EURORDIS MEMBERSHIP MEETING 2021

SHAPING THE NEXT 10 YEARS OF RARE DISEASE POLICIES: EUROPE'S ACTION PLAN FOR RARE DISEASES

#30millionreasons

SPEAKER BIOS

Vesna Aleksovska

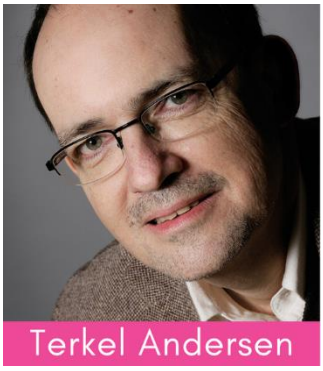
Vesna Aleksovska is a Gaucher patient with a background in journalism, NGOs, project management, and business consultancy, and a decade of experience working with patient organisations and advocating for patients' rights (through communication and cooperation with governments and international institutions). She founded Life with Challenges, an association of citizens with rare diseases (2009), and the North Macedonian National Alliance for Rare Diseases (NARDM), of which she became president in 2014 and on whose board she currently sits. She is equally a board member of the country's Alliance of Patient Organisations (APO). Other entities with which she has been involved include EURORDIS' Drug Information, Transparency, and Access (DITA) taskforce (from 2013); the European Patients' Academy on Therapeutic Innovation (EUPATI – fellow, trainer, and national ambassador since 2015); and the International Gaucher Alliance (IGA – member of the board of directors since 2014 and chairperson from 2019). Vesna strongly believes that by strengthening patient voices and raising awareness about rare diseases, we can build a better future for people living with a rare disease (PLWRD) and their families.

Kostas Aligiannis

Kostas Aligiannis joined EURORDIS in January 2020 as Public Affairs Manager – EU & National Integrated Advocacy. He is responsible for coordinating and empowering advocacy and the outreach capabilities of national rare disease patient groups (notably national alliances) by enhancing support to EURORDIS activities vis-à-vis EU institutions. He ensures both that the priorities and contributions of national alliances feed into the planning and definition of advocacy goals, and that feedback and guidance on the process to follow are regularly provided. Before EURORDIS, Kostas worked at several Brussels-based NGOs and patient organisations, focussing mostly on healthcare. His academic pursuits took him to the University of Maastricht (where he read European Studies), then onto KU Leuven (Law). He is presently pursuing a master's in Public

Health at the Free University of Brussels-VUB. As well as Greek and Dutch (mother tongues), Kostas speaks English, German, and French.

Alba Ancochea has upwards of a decade of experience and commitment in planning and implementing policies, programmes, and socio-health projects in the field of rare diseases. Since 2009, she has been actively involved with the Spanish Federation of Rare Diseases (FEDER) and its Foundation, which gathers 378 patient organisations. In her capacity as CEO (2013–), she leads a team of just under 50 people in developing around 25 specialised projects to improve the lives of PLWRD. At the international level, Alba's roles are prolific: she works for the Iberoamerican Alliance for Rare Diseases (ALIBER – volunteering as Advocacy Advisor), whose creation and activities FEDER has promoted; the Rare Diseases International (RDI) Council and Advocacy Committee; the EURORDIS Board of Directors and Council of National Alliances (CNA – representing FEDER); and the Patient Advocates Constituent Committee (PACC) of the International Rare Diseases Research Consortium (IRDiRC – representing ALIBER). In addition to all this, she has undertaken undergraduate and master's studies in psychology, special needs teaching, and NGO management.



Terkel Andersen, who himself has haemophilia, has contributed to the rare disease cause since 1983, when he joined a Nordic project mapping the problems stemming from rare diseases. In 1985, he became president of the Danish Haemophilia Society (remaining so until 2017), before cofounding Rare Diseases Denmark a year later. From 1990 to 2001, he was the first executive director of the Danish Centre for Rare Diseases and Disabilities; he served on the executive board of the World Federation of Hemophilia (WFH) between 1992 and 2002; and for six years beginning 1993, he was chairman of the European Haemophilia Consortium (EHC). He also worked with the National Council for Volunteering (Denmark) in 2018. Since its institution in 1997, Terkel has sat on EURORDIS' Board of Directors, and was elected president in May 2013, representing the organisation as such at international conferences within and beyond Europe.

Anna Arellanesová is the former and current chair, respectively, of the Czech Cystic Fibrosis Association (her child lives with CF) and Rare Diseases Czech Republic – a patient-led umbrella organisation (cofounded by Anna in 2012) which seeks to unite, represent, support, and raise awareness about PLWRD. She is also vice-chair and a spokesperson for rare diseases at the newly established Patients' Council of the Czech ministry of health, on whose working group for rare diseases she additionally sits. Together with others, she proposed legislation for an orphan drug reimbursement approval scheme to empower patients as decision-makers, and was instrumental in authoring both the Czech National Strategy and National Action Plans for rare diseases.

