D6.4 Report on the Policy Conference

March 2021

Report Information

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Project Information

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Rare 2030 Policy Conference - Objectives

Co-hosted by members of the European Parliament Frédérique Ries (Belgium) and Cristian Silviu Buşoi (Romania), the Rare 2030 Policy Conference was the occasion to officially present the results of a two-year Foresight study in presence of high-level speakers, Rare 2030 partners and experts in the field of rare diseases. The Conference, which took place online on February 23rd 2021 as part of the first edition of the European Rare Disease Week, allowed the audience to discover and gain deeper understanding of the Rare 2030 policy recommendations and to further discuss:

1. How the Rare 2030 recommendations could help shaping a new European policy framework for rare diseases, and
2. How these recommendations could be used as a road map for national initiatives across topic areas.

The conference started with a plenary session during which a number of high-level speakers recognized the value importance and utility of the Rare 2030 recommendations in achieving both objectives. Rare 2030 partners further introduced the audience to the recommendations and the second half of the day was dedicated to 7 thematic parallel break out sessions where, in small groups, participants were given the chance to brainstorm, take ownership of the Rare 2030 project's outcomes and be inspired to take action in helping the community to build a better future for people living with a rare disease in Europe.

Key Facts and Figures

719 attendees

8 outcome-based and measurable recommendations

39 high-level speakers

716 countries represented

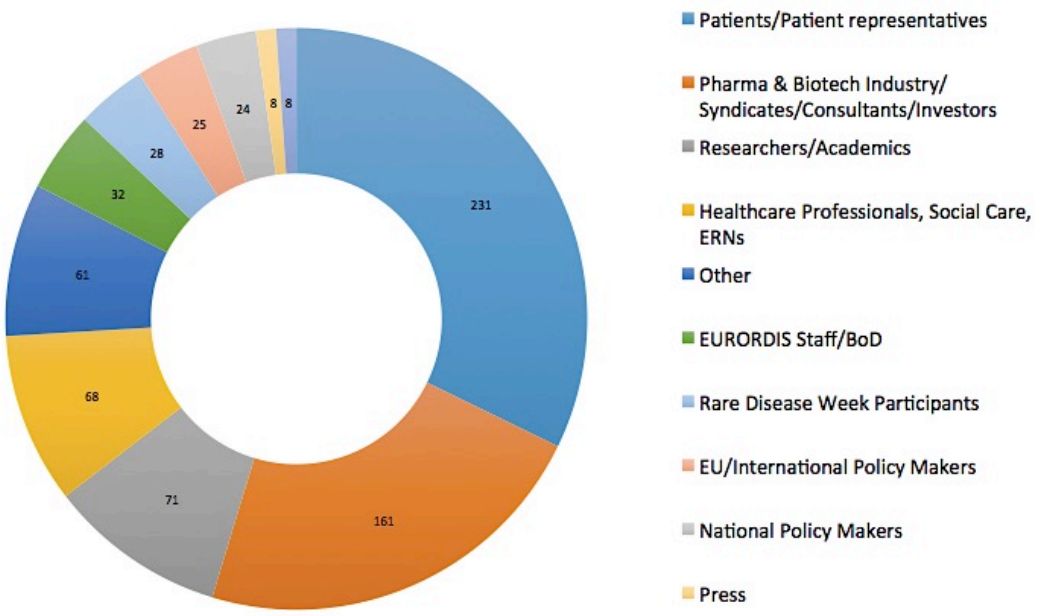
7 parallel breakout sessions

3 MEPs

2 ministers of Health

1 European Health Commissioner

1 President of the European Parliament



Agenda

The agenda below displays the structure of the day and – where applicable – corresponding videos.

Opening Plenary	
13.30 – 13.40	**Welcome** Moderator Tamsin Rose (Friends of Europe)
13.40 – 13.45	**Greetings** Frédérique Ries & Cristian Silviu Buşoi (European Parliament)
13.45 – 13.50	**Opening Remarks** Stella Kyriakides (Commissioner for Health)
13.50 – 13.55	**"Getting to know the Rare 2030 Project"** (video)
14.00 – 14.20	**Keynote Speakers** Olivier Véran (Ministry of Health, France) (video) Terkel Andersen (EURORDIS – Rare Diseases Europe) Nikolaos Milionis (European Court of Auditors) Rebecca Skarberg (Osteogenesis Imperfecta Federation Europe, Norway)
14.20 – 14.30	**Presentation of the Rare 2030 recommendations** Prof. Kate Bushby
14.30 – 15.05	**Discussion Panel** (moderated by Kate Bushby) Yann Le Cam (EURORDIS – Rare Diseases Europe) Victoria Hedley (UNEW) Prof. Maurizio Scarpa (MetabERN) Lucia Monaco (Fondazione Telethon) Ana Rath (Orphanet) Giovanna Giuffrè (ISINNOVA) Fanni-Laura Mäntyla (Rare 2030 Young Citizens)
15.15 – 15.20	**Keynote Speaker** Andrzej Ryś (European Commission, DG Health and Food Safety)
15.20 – 15.25	**"In Ten Years' Time… A word from the Rare 2030 Young Citizens"** (video)
15.30 – 16.00	**Comfort Break**
Break Out Sessions	
16.00 – 17.30	**Parallel Break Out Sessions** Diagnosis, Research, Data, Integrated Care, Treatments, Access to Healthcare, Patient Partnership
17.30 – 17.45	**Comfort Break**
Closing Plenary	
17.45 – 18.30	**Closing Remarks** Kateřina Konečná (European Parliament) Milan Macek (Rare2030 Research Advisory Board) Jan Blatný (Ministry of Health, Czech Republic) David Maria Sassoli (President, European Parliament) Yann Le Cam (EURORDIS –Rare Diseases Europe) (vidéo)

The recording of the full event can be found **here**.

Keynote Speeches Highlights

The speakers' bios can be found in the annex of this report.

Stella Kyriakides: "This journey is not ending with these recommendations. We all need to continue to take them forward. [...] Today's recommendations are the results of a major effort to inform and support rare disease policies. I can assure that rare disease policy will remain an important focus for the Commission and that patients' voices will continue to inform our approach. Current achievements need to be consolidated and further improved"

Frédérique Ries: "The Rare 2030 recommendations are our guidance to invent the innovative, tailored, and collaborative health systems of tomorrow"

Olivier Véran: "A united Europe, a closer-knit Europe, is a Europe that knows how to reduce the impact of rare diseases on those affected and their carers."

Yann Le Cam: "It is time to reset Europe's rare disease focus for the next decade: we need a policy framework adapted to today's realities, to embed excellence and bring policies in line with new technologies, values and infrastructures. Europe's efforts since 2009 have shown how much progress can be made when national plans and strategies for rare diseases are coordinated across countries and integrated at EU level. We cannot lose momentum now: the Rare 2030 recommendations set the direction we need to go"

Terkel Andersen: "We must re-incentivize the European strategy for rare diseases with measurable objectives for these unmet needs. We must renew our strategy in order to make equal access to health a priority and a distinct value of Europe. We must coordinate across research, digital, healthcare, and social welfare to harvest the synergies of European Collaboration. If we do so, the lessons taken from the Covid19 crisis combined with the recommendations of this Foresight Study will be seen as having provided important guidance for the coming years."

Katerina Konečná: "I believe that deeper cooperation and a Europe-wide solution to the situation of patients with rare diseases should be one of the topics of the forthcoming Czech Presidency of the Council."

Rebecca Skarberg: "The recommendations of Rare2030 show us that we still need a protecting hand over rare disease when priorities are set, when budgets are debated and when healthcare becomes a tug of war. If we handle rarity disease by disease we will never reach enough prevalence, documentation or profit to compete with other just causes."

Andrzej Ryś: "The golden age of networking [...] The ERNs are clearly the best examples of this networking. It makes sense, it helps us to develop things. It provides additional value to the patients and the clinicians and we should continue."

Nikolaos Milionis: "In 2019 we published a report on the implementation of the Cross-Border Healthcare Directive. Our overall conclusions was that EU actions in Cross-Border Healthcare Directive were ambitious and enhanced MS collaboration. But that they require better management, since EU patients still face challenges in accessing healthcare abroad, especially for rare diseases, and only a minority of patients are aware of their right to access healthcare across the border."

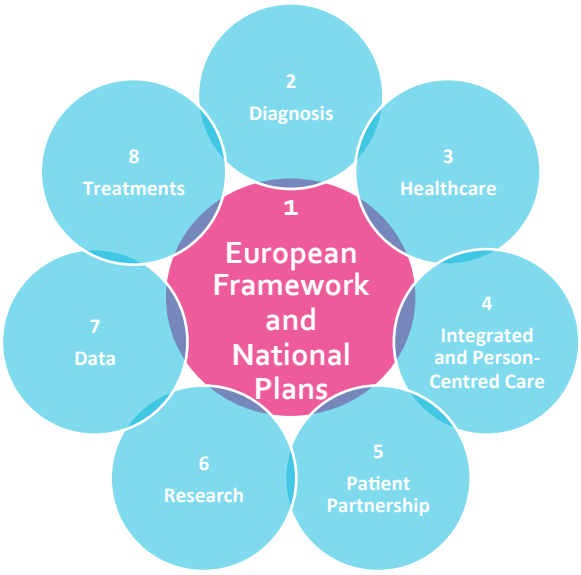
Cristian Silviu Buşoi: "This [the Rare 2030 recommendations] is yet another milestone for improving the lives of those living with a rare disease. Under the EU4Health program we call for an up-to-date rare disease strategy and for continuing the financing of the ERNs"

Jan Blatný: At the EU level, our government is looking forward to the outcomes of the Rare 2030 project. We look forward to use identified core Rare 2030 topics for the upcoming EU council trio presidency - which is French, Czech and Swedish presidencies - to be held next year. Therefore, let me conclude with the underlying motto of solidarity: we learned that national governments can't cope alone. We need EU wide efforts as is now the case with the pandemic. We should also balance national and EU-wide approaches and most importantly, we

need to place rare disease strategies within the framework of a more comprehensive approach to vulnerable and marginalised populations. Importantly, such approach is at the core of traditional European values.

