New proposals to improve rare disease patients' access to medicines through better Compassionate Use Programmes

25 April 2017, Paris – EURORDIS-Rare Diseases Europe today publishes a new position on compassionate use, calling for the adoption of measures to revolutionise patients' access to new medicines through **Compassionate Use Programmes** (CUP).

For a rare disease patient, to die knowing that a new medicine that could save their life is approaching the market is a source of unmeasurable despair.

Under CUPs, medicines that have not yet been authorised are made available to patients whose disease would otherwise reach a stage that is severe and irreversible or who may pass away before the medicine is brought to market.

François Houÿez, Treatment Information and Access Director, Health Policy Advisor at EURORDIS, commented, "**Compassionate use programmes can save lives** by providing early access to promising new medicines for rare disease patients. Patients with **chronic, seriously debilitating or life-threatening diseases should not have to wait to access a new medicine**. Patients in these situations are desperate and willing to take a higher risk to use a medicine that is not authorised."

He continued, "The new proposals included within this position paper set out why there is a **need for CPU programmes in all countries** and how first steps could be taken to implement or improve such systems. We need to aim to r**educe inequalities between countries in patients' access to CPU programmes and new medicines**."

Proposals put forward by EURORDIS

In the position paper, EURORDIS puts forward the following policy proposals as possible solutions to improve compassionate use across Europe:

1. Promote the French Temporary Use Authorisation (ATU) system so that every Member State adopts it, as it is probably the most efficient compassionate use scheme; **or**
2. Adopt European legislative measures which would confer a greater role in the organisation of CUPs upon the EMA; **and/or**
3. Apply the Directive on Patients' Rights in Cross-Border Healthcare to include compassionate use as part of the care basket so that patients can benefit from these treatments wherever they live in the EU; **and/or**
4. Apply Medicines Adaptive Pathways to Patients to all medicines, where the EU regulator may authorise a medicine at an early stage for a limited group of patients who have a great need for the product, keeping in mind that post-authorisation confirmatory studies need to be conducted afterwards; **and**
5. Amend the EMA guidelines for compassionate use so that the role of the EMA can be reinforced.

Not all countries benefit from an efficient CUP. A time difference of more than three years to access a medicine may exist between those patients benefiting from a compassionate use treatment and those who do not, depending on where they live. Having a mechanism to gain early access is crucial, but the process must be guided by research and trial data to the greatest degree possible.

Recommendations to patient organisations, industry, Member States & European authorities

The position paper also sets out recommendations to patient organisations, industry, Member States and European authorities on how to advocate for, create and manage CUPs, including:

- A call on **patients** to engage in early discussion with medicines developers to agree on if and when a CUP could be relevant, and for which patients;
- A call on **industry** to plan for an adequate supply of the product to be provided through the CUP;
- A call on **national authorities** to improve transparency of the CUPS they authorise in their Member State, so that clinicians and patients are aware of programmes and how to join them; and
- A call on the **European Commission** to compare different national schemes for CUPs in Europe.

What is compassionate use?

According to the EU regulation on pharmaceuticals (art. 83.2), compassionate use is '**making a medicinal product 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 cannot be treated satisfactorily by an authorised medicinal product**'.

- The medicine must either be the subject of an application for a marketing authorisation or must be undergoing clinical trials.
- Medicines provided through CUPs are done so before the medicine is authorised for market. A CUP is a possibility whereby patients receive a treatment at a stage where is efficacy is not yet demonstrated, and not everything is known on its toxicity, and the patient and doctor have agreed on this option.
- Regarding the evidence on which a CUP can be authorised, different Member States require different evidence on the benefits. The French regulatory scheme for CUPs requires "efficacy and safety to be highly presumed, according to the scientific knowledge available", while in Germany, efficacy needs to be also "assumed" and not fully established.

More information on CUPs:

- **List of departments responsible for CUPs in Europe** (Heads of Medicines Agencies)
- **European Medicines Agency website**
- CUPs are not clinical trials, however in Member States that do not have a regulatory scheme for compassionate use, open label trials can serve to provide a product available on a compassionate basis. Use keywords such as "compassionate" or "open label" to find them via **www.clinicaltrialsregister.eu**

About the EURORDIS position paper on compassionate use

EURORDIS members initiated this position following a survey on compassionate use and a workshop in November 2011. The paper was written by EURORDIS and the Drug Information, Transparency and Access (DITA) Task Force and developed in consultation with the Council of National Alliances, the Council of European Federations, the Therapeutic Action Group (TAG) and other EURORDIS members, and adopted by the EURORDIS Board of Directors on 23 March 2017.

Read the annexes to this position paper.

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EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 700 rare disease patient organisations from more than 60 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe.

By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services. Follow @eurordis or see the EURORDIS Facebook page. For more information, visit www.eurordis.org

Rare diseases

The European Union considers a disease as rare when it affects less than 1 in 2,000 citizens. Over 6000 different rare diseases have been identified to date, affecting over 60 million people in Europe and the USA alone. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offering inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

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