Astri Arnesen previously worked as an expert in the field of special educational needs, advising stakeholders in the sector from kindergarten right up to university level as a trained teacher in learning disabilities herself. She has degrees in developmental psychology, management, and innovative processes. Having also been active internationally as a patient representative and advocate since the mid-1980s (including engagement in EURORDIS and ERN-RND, which she coordinates as part of the ePAG Steering Committee), Astri was elected president, respectively, of the Norwegian and European (EHA) Huntington associations, holding the former post from 2004–14 and the latter since 2016 (after joining the board in 2010). Astri's story is profoundly personal: her mother developed symptoms for Huntington's a matter of years after the birth of her daughter and died of the degenerative disease in 2004.

Sharon Ashton joined EURORDIS in 2010 as Events Manager, later becoming Events Director. As part of the Communications & Development team, she is responsible for organising major EURORDIS events, including the biennial European Conference on Rare Diseases and Orphan Products (ECRD), the EURORDIS Round Table of Companies (ERTC) workshops, and the EURORDIS Black Pearl Awards (BPA). All told, Sharon has 19 years of experience managing medical and other international conferences. Based in the UK, she holds a BA in Marketing and French, and speaks the language.

Anne-Laure Aslanian joined EURORDIS in March 2019 as Patient Engagement Manager, coordinating and assisting eight of the 24 ePAGs established by EURORDIS to ensure meaningful advocate engagement across all ERN activities. She also heads the communication and knowledge management that supports the work of the ePAGs and facilitates the exchange of good practices and access to information. Anne-Laure has several years of experience developing international research project proposals and overseeing their implementation. Before EURORDIS, she spent four years at a hospital research institute and in a biomedicine centre in Barcelona, where she fostered the participation of researchers and clinicians in research grant opportunities and their increased collaboration in European healthcare initiatives. She holds a master's degree in European Studies and International Business Law from Aix-Marseille University, and speaks English, Spanish, Catalan, and basic Portuguese (as well as her native French).

Simona Bellagambi served the Associazione Sclerosi Tuberosa (TSC Association) as its National Secretary, representative in the international network, and head of its dedicated helpline from 1999 to 2009, and contributed to the setup of the Italian network of Centres of Reference for TSC (tuberous sclerosis complex). In 2005, she joined EURORDIS' Council of National Alliances as the delegate for UNIAMO (the Italian rare disease federation), before becoming a EURORDIS EUROPLAN advisor and being elected to the Board of Directors in 2012. Simona – whose niece lives with TSC – has also spoken at several international conferences and done much to promote and coordinate events within Italy as part of international awareness-raising campaigns, such as Rare Disease Day and EURORDIS' POLKA and RAPSODY projects.

Since 2018, Stefano Benvenuti has been Global Partnership Manager at Fondazione Telethon, whom he represents (2019–) on the Executive Committee of the European Joint Programme on Rare Diseases (EJP RD). Upon graduating from the University of Bologna in 2007 with a degree in International Cooperation for Development, he began working as a consultant project manager on EU-subsidised schemes. In 2010, Stefano joined the healthcare department of Veneto (Italy) as an EU project specialist, contributing to the restructure of the region's participation in EU-funded programmes. During this period, he also completed a master's degree in Health Technology Assessment at Rome's Università Cattolica. It was in 2016 that he finally made the move to Telethon to set up the foundation's EU affairs office and arrange its participation in European and other international initiatives.

Matt Bolz-Johnson joined EURORDIS in 2014 as Healthcare and Research Director, and is now ERN & Healthcare Advisor, having worked on two Joint Actions for rare diseases and helped mould the development and delivery of ERNs around patients through the creation of ePAGs. He put in place PACE-ERN (the Partnership for Assessment of Clinical Excellence in European Reference Networks) and put together the technical proposal for the ERN Assessment Manual and Technical Toolbox, the blueprint for all 24 ERNs. Before EURORDIS, Matt spent 14 years in hospital and commissioning trusts at the National Health Service (NHS), where he was responsible for strategy development, clinical turnaround, quality assessment, and contracting across the full healthcare spectrum. As a National Commissioner for ultra-rare diseases and highly specialised healthcare services, he advanced national networks to improve equitable access, cost efficiency, and clinical effectiveness, as well as to promote innovation for transplants, rare cancers, and paediatric, genetic, and highly specialised mental health conditions. Matt has an MA in Fine Art from UCL's Slade School. Now living in Köln, he is in the process of learning German to add to his native English.

Simone Boselli has helped shape European policy since 2017, with the goal of advancing rare diseases as a global public health priority. To this end, he supports the activities of RDI and the further development of the NGO Committee for Rare Diseases. He represents EURORDIS – as a member of its European and international advocacy team and in the context of the European Pharmaceutical Strategy – in discussions and solutions for improved access to innovative therapies, with a focus on initiatives to reduce delays and inequalities and to address the underlying challenges of the value assessment, pricing, and reimbursement of orphan medicines. Simone took part in finalising the paper *Breaking the Access Deadlock to Leave No One Behind*. In all, he has almost 15 years of experience in the European public affairs arena, having previously worked for two leading consultancies in Brussels and specialised particularly in health advocacy and governmental matters. His healthcare expertise are evidenced by the design and implementation of a range of national and European advocacy campaigns.