Final Recommendations

7 project partners presented the Rare 2030 recommendations through a discussion moderated by Prof. Kate Bushby, Rare 2030 Research Advisor and former leader of the EUCERD Joint Action on Rare Diseases. Each panelist was asked specific questions related to their own field of expertise and/or their involvement in the project, which also included links to the recent COVID-19 pandemic. The presentation of the Rare 2030 outputs particularly stressed the interconnectivity and interdependence of the 8 recommendations, as well as the importance of addressing all topics in European and national plans.



Discussion panel:

Yann Le Cam – Chief Executive Officer, EURORDIS

Ana Rath – Director, Orphanet

Lucia Monaco – Head of Research Impact and Strategic Analysis, Fondazione Telethon and Consortium Assembly Chair of IRDIRC

Maurizio Scarpa – Coordinator, MetabERN

Fanni-Laura Mäntylä – Rare 2030 Young Citizens representative

Giovanna Giuffrè – Foresight Expert, ISINNOVA

Victoria Hedley – Rare Disease Policy Manager, Newcastle University

The following sections detail the recommendations that were presented during the plenary of the conference.

1. Long-term, integrated European and national plans and strategies

A European policy framework for rare diseases defined by societal responsibility, equity and driven by the needs of people living with a rare disease should guide the implementation of consistent national plans and strategies, secure major investments at both the European level and by governments that are fairly shared across Europe in order to pool scarce resources, share expertise and information, scale-up good practices and provide access to timely and accurate diagnosis and the highest available quality of treatment and care for people living with a rare disease, no matter where they live in Europe. Both EU and national policies are supported by measurable outcomes that are monitored and assessed by a multistakeholder body on a regular basis.

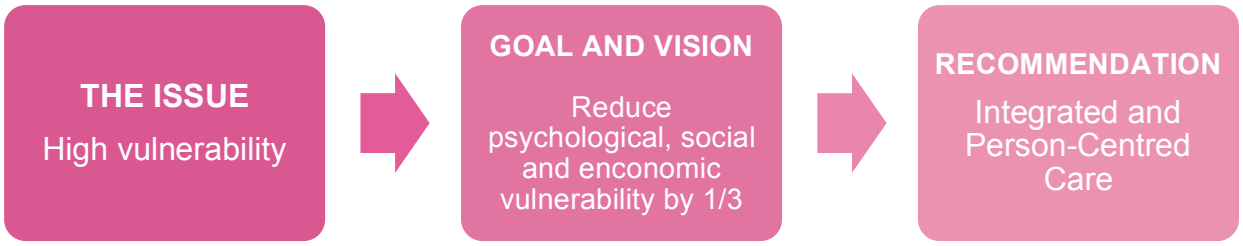
2. Earlier, faster, more accurate diagnosis

The time to diagnosis should be shortened - whilst avoiding erroneous and subsequent negative consequences - which should be achieved by better use and accessibility of currently effective and available diagnostic testing technologies, best practices and programmes. New technologies and innovative approaches must be driven by patients' needs and applied rapidly and strategically. Inequalities in access to diagnosis and ensuing care must be eradicated through the harmonisation of standards and programmes across Europe (and beyond). A particular focus is necessary for patients with undiagnosed rare diseases, which demand greater and more strategic global collaboration via data-sharing and diagnostic platforms and infrastructures.

3. Access to high quality care

Political, financial, operational and technical support at European, national and regional levels should be provided to establish a mature and highly specialized healthcare ecosystem that, in collaboration with patient organizations and all relevant stakeholders, leaves no person living with a rare disease with uncertainty regarding their diagnosis, care and treatment.

4. Integrated and person-centred care



Implement EU-wide and national actions by all stakeholders that guarantee the integration of people living with a rare disease in societies and economies, enabling them to live life to their full potential, by implementing innovative solutions and approaches to integrated and person-centred care along the full lifespan of people living with a rare disease. EU-wide and national actions must be undertaken by all stakeholders to guarantee equal opportunities and access to the labour market, active support for employment, fair working conditions, social protection and inclusion and integrated, person-centred and long-term care for people living with a rare disease and their families.

5. Partnership with patients

An overall culture, reflected in policies and funding, that encourage the meaningful participation, engagement, involvement and leadership of people living with a rare disease in the research, care and development of diagnostic tools, treatments and innovative solutions to improve the health and social status, healthcare delivery, autonomy, quality of life and well-being of people living with a rare disease in Europe. Patient partnership should be encouraged in both the public and private sectors and people living with a rare disease and their representatives may often serve as a partnering link between the two.

6. Innovative and need-led research and development

Maintain basic, clinical, social and translational research on rare diseases as a priority by increasing the funds for competitive and pre-competitive research, establishing greater incentives in more neglected areas (or in areas of high unmet needs), and supporting infrastructures required to expedite discovery and knowledge acquisition. Research in public health, social sciences, healthcare organisation, health economics and health policy research

must also be promoted, to ensure that research outputs are applied for the benefit of people living with a rare disease.

7. Optimising data for patient and societal benefit

All European data sources of relevance to addressing the challenges faced by people living with a rare disease should be federated in a continuum encompassing epidemiological, healthcare, research, quality of life and treatment-related data, and should be linked at the global level where possible. Sharing of data for care and research should be optimised across infrastructures and countries, relying upon commonly adopted codification systems (Orphanet nomenclature), harmonised standards and interoperability requirements. Cohesive data ecosystems should be developed at national level, linking seamlessly via Findable, Accessible, Interoperable and Reusable (FAIR) data approaches to an integrated European ecosystem, positioned within the European Health Data Space and centred on robust European Reference Networks (ERNs), the European Platform on Rare Disease Registration, and other key infrastructures. Legal and ethical guidelines and regulations should incentivise practices that best lead to addressing these challenges while respecting international, national and regional laws and conventions – particularly the preferences and privacy of people living with a rare disease and their families.

8. Available, accessible and affordable treatments

Establish streamlined regulatory, pricing and reimbursement policies. These policies should encourage a continuum of evidence generation along the full life cycle of a product or technology as well as the patient journey from diagnosis to treatment access. A European ecosystem able to attract investment in areas of unmet need, foster innovation, and address the challenges of healthcare system sustainability.

Break out sessions

Following the plenary and the presentation of the Rare 2030 policy recommendations, attendees were offered the opportunity to join breakout sessions dedicated to 7 Rare 2030 topics (mirroring 7 of the 8 recommendations). Topic/Recommendation 1 "Long-term, integrated European and national plans and strategies" was not specifically addressed in a breakout session as it is considered to be central to all recommendations and widely covered in the plenary sessions. Each breakout session was structured as follows: a presentation by a moderator, a presentation by a policy-maker, a case study presented by a stakeholder relevant to the session's topic. Following these presentations, attendees were divided into small discussion groups where they could discuss the recommendations and report back to the breakout main group about how they may adopt these recommendations in their work. The goals of these sessions were to allow the audience to gain deeper understanding of the recommendations, provide attendees with concrete examples of how to use the Rare 2030 project's outcomes, and to allow participants to discuss, brainstorm, give feedback and take ownership of the recommendations.

The following section addresses each breakout session and displays the theme-specific points of discussion raised by participants.

1. Earlier, faster, more accurate diagnosis

95 attendees

Moderator: Dr Luca Sangiorgi – ERN BOND

Policy-maker: Dr Holm Grassner – ERN-RND

Case study: Christine Mundlos – ASCHE

Discussion

Participants of the session praised the ambition of the Rare 2030 recommendations and, while acknowledging that a change – as well as improvements on the topic of diagnosis – is needed, questioned whether a goal of a diagnosis within six months is realistically feasible, as they point to a gap between this goal and the current six to seven years before an accurate and correct diagnosis. However, participants stressed that the goal could be reached provided that there is political willingness at both national and European levels and willingness to actually invest on national and European programs, including reimbursement of diagnostics and examinations for which people living with a rare disease typically have to pay.

They further call for the implementation of a registry for the undiagnosed patients at national level, where data from unsolved cases would be stored and potentially retrieved and used at a later stage for research purposes. Participants – echoing discussions in other breakout sessions – also report that data are currently kept in siloes, potentially hampering research and progress in diagnosis and that more education is needed to effectively guarantee that rare diseases can be detected in due time and acted upon quickly following the onset of symptoms. European Reference Networks (ERNs), differences between countries with regards to strategies, and the role of patients were also discussed.

2. Access to high quality care

70 attendees

Moderator: Victoria Hedley – UNEW

Policy-Maker: Martin Dorazil – EC, Unit B3

Case study: Ilaria Galetti – ERN Re-Connect

Discussion

The COVID-19 pandemic was discussed and generally perceived as a threat, as it may push rare diseases down in the list of priorities of in countries and their healthcare systems. Participants consider that this threat must be addressed at the EU level and that collaboration should become the goal to reach for policies to be effective and access to care to be improved. They took the example of European agriculture policies to illustrate that healthcare too would benefit from collaboration beyond national borders. Agriculture is a transnational collaborative despite great differences in climate, terrain, culture, tradition – why can't the same be true for rare diseases? Questions of European support to national initiatives and funding issues were also raised.

Multi-stakeholder approaches and clarification of the roles of ERNs were strongly encouraged by the group. On the topic of ERNs more specifically, participants said these should be embedded in healthcare systems and that more information on how to reach out to them and what they can actually help with should be shared. Participants also linked access to care to other topics and recommendations, especially with regards to data.

3. Integrated and person centred care

56 attendees

Moderator: Clara Hervas – EURORDIS

Policy-maker: Inmaculada Placencia Porrero – EC, DG Employment

Case study: Dorica Dan – Romanian Alliance for Rare Diseases

Discussion

Participants showed great interest in the recommendation on integrated care and praised the project for shedding more light on topics such as psychological support. They also acknowledged the links and interconnectivity between the different recommendations, stating that they are all equally needed to guarantee that rare diseases are considered and addressed comprehensively in future initiatives.

Participants discussed the option of having a rare disease card, similar to the already existing disability card. They believe that patients and patient organisations should communicate more on the holistic needs of the rare disease community and the impacts of disabilities and psychological needs. They defined person-centred approaches and networks as good practices and compared countries such as Austria, Germany, Ireland, Norway and Sweden with regards to national concrete actions currently taking place and concluded that following the outcomes of these initiatives would help identifying gaps in integrated care throughout Europe.

