11th European Conference on Rare Diseases & Orphan Products (ECRD) 2022

Organiser:

European Organisation for Rare Diseases (EURORDIS)

Contact:
Valentina Bottarelli
Head of European and International Advocacy

EURORDIS-Rare Diseases Europe
Rue d'Egmont 11
1000 Bruxelles
Belgium
+32 2 511 98 91
Valentina.bottarelli@eurordis.org

With the support of:

The European Commission:

Co-funded by the Health Programme of the European Union

AFM-Telethon:

Stella Kyriakides
Commissioner for Health and Food Safety
European Commission
Rue de la Loi / Wetstraat 200
1049 Brussels
Belgium

Friday, 29 July 2022

Re: Call for a European Action Plan on rare diseases from organisers and partner organisations of ECRD 2022

Dear Commissioner Kyriakides,

On behalf of the organisers and partners of the 11th European Conference on Rare Diseases and Orphan Products (ECRD), we would like to thank you for your contribution to this key event for the rare disease community. We appreciated your reassurance that "*access to knowledge, diagnosis and treatment for rare disease patients will remain central to EU policy*".

However the question remains: how can we ensure the interconnectedness and synergy of the different legislative and non-legislative initiatives you mentioned, across different directorates, without a strategy in place that would bring the existing actions on rare diseases under a common EU umbrella, steered by the same measurable goals?

Over the five days of the European Conference on Rare Diseases, 850 stakeholders gathered to discuss exactly how this rare disease policy should be put into action in Europe to work towards meaningful goals, aligned with the SDGs, for people living with a rare disease. These goals were the themes of the conference: to improve health and well-being, to reduce inequalities and to foster innovation.

At the conference it was clear: the rare disease community needs a coordinated, cross-border and cross-sector European action plan on rare diseases. This call, reiterated by this letter, comes from all stakeholders: from patient advocates, clinical and research groups, industry umbrella groups, to Members of the European Parliament and Member States. The 30 million Europeans living with a rare disease at the European level can no longer wait for "accidental progress", but require success by design.

As you so duly noted,

- It is important to the rare disease community that there is a strong European Health Data Space that upholds strong ethical principles of digital health and that includes a specific rare disease codification standard;

- It is important to us that the regulatory system, through the revision of the Orphan Medicinal Products and Paediatric Regulation, the General Pharmaceutical Legislation and the implementation of the HTA regulation, positions the EU as a global leader in medicine development to address the unmet needs of the rare disease community;

- It is important to us that European Reference Networks improve standards of care by being well integrated into national health care systems through the upcoming joint action;

- It is important to us that rare disease research is prioritised and has a direct impact on people living with a rare disease. This is an area where you recognised the benefits of working together at EU level, and we would stress the need for this to be cross-border but also cross-sector working with strong links between research, data and healthcare.

We recognise these tremendous efforts and progress over the last years.

Yet, what is equally important is that all these actions work towards common goals through one coordinated strategy.

Europe's Action Plan for rare diseases is the necessary step to improve the lives of European citizens living with a rare disease. Success by design would mean linking the needs of a person living with a rare disease from diagnosis, through to data, research, treatment, care and inclusion; enshrining rights to accessing quality care, education and employment; and ensuring that this population can benefit from the cutting edge science and technology that the field is known for. It would strive towards the objectives of both EU4Health and Horizon Europe.

It does not take much more than what we have already. The common view of the rare disease community is that all legislative and non-legislative actions should be federated under a new Commission Communication and Council Recommendation on rare diseases, revising the framework from 2009. This should see the whole European Union work towards the same common goals, through:

- All legislative files with relevance to rare diseases connected and reported on;
- Encouragement for Member States to drive national plans and strategies for rare diseases;
- Innovative actions to accelerate progress in certain areas;
- Measurable goals aligned with SDGs introduced to track progress.

On the last day of the European Conference on Rare Diseases and Orphan Products, the **Czech Republic committed to supporting an EU Action Plan on rare diseases during its six-month EU Presidency,** following on from France as part of the Trio of the EU Council Presidencies' priorities.

We appreciate your commitment to rare diseases and have no doubt that under your leadership, we will see a new strategy for rare diseases by the end of this Commission's mandate.

We count on your support and look forward to working with you further.

Signed by:

Co-organisers

1. EURORDIS-Rare Diseases Europe
2. Orphanet

Full and Associate Partners

3. C4C (Conect4Children)
4. EAHP - European Association of Hospital Pharmacists
5. Ethical Medicines Industry Group
6. EFPIA - European Federation of Pharmaceutical Industries and Associations
7. EUCOPE - European Confederation of Pharmaceutical Entrepreneurs
8. Europabio
9. European Burden of Diseases Network
10. European Joint Programme on Rare Diseases
11. Fipra
12. Institut Imagine
13. Isinnova
14. John Walton Muscular Dystrophy Research Centre
15. MedTech Europe
16. Telethon Italia
17. Together for Rare Diseases
18. The Wilhelm Foundation – The Undiagnosed

Coordinating centres of the ERNs:

19. Endo-ERN
20. ERKNet
21. ERN EYE
22. ERN EURACAN
23. ERN EuroBloodNet
24. ERN eUROGEN
25. ERN EURO-NMD
26. ERN GENTURIS
27. ERN GUARD-HEART
28. ERN ITHACA
29. ERN RARE-LIVER
30. ERN-RND

31. ERN TRANSPLANT-CHILD
32. MetabERN
33. VASCERN

Council of Rare Diseases National Alliances

34. Pro Rare Austria Allianz für seltenen Erkrankungen
35. RaDiOrg Rare Disease Organisation Belgium ASBL/VZW
36. Bulgarian National Alliance of People with Rare Diseases
37. Rare Diseases Croatia
38. Cyprus Alliance For Rare Disorders
39. Rare Diseases Czech Republic (Ceska Ascociace Pro Vzacna Onemocneni)
40. Rare Diseases Denmark (Sjaeldne Diagnoser)
41. Harso-Rare Disease Alliance Finland
42. Alliance Maladies Rares
43. Allianz Chronischer Seltener Erkrankungen e.V.
44. 95 Rare Diseases Alliance, Greece
45. HUFERDIS – RIROSZ - Rare Diseases Hungary
46. Rare Diseases Ireland
47. UNIAMO- Rare Diseases Italy
48. Latvian Alliance for Rare Diseases
49. Rare Diseases Lithuania (Vaikų retų ligų asociacija)
50. Alan - Maladies Rares Luxembourg
51. National Alliance For Rare Diseases Support - Malta
52. VSOP - Vereniging Samenwerkende Ouder En Patiëntenorganisaties
53. Polish National Forum on the treatment of Orphan Diseases – Orphan
54. RD-Portugal - União de Associações das Doenças Raras de Portugal
55. Romanian National Alliance For Rare Diseases
56. Slovak Alliance of Rare Diseases
57. FEDER - Federación Española De Enfermedades Raras
58. Rare Diseases Sweden (Riksförbundet Sällsynta Diagnoser)