G.A.P.

GEARING UP ACCESS
PROPOSAL FOR V4

GAPV4 | EDITION 2025

GOALS & METHODS

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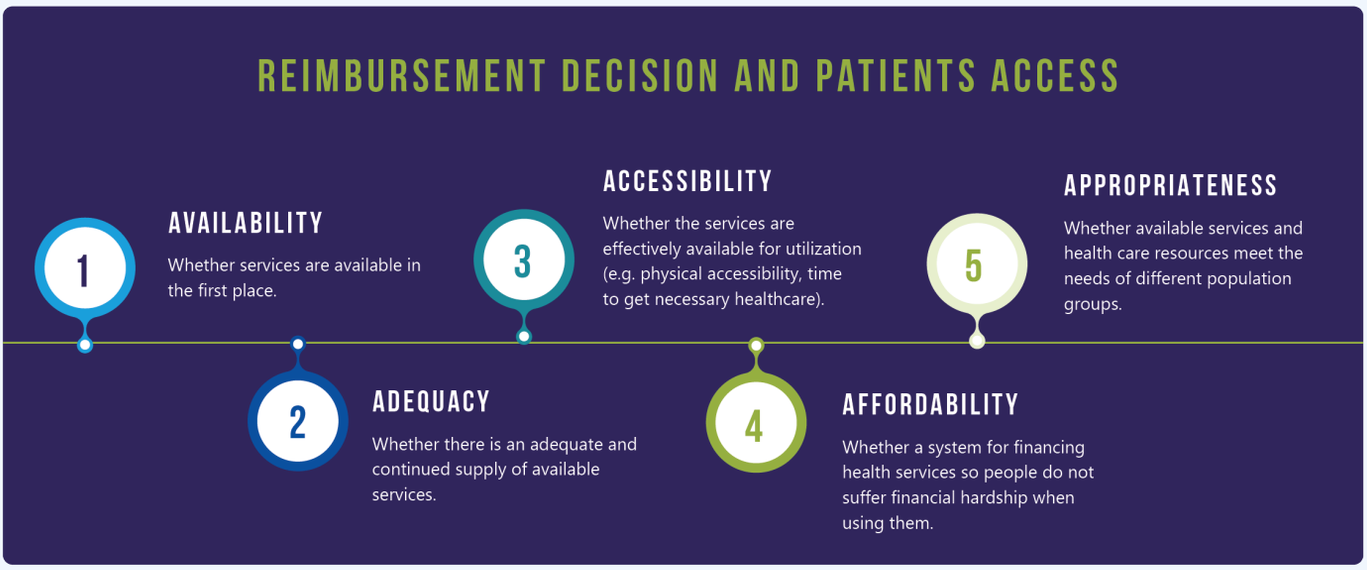
INTRODUCTION

Improving the availability of medicines authorised in the European Union (EU) is a key priority for the European medicines regulatory network and for the pharmaceutical industry according to the new Pharmaceutical Policy in the EU. Member State countries, in line with the health national policies, are gearing up for universal coverage in the healthcare sector, including equalisation of the access to medicines. These objectives need to take account the "lifecycle" of a pharmaceutical product and the different regulatory levers and policy interventions that take place over its course. In recent years, policymakers in European countries have been increasingly concerned about developments in the pharmaceutical sector that have been challenging the affordability of new medicines and the financial sustainability of solidarity in the publicly funded health care systems[1,2]. The '5 As' definition is widely used to capture the different aspects of patient access, including availability, adequacy, accessibility, affordability and appropriateness[4,5,6].



[1] *World Health Organization. Regional Office for Europe, Policies EO on HS and, Vogler S, Paris V, Panteli D. Ensuring access to medicines: how to redesign pricing, reimbursement and procurement?. 2018*

[2] *Godman B, Hill A, Simoens S, Selke G, Selke Krulichová I, Zampirolli Dias C, Martin AP, Oortwijn W, Timoney A, Gustafsson LL, Voncina L, Kwon H-Y, Gulbinovic J, Gotham D, Wale J, i in. (2021) Potential approaches for the pricing of cancer medicines across Europe to enhance the sustainability of healthcare systems and the implications. Expert Rev. Pharmacoecon. Outcomes Res. 21(4):527–540.*

[3] *Penchansky R, Thomas JW. The concept of access: definition and relationship to consumer satisfaction. Med Care. 1981;19(2):127-140.*

[4] *The model by Penchasky & Thomas was readapted by the multi-stakeholder Patient Access Partnership (PACT) network and the same approach was accepted by the European Patients Forum in 2016. 5As are complemented by „Timeliness" in the Towards a fairer and more effective measurement of access to healthcare across the EU report.*

