EURORDIS GENERAL ASSEMBLY
22 MAY 2024

CANDIDATES TO THE BOARD OF DIRECTORS' ELECTION

ANNA ARELLANESOVÁ, RARE DISEASES CZECH REPUBLIC

I am the chair of Rare Diseases Czech Republic, a Czech patient-led umbrella association for rare diseases. Rare Diseases Czech Republic's mission is to unite, represent interests and support its members – patient organizations and individual persons with RDs in the Czech Republic and to strengthen awareness about rare diseases among experts, public and officials.

I am also a member of the board and former chair of the Czech Cystic Fibrosis Association. Shortly after my child was diagnosed with cystic fibrosis, I became actively engaged in the community. In 2012, together with other patient organization representatives, we founded Rare Diseases Czech Republic.

Being in the role of a vice-chair of the newly - established Patient Council of the Ministry of Health, where I represent rare diseases, I was able to participate in the creation of proposed new legislation for an orphan drug reimbursement approval system, one which counts on the active participation of patients as decision makers. I am also a member of the interdepartmental working group for rare diseases at the Ministry of Health. Thanks to our long-term cooperation with Czech RD experts, we work together on improving timely diagnosis and access to multidisciplinary care within the centers of expertise that are part of ERNs.

I have a bachelor's degree in humanities from Charles University and live in Prague.

I am fully aware that international cooperation is crucial for RD community as it was proved during the Czech presidency in 2022, during which we presented a Call to Action to have a European Action Plan for Rare Diseases which was supported by over 20 MS. That is why I would like to continue to offer my experience, skills, and time to help as a member of the Board of Directors of EURORDIS.

SIMONA BELLAGAMBI, UNIAMO – RARE DISEASES ITALY

My niece Alice was born with Tuberous Sclerosis 26 years ago and this is exactly the time of my involvement in Rare Diseases. Helping her and my sister to find answers to the questions they had about living with a rare disease was my first commitment that evolved to all members of the Italian TSC Association that I joined to serve as national secretary and representative in the international network.

I was also in charge of the dedicated help line service and contributed to the set-up of the Italian network of Centres of reference for TSC. I firmly believe in a global approach to face the common needs of all rare disease patients and families thus I also offered my collaboration to the Italian Federation for Rare Disease- UNIAMO- when I became the organisation's representative in the EURORDIS Council of National Alliances and international relations. In 2012 I was elected for the first time to the EURORDIS Board of Directors.

In these years I've represented EURORDIS and UNIAMO at several national and international conferences and in the drafting of documents and position papers contributing in bringing in the voice and the perspective of RD patients on different issues as newborn screening, undiagnosed conditions, inequality in access to treatments, the engagement of patients and the importance of having a holistic care approach that includes the social aspects.

I also served as EURORDIS Advisor to both rounds of EUROPLAN national conferences and RD-ACTION Workshops, which were aimed at the development and implementation of national rare disease plans, supervising Spanish, Greek and Italian Conferences gaining knowledge on the broader scenario and at the same time of the different national health systems.

Over these years of service, I have seen the growth of this organisation and of its the areas of action. It was natural for me to get engaged in those activities where I could give my contribution to EURORDIS and its members.

In the last 6 years I've been appointed as Board Member to the Rare Disease Day for which I was also involved in its strategic review aiming at reinforcing the engagement across key audience, strengthening our worldwide community and better addressing the challenges that the evolution from international to global campaign has brought in. I truly believe that, increasing the visibility of RDs and their impact in the daily life of PLWRD and families is the crucial step to advocate for the rights of PLWRD through a common message adapted at national level to fit in national agendas. Furthermore, I deem that the increased visibility gained played an important role in achieving the first UN Resolution on the right of PLWRD and their families in 2021.

As member of the Panel of Experts, I was involved in the enriching adventure of the Rare2030 Foresight study where through a consultative multi-stakeholder process the desirable scenario for a new policy framework was clearly indicated.

