

`Ladies and Gentlemen

Thank you very much for the invitation to this important event.

I think it is not my role to discuss about Leaving Europe and How. This is a political issue, a popular vote has been expressed and an important negotiation is ongoing.

However, I think it is my role, as a physician and a scientist, to thank the United Kingdom for its contribution to the basic and medical sciences and for the leadership that this country has always represented traditionally in human sciences.

I would not be here to speak to you as the Leader of the European Reference Network For Metabolic Disorders, if Sir Archibald Garrod would not have started, for the first time in 1902 in Cambridge, the unravelling of complex metabolic diseases.

He described 4 diseases linked together by a common metabolic pathway, now we know now more than a 1000 diseases for which, thanks also to the studies of important UK scientists, we have understood the causes, how to diagnose patients and how to identify treatments.

Care for patients is our collective ethical duty.

The ERNs are one of the most ambitious and complex collaborations between expert healthcare providers and clinicians legally based on an EU directive (The Cross Borders Directive published in 2011) gives patients the right to seek diagnosis and treatment across borders if it is not available in their own countries under certain provisions.

24 brave clinicians have risen to this challenge and have realized the most outstanding network, unique in the world, involving about 20k healthcare professionals in 300 hospitals in 26 countries, and taking care of about 1M rare disease patients.

Approximately 150k patients are under the care of the 35 UK Hospitals and 129 HCP inside 22 ERNs. I have estimated about 20-25% of all ERNs patients.

We must think about these patients. All patients with a rare or highly complex condition, requiring a multidisciplinary team, highly technological research and the support of therapies developed by industries translating brilliant ideas into innovative therapies.

This is what UK represented so far, the leader in ideas, proof of concepts and innovative therapy.

One example. In the metabolic field, the Manchester University and the GOSH have always been the European leader in experimenting new therapies, most of them are available worldwide, and Cambridge University and Oxford Universities top leaders in creating new therapies, gene therapies, small molecules, all of them certain to be translated into effective therapies by a private-public interaction under EU grants. These are results obtained by sharing, networking, competence and productive competition linked to transparency.

Why it is important to stay together inside the ERNs:

Quoting the UK Rare Disease Forum statement published last February 2018,

- Collaboration (to strength each other at all levels)
- Evaluation (to develop concise and tangible metrics to measure the real impact of action on patients affected by RD, to create a benchmark for RDs, and to optimize services always considering the impact on health economy)
- Consolidation (building and transforming the best of existing provision to fully realize their potential)

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Patients must be at the center of our work. They need us to collaborate, we need them to evaluate our work so that we can consolidate our work to realize our full potential in developing new therapies.

The EU has created tools (such as the Clinical Patient Management System) to speed up virtual consultations among experts to create faster access to new therapies and procedures.

More than 200 patients have been evaluated so far and 2 UK ERNs, the ERN Eurogen lead by a UK leader Prof. C. Chapple and ITHACA lead by another UK prof. Jill Clayton Smith, are the current most active users of this platform.

Each ERN is developing guidelines, new methods for diagnostics, systems to evaluate the efficacy and safety of therapies, connection with important stakeholders (EMA, IRDIRC, technological and biological infrastructures etc.), to help every Country in offering the best follow up help to each patients affected by RDs and highly complex conditions.

As Coordinator of the MetabERN and as representative of the leading team of the ERN-CG I can ensure that we will do everything to continue to collaborate our UK Colleagues in the interest of the patients.

Nobody will be left behind, no patients will be left without the help of the ERNs when needed.

I want to end with a very famous quote by Winston Churchill:

A pessimist sees the difficulty in every opportunity; an optimist sees the opportunity in every difficulty.

I believe that this difficult historical moment for the world and for the EU, when everything is put under discussion, when even what we thought were undiscussable social and political achievements, now are argued to be not sustainable, this historical moment will move us in finding new opportunities to better work together, to continue to share aims and vision, to help better our patients to whom we must continue to be faithful and whom should drive our decisions.

Our patients will remain at the heart of everything we do.
Thank you for giving me the opportunity to speak to you
today.