[5] *Garcia MM, Barbosa MM, Silva RM, Reis EA, Alvares J, Assis Acurcio F de, Godman B, Guerra Junior AA. (2019) Indicator of access to medicines in relation to the multiple dimensions of access. J. Comp. Eff. Res. 8(12):1027–1041.*

[6] *Barbosa MM, Moreira TA, Nascimento RC, Nascimento MM, Acurcio FA, Godman B, Guerra AA, Alvares-Teodoro J. (2021) Access to medicines in the Brazilian Unified Health System's primary health care: assessment of a public policy. J. Comp. Eff. Res. 10(10):869–879.*

Access to new therapies and diagnostics varies significantly between countries in the EU. This has been seen with the utilisation of new biological medicines to treat immune diseases, including rheumatoid arthritis and inflammatory bowel diseases[7,8,9]. In addition, for medicines to treat rare diseases and cancer[10]. The importance of attaining affordable access to pharmaceutical products in the global efforts towards universal health coverage has long been recognized. The increasing wave of scientific innovation approved by EMA over the last 10 years could generate substantial health benefit on a population level. A new Pharmaceutical Strategy, adopted in October 2021 by the EU Commission, promises patient access to affordable medicines and aims to support competitiveness and innovation in Europe's pharmaceutical industry. To ensure access to affordable medicines for patients, it also addresses unmet medical needs (e.g. oncology, rare diseases) and promotes a high level of quality, efficacy and safety standards.

In Europe, multiple organizations have developed and/or published healthcare indicators, ranging from large-scale, regularly updated databases and reports[11] to ad hoc, disease-specific surveys and reviews commissioned by authorities, patient associations or commercial projects. **In this project, a unique multi-level indicator framework for measurement of patient access to therapies and diagnostics was developed by a group of experts. Its aim is to create a benchmark for the V4 region from the angle of clinical standards with the same set of 8 indicators capturing different aspects of patient access, including availability, affordability, and accessibility of pharmaceutical products, as well as diagnostics. All indicators were adapted to specific therapeutic areas in terms of relevant medicines and diagnostic tests.** At the highest level of analysis, to give an overall assessment, a single cumulative indicator was calculated for each country, based on the disease-level indicators. **It allows us to draw direct comparisons between the healthcare systems in V4 in the investigated areas.**

Better access to the diagnostic procedures and therapies is one the key elements within the entire healthcare service system, which is necessary to improve survival and quality of life (as measured in DALY) in a given population. All disease areas selected for the purposes of the current project are important from the public health perspective.

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[7] *Putrik P, Ramiro S, Kvien TK, Sokka T, Pavlova M, Uhlig T, Boonen A, Working Group 'Equity in access to treatment of rheumatoid arthritis in Europe'. (2014) Inequities in access to biologic and synthetic DMARDs across 46 European countries. Ann. Rheum. Dis. 73(1):198–206.*

[8] *Baumgart DC, Misery L, Naeyaert S, Taylor PC. (2019) Biological Therapies in Immune-Mediated Inflammatory Diseases: Can Biosimilars Reduce Access Inequities? Front. Pharmacol. 10:.*

[9] *Kostić M, Djakovic L, Šujić R, Godman B, Janković SM. (2017) Inflammatory Bowel Diseases (Crohn´s Disease and Ulcerative Colitis): Cost of Treatment in Serbia and the Implications. Appl. Health Econ. Health Policy. 15(1):85–93.*

[10] *EURORDIS. (2018) Breaking the Access Deadlock to Leave No One Behind. Dostęp: http://download2.eurordis.org.s3.amazonaws.com/positionpapers/eurordis_access_position_paper_final_4122017.pdf.*

[11] *e.g., Euro Health Consumer Index by Health Consumer Powerhouse; European Core Health Indicators and Eurostat data by EU; OECD Health data and reports; WHO Health reports and Core Health Indicators in the WHO Europe Region; measurements by national authorities etc.*

#	KPI		5 "A"
1	RESTRICTIONS ON REIMBURSEMENT	THERAPY	Accessibility **Availability**
2	TIME TO AVAILABILITY		Accessibility
3	COMPLIANCE WITH INTERNATIONAL GUIDELINES		**Availability** Adequacy Accessibility
4	EARLY ACCESS PROGRAMS		Accessibility
5	SHARE OF PATIENTS ON A SPECIFIC NOVEL TREATMENT		Accessibility Appropriateness
6	NOVEL TREATMENT DEPLOYMENT		**Availability** Adequacy Appropriateness
7	REIMBURSEMENT OF MOLECULAR TESTS	DIAGNOSTICS	Adequacy Accessibility
8	ACCESS TO ADVANCED DIAGNOSTICS		Adequacy Accessibility

The aim of this analysis is to facilitate a better understanding of the deeply complex problem of patient access to medicines. Our results may be hopefully supporting for decision makers and other stakeholders in preparing for future innovations in healthcare among the V4 countries and how these may become more accessible. The disclosed differences may become motivators to promote equal access to health care across the V4 countries as well as to show key stakeholders potential actions for improvement where there are concerns.