4. Partnership with patients

90 attendees

Moderator: Virginie Bros-Facer – EURORDIS

Policy-maker: Antoni Monsterrat Moliner – ALAN

Case study: Fanni Petridis – Roche

Discussion

Amongst the challenges in partnerships with patients discussed in this breakout session, participants put the stress on the perceptions that stakeholders may have of each other. They particularly reported that, while the

benefits of working with patients are numerous, the medical community may be quite reluctant to collaborate closely with people living with a rare disease as the latter might not be sufficiently skilled for effective partnerships. Regulatory issues related to the involvement of patients were also part of the debate. Another challenge at the centre of discussion was the issue of the "rare disease bubble". The group noted that people recognised as actual partners are often the same ones and participants thus deplore a lack of inclusiveness and reporting comprehensiveness. In other words, one may not have a comprehensive understanding of the rare disease community, as only a few individuals are visible and invited to collaborate: How to avoid the problem of the "usual suspects".

Participants therefore called for more education around rare diseases and across stakeholders, to guarantee that they are all working with a minimum educational background. The long-term of these educational programs has also been briefly addressed. They further encourage bringing patient representatives to the next level, with clearer roles and responsibilities, with regulations and adequate training in place to build a strong patient community active in research, development, etc.

5. Innovative and need-led research and development

82 attendees

Moderator: Stefano Benvenutti – Fondazione Telethon

Policy-maker: Catherine Berens – EC, DG Research

Case study: Inès Alves – Patient Expert

Discussion

The group highlighted that all recommendations are highly interdependent and regularly established links between them in their discussion.

Participants stressed the need for a strong signal from the EU when it comes to research and areas where research should be particularly emphasised. They discussed the importance of partnerships at various levels, such as across countries, disciplines and qualifications. The benefits of involving patients in research were specifically emphasised (meaningful endpoints, broader perspectives) and thus called for patient experts mandates to be implemented.

The also acknowledged the significant progress t made in research in the past decade but firmly believe there is room for further improvements through stronger collaboration, sharing of good practices and participatory ecosystems being encouraged and facilitated.

6. Optimising data for patient and societal benefit

63 attendees

Moderator: Ana Rath – Orphanet

Policy-maker: Ceri Thompson – EC, DG Communications Networks, Content and Technology

Case study: Anne Charlet – Lupus Europe

Discussion

Participants in the breakout session on data called for increased and more efficient sharing of data and a wider uptake of electronic health records throughout Europe, while acknowledging that more education and harmonization of data collection/storage/use were crucial conditions for a comprehensive and successful data

strategy. Orpha Coding was cited as an example of good practice and its broader use and uptake was strongly encouraged. The establishment of registries, with the example of Switzerland, was also firmly supported.

With regards to people living with a rare disease specifically, participants believe that they have a role to play in data collection. The use of patient-reported outcomes and means of data collection through smartphones and other personal devices were taken as an example, therefore moving from a classical registry to a more modern approach to data collection. They further call for more education on secondary use of data and 40% of participants considered that helping coordination of European initiatives around data is a priority.

7. Available, accessible and affordable treatments

97 attendees

Moderator: Simone Boselli – EURORDIS

Policy-maker: Kaja Kantorska – EC, DG Health and Food Safety

Case study: Diego Ardigo - Chiesi

Discussion

The competitiveness of Europe was a key point of discussion in the breakout session. The topic of incentives was especially stressed and participants exchanged views on the areas where these incentives should be used the most. Rewarding all innovations was strongly encouraged, although the appropriate allocation of resources between rare diseases and extra rare diseases was recognized as a challenge, particularly when incentives would be granted to specific innovations to the detriment of others intended for more "common" conditions. The definition of unmet need, which would have its influence on the granting of incentives, was also discussed. The EU and US approaches were addressed and compared and EU-level HTA was identified as the high hope for the rare disease community as a whole.

Linking the recommendation on treatments to other topics, participants called for more sharing and harmonization of data regardless of geographical considerations/concerns. Echoing other groups, such as "Partnerships with Patients", the involvement of patients at an early development stage and the gap between academics and regulatory spheres were also discussed. Particularly with regards to patient involvement and collaboration between stakeholders, participants believe that a better definition of (added) value would greatly benefit from the inclusion of all stakeholders (including patients) in research and regulatory processes.

Key Takeaways

The following takeaway messages were extracted from the closing speech of Yann Le Cam, Coordinator of the Rare 2030 project.

The Immediate Impact of the Rare 2030 Foresight Study

This foresight study has had three benefits.

1. An immediate benefit has been to digest the ongoing policies to inform immediate actions.
2. It has allowed preparing for the future and for the preferred scenario ("Investment for Social Justice") while, at the same time, also foreseeing what actions should be considered and/or taken should the least favorite scenario happen.
3. It has prepared a large audience and equipped this audience with a shared vision and road map to be implemented in the next 10 years. More than 200 people were involved in the Rare 2030 project but,

through this online conference, more than 700 people – possibly more – were reached and given the possibly to share a common goal.

The Rare 2030 policy recommendations form a roadmap, which has immediate impacts on the new European programs within the long-term EU Multiannual Financial Framework 2021-2027, which includes the Horizon Europe Research program, the EU for Health Program and the European Digital Health Space, all which have great importance for the rare disease community. Another example is the European Structural Funds, which are particularly important at the member state level to fund activities. The Rare 2030 recommendations can also help inform the ongoing revision of policies, such as the EU pharmaceutical strategy in terms of unmet needs and access to medicines, the Orphan Drug Regulation, the Pediatric Regulation, as well as the incentives granted based on these regulations.

In the last few years, a number of messages of the rare disease community have been heard, resulting in for example, the implementation of 24 European Reference Networks. However, many other messages were not heard or sufficiently understood – a European Reference Network on rare infections or pregnancies. There remain many important gaps and needs and much remains to do to addressed them. Current policies lack achievable milestones and results on topics covered by the Rare 2030 Project. A holistic approach, going from research to actual care, is crucial and should be set as one of the main policy goals to avoid fragmentation of efforts. Alongside resources and goodwill, coordination is therefore key. This is essential if, as agreed in the Sustainable Development Goals including that of Universal Health Coverage, we strive to leave no one behind. The Rare 2030 recommendations now need to be translated into actions as part of an actual strategic action plan to address the needs of the 30 million people living with a rare disease in Europe.

Europe has been an inspiration to the world over the past decades. When the strategy of National Plans was adopted in 2009, it inspired countries such as Canada, Mexico, Argentina, Colombia and, progressively, most of Latin and Central America in addition to New-Zealand and Australia. Just because Europe has been a role model in the past, does not mean that efforts should be stopped or limited now. On the contrary, it shows that Europe must continue to serve as a leader and develop further actions and initiatives at the national, European, and even global levels.

The Context of Covid 19

The recent COVID-19 pandemic has been mentioned by many speakers today and could be closely linked to rare diseases. The European Funds Network was immediately useful at the beginning of the pandemic, allowing for coordination between professionals. A European Reference Network on rare infections would have helped even further, but the hope is we will get back to establishing this Network in the future. From another perspective, some people with COVID-19 may have symptoms long after their infection, two to five months later. Today, these "long COVID" patients are in a maze and do not know where to go. In most cases, they go to rare disease centers for neuromuscular diseases or for rare lung diseases. The need to tackle this at the European level is of common interest for society: there is only going to be a few of these cases and this type of infection will not become a prevalent condition. It is a rare disease and it is an immediate challenge.

Another example of the link between Covid 19 and rare diseases is the vaccine. The RNA vaccines from Pfizer and Moderna are both based on research performed in Europe on rare diseases and rare cancers. Significant investments and accelerated access to vaccines was enhanced because the science was already there. The return on investment for Europe has thus already been quite high and clearly demonstrates the benefits for the whole society in investing in rare diseases. What seemed impossible yesterday has become possible today. Member States come together, under the European Commission, to negotiate with laboratories for the vaccines and to do joined purchasing. It is the hope of the rare disease community that this will also happen for rare disease therapies – perhaps not for all of them, but at least for the most complex ones, such as gene and cell therapies. If we do not follow this path, the lack of coordination and collaboration will only result in growing inequalities and ultimately innovation will not reach those in need.

This Conference platform welcomed the support of the Health Commissioner, the call from Court of Auditors for a revision and adjustment of the policies, a push from the members of Parliament for an improved framework for the next years and a message from the President of the European Parliament himself of the importance to implement the Rare 2030 recommendations. Rare diseases are on the forefront of collaboration between Member States and of integration of health strategy between them. This is a precious input when we are

confronted with health challenges. We are faced with a pandemic today but other challenges may arise tomorrow and collaboration is Europe's best weapon. We need to encourage collaboration. France and the Czech Republic said rare diseases will be a priority in their respective semesters of the EU presidency. We thus hope that Member States, the European Parliament and the European Commission will be on board for a new Council recommendation on rare diseases, to prepare a comprehensive and integrated approach so that, not only do we have a protective hand on people living with a rare disease in Europe, on what has been done in the past, but also a helping hand for the rest of the population, focused on measurable public health objectives to be proud of 10 years from now.

Media Coverage

The following sections displays materials and links related to the media coverage of the Rare 2030 Policy Conference.

Media

Mediaplanet published two articles, both featured in a supplement of The Guardian (United Kingdom) on the topic of rare diseases. The first article features Ana Rath (Orphanet) and Yann Le Cam (EURORDIS) as authors (February 16th 2021). Find the article "The future of rare diseases starts now". The second article features Rebecca Skarberg (OIFE) as author (February 17th 2021). Find the article "A better future for rare diseases"

Yann Le Cam (EURORDIS) gave an interview February 19th 2021 on the show Tendances Premières at Radio-Television Belge de la Communauté Française (RTBF) on the topic of Rare Diseases and COVID-19. See the video

Following the intervention of French Minister for Health, Olivier Véran, Pharmaceutics published an article on February 25th 2021 "*Les maladies rares: priorité de la prochaine présidence Française de l'UE*". Find the article

Social media

Several materials were shared on social media (Twitter, Facebook, LinkedIn) from both EURORDIS and Rare 2030 accounts. The materials mainly focused on registrations (reminder to register), the recommendations, interventions from keynote speakers, and videos broadcasted during the conference. The figures below are retrieved from EURORDIS and Rare 2030 accounts from February 23rd 2021.

