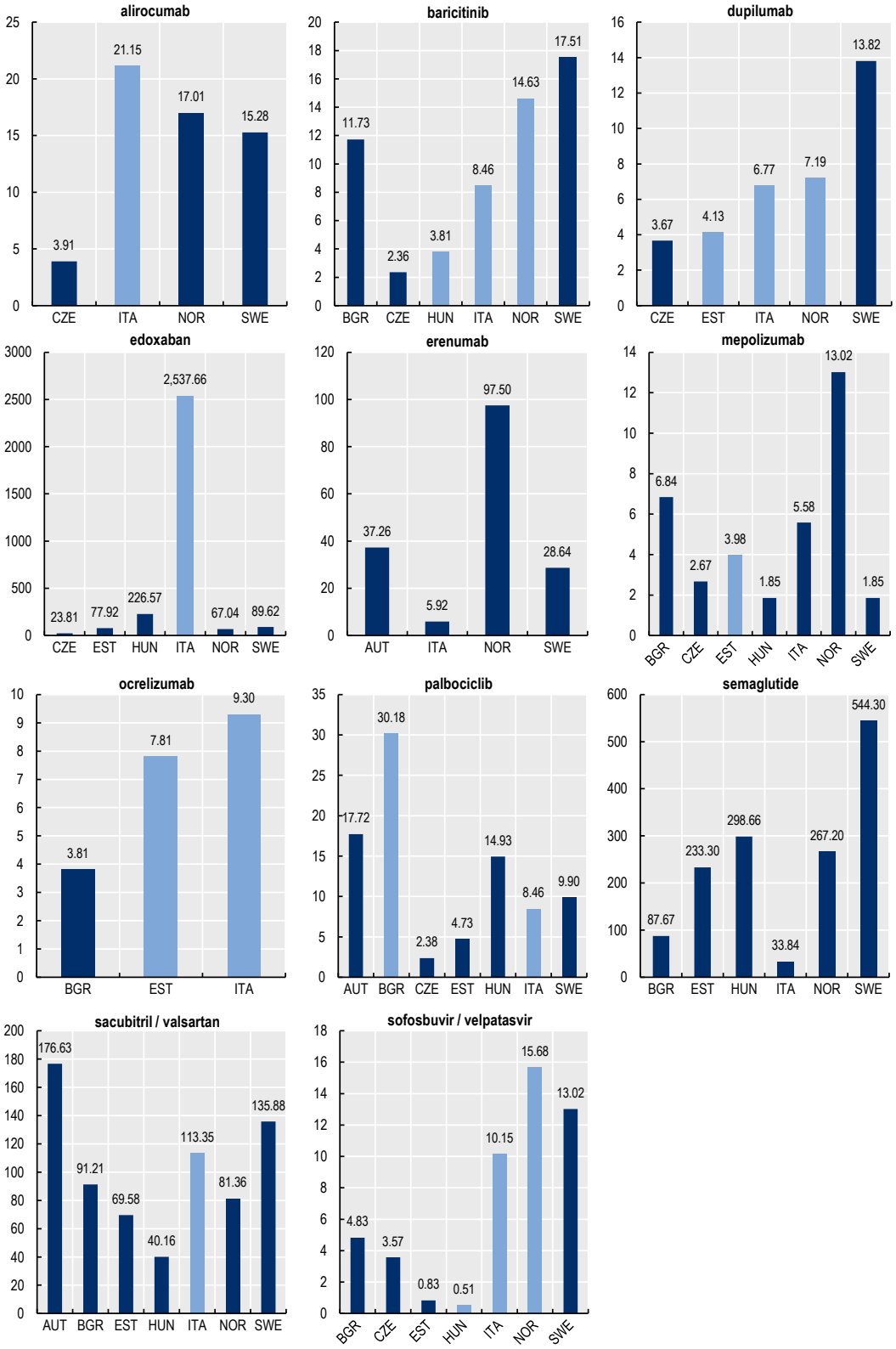
Consumption in milligrams per 1000 population per day



Note: most frequently administered in primary or ambulatory care; most frequently administered in the inpatient setting.
Asfotase alfa, nusinersen, niraparib and tivozanib were excluded due to data availability. Scales differ by medicine.
Estimated using total consumption over a yearly period: (a) 12 months prior to 01/10/2021; (b) 2021; (c) 30/07/2020 to 30/06/2021; (d) 2020.
Source: (1) OECD survey on access to novel medicines; (2) EURIPID database.

Figure A G.3. Approximate patient numbers for the index product/indication pairs across countries

Estimated number of patients per 100 000 population treated in the 12 months prior to 01 October 2021



Note: most frequently administered in primary or ambulatory care; most frequently administered in the inpatient setting.
Asfotase alfa, nusinersen, niraparib and tivozanib were excluded due to data availability. Scales differ by medicine. Swedish data refer to 2021. Norwegian data refer to 2020. Source: OECD survey on access to novel medicines 2021.

Table A G.1. Summary of utilisation data able to be provided by survey respondents

COU (N=20)	Total consumption, regardless of pharmaceutical form/dose/strength	Approximate number of patients treated
AUT[1]	√	√
BEL[2]	√	
BGR	√	√
CYP		
CZE	√	√
DEU	Not available	Not available
ESP[1]	√	√
EST	√	√
FIN	None provided	None provided
FRA	√	
GRC	Not provided – considered commercially sensitive	Considered commercially sensitive
HUN	√	√
ISL	√	Not accessible
ITA	√	√
LTU	No indication about sales given	No indication about sales given
LUX	Not provided	
NOR	√	√
POR	√	
SVN	√	
SWE	√	√

Note: √ = yes. Grey = covered and sold, but data not available. Not applicable to Malta as none of the sample pairs were covered.
1. Data may be available but not necessarily able to be publicly disclosed at the level of the individual active substance.
2. Belgium does not have complete data regarding approximate number of patients treated (they have data for public pharmacies but no hospital data).
Source: OECD survey on access to novel medicines 2021.

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