Valentina Bottarelli joined EURORDIS in 2007 as European Public Affairs Senior Advisor, analysing and offering strategic advice on EU policies and managing European-funded projects for EU public institutions. She also coordinates activities aimed at fostering the development of rare disease national plans in EU countries within the framework of the EUROPLAN project. On top of this, Valentina has worked as a consultant in public affairs and communications, and at the European Commission's Directorate-General for External Relations. She holds an MA in European Political and Administrative Studies from the College of Europe in Bruges, and speaks Italian, French, English, and Spanish.

Michele Calabrò works with the EU4Health Civil Society Alliance and is Policy Advisor at the European Patients' Forum (EPF), respectively co-leading (with colleagues of the European Public Health Alliance) and leading their activities in the field of digital health policy. Before joining the EPF in 2020, Michele was Policy and Communications Manager at the European Health Management Association (from 2016), where he coordinated policy, communications activities, and work relating to EU projects such as TO-REACH, which focusses on health systems research. Michele is also part of several youth health policy networks, including the Young Forum Gastein (YFG) – with whom he is a Task Force member – and the European Health Parliament (EHP) alumni group.

Marta Campabadal joined EURORDIS in 2012 as RareConnect Coordinator and currently holds the title of Patient Engagement Manager – Online Communities, RareConnect Programme Lead. She works within the communications team based in Barcelona, bringing together and engaging patient organisations and advocates from all over the world to build successful online communities. Marta runs RareConnect's social media channels while developing and maintaining rare disease communities on the platform. This helps her identify the needs of patients to ensure that the resources, services, groups, and contacts available all correspond to them. Before EURORDIS, she was employed by the Catalonian government in its internal communications and promotional activities, having graduated in Advertising and Public Relations at the Pompeu Fabra University, Barcelona. Marta's mother tongue is Catalan, in addition to which she speaks Spanish and English.

Raquel Castro joined EURORDIS in 2012. Currently based in Barcelona, she coordinates the operations, strategic development, and partnerships of the EURORDIS Open Academy and its training programmes, and is responsible for leading EURORDIS' social policy projects – a role in which she draws attention to the everyday needs of PLWRD and their carers, sharing good practices and supporting the integration of rare diseases into social services while advancing holistic and integrated care. Raquel formerly headed EURORDIS' activities within the EU-funded INNOVCare initiative, and partook in the European Joint Actions for rare diseases, contributing to the elaboration and implementation of the Commission Expert Group on Rare Diseases' *Recommendations to Support the Integration of Rare Diseases into Social Services and Policies* (2016). Before EURORDIS, Raquel designed and ran the Portuguese helpline for rare diseases, having earlier participated in development projects in Europe and India. A Portuguese national, she speaks fluent English, French, and Spanish, and holds a BA in Communication and a postgraduate degree in Project Management at the Lisbon School of Economics and Management.

After 32 years as a global finance executive with Procter & Gamble, Alain Cornet decided to repurpose his skills for the rare disease cause, becoming treasurer of CLAIR (*Contre les Affections Rhumatismales Inflammatoires*) and later of EURORDIS. He also holds the position of General Secretary of Lupus Europe and is engaged in multiple projects, including but not limited to: ERN ReCONNET ePAG; the EJP RD's WP20 on clinical trials; and the EULAR School of Rheumatology: PARE (People with Arthritis and Rheumatism).

Sandra Courbier joined EURORDIS as Patient Voices Manager in June 2015, later becoming Social Research Director – Rare Barometer Programme Lead. Paris-based, she is responsible for consulting patients and their representatives to define the direction of EURORDIS' policymaking campaigns. This is accomplished for the most part through the Rare Barometer survey programme, which seeks to collect both qualitative and quantitative data on the experiences and expectations of PLWRD and their families, in the aim of incorporating patient perspectives into EURORDIS' advocacy work. Sandra also helps crystallise patient opinions on ethical, legal, and social issues surrounding rare disease research through EC-funded projects. Before EURORDIS, she worked for the French government's information service, acquiring a wealth of experience in health-related studies and polls and in public affairs and communication. While at TNS Opinion in Brussels – the company that produces the Eurobarometer – she obtained an insight into international

survey research. A French and English speaker, Sandra holds a master's degree in survey methodology applied to political science and sociology from the Grenoble Institute of Political Studies.