Twitter

Rare 2030 account:

- Reach/Impressions: 37.676
- Engagement: 627

In comparison to February 2020, the overall engagement for the whole month on the Rare 2030 Twitter account more than doubled (20.600 in 2020 compared to 51.000 in 2021)

EURORDIS account:

- Reach/impression: 51.021
- Engagement: 1.867

<u>Facebook</u>

EURORDIS account:

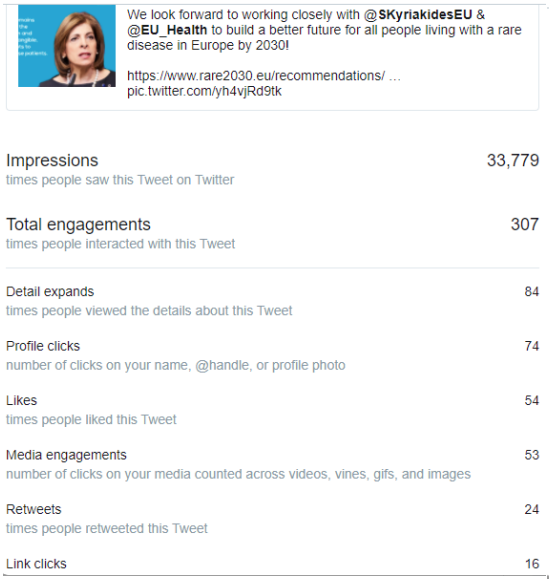
- Reach/impressions: 24.676
- Impression: 853

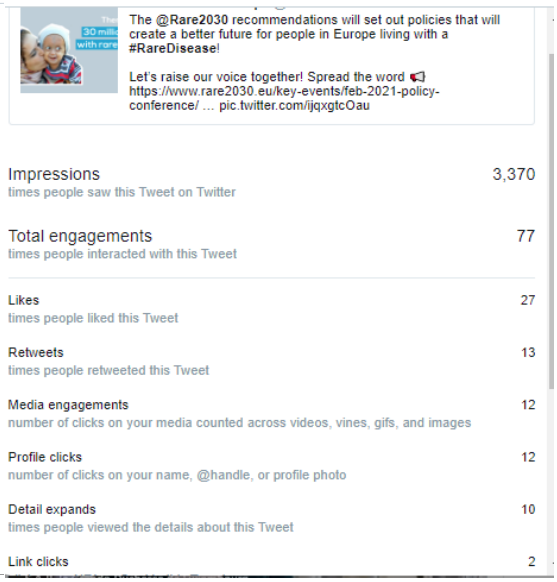
<u>LinkedIn:</u>

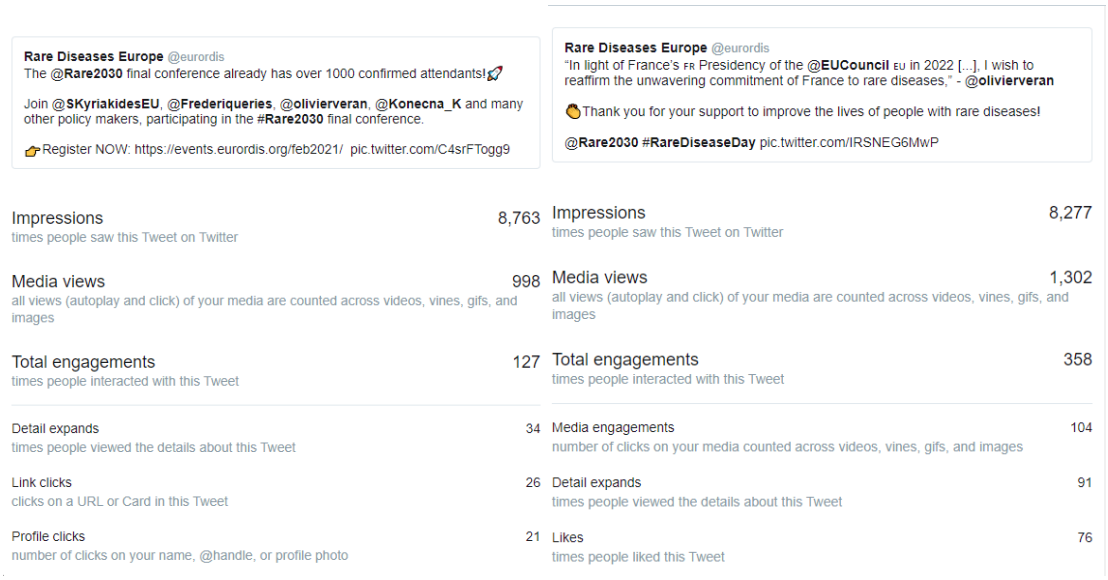
EURORDIS account

- Reach/impressions: 16.292
- Impressions: 494

Screenshots of Tweets and posts (as well as performance analysis) are displayed below





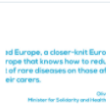


Rare Diseases Europe @eurordis
Thank you to all patient advocates, practitioners and policy makers who joined us at the **@Rare2030** final event.

The resulting recommendations will guide policy changes for the next 10 years, and we are grateful to have your full support!🙌

ℹ https://www.rare2030.eu/recommendations/ …
pic.twitter.com/XSh2eEQZAH

Impressions times people saw this Tweet on Twitter	4,034
Total engagements times people interacted with this Tweet	113
Likes times people liked this Tweet	38
Media engagements number of clicks on your media counted across videos, vines, gifs, and images	20
Detail expands times people viewed the details about this Tweet	19
Retweets times people retweeted this Tweet	15
Profile clicks	11



Rare Diseases Europe @eurordis
The **@Rare2030** final event detailed a set of rare disease policy recommendations in 8□ key areas.

Which one is the most important to you? 👇
https://www.rare2030.eu/recommendations/ … **#Rare2030**
pic.twitter.com/z3lGpmQl48

Impressions times people saw this Tweet on Twitter	2,748
Total engagements times people interacted with this Tweet	101
Likes times people liked this Tweet	26
Detail expands times people viewed the details about this Tweet	23
Profile clicks number of clicks on your name, @handle, or profile photo	16
Retweets times people retweeted this Tweet	14
Link clicks	13

EURORDIS - European Rare Diseases Organisation
Published by Hootsuite · 23 February at 18:49 ·

Thank you to all patient advocates, practitioners and policy makers who joined us at the Rare 2030 final event.

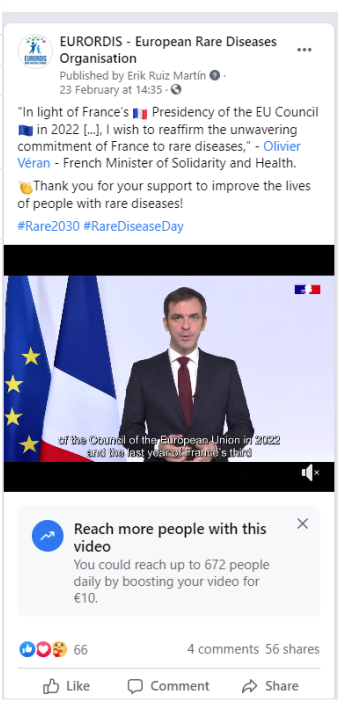
The resulting recommendations will guide policy changes for the next 10 years, and we are grateful to have your full support!🙌

https://www.eurordis.org/.../better-future-design-call...

82 1 comment 34 shares
Like Comment Share

Performance for your post

6053 People reached
234 Likes, comments and shares
89 Post clicks
6 Photo views | 17 Link clicks | 66 Other Clicks
NEGATIVE FEEDBACK
0 Hide all posts | 1 Hide post
0 Report as spam | 0 Unlike Page
234 Likes, comments and shares
BRANDED CONTENT DISTRIBUTION — View Breakdown
6032 Total reach | 6053 Organic reach | 0 Paid reach
6032 Total impressions | 6032 Organic impressions | 0 Paid impressions



EURORDIS - European Rare Diseases Organisation
Published by Erik Ruiz Martín · 23 February at 14:35 ·

"In light of France's 🇫🇷 Presidency of the EU Council 🇪🇺 in 2022 […], I wish to reaffirm the unwavering commitment of France to rare diseases," - Olivier Véran - French Minister of Solidarity and Health.

👏Thank you for your support to improve the lives of people with rare diseases!

#Rare2030 #RareDiseaseDay

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You could reach up to 672 people daily by boosting your video for €10.

