

Glossary of Pre-approval Access Terminology

Agencia Espanola de Medicamentos y Productos Sanitarios (AEMPS): The AEMPS is a state agency in Spain attached to the Ministry of Health. It is responsible for guaranteeing to society, from a public service perspective, the quality, safety, efficacy and correct information of medicines and health products, from their research to their use, in the interests of protecting and promoting human health, animal health and the environment. For more information: <https://www.aemps.gob.es/la-aemps/quienes-somos/?lang=en>

ANSM: The National Agency for the Safety of Medicines and Health Products is the public actor which, on behalf of the State, allows access to health products in France and which ensures their safety throughout their life cycle. For more information: [https://www.ansm.sante.fr/L-ANSM/Une-agence-d-expertise/L-ANSM-agence-d-evaluation-d-expertise-et-de-decision/\(offset\)/0](https://www.ansm.sante.fr/L-ANSM/Une-agence-d-expertise/L-ANSM-agence-d-evaluation-d-expertise-et-de-decision/(offset)/0)

BASG: BASG is the national authority for drugs, medical devices, blood and tissue in Austria. The BASG monitors - nationally and in concert with the European sister agencies - the drugs and medical devices that are already on the market with regard to their effectiveness, possible side effects, their production, transport and storage. For more information: <https://www.basg.gv.at/ueber-uns>

Bulgarian Drugs Agency: The Bulgarian Drug Agency at the Ministry of Healthcare is defined as a body for the supervision of the quality, efficiency and safety of medicines. For more information: <https://www.bda.bg/en/about-bda/history#from-nimp-to-bda>

Clinical Trial(s): Interventional study (clinical trial) is a study in which participants are assigned to groups that receive one or more intervention/treatment (or no intervention) so that researchers can evaluate the effects of the interventions on biomedical or health-related outcomes. The assignments are determined by the study's protocol. Participants may receive diagnostic, therapeutic, or other types of interventions. for more information: <https://www.clinicaltrials.gov/ct2/about-studies/glossary>

ClinicalTrials.gov: ClinicalTrials.gov is an online database of publicly and privately supported clinical trials conducted around the world. Doctors and patients may consult this database when seeking access to an experimental drug, via either a clinical trial or pre-approval access. While ClinicalTrials.gov is operated by the United States National Institutes of Health (NIH), being listed in the database should not be seen as an endorsement by the NIH or the U.S. Food and Drug Administration (FDA) of the value of any agent or product.

Commercially Available: A product that has received local health authority approval and the product is able to be prescribed.

Compassionate Use: “Compassionate use” is access to experimental drugs that have not yet been approved for sale or use by the FDA or relevant regulatory authority, such as Health Canada. The term is often used interchangeably with “expanded access” and “pre-approval access.” Often experimental drugs may be available to patients through clinical trials. Compassionate use is the provision of the drug to patients who are unable to participate in a clinical trial because of severity of illness or some other factor. Sponsors decide whether to make their experimental drugs available to patients via compassionate use. Should a sponsor, often a private company, be willing to provide the experimental drug, the FDA must approve the planned use of that drug. FDA regulations specify two groups of people eligible for compassionate use: 1) those with life-threatening diseases or conditions for which “there is a reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment” and 2) those with serious diseases or conditions that have a “substantial impact on day-to-day functioning” (21 Code of Federal Regulations 312.300(b)). In most cases, patients who seek compassionate use must have exhausted all approved therapies for their condition and be unable to enroll in a clinical trial.

Danish Medicines Agency (DMA): The Danish Medicines Agency authorises and inspects pharmaceutical companies and licenses medicinal products in the Danish market, monitors adverse reactions from medicinal products, authorises clinical trials, decides which medicines are eligible for reimbursement, monitors medical devices available in Denmark and supervises adverse incidents involving medical devices, appoints proprietary pharmacists, organises the pharmacy structure and supervises pharmacies and retailers. They perform most of their tasks in close cooperation with colleagues from regulatory authorities and organisations in the other EU countries. For more information: <https://laegemiddelstyrelsen.dk/en/about/>

Early Access to Medicines Scheme (EAMS): The Early Access to Medicines Scheme (EAMS) in the United Kingdom aims to make promising new medicines available to patients sooner. It was set up in 2014 and is run by the Medicines and Healthcare Products Regulatory Agency (MHRA). For more information: <https://www.cancerresearchuk.org/about-cancer/cancer-in-general/treatment/access-to-treatment/early-access-to-medicines-scheme>

Eligibility Criteria: Eligibility criteria are the key requirements for entry into a clinical study or other investigational medicine program such as expanded access. For more information: <https://www.clinicaltrials.gov/ct2/about-studies/glossary>

Ethics Committee: Ethics committees are a group of individuals formed to protect the interests of patients and address moral issues. It normally includes a board member of the institution, a lay person, and an administrator. Most ethics committees work in an advisory capacity; they can help patients and families reach informed decisions and work with health care providers in order to make complex and difficult decisions.