Avril Daly

Avril Daly was diagnosed with retinitis pigmentosa at age 23, and has worked on the development and implementation of health policy for eye diseases since the turn of the century. Having been CEO of the Irish NGO Fighting Blindness for eight years, she now runs Retina International (a patient led global umbrella organisation which represents the voice of 43 research- and policy-oriented charities) in the same capacity. From 2006 to 2019, she was chair of Rare Diseases Ireland, and has sat on the EURORDIS Board of Directors since 2009, assuming the post of vice-president in 2012. The previous year, Avril had been appointed by the Irish minister for health to a steering committee tasked with preparing the National Rare Disease Plan for Ireland (2014). Other government bodies and projects of which she has been a part include the Irish Clinical Programme for Rare Diseases at the Health Service Executive (2014) and the Technology Review Committee for Rare Diseases and National Centre for Pharmacoeconomics (both 2019), to review orphan therapies that fail initial HTAs for statutory reimbursement. At the European level, Avril has held board positions with the EPF and the European Platform for Patients' Organisations, Science, and Industry (Epposi). She has spoken to the European and national parliaments to petition for the inclusion of patient perspectives in every element of healthcare, from concept right the way through to the delivery of diagnosis, treatment, support, and rehabilitation. A firm believer in the paramountcy of patient voices in expediting the development of appropriate medical interventions, Avril has been a board member at Health Research Charities Ireland (HRCI) and IPPOSI, the Irish Platform for Patient Organisations, Science, and Industry.


Dorica Dan

In 2011, Dorica Dan founded the Romanian Rare Cancers Association (over which she still presides) and was elected to EURORDIS' Board of Officers, four years on from joining the Board of Directors and establishing the Romanian National Alliance for Rare Diseases (RONARD) – of which she also remains president – through a project funded by the CEE Trust. Four years earlier still, in 2003, she set up RPWA, the Romanian Prader Willi Association (which she currently chairs), coordinating the opening and continued running of the country's Pilot Reference Centre for Rare Diseases (2011–) and first Centre for Information about Rare Genetic Diseases (2005–). Having also headed Romania's National Plan for Rare Diseases, Dorica was named an Ashoka Fellow in 2018, and is an ePAG cochair for ERN ITHACA.

Saskia de Vries is Head of Philanthropy and Major Donor Fundraising at the Allianz Chronischer Seltener Erkrankungen (ACHSE) e.V., which was founded in 2004 as the German umbrella organisation of and for PLWRD and their families (as represented through more than 120 patient associations).

Davor Duboka joined EURORDIS' communications team in 2018. As Web Technology Manger, he is responsible for the technical, project, and content management aspects of all EURORDIS websites, as well as for providing technical support and training on other web tools and technologies to his colleagues. Before EURORDIS, he worked as Sound Designer and IT Maintenance Manager at the Atelje 212 theatre in Belgrade, and was already involved in patient advocacy, holding the position of Executive Director at the National Organisation for Rare Diseases of Serbia (NORBS). An English, French, and Russian speaker (as well as his native Serbian), Davor holds degrees both in Automotive Engineering from the University of Belgrade's Faculty of Mechanical Engineering, and in Sound Design from the city's Faculty of Dramatic Arts. He is also a father to two boys, the elder of whom is living with Gaucher disease.

Catherine Fowler is the cofounder and a trustee of the Aortic Dissection Charitable Trust and the Patient and Public Voice representative at the NHS England Cardiac Clinical Reference Group (CRG). After losing her father to a misdiagnosed aortic dissection in 2015, she launched a petition and awareness-building campaign requesting the NHS and the UK Health and Safety Executive to implement changes across four key pillars (policy, diagnostics, education, and process). The petition currently has over 6,600 supporters and is growing daily. Catherine's tireless efforts on radio, television, and at medical conferences on a national and European scale continue to be a catalyst for positive change across the aortic dissection diagnosis and treatment pathway.

As well as being vice-president of the Federation of European Scleroderma Associations (FESCA), Ilaria Galetti is an ePAG representative for systemic sclerosis at ERN ReCONNET (with whom she has helped review clinical practice guidelines and develop sclerosis patients' care pathways), as well as being a member both of the DITA taskforce and of the Rare 2030 Panel of Experts and Italian core group that brought together different stakeholders and policymakers. Ilaria interacts with both clinicians and the wider rare disease community to ensure the needs of PLWRD are met and their quality of life improved. As a patient representative, she is actively involved in consensus meetings aimed at creating recommendations and risk scores, and in Community Advisory Boards (CABs) for the research and development of novel sclerosis therapies.

Gulcin Gumus joined EURORDIS in June 2018 as Research and Policy Project Manager to support EU-funded clinical research and research infrastructure projects, as well as assisting the Scientific Director and public affairs team on advocacy issues relating to rare disease research and policy. Before EURORDIS, she coordinated projects revolving around social entrepreneurship and education for several national and European NPOs. She is also a youth facilitator and antidiscrimination peer trainer. Gulcin studied Molecular Biology and Genetics at undergraduate level, and holds a PhD in Foetal and Perinatal Medicine from the University of Barcelona (where she worked in R&D for preclinical therapies targeting rare prenatal and childhood diseases). She speaks Turkish, English, Spanish, and some Italian.

Karolina Hanslik is a member of ABeFAO (the Belgian Association for Families Affected by Esophageal Atresia), and joined EURORDIS in 2019 as Project Senior Manager of Rare Impact. Before EURORDIS, she worked for the European Hospital and Healthcare Federation (HOPE) for seven years, then in data protection, eHealth (patient registries), HTA (helping coordinate EUnetHTA), and other policy areas at DG SANTE. Karolina studied at Sciences Po Bordeaux and at the University of Silesia's Institute of Political Sciences, as well as obtaining a master's degree in European Studies from the Université libre de Bruxelles (2001). A Polish national, she also speaks English, French, Italian, and some Spanish.

