



CDDF Live Webinar

“Compassionate Use and EAP for Cancer Drugs – From Fragmentation to a Common Framework in Europe under the New Legislation”

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Compassionate Use

A potential lifeline for patients

Compassionate Use (CU) = Managed Access Programs (MAP) at Novartis

Provision of an investigational or locally unapproved or unavailable product to a patient outside of a clinical trial, to address an unmet medical need subject to fulfilment of MAP criteria.

Intent: Access to treatment

- 🔗 *Novartis commitment to patients and caregivers*
- 🔗 *Novartis Position on Access to Unauthorized Novartis Products through Managed Access Programs (MAP)*

MAP Criteria

- ❖ **Independent request** from a **physician**
- ❖ **Patient** with a **serious** or **life-threatening** disease with **no comparable or satisfactory alternative therapy** available
- ❖ **Clinical trial participation not possible**
- ❖ **Potential patient benefit** justifies the potential risk of the treatment use
- ❖ **Other medical criteria** established by Novartis medical experts are met
- ❖ **Supply is available**, no interference with clinical development program
- ❖ **Provision is allowed by local laws/regulations**



Compassionate Use

Our research – Factors impacting CU access

JAMA Health Forum*

Novartis analysis of <30,000 CU requests (2018-2020)



- 31,711 requests received by Novartis from 110 countries, over a 3-year period (~95% approval rate)
- Requests largely for cancers (40%), rare diseases, infectious diseases and neurological conditions

Evaluation of factors impacting CU access concluding that:

- **Public availability and awareness of regulations**

and

- **Local clinical trial activity**

are modifiable country-level factors that could potentially facilitate early patient access to novel, life-saving medicines via CU



**Aliu P, Sarp S, Reichenbach R, et al. International Country-Level Trends, Factors, and Disparities in Compassionate Use Access to Unlicensed Products for Patients With Serious Medical Conditions. JAMA Health Forum. 2022;3(4):e220475. doi:10.1001/jamahealthforum.2022.0475*

Key takeaways for the CU community

- Enact **local CU regulations** adopting best practices yet tailored to the local situation
- Establish simple, **streamlined local CU processes** that facilitate access (avoid bureaucracy, create clarity on operational requirements etc.)
- Create **awareness** among local HCPs, healthcare institutions and patient groups about the regulations and ease of use of the pathway
- Enable and facilitate the **conduct of clinical trials locally**



Compassionate Use

European Pharma Legislation

Current EU CU law is fragmented

Article 83

Regulation (EC) No 726/2004

Groups of patients

Scope: Compassionate Use - Access to unauthorised medicines to groups of patients with no satisfactory authorised alternatives

Article 5 (1)

Directive 2001/83/EC

Individual patient supply

Scope: Supply of unauthorised medicines to individual patients upon an unsolicited HCP request

Member States implement both routes, but national requirements differ across the EU.

⚠ **Different local interpretations of EU Article 83 and Article 5 (1)**

→ No common CU regulatory framework for cohorts AND individual patient requests

⚠ **Variety in local terminologies, scope and definitions – lack of awareness**

→ No common approach leads to uncertainties and confusion

⚠ **Different local operational requirements**

→ No EU CU standards for topics like patient transition, product labeling, supply, QP release, data collection

Opportunities with new EU Pharma Legislation

Article 27 - CU (2028)



1 Unified Legal Basis

Single Regulation for Cohorts and Individual Requests

- Creates a coherent EU-wide framework applicable to all forms of CU
- **Opportunity to harmonize terminology, scope and operational requirements**

2 Transparency

Member State Notification & Public EMA Disclosure

- Member States required to notify the EMA when granting CU
 - The EMA will make CU notifications publicly available
- **Opportunity to increase awareness and visibility**

3 Real-World Data (RWD)

EMA Will Use RWD from CU

- EMA will make use of RWD taking reliability of data into account
 - Creates a formal link between CU data and marketing authorisation support
- **Opportunity to establish a fit-for-purpose RWD collection framework for CU**

EU reform: Opportunities depend on implementation

Foster Common Approach & Clarity | Update EU CU Guideline | Support equity in patient access

Call for action

- Create awareness and offer education through your forum
- Support the implementation of the EU pharmaceutical legislation
- Advocate for CU and for patients



Thank you!