ERGOMED

# Compassionate Use Programs

## The Expertise to Develop Drugs Faster to Improve and Save Lives

**Compassionate use programs (CUP) are early access programs intended to facilitate the availability of new medicines to patients suffering with life threatening disorders or diseases.**

In general, CUP are considered during late stage of development where patients get pre-launch access to the investigational drugs or drugs not yet authorized in the country. Unlike clinical trials, which are protocol driven and where participants have to meet certain inclusion and exclusion criteria, CUP allows patients without considering any criteria. However, CUP enrol patients as per the laws and regulations outlined for the program. CUP are also considered the first opportunity to observe the medicine's safety in a real-world setting, as opposed to a clinical trial setting.

The European Medicines Agency (EMA) defines "compassionate use" as a treatment option that allows the use of an unauthorized medicinal product that is under development. In addition to CUP, there is also possibility to ask for Named-patient program on the basis of a physician's request on behalf of specific or "named" patients.

The EMA provides recommendations for compassionate use through the Committee for Medicinal Products for Human Use (CHMP) for medicinal products eligible to be authorized via the Centralized Procedure.

Laws and regulations are set by the EMA for compassionate use in the European Union (EU) and member states have in turn developed their own legislation for CUP based on this legal framework.

Most of the EU Member States have special programs that facilitate early patient access to new medicines through a national authority. The prevailing early access programs are known by various names in each country such as CUP, special access program, Named Patient Program (NPP), managed access program etc. Moreover, these terms vary based on geographic location and are often used interchangeably. They can imply different ideas with respect to the geographic area. Nonetheless, all these programs make a drug available to a patient prior to authorization and commercial launch in the country.

In the US the regulations for Expanded Access provide pathways to cover access for individual patients (including for emergency use), for intermediate-size patient populations or for widespread treatment use through a treatment IND or treatment protocol (designed for use in larger patient populations) (21 CFR 312).

Our team of regulatory experts support Sponsor's endeavour in providing early access to promising life-saving drugs by providing in-depth local and global knowledge to navigate through the regulatory framework in each country. As there can be a high degree of variability that exists not only between US and EU, but also between the individual EU Member States in terms of the type of data collection permitted, it is important to consider this aspect along with all the other variables (the application and language requirements, time limit or expiry of the CUP approvals, commitments and timelines for submitting marketing authorisation application, impact on reimbursement, labelling and import license requirements) to ensure successful implementation of a global CUP. It is therefore crucial to note that there is no substitute for highly skilled local experts, who are experienced and knowledgeable on regulatory and operational requirements of compassionate use programs.

Ergomed has successfully set up and executed a number of CUP, including the ones related to COVID-19 treatment.

Ergomed's experts develop and implement CUP strategies, including:

- Development of the treatment protocol and patient related materials;
- Designing a tailored data capture solution, focusing on real-world data collection;
- Providing local staff to liaise with treating physicians;
- Safety reporting in line with local country specific requirement, and
- Logistical support for providing the required medicinal product.

Ergomed, being a specialist CRO focused on rare disease research has over 20 years of expertise facilitating high quality clinical research in hard to find patient populations. We also pride ourselves in facilitating after care treatment via named patient, compassionate use, and expanded access programs globally.

**To learn more about Ergomed's expertise in running successful Rare Disease clinical trials, including our unique Site Support Services, please contact us on:**

**+44 (0)1483 503205** or email **info@ergomedplc.com**