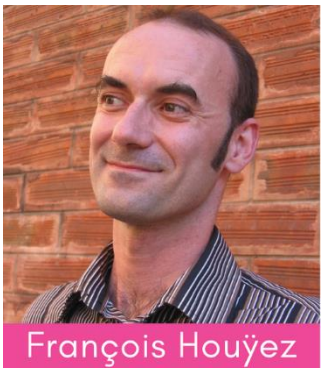
Victoria Hedley has been an active participant in the formulation of rare disease policies and outputs since 2012. She led both the knowledge-based activities of the Rare 2030 foresight project and – prior to that – the policy component of the EU Joint Action for rare diseases, RD-ACTION (after helping coordinate the preceding Joint Action of the now-defunct European Union Committee of Experts on Rare Diseases). In the above roles, Victoria has contributed to the articulation of EU-level recommendations on topics such as cross-border genetic testing; the incorporation of rare diseases into social services and policies; patient registration and data collection; national plans and strategies; and, most notably, European Reference Networks (ERNs), having headed RD-ACTION support for their conceptualisation and implementation and designed key workshops to assist them in addressing shared policy challenges. For five years, she has taken the lead in putting together the yearly *Overview Report on the State of the Art of Rare Disease Activities in Europe*. In a research capacity, Victoria is a seed member of the Rare Diseases Global Open FAIR Implementation Network (RDs GO FAIR), and co-leads both the data-related operations of connect4children and the recently established Newcastle University Centre of Research Excellence for Rare Diseases. Her efforts extend beyond campus and into the local area, where she founded the Newcastle rare diseases community to consolidate the diversity of regional disease-focussed and methodological expertise with a view to optimising patient-centred research and innovation. For her outstanding contribution to the rare disease cause, Victoria received the EURORDIS European Rare Disease Leadership Award at the Black Pearl ceremony in February this year.

Aside from being an internationally renowned in-person and virtual speaker and moderator, Gerrit Heijkoop is an expert in meeting design who has worked at the junction of events and social media for years and knows exactly how the two complement one another. As your online host, Gerrit will guarantee your ease and active participation in order to make everyone feel welcome and optimise the online communicative experience.

Inés Hernando joined EURORDIS as ERN and Healthcare Director in February 2018 to manage the monitoring of the European rare disease healthcare landscape from a policy and advocacy perspective. She supports ERNs and their establishment through her leadership of the team that oversees the 24 ePAGs ensuring patient advocate engagement across the board. Before EURORDIS, she was eHealth Senior Manager for two years at COCIR (the European Coordination Committee of the Radiological, Electromedical, and Healthcare IT Industry), where her primary concerns were with digital health – specifically, data protection, interoperability, and cross-border data flows. She also spent eight years as eHealth Coordinator at the Spanish Electronic Health Record (EHR) initiative, providing general leadership of the programme and helping integrate it into regional health authorities and the Ministry of Health. Inés holds a master's in Health Economics, Policy, and Management from LSE.

Clara Hervás works in EURORDIS' European and international advocacy team as Public Affairs Manager. She develops and coordinates policy activities pertaining to political issues that affect PLWRD globally, such as universal health coverage and respect for human rights – in particular, those of people with disabilities. Clara is also public affairs manager with RDI and policy coordinator at the NGO Committee for Rare Diseases, a platform for 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 WHO and the OHCHR. A Spanish national, Clara holds a BA in Human Geography from the University of Cambridge and an MSc in Global Politics from LSE.

Virginie Hivert joined EURORDIS in 2014 as Therapeutic Development Director. In this post, she trains and ensures the engagement of patient representatives in therapeutic development activities. As an observer on the Committee for Orphan Medicinal Products (COMP) at the EMA, she is responsible for following the development of orphan medicinal products. She is also vice-chair of IRDiRC's Therapies Scientific Committee (TSC) and coordinates the group of high-level EURORDIS representatives and volunteers who sit on the various scientific committees and working parties at the EMA (known as the Therapeutic Action Group – TAG), as well as herself being the alternate member representing patients on the Pharmacovigilance Risk Assessment Committee (PRAC). Before EURORDIS, Virginie coordinated data collection on facilities and services relating to rare diseases (such as expert centres, medical laboratories, research projects, and clinical trials) at Orphanet. A French national, she speaks English and French, and has both a PharmD and a PhD in Biological Sciences, for which she carried out basic research on pathophysiological pathways in oncology.