66 4 comments 56 shares
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Performance for your post

7356 People reached
244 Likes, comments and shares
430 Post clicks
17 Clicks to Play | 4 Link clicks | 409 Other Clicks
NEGATIVE FEEDBACK
0 Hide all posts | 2 Hide post
0 Report as spam | 0 Unlike Page
244 Likes, comments and shares
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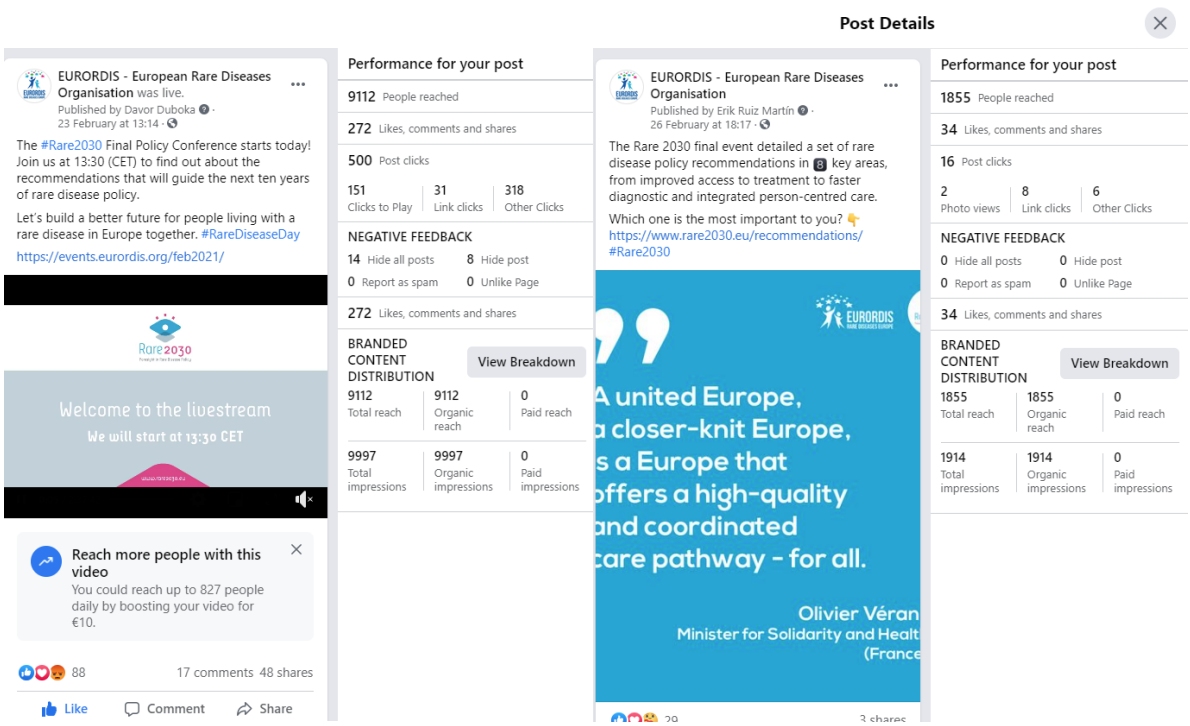
7718 Total impressions | 7718 Organic impressions | 0 Paid impressions



Press releases/articles

A sampling of press releases and article have also help disseminate the outcomes presented during the conference to a wide audience:

- European Medical Journal - RARE 2030 FINAL POLICY CONFERENCE: SUMMARY OF THE RECOMMENDATIONS OF THE RARE 2030 FORESIGHT STUDY - https://www.emjreviews.com/european-medical-journal/article/rare-2030-final-policy-conference-summary-of-the-recommendations-of-the-rare-2030-foresight-study/
- EURORDIS - A better future by design: call for a new European policy framework for rare diseases - https://www.eurordis.org/content/better-future-design-call-new-european-policy-framework-rare-diseases
- European Commission - Speech by Commissioner Kyriakides to the RARE2030 Final Conference - "The Future of Rare Diseases Starts Today: Recommendations from the RARE 2030 Foresight Study" - https://ec.europa.eu/commission/commissioners/2019-2024/kyriakides/announcements/speech-commissioner-kyriakides-rare2030-final-conference-future-rare-diseases-starts-today_en
- European Reference Networks:
    - eUROGEN - Rare 2030 Final Virtual Conference - https://eurogen-ern.eu/rare-2030-final-virtual-conference/
    - ERN Cranio - https://ern-cranio.eu/news-events/events/rare-2030-final-policy-conference/
- EU- IPPF - Rare diseases Eyurope: call for a new European policy framework for rare diseases - https://www.eu-ipff.org/news/article/press-release-from-eurordis-rare-diseases-eyurope-call-for-a-new-european-policy-framework-for-rare-diseases

Annex: Speakers bios

Rare 2030
Foresight in Rare Disease Policy

FINAL POLICY CONFERENCE

The future of rare diseases starts today

Recommendations from the Rare 2030 Foresight Study

Speaker biographies

Co-hosting MEPs

Frédérique Ries

Frédérique Ries has been a Member of the European Parliament since 1999 and represents the Belgian Mouvement Reformateur in the Renew Europe Group. She is part of the the Committee on the Environment, Public Health and Food Safety (ENVI) sector and is Vice-Chair of Renew Europe.

A journalist and former Secretary of State for European and Foreign Affairs, she has been a pioneer in the implementation of patient-centric health policies that focus on European Union-added value, especially in the field of rare diseases.

She is a sponsor of the pilot project Rare 2030 (initiated by the European Parliament and co-funded by the European Commission), a foresight study that gathers the input of a large group of patients, practitioners and key opinion leaders to propose policy recommendations that will lead to improved policy and a better future for people living with a rare disease in Europe. Frédérique Ries is also the recipient of the 2017 EURORDIS Policy Maker of the Year Award.

Cristian-Silviu Bușoi

Cristian-Silviu Bușoi is a Member of the European Parliament and Chair of the Industry, Research and Energy Committee. He is also Member of the Delegation for Southeast Asia, as well as substitute Member of the Environment, Public Health and Food Safety (ENVI), Committee on Economic and Monetary Affairs (ECON) and the Delegation for the Arab Peninsula.

Mr. Busoi studied Medicine at Carol Davila University in Bucharest, Law from Titu Maiorescu University in Bucharest, and Diplomacy from the Romanian Diplomatic Institute in Bucharest. His political career started in 1996 when, as a student, he became a member of the National Liberal Party. Eight years later, he entered the Romanian Parliament and, in 2013, became President of the Romanian National Health Insurance House. Since 2007, he has been elected to the European Parliament for three consecutive terms.

Speaker biographies

Alphabetical order

Terkel Andersen

Terkel Andersen was elected President of EURORDIS-Rare Diseases Europe in May 2003 and has been a member of the EURORDIS Board of Directors since 1997, when the organisation was founded.

A person with haemophilia himself, Terkel has broad experience in disability and health issues becoming involved in the rare disease field in 1983 when he joined a Nordic project on the mapping of problems related to rare diseases. Terkel served as president of the Danish Haemophilia Society 1985-2017. He was one of the founders of the Danish Alliance of Rare Disorders in 1986 and worked as the first Executive Director of the Centre for Rare Diseases and Disabilities of the Ministry of Social Affairs in Denmark from 1990 to 2001. From 1992 to 2002, he served on the executive board of the World Federation of Hemophilia; and from 1993 to 1999, he was chairman of the European Haemophilia Consortium.In his professional capacity Terkel worked with the Danish National Council for Volunteering until October 2018.

Terkel represents EURORDIS at International Conferences throughout Europe and beyond.

Diego Ardigò

Diego is currently the Global Head of Research & Development in Rare Diseases at Chiesi Group. Before this role, he was leading Chiesi's development projects in rare diseases and advanced therapies and has more than 20 years experience in medical research and more than 10 in drug development, spanning from pre-clinical to commercial phase.

Diego is an MD with a specialization in Internal Medicine. He obtained a PhD at the University of Parma (Italy) in cardiovascular pathophysiology and a post-doctoral fellowship in cardiovascular genomics at Stanford University (California, US).

Before joining the industry, he worked at the University of Parma (Italy) in the field of cardiovascular and metabolic genomics, and as free-lance consultant for various academic institutions. He joined Chiesi in 2010, where he acted as Clinical Lead in the registration of the first stem cell therapy in EU and led the cross-company team (with uniQure BV) treating the first patient with a commercial gene therapy in EU.

Diego is serving as chairman of the Therapies Scientific Committee of IRDiRC (International Rare Diseases Research Consortium), where he led the Orphan Drug Development Guidebook initiative, and is a board member of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE). He is author of 50+ indexed papers and frequent speaker at international medical and industrial congresses.

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Inês Alves

Patient expert in rare bone diseases. Degree in veterinary medicine, post-graduated in surgery. 2 years of Ph.D. studies in veterinary sciences, incomplete degree. Creator of Beyond Achondroplasia in 2013, founder and president of ANDO Portugal, the Skeletal Dysplasia National Association. Vice-president of EUPATI Portugal. EUPATI fellow and Fundamentals trainer. Patient representative at ERN BOND, the European Reference Network for Rare Bone disorders, and member of the Steering committee.

Lecturer on patient involvement in R&D. Health Parliament Portugal Deputy at Technology and Care Integration Committee. Co-chair of Patient Outcomes for EuRR-Bone, the European Registry for rare bone and mineral disorders. Patient expert for EMA and IMI; Member of the management committee and communication co-leader of COST action Gemstone, Genomics of musculoskeletal traits EURORDIS volunteer evaluator in European Joint Program for rare diseases, EJP RD, and member in the E-rare and Rare 2030 expert panel. Founder and chair of the European Rare Bone Forum (ERFB). Based in Évora, south of Portugal. Mother of 3 children, one has achondroplasia, a rare bone condition.

Catherine Berens

Dr Catherine Berens was trained as a pharmacist and obtained her PhD in Pharmaceutical Sciences from the Catholic University of Louvain in Belgium. After having worked for the European Directorate for the Quality of Medicines & HealthCare (European Pharmacopoeia, Council of Europe, Strasbourg), she joined the European Commission in Brussels in 2002, as a Scientific Officer in charge of Rare Diseases in the Health Directorate of Directorate-General for Research and Innovation (DG RTD). From 2012, she worked as a Policy Officer for Pharmaceuticals in then-DG Enterprise and Industry, dealing with pricing and reimbursement of pharmaceuticals, and access to medicines. In 2014 she was appointed Head of Sector Neuroscience in DG RTD, and from September 2016, worked as the Assistant to DG RTD's Deputy Director-General in charge of Research Programmes. She was appointed Deputy Head of Unit 'Strategy' in DG RTD's Health Directorate in 2018, and became Deputy Head of Unit 'Healthy Lives' in 2019.

Jan Blatný

Jan Blatný M.D., Ph.D., Minister of Health of the Czech Republic studied general medicine at the Faculty of Medicine of Masaryk University in Brno, where he graduated in 1994 and where he also received his Ph.D, and in 2018 he was appointed as Associate professor of pediatrics at his home university. After his studies he spent various lengths of time at internships in Switzerland and the UK. From 2006 to 2008, he worked as a physician at the Primary at the Children's University Hospital in Dublin, Ireland, being registered both in Czechia, the UK and Ireland. However, he spent most of his professional life in the University Hospital Brno, especially at its children's hospital.

