Press release

EURORDIS Chief Executive Officer addresses Health Ministers at WHO to improve access to medicines for rare diseases

***13 September, Paris* – For the first time, Yann Le Cam, Chief Executive Officer of EURORDIS-Rare Diseases Europe, addressed 26 European Health Ministers at the WHO Regional Committee for Europe on their role in improving access to medicines for people with rare diseases.**

Le Cam spoke at the WHO Regional Committee for Europe Meeting in Tel Aviv on Monday 12 September. He addressed 26 Health Ministers from across the wider European region on the role that they could play in improving access to medicines for people living with rare diseases. This is an important milestone for the whole rare disease community, whose voice was heard at the level of global decision makers.

At the meeting, delegates adopted a WHO Europe Statement, giving a mandate to WHO Europe to create a Joint Stakeholder Platform and take concrete actions on access to effective, novel, and high-priced medicines. Given the current geopolitical and economic circumstances, leaders from around Europe believe that now is the right time, and the WHO is the right forum to be discussing and pushing these actions that EURORDIS has pushed for a long time.

This statement builds on the work carried out by the Oslo Medicines Initiative - WHO in 2021, aiming at fostering multi-stakeholder dialogue to reshape political discourse and create partnerships to build a movement for change. EURORDIS participated actively in the Initiative, through responses to consultations, interviews, meetings with advisors, participation in webinars, and in sharing its proposals.

Addressing the delegates, including Director General of the WHO, Dr Tedros Adhanom Ghebreyesus, Regional Director of WHO-Europe Dr Hans Kluge, 26 European Health Ministers and 600 other participants, Yann Le Cam said

> *"Among the novel, effective, high-priced medicines, those for rare diseases are impacted and a growing part. Science and industry investments are delivering lifesaving medicines or disease transforming therapies for unmet medical needs, particularly gene and cell therapies."*

It is expected that in the next 5 years, 20-30 new transformative therapies will be approved to treat 10 or 50, or 500 patients, or a few hundreds of thousands. Their timely availability will depend on the political will that governments are able to muster in favour of more collaborative policies, to drive more equitable outcomes.

> *"The current fragmented European market doesn't work for these new medicines. Only a few countries will be served. We are ready for failure, drama and media scandals, and having thousands of patients die while they are waiting. Their challenges can only be addressed through European collaboration," continued Le Cam.*

The concrete proposals put forward by the rare disease community included:

- Collaborating now on the very low prevalence diseases and complex treatments.
- Organising structured access for very rare diseases to go beyond our entrenched barriers.
- Using the unique European solutions at hand, such as a centralised EMA and HTA framework, European Reference Networks for rare diseases, and evidence emerging from pilot trials on the collection of Real-World Evidence and between payers.

> *"What we need now is for Member States' buyers to come together. To have a one-stop shop. To have one European negotiator, using the strength of their collective bargaining power,"* added Le Cam. *"Circumstances are unique and offer opportunities which cannot be missed. You have the power in this room to form the 'Coalition of the Willing', to*

> *develop a European Model of Care with greater solidarity between the Member States, more equity across citizens in Europe which in turn will make Europe more attractive, whilst leaving no one behind."*

The adoption of this WHO Statement is a positive step forward, and EURORDIS looks forward to participating in the Joint Stakeholder Platform in the months to come to deliver on the promises of the milestone document.

About rare diseases

The European Union considers a disease as rare when it affects less than 1 in 2,000 citizens. Over 6,000 different rare diseases have been identified to date, affecting an estimated 30 million people in Europe and 300 million worldwide. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offerings are inadequate, and research is limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of 1000 rare disease patient organisations from 74 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 eurordis.org.

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