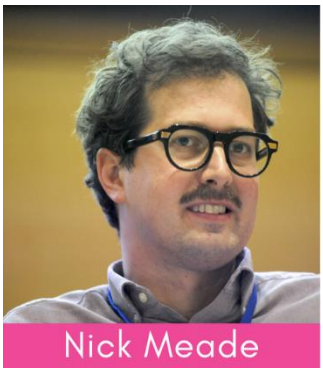


François Hoüyez is a patient and has advocated within such bodies as ACT UP, the European AIDS Treatment Group (EATG), and the EMA (with whom he pioneered such advocacy as part of the first patients' delegation engaged in dialogue with it in 1996) since the early 1990s. He began with EURORDIS in May 2003 and now works as Information & Access to Therapies Director & Health Policy Advisor, representing EURORDIS at the EMA's Patients' and Consumers' Working Party; at the Health Technology Assessment (HTA) Network; and in the CIOMS Working Group XI on patient involvement in the development and safe use of medicines. François supervises EURORDIS' Community Advisory Board (CAB) programme and the European Network of Rare Diseases Help Lines (ENRDHLs). He also collates information on trends and regularly fields questions from rare disease patients with difficulties accessing treatment (tackling such issues as marketing authorisations; health technology assessment, pricing, and reimbursement; compassionate use; shortages; and pharmacovigilance). Please feel free to contact him on any of the above topics.

Anna Kole is a public health professional with more than ten years' experience in the field of rare diseases across the US and Europe. She first joined the EURORDIS team in 2009 to publish *The Voice of 12,000 Patients*. These days, she is in charge of the two-year Rare 2030 foresight study, which employs a participatory approach to propose rare disease policy recommendations for the decade to come. Before this, she managed EURORDIS' involvement in projects and networks centred on patient registries, biobanks, and clinical bioinformatics/omics (among which RD-Connect, IRDiRC, and TREAT-NMD) in an effort to empower PLWRD and their representatives to partake in the research process. During her prior spells with Orphanet and the US' National Organization for Rare Disorders (NORD), Anna oversaw policymaking, analysis, and patient education/registries. In her free time, she organises charity clothes sales to raise money for rare disease patient organisations. A Polish and American national, she speaks Polish, English, and French, and holds a master's degree in Maternal and Child Health from the UNC Gillings School of Global Public Health.

Yann Le Cam has dedicated a quarter-century of professional and personal effort to patient advocacy in the fields of cancer, HIV/AIDS, and rare diseases through support for health and medical research NGOs in France, Europe, and the US. He has three daughters, the eldest of whom lives with cystic fibrosis, and was one of the founders of EURORDIS back in 1997 (serving as its CEO since 2001). He is also among the architects and current members of the RDI Council, and has taken part in the revision and adoption of European regulations impacting the lives of PLWRD, including the EU regulation on orphan medicinal products. He was one of the first patient representatives appointed to the Committee for Orphan Medicinal Products (COMP) at the EMA, with whom he spent nine years (six of which as vice-chair); he sat on the management board and executive committee of the French HTA agency for five years and on the DIA Advisory Committee Europe for another three; he was EUCERD vice-chairman from 2011–13, and was renominated to the Commission Expert Group on Rare Diseases (2013–16) along with seven other patient representatives. Yann is also a member and most recent former chair of IRDiRC's Therapies Scientific Committee, and was elected in June 2016 to the EMA's management board.

Elvira Martínez has been working at FEDER for a year and a half in advocacy and institutional relations and as a contact point for such organisations as EURORDIS, RDI, and ALIBER, coordinating Spain's Rare 2030 event. Before FEDER, she worked in journalism, communication, and events in Madrid, Paris, and Brussels.



Having worked there for more than 15 years, Nick Meade is currently Director of Policy at Genetic Alliance UK, a charity of over 200 patient organisations supporting all those affected by genetic, rare, and undiagnosed conditions. The alliance's policy work focusses on supporting research and innovation to facilitate progress towards cures and treatments for unmet health needs; on the commissioning of healthcare services and access to therapies; on diagnosis, including screening and genomic technologies; and on reproductive choice.

Maria Montefusco is the chairperson of Rare Diseases Sweden (2019–), a senior advisor for disability issues at the Nordic Welfare Centre, and the secretary at the Nordic Council of Ministers' disability council. With EURORDIS, she has sat on the board since 2020 and been a member of its Social Policy Action Group (formerly Social Policy Advisory Group) since 2015. Previously, she was a part of the expert group at the Swedish Infocentre for Rare Diseases (University of Gothenburg), which reported to the National Board of Health and Welfare. Maria has BAs in Political Science and Social Anthropology (Stockholm University), and a diploma in executive strategic project management from the Berghs School of Communication.

Antoni Montserrat Moliner, himself a PLWRD, was responsible for rare disease and cancer policies at the European Commission's Directorate for public health from 2004 to 2017. In this post, he spurred the creation of the EUCERD and other expert committees on rare diseases, as well as putting several of the EU's Joint Actions (on rare cancers, national plans, registers, etc.) into practice. He remains active within the directorate. In 2018, Antoni was admitted to the Board of Directors at ALAN – Maladies Rares Luxembourg. Having sat on the Steering Committee of Italy's National Centre for Rare Diseases (CNMR), he was appointed Vice-President (and acting President) earlier this year. Antoni has additionally supervised the compilation of Luxembourg's rare disease register, and is a member of the EURORDIS Newborn Screening Working Group (NBS-WG) as well as the Scientific Committee at FEDER. He has an MBA from the London School of Economics and Political Science (Essentials Course), and another from the Solvay Brussels School of Economics and Management (in Innovation and Leadership).