His main fields of interest have always been haemophilia, life threatening bleeding, and thrombosis in children. In this regard, he has been the Coordinator of the Czech National Haemophilia Programme (the authority managing and overseeing haemophilia care in the country). He co-operates with the Perinatal/Paediatric Scientific and Standardization Committee (SSC) of the ISTH, serves on the Executive Committee of EAHAD, on the Working Group for Pharmacokinetics and Population Pharmacokinetics of Factor Concentrates at SSC for Factor VIII, Factor IX and Rare Coagulation Disorders of the ISTH, on the Working Group on Paediatric Haematology of the Czech Paediatric Society, as well as on the Steering Committee of the Czech Society for Thrombosis and Haemostasis. He is a member of Medical Advisory Group (MAG) of EHC (European Haemophilia Consortium). He has authored and/or contributed to over 90 scientific papers and books and serves as a reviewer of several scientific journals, including Haemophilia Journal. He is a member of the committees of a number of national and international professional societies with this focus.

Stefano Benvenuti

Stefano Benvenuti is Global Partnership Manager at Fondazione Telethon since 2018. He is an experienced manager of an EU funded project and an expert of EU research policy. Since 2019 he represents Fondazione Telethon in the Executive Committee of the European Joint Programme on Rare Disease (EJP-RD). After graduating in International Cooperation for Development at the University of Bologna in 2007 he started as a consultant project manager of EU funded projects. In 2010 he joined the healthcare department of Regione Veneto working as EU project specialist where he contributed to re-design the regional system of participation in EU funded programs. During this period he also completed a master degree in Health Technology Assessment at Università Cattolica in Rome. Finally, in 2016, he joined Fondazione Telethon to set-up the EU affairs office and coordinate the participation of Fondazione Telethon in European and International initiatives.

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Virginie Bros-Facer

Virginie joined EURORDIS in 2015 as Research Infrastructure Project Manager, later becoming Scientific Director. Her responsibilities include managing all EURORDIS activities related to infrastructures and technologies facilitating rare disease research. Activities include fostering patient engagement in RD research projects, registries, clinical bioinformatics, genetics and genomics as well as ethical issues surrounding this pre-clinical research.

Prior to joining EURORDIS, Virginie worked for several research funding organisations in the UK, including as Director of Medical Research for Sparks, a children's medical research charity based in London.

Virginie holds an MSc and a PhD in Neuroscience from King's College London and also worked at the UCL Institute of Neurology on several research projects aiming to develop new therapeutic strategies for motor neuron disease and other neuromuscular disorders.

Simone Boselli

A member of the European and International Advocacy team, Simone has contributed to European policy development since 2017. Specifically, he represents EURORDIS in policy discussions on access to therapies, with a focus on reducing delays and inequalities, on the underlying challenges in the field of the value assessment, pricing and reimbursement of orphan medicines, and on current initiatives towards improved access. He has contributed to the finalisation of the paper on 'Breaking the Access Deadlock to Leave No One Behind' and is engaged in finding new policy solutions for improving access to innovative therapies for people with rare diseases in the context of the European Pharmaceutical Strategy.

Simone has almost 15 years of experience in the European public affairs arena, having previously worked for two leading consultancies in Brussels and specialised in health advocacy and government affairs in particular. He has expertise in healthcare having devised and implemented advocacy campaigns at EU and national level on a range of global health issues.With a view to advance rare diseases as a public health priority at a global level, Simone also supports advocacy activities at Rare Diseases International and the further development of NGO Committee for Rare Diseases.

Kate Bushby

Professor Kate Bushby joined the pioneering department of Human Genetics at the University of Newcastle in 1989. She built on the strong tradition of neuromuscular disease research there to develop a world leading team in patient care and clinical trial design and execution including leading the TREAT-NMD network of excellence for translational research in neuromuscular diseases.

Professor Bushby subsequently worked in rare disease policy development as a Vice President of the EU Committee of Experts on Rare Diseases and via a series of Joint Actions worked on development of the policy underpinning European Reference Networks for Rare Diseases.

Following the diagnosis of her husband with a glioblastoma, Prof Bushby stood back from her career, though she is now working in a voluntary capacity with the brain tumour community, specifically the strategic programme of the Tessa Jowell Brain Cancer Mission on the development of centres of excellence for brain tumour care in the UK.

Anne Charlet

Anne Charlet is Lupus Europe's Vice-Chair and a Lupus France board member. When Anne's daughter was diagnosed with Lupus at the early age of 16, it was a natural step to get involved, which led her to these patient representative organisations.

Anne is a trilingual American, with a deep interest in the subject of cultural intelligence. She has worked in the USA, UK and Germany before settling with her family in France, where she now provides Global Marketing support for Europe's largest IT service provider. As a member of the Lupus Europe Board of Directors and representing the European Lupus community, Anne is actively involved in several work-groups, specifically in the Digital Health area, for EPF, and the Patient Think Tank for EFPIA. Living in France, Anne has taken part in several initiatives by Lupus France such a crowd-funding for lupus research, online "meet the doctor" sessions with French experts, but obviously also the broader reaching Telethon.

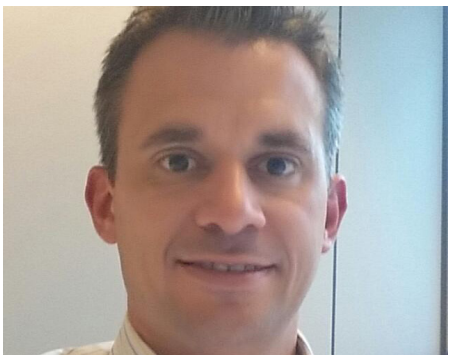
One of her main drives is to achieve better recognition of lupus throughout the world, and shorter time to diagnosis for lupus patients until we can one day live in a world without lupus.

Dorica Dan

Dorica Dan was elected to the Board of Officers in 2011 and has been a member of the EURORDIS Board of Directors since 2007.

Dorica initiated RPWA (Romanian Prader Willi Association) in 2003, established RONARD (Romanian National Alliance for Rare Diseases) through a project funded by CEE Trust in 2007 and Romanian Rare Cancers Association in 2011. She has opened the first Centre for Information about Rare Genetic Diseases in Romania and initiated the National Plan for Rare Diseases in Romania. In June 2011 she opened the Pilot Reference Center for Rare Diseases "NoRo" through a project implemented in partnership with Frambu Norway and funded by Norway Grants. She became an Ashoka Fellow in 2018.

Today she is the chair of the Romanian Prader Willi Association, Romania (RPWA); president of the Romanian National Alliance for Rare Diseases(RONARD); president of the Romanian Association for Rare Cancers and the coordinator of NoRo Center. She is also an ePAG co-chair in ITHACA.



Martin Dorazil

Martin Dorazil works since September 2019 as the Deputy Head of the Unit "European Reference Networks and Digital Health" in the European Commission's Directorate-General for Health and Food Safety. He has a background in law. He joined the European Commission in 2004 and since then he has worked on issues related to patients' rights in cross-border healthcare (DG SANCO), antitrust issues in the pharmaceutical sector (DG COMP) and legal, regulatory and policy issues related to marketing authorisations of medicinal products (DG SANTE).

Ilaria Galetti

I am vice president of FESCA, the Federation of European Sclero-derma Associations, Systemic Sclerosis ePAG representative in ERN (European Reference Network) ReCONNET, member of the DITA Task Force and member of the Panel of experts of Rare 2030, where I participated in the development of the Recommendations that aspire to improve the lives of RD patients in the next decade. I was part of the core group of Rare 2030 Italian stage that involved different stakeholders and policy makers.

I always try to collect the needs of the wider community and to interact with clinicians to improve the cure and the QoL of rare disease patients. As patient representative, I often participate in consensus meetings aiming at creating diagnostic recommendations and risk scores or in CABs for the research and the development of new potential therapies for SSc.

In ERN ReCONNET I have been involved in several activities such as in the State of the Art of CPGs and in SSc patient's care pathways, always collecting the voice of the European SSc community and giving back the outcomes of the activities.

Giovanna Giuffrè

Giovanna Giuffrè has worked with ISINNOVA since 2008, and has been a partner since 2010. Giovanna has been involved on the management of several IEE, FP7 and Horizon 2020 projects in the research and innovation, sustainability and health fields.

Through her work on scenario-building research projects, Giovanna has gained experience analysing trends and policies, facilitating workshops, and redacting policy recommendations. These projects include Foresight on Demand (FoD), BOHEMIA - "Foresight in support of future EU Research and Innovation Policy; RARE2030 - Foresight in Rare Disease Policy"; FRESHER– Foresight and modelling for European Health policy and regulation; FLAGSHIP - Forward Looking analysis of grand societal challenges and innovative policies; PASHMINA - Paradigm Shift Modelling and Innovative approaches.

In the framework of EU projects, Giovanna has researched and analysed policies in order to evaluate their impacts and effectiveness and has offered support to European and national authorities in planning and managing sustainable policies. Previous experience has included working for networks of local authorities (EUROCITIES and ICLEI) and for a network of non-governmental organisations (Medlink - Mediterranean links). Giovanna holds a Law degree from La Sapienza University, Rome, Italy and has a Masters in European Studies, College of Europe, Warsaw.

Holm Graessner

Holm Graessner has graduated in Biomedical Engineering, Electrical Engineering, German Language and Literature, Philosophy as well as Business Administration. He received his PhD "Summa cum laude" in 2004 and, then, he obtained his MBA degree in 2008.

He has been Managing Director of the Rare Disease Centre, since 2010, at the University and University Hospital Tübingen, Germany. He is Coordinator of the European Reference Network for Rare Neurological Diseases (ERN-RND). Together with Olaf Riess, he coordinates the H2020 Solve-RD project on "Solving the unsolved rare diseases". www.solve-rd.eu

From 2003 until now, he has been coordinating and managing more than 10 EU funded collaborative projects. The main focus of these projects are rare and neurological diseases, among them EUROSCA, MEFOPA, SENSE-PARK, MULTISYN, NEUROMICS and PROOF.

He has been co-leading one of the four working groups of the German Action Plan for Rare Diseases. Since 2020, as a fellow of the European Academy of Neurology (EAN) and in his function as the coordinator of ERN-RND, he is a personal member of the Rare Disease Coordinating Panel of the EAN.