Within its Recommendations, the diagnostic pathway, and more precisely, reducing the time to an accurate diagnosis and the inequalities in accessing the NBS across EU countries, has become my main commitment also based on the experience of the Italian NBS Programme . With the aim of contributing to achieving this goal, I've joined the EURORDIS Working group on Newborn Screening. Now this WG is also involved in the Screen4Care Research Project exploring the use of genetic newborn Screening and digital technologies where I'm member of the Patient Advisory Board: It's a challenging project that involves many aspects as ethics,

use of Artificial Intelligence and of secure personal data management where I'm aware of the responsibility to represent our perspective.
I've been also member of the Task Force on Undiagnosed conditions in the SOLVE-RD Project and I'll continue my involvement in the just started JA JARDIN on the integration of ERNs in the National Health Systems.
After years of involvement in the activities of EURORDIS, I increasingly believe in the strength of EURORDIS and its capacity to be the European voice to the common needs of people affected by rare diseases. The voice of people with rare diseases and their families must be heard even louder at this time in order to keep Rare Diseases a public health priority on the agenda of the new European Parliament and Commission and to harmonize policies at national level.
I would like to continue to contribute to our cause by putting the experience gained over these years of participation in specific activities and projects, at the service of EURORDIS and its members with the aim of contributing in reaching concrete advancements in all fields for the benefit of all PLWRD and their families
I therefore ask you to trust me and vote for me

AVRIL DALY, RETINA INTERNATIONAL, IRELAND

My name is Avril Daly, I live in Dublin, Ireland and I am a person living with the rare disease, Retinitis Pigmentosa or RP as it is also known. RP is an inherited retinal degeneration (IRD) that causes night blindness, tunnel vision and results in severe vision impairment. Over three hundred genes have been identified that are known to cause this condition. In 2017 I received a genetic test and learned that my condition is dominantly inherited but is de novo in me, this means I am the first in family to have this condition.
I began working in patient advocacy on June 12th, 2000, as Public Affairs Executive at the Irish charity Fighting Blindness. My role focused on advocating for better infrastructure and supports to foster medical research in rare eye disease in Ireland. In 2009 I was appointed CEO of the organisation and in 2016, I moved to Retina International taking on the role of CEO.
Retina International is a global patient-led umbrella group dedicated to the promotion of research into rare and inherited retinal degenerations and acts as a voice for patients on policy issues that affect them. My work has allowed me the privilege of working with patients, health care providers, basic researchers and social scientists from many countries to find ways to demonstrate the significant impact and high unmet need faced by people who live with rare eye diseases. Retina International is a supporting partner of the ERN-EYE and prioritises European collaboration across stakeholder groups.
I have been President of EURORDIS-Rare Diseases Europe since November 2022. I was first elected to the EURORDIS Board of Directors in 2009 and served as Vice-President of EURORDIS from 2012 to 2022. At home in Ireland, I am a board member of Rare Disease Ireland, the Irish National Alliance for Rare Diseases, and I am working with them in the development of a new Irish National Plan for Rare Diseases. I am currently representing patients on the

implementation committee for the Irish Genes and Genomics Strategy, under the auspices of the Irish Health Service Executive (HSE).
Ultimately, I am a person living with a rare disease. I have members of my immediate and extended family affected by different rare diseases, disabling, life changing and life limiting. I understand what it is like to live with a degenerative condition that many people have never heard of and do not understand. I know what it is like to struggle to find a diagnosis and the challenge we face to be heard.
I am passionate in my belief that the patient voice should be central in all decision-making processes that affect us. I believe strongly that rare disease patients, their parents, families and loved ones are the true experts in understanding the challenges and needs of each condition and can, if truly listened to, find solutions to address the challenges faced every day. As patients with must be listened to, and with thousands of diseases, diverse need, cultural, linguistic, and political barriers, we must continue to work together to raise our voice to demonstrate the significant and high unmet medical need that still exists in our community.
While we strive to support research that has the potential to bring about treatments and cures, we must also consider the day to day lives of patients and those who care for them. We need to find ways to convey the support needed by people living with rare disease from when they get up in the morning until they go to bed at night and why that must be valued and understood by society at large. We need to continue to work with all stakeholders, including ERNs, in the development care pathways to deliver end to end care including early detection, diagnosis, care, mental health supports, rehabilitation, respite and treatment.
While there is much political support for a new framework for Action on Rare Disease in Europe, challenges and uncertainties lie ahead. Maintaining our structured approach to collaboration will allow this community to ensure that rare disease remains a public health priority in Europe.
If I am elected to the EURORDIS Board, I will work to ensure that our voices are heard and are stories listened to, so that we can help to lead the change needed in Europe to address our collective challenges, achieve our shared goals and leave no one behind.
Thank you!