Cor Oosterwijk is the Managing Director of the Dutch Patient Organisation for Rare and Genetic Diseases (VSOP), which has a membership of more than 90 patient organisations and 15 professional employees. A medical biologist by education, Cor has a range of experience in the field of biomedical and clinical research, and has been a patient advocate since 2001. As such, he has participated in several national and international committees, projects, and organisations concerned with health policy, rare diseases, and genetics.

Stanislav Ostapenko joined EURORDIS this year as Communications Manager. He drafts all public communications produced by EURORDIS, as well as overseeing weekly operations to ensure that all published content (in newsletters and press releases and on websites and social media) is fully accurate, timely, and professional. Before EURORDIS, Stanislav spent two years with UNESCO as a Communications Consultant, specialising in human rights, AI ethics, and social and human sciences. He also worked at the OSCE Office for Democratic Institutions and Human Rights (ODIHR) in Warsaw and at American Councils Ukraine in Kyiv, where he created and delivered communications plans and provided support for various events. Stanislav holds a master's in European Public Affairs and Policy from the College of Europe, and speaks English, French, Russian, and Ukrainian.

As the mother of an SMA patient, Marie-Christine Ouillade has campaigned on behalf of PLWRD for more than two decades in her roles as board member at AFM-Téléthon and with such bodies as TREAT-NMD and IRDiRC. From 2015 to 2017, she was president of SMA Europe and sought to accelerate access to new therapies while improving the standard of care. She has since coordinated the organisation's biennial scientific congresses, the next of which is due to be held in Barcelona (in 2022), and was named chair of the steering committee of the recently inaugurated SMA NBS Alliance at the project's inception in March last year. Officially launched in August 2020, the alliance tasks itself with putting on events and publishing papers to advocate for SMA newborn screening, while supporting national organisations in its implementation.

Having been diagnosed with homozygous familial hypercholesterolemia (HoFH) at age four and put on the only lifesaving treatment then available, Athanasios ("Thanos") Pallidis helped found the awareness-raising Greek FH patient association in 2013 as vice-president of the board (before becoming president two years later for a three-year term). During his presidency, the board successfully advocated for a ruling to promote the admission of HoFH students to public universities. Thanos contributed a great deal to the policy paper *The Global Call to Action on FH*, and has successfully negotiated with Greece's Ministry of Health to improve the care and resources devoted to FH patients and their families and caregivers.

Matteo Scarabelli joined EURORDIS in 2017. He deals with all national and European matters relating to health technology assessment as Patient Engagement Manager – HTA, with a view to strengthening patient participation and positively influencing EU policy. Before EURORDIS, Matteo had a range of experiences: he assisted in setting up a helpdesk for refugees and asylum seekers in Paris; he then had a spell interning at the European Parliament as an MEP's assistant; and, most recently, he ran the Brussels office of an Italian think thank, where he was involved in research into HTA and staged several events. Matteo has a PhD in Philosophy from Paris 1 and the University of Milan, and speaks English, French, and Italian.

Annalisa Scopinaro has been involved in the leadership of UNIAMO since 2009, when she was received on the Board of Directors (occupying this post until 2014 as a liaison with regional representatives). In her last two years on the board, she assumed the role of treasurer, and since 2019 has been the federation's president, a job which entails chairing its governing council, coordinating its activities, and managing its employees and partner relations.

Rebecca Tvedt Skarberg is a trained social worker and international volunteer for EURORDIS and the Osteogenesis Imperfecta Federation Europe (OIFE), with degrees in psychology and counselling. Having been born with OI, she has been personally invested in disability rights, in awareness and knowledge building, and in patient equity, advocacy, and autonomy from a young age. From 2001 to 2014, she worked at the Norwegian Labour and Welfare Administration (NAV), and has since been engaged in strengthening patient involvement with the Norwegian National Advisory Unit on Rare Disorders (NKSD). In 2015, she contributed to the formation of ERN BOND (the European Reference Network on rare bone diseases) from her position on the steering committee, and was elected to the ePAG at its launch the following year. Rebecca attended the inaugural EURORDIS Leadership School in 2019 and, since 2020, has been involved in the patient participation component of the creation of the European Registries for Rare Bone and Mineral Conditions (EuRR-Bone). Throughout these two years, she also served on the Rare 2030 Panel of Experts. In 2021, Rebecca's remarkable record as a volunteer was recognised with the bestowal of the EURORDIS Volunteer Award.

Claudia Sproedt is the founder of both Cystinose-Selbsthilfe e.V. – the German patient organisation for cystinosis patients and their families (of which she was chair or vice-chair from its genesis in 1991 up to 2014) – and the Cystinosis Network Europe (which she chaired for the first three years of its existence, before becoming a trustee in 2019). Until last year, she sat on the board at FEDERG (the European Federation for Rare and Genetic Renal Diseases), and remains a board member of ACHSE e.V. (the Alliance for Chronic Rare Diseases, Germany). Claudia is also a patient representative at ERKNet (the rare kidney disease ERN) and chair of its ePAG. In 2019, her sustained efforts in the field of rare diseases were rewarded with the Order of Merit of the Federal Republic of Germany.

