

Compassionate use program

The EU regulatory framework makes it possible for non-authorized medicines to be made available under certain circumstances. This is achieved through a compassionate use program.

Relevant regulation

According to article 83 of Regulation (EC) No 726/2004, medicinal products without a Marketing Authorisation *may be made available for compassionate reasons to a group of patients with a chronically or seriously debilitating disease or whose disease is considered to be life-threatening, and who can not be treated satisfactorily by an authorized medicinal product.*

National jurisdiction

Compassionate use programs falls under national jurisdiction and, in most Member States under the remit of National Competent Authorities (NCA). Article 83 of [Regulation](#) (EC) No 726/2004, states that the Committee for Medicinal Products for Human Use (CHMP) has an advisory role at the request of a Member State. The individual NCA decide whether or not to approve the use of medicinal products without a market authorization.

The NCA in the Member State decides if such a program fulfils an unmet medical need according to their clinical practices and available alternatives. Some Member States have a long tradition on early access programs, including compassionate use, and others have different provisions in their national legislation.

Most of the compassionate use program notifications are submitted directly to the NCA within the different Member States.

NCA's that publish guidance on their compassionate use programs within their Member States:

- Austria - Austrian Medicines and Medical Devices Agency
<http://www.basg.gv.at/en/medicines/prior-to-authorisation/compassionate-use/>
- Belgium- Federal Agency for Medicines and Healthcare Products (FAMHP)
http://www.fagg-afmps.be/nl/MENSELIJK_gebruik/geneesmiddelen/geneesmiddelen/onderzoek/ontwikkeling/gebruik_in_schrijnende_gevallen_medische_noodprogrammas/

- Czech Republic – State Institute for Drug Control (SUKL)
<http://www.olecich.cz/encyklopedie/dostupnost-leciv-v-cr>
Information about actual use of non-authorised medicinal products is published regularly at <http://www.sukl.cz/hodnoceni-neregistrovanych-lp>
- Denmark -Danish Medicines Agency
<http://sundhedsstyrelsen.dk/da/medicin/regulering/udleveringstilladelser>
- Estonia – Ravimiamet agency
<http://www.ravimiamet.ee/en/compassionate-use-programs-under-estonian-legislation>
- Germany - Paul-Ehrlich-Institut (PEI)
<http://www.pei.de/EN/information/license-applicants/clinical-trial-authorisation/compassionate-use/compassionate-use-node.html>
- Ireland – Health Products Regulatory Authority (HPRA)
<https://www.hpra.ie/homepage/medicines/regulatory-information/medicines-authorisation/access-to-medicines-prior-to-authorisation>
- Italy – Italian Medicines Agency (AIFA)
<http://www.agenziafarmaco.gov.it/it/content/normativa-di-riferimento-sperimentazione-clinica>
- Latvia - State Agency of Medicines of Latvia (ZVA)
<http://likumi.lv/doc.php?id=159645>
- Liechtenstein – Amt für Gesundheit (AG), direct link to Swissmedic
<https://www.swissmedic.ch/bewilligungen/00349/index.html?lang=de>
- Norway - Norwegian Medicines Agency (NOMA)
http://www.legemiddelverket.no/Godkjenning_og_regelverk/Klinisk-utproving/Compassionate-use/Sider/default.aspx
- Romania – National Agency for Medicines and Medical Devices (NAMMD)
http://www.anm.ro/anmdm/med_tratamente_ultima_instanta.html
- Slovenia - Agency of the Republic of Slovenia for Medicinal Products and Medical Devices (JAZMP)
http://www.jazmp.si/zdravila_za_uporabov_humani_medicini/uvoznos_zdravil_ki_nimajo_dovoljenja_za_promet_in_riz_zdravil/
- Spain - Spanish Agency of Medicines and Medical Devices (AEMPS)
<http://www.aemps.gob.es/medicamentosUsoHumano/medSituacionesEspeciales/home.htm>

- Sweden – Medical Products Agency (MPA)
<http://www.lakemedelsverket.se/malgrupp/Foretag/Lakemedel/Compassionate-Use-Program/>
- The Netherlands - Medicines Evaluation Board (MEB)
<http://english.cbg-meb.nl/human/for-marketing-authorisation-holders/contents/prior-to-marketing-authorisation-application/compassionate-use-programme>
- United Kingdom – Medicines and Healthcare products Regulatory Agency (MHRA)
UK Special's Scheme:
<http://www.mhra.gov.uk/Howweregulate/Medicines/Doesmyproductneedalicence/Medicinesthatdonotneedalicence/index.htm>
and
<https://www.gov.uk/guidance/apply-for-the-early-access-to-medicines-scheme-eams>