Victoria Hedley

Victoria has been an active participant in the generation of rare disease (RD) policies and policy-related outputs for a decade. She currently leads the knowledge-base activities of the Rare 2030 foresight project and previously led the policy arm of RD-ACTION, the EU Joint Action for RD.

She has experience and understanding of a wide range of topics under the RD 'umbrella' and has contributed to the development of EU-level Recommendations around topics such as Cross-Border Genetic Testing; the Incorporation of RD into Social Services and Policies; Patient Registration and data collection; National Plans and Strategies; and, most prominently, European Reference Networks.

She led RD-ACTION support for the conceptualisation and implementation of ERNs and is now co-lead of the new Newcastle Centre for Rare Diseases, which she brought to fruition in 2020, and which is now consolidating the broad range of disease-focused and methodological expertise in the Newcastle region, to optimise patient-centred research and innovation.

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Clara Hervás

Clara Hervás is a Public Affairs Manager within the European and International Advocacy team of EURORDIS. In this role, she is responsible for developing and coordinating policy activities on issues that affect people living with a rare disease, such as respect for human rights, universal health coverage or the rights of people with disabilities.

Clara Hervas is also public affairs manager within Rare Diseases International (RDI) and policy coordinator at the NGO Committee for Rare Diseases, established as a platform to exchange with the United Nations. As such, she is responsible for institutional relations with the UN, its Member States, and its agencies, such as the World Health Organization (WHO).

A Spanish national, Clara Hervás holds a Bachelor of Arts in Human Geography from the University of Cambridge and a Master of Science in Global Politics from the London School of Economics (LSE) in the UK.

Kaja Kantorska

Kaja is a Policy Officer in Unit B5 dealing with Medicines: policy, authorization and monitoring at the Directorate General Health and Food Safety. Her work centers around the development of the pharmaceutical legislation and policies concerning authorization and manufacturing and use of medicinal products in the European Union, in particular with respect to orphan medicinal products.

She worked previously as Policy Officer in the Biotechnology field developing policy and legislation, as well as managing existing legislation, in relation to genetically modified organisms.

She has a university degree in Master Engineer in Biotechnology field from Lodz University of Technology in Poland.

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Kateřina Konečná

Kateřina Konečná, Member of the European Parliament. In the year 2009, she successfully graduated from Masaryk University and received an engineer's degree in the Public Administration field. She also has a bachelor's degree from Masaryk University, Faculty of Law. Her interest in the world surrounding us brought her to politics and resulted in her candidacy to the Czech Chamber of Deputies in 2002. As the youngest member of the Chamber of Deputies, she became a member of the Foreign Affairs Committee. She became a member of the Environment Committee after the elections to the Chamber of Deputies in 2006. She led the list of Communist candidates for elections in May 2010. After her re-election, she continued the promotion of practical and effective protection of the environment that will help people and will not take their jobs.

She also led the list of Communist candidates in the elections to the European Parliament in May 2014 as well as in May 2019. As a member of the European Parliament, she continues to engage in the Environmental Protection Policy and Public Health Policy as a member of the ENVI Committee. She focuses on the topic of Access to Healthcare, deals with the Public Health issues and cooperates very closely with patients and their organizations. She also supports the development of the Patients Academy project in the Czech Republic. During her mandate, she worked on the HTA regulation, Medical devices and In-vitro medical devices regulations or EU4health program. In the term 2014-2019, she was the European Parliament's sole contact person responsible for the ECDC agency, group's coordinator in the ENVI Committee and vice-chair of the EMIS Inquiry committee and PEST Special Committee. From September 2020 she is also a group's coordinator in the IMCO Committee and BECA Special Committee.

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Stella Kyriakides

On 1 December 2019, Ms. Kyriakides became the European Commissioner for Health and Food Safety. During the COVID-19 crisis, she has been leading the Commission's work to coordinate the EU's health response and support Member States to tackle the pandemic. She is responsible for the EU's Vaccine Strategy in order to procure safe and effective vaccines for all EU Member States and Europe's broader neighbourhood.

She is also responsible for the establishment of a strong European Health Union and supporting Member States to strengthen healthcare systems and deliver better patient outcomes for all EU citizens. This includes leading the reinforcement of EU health agencies, putting in place a stronger framework of cooperation against health threats, setting up a Health Emergency Preparedness and Response authority, rolling out Europe's Beating Cancer Plan to help improve cancer prevention and care, the new Pharmaceutical Strategy to ensure that Europe has enough affordable medicines to meet its needs, creating a European Health Data Space and the implementation of the new and ambitious EU4Health Programme.

On food safety, Commissioner Kyriakides is leading the new 'Farm to Fork' strategy for sustainable food, covering every step in the food chain from production to consumption. Her responsibilities also include ensuring enforcement of animal welfare laws and promoting European standards globally, as well as ensuring enforcement of EU laws on food safety and animal and plant health and leading the work to protect plant health, reduce dependency on pesticides and support low-risk and non-chemical alternatives.

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Yann Le Cam

Yann was one of the founders of EURORDIS-Rare Diseases Europe in 1997. He has been the organisation's Chief Executive Officer since 2000.

Yann initiated Rare Diseases International (RDI) in 2009. He is an elected member of the RDI Council and Chair of the RDI Advocacy Committee. He is a founding member of the NGO Committee for Rare Diseases (United Nations, New York) in 2014 and its Vice-Chair. Yann is a Co-Chair of the Global Commission to End the Diagnostic Odyssey of Children with Rare Diseases since its launch in 2018. Yann is a member of the World Economic Forum's Health Stewards Board from 2020 and of its Global Precision Medicine Council since 2019.

Recent past positions include: member of the Management Board of the European Medicines Agency (EMA) 2017-2019; Chair of the Therapies Scientific Committee of the International Rare Diseases Research Consortium (IRDiRC), 2013-2017; Vice-Chairman of the EU Committee of Experts on Rare Diseases (EUCERD), 2011 – 2013; and a member of the Commission Expert Group on Rare Diseases, 2014 – 2017; member of the Committee for Orphan Medicinal Products (COMP) at the EMA, served 9 years, two elected mandate as vice-chair for 6 years. Yann holds an MBA from HEC Paris. He has three daughters, the eldest of whom is living with cystic fibrosis.

Milan Macek

Professor Milan Macek Jr. MD, DSc is the chairman of the Department of Biology and Medical Genetics at the Charles University in Prague – the largest academic medical and molecular genetics institution in the Czech Republic. He was a past President of the European Society of Human Genetics (ESHG), currently a board member of the European Society for Human Reproduction and Embryology and of the European Cystic Fibrosis Society (ECFS).

His institute contributes to dissemination of knowledge in genetics gathered within various international European projects, such as CF Network, EuroGentest, EuroCareCF or Techgene, to Central and Eastern Europe. Prof. Macek did his postdoctorates at the Institut of Human Genetics in Berlin and at the McKusick-Nathans Centre for Genetic Medicine, Johns Hopkins University in Baltimore. During that time, he was also a fellow at Harvard School of Medicine in Boston. Prof. Macek is national coordinator of Orphanet, active member of Eurogentest, has been the chief advisor of the Czech EU Council Presidency under which the "EU Council recommendation on an action in the field of rare diseases was adopted in June 2009.

He also serves at the EUCERD committee on rare diseases and is involved in the rare disease-focused initiatives EURenOmics and RD-Action.

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Fanni-Laura Mäntylä

Fanni-Laura Mäntylä is a Mental Health Activist, a Patient Advocate and Mental Health and Substance Abuse Work Professional. Mäntyla was Chair of the 5th Edition of European Health Parliament: Committee on Mental Health and Healthy Workforce.

Speaking openly about her personal experience of work related burnout and young adulthood with severe depression, Mäntylä in her 30s is now an active influencer about mental health in her native country Finland as well as in the EU.

Being a type-1 diabetic, Mäntylä urges for more discussion about health in a holistic manner and calls for all stakeholders together on the agenda. Mäntylä is a strong voice for young people affected by mental ill-health and a living proof that issues with health do not define a person.

Nikolaos Milionis

Nikolaos Milionis has been a Member of the European Court of Auditors since the 1st of January 2014. He graduated from the Hellenic National School of Public Administration and entered the Hellenic Court of Audit as a First Instance Judge (Auditor) in 1989. He was appointed Appellate Judge (Conseiller Référendaire) in 1997 and Councillor (Conseiller-Maître) in 2004. He now holds the position of Vice-President of the Hellenic Court of Audit.

He acquired a PhD in Public Finance Law in 1998 (University of Athens), writing his thesis on 'The Institutional Role of the Hellenic Court of Audit'. He has taught at the Hellenic National School of Public Administration (1997-1998) and at the Hellenic National School for the Judiciary (1998-2012). Since 2013 he has been an Assistant Professor in Public Finance Law in Panteion University of Social and Political Sciences.

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Lucia Monaco

Lucia Monaco is the current Consortium Assembly Chair of the International Rare Diseases Research Consortium (IRDiRC), where she represents Fondazione Telethon, a charity committed to rare genetic diseases research. She is the head of Research Impact and Strategic Analysis and former Chief Scientific Officer of the foundation. She previously worked as a researcher at the San Raffaele Scientific Institute in Milan and earlier at Farmitalia Carlo Erba in Milan, Italy. She graduated in chemistry at the University of Pavia, Italy and received her training in biochemistry at the University of Iowa in Iowa City, USA and in molecular biology at the European Molecular Biology Laboratory in Heidelberg, Germany.

She is/has been member of several international committees and boards, among which: the Policy Board of the European Joint Program on Rare Diseases (EJP RD) and the Horizon 2020 Advisory Group for Societal Challenge 1 – Health, demographic change and well-being.

Antoni Montserrat Moliner

Antoni Montserrat Moliner (born in Barcelona and living in Luxembourg. Having both nationalities). He has been the Policy Officer in charge of the EU Rare Diseases policy and the EU Cancer policy in the Directorate of Public Health in the DG SANTE of the European Commission during 2005 and 2018. In the origin of the Commission Communication 2008 and the Council Recommendation 2009 on rare diseases as well as from many other EU initiatives in the fields of rare disease and cancer.