IT IS TIME TO ACT.

GOALS & METHODOLOGY OF THE PROJECT

Ensuring patient access is the key determinant to deliver value of innovative treatments to further improve the health of the population health. Universal coverage and access to pharmaceutical innovations have appreciably improved patient outcomes across the different indications. However, this can only be achieved by full access to advances on health care.

Our goal is to support key decision makers to identify the gaps in patient access to innovative therapies throughout the Visegrád Group countries.

To present different dimensions of patient access, we selected a total of 12 indications (therapy areas) divided in 3 categories: oncology, chronic diseases and rare diseases. These categories may serve as representative examples for describing and evaluating patients' access to innovative therapies in the Visegrád Group countries. Evaluated therapy areas were those for which the highest number of new innovative treatments had been approved in Europe between 2010 and 2023; consequently, it was possible to assess the extent to which they have become available to patients in actual practice.

All therapeutic areas are evaluated using the same set of 8 indicators which capture different aspects of patient access, including availability, affordability, and accessibility of innovative pharmaceutical products, treatments recommended by clinical guidelines as well as diagnostics; furthermore, real-life utilization of selected therapies was also considered. All indicators were adapted to the specific therapeutic area in terms of relevant pharmaceutical products and diagnostic tests.

Patient access indicators are descriptive measurements that evaluate the level of patient access by defining a set of benchmarks comparing several aspects of treatment and diagnostic process. After conducting a review of existing and publicly available patient access indicators in international measurements, we developed a multi-level indicator framework in which indicators are aggregated for each therapeutic area and for each country. As a result, making it possible to compare diseases and countries in a flexible way.

Data on which indicators are based on those published by national authorities in each country, including national reimbursement databases, reimbursement protocols, and prescription databases. Furthermore, we used international guidelines published by professional organizations as a benchmark. In cases when certain necessary data points were not publicly available for all countries, we used estimates based on the available data.

#	INDICATOR (KPI) NAME	DESCRIPTION AND RATIONALE
1	RESTRICTIONS ON REIMBURSEMENT	refers to the availability of reimbursed treatment options at the country level by calculating the proportion of innovative medicines approved by EMA that are available under the regular or irregular reimbursement. Partial reimbursement (restrictions compared to EMA label) is given less weight than a full indication reimbursement.
2	TIME TO AVAILABILITY	refers to the length of time (measured as number of days) between the EMA marketing approval and the date of availability under regular reimbursement for patients.
3	COMPLIANCE WITH INTERNATIONAL GUIDELINES	refers to the proportion of therapies reimbursed in a given country. The main points of relevant international guidelines of professional organizations (e.g., ESMO guidelines) were identified for each indication.
4	EARLY ACCESS PROGRAMS	refers to the availability of early access programs (excluding compassionate use) before regular reimbursement. Early access programs are a pathway to provide innovative treatments through individual application in some countries .
5	SHARE OF PATIENTS ON A SPECIFIC NOVEL TREATMENT	refers to the utilization of specific therapies. The indicator refers to the share of patients on a specific innovative treatment (or a category of innovative drugs) in 12 months, compared to the total number of eligible patients. The number of eligible patients is calculated using the same population data e.g. epidemiological data were identified in each indication.
6	NOVEL TREATMENT DEPLOYMENT	refers to the utilization of a specific ATC code category (in absolute value) per total population per 12 months.
7	REIMBURSEMENT OF MOLECULAR TESTS	refers to the public reimbursement of molecular or other tests required when the qualification to the therapy is conditioned by the presence of a specific biomarker. Relevant tests were selected for each indication based on guidelines.
8	ACCESS TO ADVANCED DIAGNOSTICS	refers to the typical waiting time for elective diagnostics following the referral, which may affect the time to treatment initiation or further diagnostics. Relevant diagnostic tests were specified for each indication based on guidelines.

As part of the V4 GAP project in the 2025 edition, we conducted an update for selected disease areas. We wanted to be able to continue to measure the limitations in access to new treatments by referring to such therapies that set the standard for modern treatment ***today.***

To this purpose, we decided to replace the medicines whose availability is assessed under 2 KPI's: "Share of patients on a specific novel treatment" and "Novel treatment deployment" for the selected diseases with products that today set the quality standard we want to strive for.

The list of interventions we refer to under these indicators is summarised in the table below.