Expanded Access: “Expanded access” (EA) is the FDA’s term for access outside clinical trials to experimental drugs that the agency has not yet approved. Expanded access is an umbrella term that applies to single patient requests and programs for groups, either intermediate-size or larger. Treatment of the patient, rather than collection of data, is the primary goal. This is also known as a “Group” or “Cohort” program.

Expanded Access Programs (EAPs): EAPs are designed to permit larger groups of patients to access an experimental drug. For both single patient requests and EAPs, treatment of the patient, rather than collection of data, is the primary goal. However, in an EAP, data are frequently collected from patients enrolled in the program.

Federal Agency for Medicines and Health Products (FAMHP): The FAMHP is the competent authority responsible for the quality, safety and efficacy of medicines and health products in Belgium. For more information: <https://www.famhp.be/en/famhp>

FIMEA: Operating under the Ministry of Social Affairs and Health, the Finnish Medicines Agency Fimea oversees and develops the pharmaceutical sector. For more information: https://www.fimea.fi/web/en/for_public/fimea-s-services-to-citizens

Form FDA 3926: Form 3926 is an FDA form for use by physicians when submitting requests for expanded access to investigational drugs, including emergency requests. This form is designed specifically for single patient requests, not for EAPs. In 2016, this form was created as a shorter, streamlined alternative to Form FDA 1571, which still must be used for EAPs.

Health Authority: A Health Authority is a government agency that is responsible for National Health Services care in a particular area.

Health Canada: Health Canada is a federal institution that is part of the Health portfolio that is responsible for helping Canadians maintain and improve their health. It ensures that high-quality health services are accessible, and works to reduce health risks.

Individual Request: Named patient requests based on an application made by the physician and/or local importer e.g. pharmacy or wholesaler.

Infarmed: National Authority for Medicines and Health Products, IP, abbreviated as Infarmed, is a public institute with a special regime, under the terms of the law, integrated in the indirect administration of the State, endowed with administrative, financial and own assets. Infarmed continues the duties of the Ministry of Health, under the supervision and supervision of the respective minister. For more information: <https://www.infarmed.pt/web/infarmed/apresentacao>

Institutional Review Board (IRB): An IRB, sometimes referred to as a research ethics committee, is a committee charged with reviewing, approving, and monitoring biomedical and behavioral research involving humans. In the U.S., pre-approval access requires review and approval by an IRB. IRB review comes after the drug company agrees to provide access to an investigational product and the FDA reviews and accepts the proposed treatment plan.

Investigational New Drug (IND): Federal law requires that a drug or other therapeutic agent be approved for use before it can be transported and distributed in the U.S. An IND exemption is the means through which a sponsor obtains permission from the FDA to distribute the agent before it has this approval. Under an IND, sponsors may distribute an experimental drug to study it in clinical trials needed for approval. An IND must also be submitted to receive access to an experimental drug for compassionate use. There are four types of expanded access INDs: 1) individual patient expanded access, which allows compassionate use of a drug by a single patient; 2) Emergency Use IND, which allows the FDA to authorize the use of an experimental drug in an emergency situation, as it did during the 2014 Ebola outbreak; 3) Intermediate-Size Patient Population IND, which allows multiple patients to gain compassionate use access to an experimental drug, and 4) Treatment IND, which is submitted for the widespread use of experimental drugs showing promise in clinical testing for serious or immediately life-threatening conditions."