Jenny Steele returned to EURORDIS in September of last year, having been a member of the advocacy team in Brussels from 2015–16 and an intern in communications and events in Paris during her university year abroad two years earlier. In the intervening period, she worked as Senior Policy Lead at the UK's leading breast cancer charity, managing high-profile campaigns on a range of important policy issues from adequate workforce planning to improving guidance on commissioning. She currently lives in Moscow.

A translator and interpreter by training, Dominique Sturz embarked on her journey in patient advocacy as an ambassador for early bilateral cochlear implantation at Cochlea Implant Austria in 1997, after her infant daughter was identified as deaf and treated with said procedure. She was chosen (2005) as a delegate to the European Association of Cochlear Implant Users (EURO-CIU), and in the same year further examinations culminated in diagnoses not only of Usher syndrome, but also of retinitis pigmentosa (RP). Subsequently, with the support of the Austrian ENT Society, Dominique spearheaded a multidisciplinary approach to early diagnosis of Usher syndrome through immediate genetic testing upon detection of an irregularity in the newborn screening process. She also cofounded and initially vice-chaired the national Forum for Usher Syndrome, Hearing Impairment, and Deafblindess (2016), and has led the Usher Initiative Austria since 2014. Her years of activism have allowed her to build a strong knowledge base, and she is accordingly a registered patient expert for Usher syndrome and other hearing disorders at the EMA. Her work in the field of visual impairment brings together her daughter's two conditions: as well as being involved with ERN-EYE ePAG, she is a part of Retina International's Usher Syndrome Special Interest Group (RI-USH SIG). With EURORDIS, Dominique has graduated the summer, winter, and digital schools, and volunteered in its taskforces.

Currently, she is a member of the core team at EUPATI Austria and vice-chair of Pro Rare Austria, the country's national alliance.

Elizabeth Vroom, the mother of an adult son with Duchenne muscular dystrophy (DMD), is the founder and president since 1995 of the Duchenne Parent Project Netherlands (DPP NL), and the cofounder and current chair of the World Duchenne Organization. She serves on multiple national and international advisory boards concerned with care, research, ethics, regulatory issues, and the development of new medicines (for example, as chair of the ethics board and a member of the executive committee of TREAT-NMD); she works with the EMA as a patient expert; she trains fellow advocates and experts as a volunteer at EURORDIS' annual Summer School; and she has participated in a number of EU-funded projects, including ASTERIX, ADAPT SMART, RD-Connect, VISION-DMD, Share4Rare (S4R), and BIND. Elizabeth is also involved in several publications revolving around standards of care and drug development for DMD (looking at outcome measures, biomarkers, trial design, data usage, etc.), and has co-authored a handbook on the disease's psychosocial aspects.

Geske Wehr is General Secretary of the EURORDIS Board of Directors (to which she was elected in 2012), the chief executive on a voluntary basis of the European Network for Ichthyosis (whose establishment she spearheaded from 2004–10), and a member of the Council of European Rare Disease Federations. Since 1997, she has been involved with the German patient association Selbsthilfe Ichthyose e.V. as co-organiser of their annual meetings (2000–), on the board of directors (2001–6), and in supporting claims to health authorities and insurance companies (present). Geske has also been a member of the board of directors at ACHSE e.V. for a number of years, and has three children, the eldest of whom lives with autosomal recessive congenital ichthyosis.

Ariane Weinman joined EURORDIS in 2004 and works in public affairs, on European projects which EURORDIS either leads or in which it is a partner, such as RD-ACTION. In particular, she supports ePAGs in the four ERNs associated with rare cancers (EURACAN, PaedCan, EuroBloodNet, GENTURIS), as well as the efforts of patient advocates in both implementing EU integrated national plans for rare diseases, and in incorporating rare adult and paediatric cancers in all national plans for cancer control. Ariane has considerable knowledge of international medicine, public health, and research, which are recurring motifs in her professional experience at such institutions as the Hospital Federation of France and the scientific department of the French embassy in China. She has an MA in International Policy Studies from the then-named Monterey

Institute of International Studies, and speaks French, English, and Mandarin Chinese.

Lenja Wiehe joined EURORDIS in resource development in 2014, and currently manages nine of the 24 ePAGs as well as devising the content for the EURORDIS Leadership School. As such, she belongs to a team responsible for engaging patient organisations in the development, programming, and evaluation of ERNs. Before EURORDIS, she was involved in several community health projects, racking up experience in health systems management while at the German Society for International Cooperation (GIZ) and with the WHO's emergency humanitarian action unit in Indonesia, UN Women's monitoring and evaluation unit in India, and the Women and Health Alliance (WAHA) International in France. Lenja has master's degrees from the University of Bonn (Southeast Asian Studies and Political Science) and the EHESP French School of Public Health (Public Health), and speaks German, English, French, and some Spanish.