	2022-2024	2025
BREAST CANCER	cyclin-dependent kinase inhibitor (abemacyclib, ribocyclib, palbocyclib)	**Novel conjugates (trastuzumab deruxtecan, sacituzumab govitecan)**
NON-SMALL-CELL LUNG CANCER	PD-1/PDL-1 inhibitors	PD-1/PDL-1 inhibitors
OVARIAN CANCER	PARP inhibitors	PARP inhibitors
PROSTATE CANCER	new generatoin hormone therapy	new generatoin hormone therapy
ACUTE MYELOID LEUKAEMIA	innovative treatment (kinase inhibitors, BCL-2 Inhibitor, next generation FMS-like tyrosine kinase 3 (FLT3) inhibitors)	innovative treatment (kinase inhibitors, BCL-2 Inhibitor, next generation FMS-like tyrosine kinase 3 (FLT3) inhibitors, **(IDH1) inhibitor)**
LYMPHOMA	Selected treatments (brentuximab vedotin, nivolumab, ibrutinib	**CAR-T therapy**
SPINAL MUSCULAR ATROPHY	Gene therapy	Gene therapy
CYSTIC FIBROSIS	Triple therapy	Triple therapy
DIABETES MELLITUS*	All treatments authorised since 2010	**GLP-1 agonists in injection**
MULTIPLE SCLEROSIS	sphingosine 1-phosphate receptor modulators	sphingosine 1-phosphate receptor modulators
PARKINSON'S DISEASE**	All treatments authorised since 2010	All treatments authorised since 2010
ASTHMA**	Biologic drugs	Biologic drugs

** In 2021 Long-acting insulin analogues.*

*** From 2023 edition.*

Regardless of the change in selected drugs, we have also decided to include the US NCCN guidelines for haemato-oncological malignancies (AML and lymphomas). The European ESMO guidelines, which had been the benchmark for therapeutic recommendations for cancer patients since the start of the GAP V4 project, have not been updated for haematological malignancies since 2020 (and even earlier for some indications). In the context of the enormous dynamism of this area, this must result in their limited relevance. In order to keep pace with the new standards, we decided to change the original focus to European recommendations and to reach for guidelines that recognise these new therapies.

In addition, we decided to group the preparations used in the treatment of diabetes into subsets designated by their active substances (used alone or in combination with classic anti-diabetic drugs, such as metformin).

DISEASE BURDEN IN VISEGRÁD GROUP COUNTRIES

All selected diseases are important from a public health perspective and their population burden can be assessed by evaluating the quality of life with increasing disability or loss of life years of the burdened population.

DALY (*Disability Adjusted Life-Years*) is a well-recognised tool, **that allows international comparison between countries to determine the health status of the population[12,13,14]. One DALY represents the loss of the equivalent of one year of full health.** DALYs is the sum of the years of life lost due to premature mortality (YLLs) and the years of years of healthy life lost due to disability (YLDs) for people living in states of less than good health resulting from the specific disease.

INDICATIONS SELECTED FOR ACCESS GAP ASSESSMENT

THE DISEASE WAS SELECTED BASED ON THE 10 YEARS ANALYSIS OF EMA AUTHORIZATION.

ONCOLOGY	ORPHAN	CHRONIC DISEASES
NSCLC	CYSTIC FIBROSIS	DIABETES MELLITUS
BREAST CANCER	ACUTE MYELOID LEUKEMIA (AML)	MULTIPLE SCLEROSIS (MS)
PROSTATE CANCER	LYMPHOMA	ASTHMA
OVARIAN CANCER	SPINAL MUSCULAR ATROPHY (SMA)	PARKINSON'S DISEASE

[12] *Global Burden of Disease Cancer Collaboration, Fitzmaurice C, Akinyemiju TF, Al Lami FH, Alam T, Alizadeh-Navaei R, Allen C, Alsharif U, Alvis-Guzman N, Amini E, Anderson BO, Aremu O, Artaman A, Asgedom SW, Assadi R, i in. (2018) Global, Regional, and National Cancer Incidence, Mortality, Years of Life Lost, Years Lived With Disability, and Disability-Adjusted Life-Years for 29 Cancer Groups, 1990 to 2016: A Systematic Analysis for the Global Burden of Disease Study. JAMA Oncol. 4(11):1553–1568.*

[13] *Masaebi F, Salehi M, Kazemi M, Vahabi N, Azizmohammad Looha M, Zayeri F. (2021) Trend analysis of disability adjusted life years due to cardiovascular diseases: results from the global burden of disease study 2019. BMC Public Health 21(1):1268.*

[14] *Feigin VL, Nichols E, Alam T, Bannick MS, Beghi E, Blake N, Culpepper WJ, Dorsey ER, Elbaz A, Ellenbogen RG, Fisher JL, Fitzmaurice C, Giussani G, Glennie L, James SL, i in. (2019) Global, regional, and national burden of neurological disorders, 1990–2016: a systematic analysis for the Global Burden of Disease Study 2016. Lancet Neurol. 18(5):459–480.*