JAZMP: JAZMP of Slovenia's primary mission is to protect public health through the regulation and supervision of medicinal products, medical devices, blood, tissues and cells and associated activities in the private and public sector. For more information: <https://www.jazmp.si/en/>

Malta Medicines Authority: Malta's Medicines Authority's vision is to be a centre of excellence in advancing effective and innovative regulation and promoting quality and scientific rigour in the work we do. For more information: <http://www.medicinesauthority.gov.mt/missionobjectives?l=1>

Medical Product Agency (MPA): The Medical Products Agency of Sweden is the responsible for regulation and surveillance of the development, manufacturing and sale of drugs and other medicinal products. For more information: <https://www.government.se/government-agencies/medical-products-agency-lakemedelsverket-lv/>

MHRA: The Medicines and Healthcare products Regulatory Agency regulates medicines, medical devices and blood components for transfusion in the UK.

Ministere-Direction de la Sante: The missions of the Ministere-Direction de la Sante, the Ministry of Health of Luxembourg, are the definition and application of government health policy, monitoring the application of health laws and regulations, supervision of health institutions and services. For more information: <https://sante.public.lu/fr/politique-sante/ministere-sante/index.html>

Ministry of Health: The Ministry of Health of Poland is a government administration office that supports the minister of health. For more information: <https://www.gov.pl/web/zdrowie/podstawowe-informacje>

Named Patient Program: A program to provide access on a named patient (individual) or group/cohort basis.

National Agency for Medicines and Medical Devices of Romania (NAMMD): The National Agency for Medicines and Medical Devices (NAMMD) is a public institution subordinated to the Ministry of Health of Romania. The NAMMD mission is to help protect and promote public health. For more information: <https://www.anm.ro/en/despre-institutie/despre-noi/>

OGYÉI: OGYÉI is responsible for the tasks performed by the General Directorate of the GYEMSZI Institute of Pharmacy, the Directorate of Device Qualification and Hospital Technology, the Technology Evaluation Department, the GYEMSZI, the ÁNTSZ and the National Food and Nutrition Institute for Food and Nutrition. For more information: <https://ogyei.gov.hu/magunkrol>

Phase 2 Clinical Trial(s): Phase 2 Clinical Trial is a phase of research to describe clinical trials that gather preliminary data on whether a drug works in people who have a certain condition/disease (that is, the drug's effectiveness). For example, participants receiving the drug may be compared to similar participants receiving a different treatment, usually an inactive substance (called a placebo) or a different drug. Safety continues to be evaluated, and short-term adverse events are studied. For more information: <https://www.clinicaltrials.gov/ct2/about-studies/glossary>

Phase 3 Clinical Trial(s): Phase 3 Clinical Trial is a phase of research to describe clinical trials that gather more information about a drug's safety and effectiveness by studying different populations and different dosages and by using the drug in combination with other drugs. These studies typically involve more participants. For more information: <https://www.clinicaltrials.gov/ct2/about-studies/glossary>

Pre-Approval Access (PAA): PAA is an umbrella term encompassing access to investigational medicines, such as expanded access programs and compassionate use. PAA refers to any use of unapproved drugs outside of clinical trials, particularly if the intent is therapeutic rather than to gain data (research).

Single Patient Request (SPR): Investigational access request outside of a clinical trial for one patient outside of an expanded access program.

Sponsor: A sponsor is the person or entity that takes responsibility for and initiates a clinical trial of an investigational agent. In the context of pre-approval access, the sponsor is typically a pharmaceutical or biotech company.

State Agency of Medicines (SAM) Estonia: State Agency of Medicines is a governmental body under the Ministry of Social Affairs in Estonia. Its main responsibility is the protection and promotion of public and animal health, through the supervision of medicines for human and veterinary use. For more information: <https://www.ravimiamet.ee/en/sam>

State Agency of Medicines (SAM) Latvia: The State Agency of Medicines of Latvia (hereafter SAMLV) is a State institution under the supervision of the Ministry of Health of the Republic of Latvia. The operational objective of SAMLV is to implement local and international pharmaceutical legislation in order to ensure that the products (medicines, medical devices, blood, cells, tissues and organs) used in health care, as well as the involved companies and their activities comply with certain requirements. For more information: <https://www.zva.gov.lv/en/about-us/about-agency>

State Medicines Control Agency of Lithuania: The State Medicines Control Agency (SMCA) is a governmental body of the Republic of Lithuania with headquarters in Vilnius. Its main responsibility is the protection of public health, through the evaluation and supervision of medicines for human use. For more information: <https://www.vvkt.lt/index.php?2380224066>

Treating Physician: Treating Physician is a physician who is providing medical treatment for a condition specific to expanded access requests.

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