From 2018 he's a member of the Board of Directors of ALAN Maladies Rares in Luxembourg. Member of the Luxembourg Comité National Maladies Rares where he's the supervisor of the Working Group in charge of the creation of the National Register of Rare Diseases. He's also member of the Scientific Advisory Committee of the Spanish FEDER, a member of the EURORDIS Working Group on Newborn Screening, as well as a consultant for several public and private organisations on rare disease affairs.

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Christine Mundlos

After finishing medical school at the University of Mainz in 1989 Christine Mundlos first worked in cytogenetics (diagnostics) and later in molecular genetics (research). During this period (1989-1996) she spent several years living and working abroad in Australia and the USA. She moved to Berlin in 2000 with her family and was first involved in a project introducing the German DRGs (patient classification system) by Lohmann & Birkner Health Care Consulting at the Charité.

From 2005 to 2007, she completed the master's degree program on "Science Marketing and Science Communication" at the Technical University of Berlin. Since the end of 2008, she has been working for the Alliance for Chronic Rare Diseases Germany (ACHSE e.V.) at the interface between patient-organisations, medicine and research. She is the head of the ACHSE counselling service, and meanwhile the deputy managing director for the Alliance of Chronic Rare Diseases (ACHSE) e.V..

Fani Petridis

Fani is a Senior Director at the Global Patient Partnership team at Roche, which is responsible for facilitating early and systematic engagement with patients across the product lifecycle through industry-leading partnerships with patients. In this role, she leads the patient partnership strategy and engagement across rare neuromuscular diseases.

She is strongly committed to ensuring that the patient and caregiver voice is fully embedded in decision-making starting from the earliest stages of a medicine's development and throughout the whole lifecycle.

A trained economist, Fani has nearly 10 years of experience in patient engagement at a country level and internationally.

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Inmaculada Placencia Porrero

Inmaculada Placencia Porrero is a Senior Expert in Disability and Inclusion at Directorate-General Employment, Social Affairs and Inclusion at the European Commission. Her unit is responsible for the coordination of European policies for persons with disabilities. She works on European disability policies including the European Disability Strategy 2010-2020, and the EU implementation of the UNCRPD. Inmaculada holds a degree in Physics and Computer Science and worked in research and development before joining The European Commission in 1991. She has worked on research programmes addressing accessibility as well as assistive technologies and was Deputy Head of Unit for various disability-related units in the Commission.

Her work in the "e-Inclusion" unit of the Directorate-General for Information Society and Media addressed accessibility policy. While at the Directorate-General for Justice she contributed to disability-related antidiscrimination legislation. She was responsible for the Task Force for the preparation of the European Accessibility Act and remains responsible since its adoption in 2015. Currently under the Directorate General for Employment, Social Affairs and Inclusion, she is also working on the preparation of the post 2020 European Disability Strategy.

Ana Rath

Ana Rath is a medical doctor with a background in general surgery and a Masters degree in Philosophy. She oriented her career to medical information and terminologies in 1997 and joined Orphanet in 2005, where she was Manager of the Orphanet Encyclopaedia, then Scientific Director, and Director of Orphanet and Coordinator of the Orphanet network since 2014.

Ana was the coordinator of RD-ACTION, the EU Joint Action for rare diseases (2015-2018) and of the IRDiRC's Scientific secretariat until 2017. She chairs the Orphanet Rare Disease Ontology (ORDO), and was member of the WHO's ICD11 Revision Steering Committee. She currently coordinates the RD-CODE on implementation of RD codification in EU member states project and co-chairs the EJP RD Pillar 2 on data and resources ecosystem for RD research in Europe.

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Tamsin Rose

Tamsin Rose is Senior Fellow at Friends of Europe. Having studied international relations, she has 25 years of experience working across the European continent from Ireland to Mongolia.

A natural communicator, Tamsin has been a radio reporter, worked on press for the EU Delegation in Moscow and is currently a member of the external speaker team for the European Commission Directorate General for Communication, describing how the EU works and key policies to visitor groups from around the world.

Since 2002 she has specialised in public health and public participation issues, serving as Secretary General of the European Public Health Alliance (EPHA), and providing strategic advice for health groups on how to engage successfully with the EU.

Andrzej Ryś

Dr. Andrzej Ryś is a medical doctor specialised in radiology and public health, graduated from Jagiellonian University, Krakow, Poland. He founded in 1991 and ran as Director until 1997 the School of Public Health at the Jagiellonian University. Thereafter, from 1997 -1999, he served as Director of the Krakow's City Health Department. Between 1999 -2002, he continued his career as Deputy Minister of Health in Poland where he was member of the Polish EU accession negotiators team for the harmonisation of the Polish Health Care Law with the EU's Acquis Communautaire.

After becoming Senior Consultant of "Health and Management Ltd" for the World Bank (WHO) and EAR in Serbia (2002), he founded (2003) the "Center for Innovation, Technology Transfer and University Development" (CITTRU) at the Jagiellonian University, where he was Director until 2006.

In 2006, he became Director for Public Health and Risk Assessment at the Directorate-General for Health and Consumers (DG SANCO), in the European Commission. From 2011-2014, he assumed the position of Director for Health Systems and Products in DG SANCO. Since 2014, Dr. Andrzej Rys is the Director responsible for Health Systems, Medical Products and Innovation in DG SANTE.

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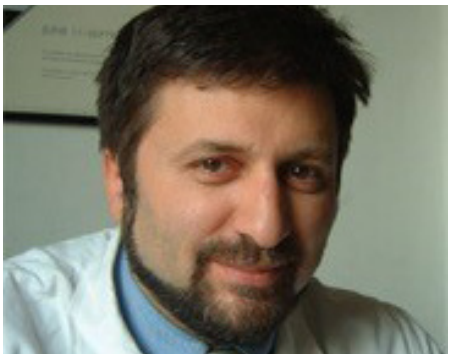

Luca Sangiorgi

Luca Sangiorgi, Director of Department of Rare Skeletal Diseases, is the coordinator of the Rare Diseases Centre of Rizzoli Ortopaedic Institute and responsible of 4 National Registers of Rare Disease (Multiple Hereditary Exostoses, Osteogenesis Imperfecta, Ehlers-Danlos Syndrome, Ollier Disease and Maffucci Syndrome).

He's the coordinator of BIOGEN, diagnos-tic and research genetic biobank, and of Telethon Network of Genetic Biobanks. He's been nominated Delegate for the European Research Infrastructure BBMRI working on Rare Dis-ease Biobanks. From March 2017, is the coordinator of the European Reference Network on Rare Bone Disorders - ERN-BOND.

He's the first author who has contributed to more than 60 articles published in impacted journals such as Nature Genetics, American Journal of Human Genetics, PLOS Genetics, Hu-man Mutation, Orphanet Journal of Rare Diseases (Index H: Scopus 25 Google Scholar 27).

David Sassoli

David Sassoli was born in Florence in 1956. During the 1970s, he graduated in political science at the University of Florence. He began his journalistic career by collaborating with small local newspapers and news agencies, before moving on to the Roman editorial office of the newspaper Il Giorno. He worked for several years as news reporter and then anchorman for TG3 and TG1, becoming one of the most notable and popular journalists in Italy. In 2007, he became deputy director of TG1.

In 2009, Sassoli joined the centre-left Italian Democratic Party. He became a Member of the European Parliament a few months later, was elected Vice President in 2014 and then President in 2019.

Rebecca T. Skarberg

Rebecca Tvedt Skarberg, (45) lives in Oslo, Norway. Her personal experience living with a rare condition comes from being born with osteogenesis imperfecta (OI), type 3. OI is a rare bone condition that affects the production of collagen. Rebecca has had approx. 100+ fractures, is short statured and uses a powered wheelchair to get around. She lives independently with her husband Knut Erik and their two cats.

Rebecca started volunteering from an early age. In the 90's she served on the board of the Norwegian OI Organization (NFOI) and also on the board of the first resource center for OI and other similar conditions in Norway (TRS). From 2007 - 2015 she served on the board of The Norwegian Federation of Organizations of Disabled People (FFO) and had many other commitments tied to that role. One of the most meaningful tasks was serving on the panel of experts for the Norwegian Directorate for Children, Youth and Family Affairs (BUFDIR) and also the project group that led to the establishment of the Norwegian National Advisory Unit on Rare Disorders (NKSD). From 2017-2019 she served on the patient board of Sunnaas Rehabilitation Hospital in Norway.

From 2014 Rebecca has worked for the Norwegian National Advisory Unit on Rare Disorders (NKSD). In 2020 she was head of the programme committee for the Rare Disease Day Norway.

Ceri Thompson

Ceri Thompson is Deputy Head of the eHealth, Well-Being and Ageing Unit in DG CNECT and working on the digital transformation of health and care, and the development of a European Health Data Space.

Prior to working on digital health policy within the Commission, Ceri worked for Eurostat, and for DG SANTE. Before joining the EU institutions in 2002, Ceri worked on global health policy at the UK's Department for International Development, for KPMG's international healthcare practice, and on epidemiological research projects in the UK and Brazil.

She has a degree in Mathematics from Durham University, UK and a Doctor of Public Health (DrPH) from the London School of Hygiene and Tropical Medicine.

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Olivier Véran

Olivier Véran is a French neurologist and politician who has been serving as Minister of Solidarity and Health in the governments of successive Prime Ministers Édouard Philippe and Jean Castex since 2020. A member of La République En Marche! (REM), he previously was the member of the National Assembly for the first constituency of the Isère department from 2017 until 2020.

Véran worked as a neurologist at the Grenoble-Alpes University Hospital. He has served as president of the Association of Hospital Assistants in Grenoble, spokesperson for the National Intersyncal of Hospital Interns, and advisor to the Departmental Order of Physicians of Isère.

Véran was first elected to the National Assembly in the 2012 elections, as a member of the Socialist Party. During his time in parliament, he was mandated by Prime Minister Jean-Marc Ayrault with a government inquiry into the regulatory framework for blood products.

In 2015, Véran resigned as an MP to compete in the 2015 departmental elections where he became a member of the regional council of Auvergne-Rhône-Alpes. In 2016, the Minister of Health Marisol Touraine appointed him to steer a committee in charge of drafting reform proposals for France's hospital financing.