JOHAN P DE GRAAF, NEDERLANDSE HYPOFYSE STICHTING (DUTCH PITUITARY FOUNDATION) THE NETHERLANDS

The diagnosis of a rare disease, involving a tumor in the hypothalamic and pituitary region, changed my life. Though I was unaware of the fact that having this disease was already affecting my health much earlier, most probably since my early adulthood. I needed quite some years to accept and even embrace my disorder, it's now part of my life and it has enriched my life as well!
Before my diagnosis I worked at a large international bank in several functions in the field of auditing, controlling and financial management accounting. Due to the illness I lost my job, but the bank gave me the opportunity to get socially engaged and I joined the Dutch Pituitary

Foundation in 2012. In 2015 I became the chair of this organisation, and together with a great board and numerous volunteers we saw an increase of almost 60% in membership. With over 2.300 members and counting we are recognised as a large patient organisation.
In the last few years I've attended a number of courses to gain useful knowledge to support my new career as a patient advocate. I have attended several European educational opportunities such as the European Patients' Academy on Therapeutic Innovation (Eupati), the Eurordis Open Academy School on Medicines Research & Development, the Open Academy School on Scientific Innovation & Translational Research and Leadership school.
In 2017 I joined the European Reference Network on Rare Endocrine Conditions (Endo-ERN) as steering committee member of the main thematic group Hypothalamic and Pituitary disorders and also several project Work Packages. In addition to my activities within Endo-ERN, I'm involved in the European Registry for Rare Endocrine Conditions (EuRRECa). Endo-ERN provided me with the opportunity to conduct research on the patient's unmet medical and social needs and quality of life. This has led to several scientific publications; something I never even had thought about before getting a diagnosis of a rare disorder.
Since 2019 I am a member of the scientific evaluation committee of the European Joint Program Rare Diseases (EJP RD) and I assess proposals for scientific research. In 2020 I was appointed to the patient expert board of the Brain Foundation Netherlands. This board assesses proposals regarding scientific brain research from the patient perspective and provides the Brain Foundation with advice such as recently on the needs of different groups of brain patients. Since December 2022 I'm involved in the Health Technology Assessment (HTA) Taskforce of Eurordis. This Taskforce advises Eurordis on all aspects regarding HTA policies and procedures. In January 2023 the European Endocrine Society (ESE) created a Patient Advocate Board, and I was invited to be one the first three co-chairs. This gives patients the opportunity to prioritise important matters on the agenda of the ESE. I initiated a project with the ESE mapping endocrine medicine shortages throughout Europe which is currently in progress.
I would be honored to join the Eurordis Board of Directors and contribute to your vision of better lives for people with a rare disease. I am genuinely enthusiastic about the volunteering work that I am able to do and I get energy from working with others and I always look for connections that may not be that obvious at first glance. Especially in the field of rare diseases being able to see what there is outside your own rare disorder bubble is key, since numbers are small and I never stop to enjoy meeting new people and finding new possibilities to work together.
I usde to work within an international bank, but it took a rare disease to really get to know the world. And to be honest: working for and with rare disease patients gives me much more energy than any previous career.

TETIANA KULESHA, NGO "RARE DISEASES OF UKRAINE", UKRAINE

Chairman of the Board of the NGO "Rare Diseases of Ukraine", co-founder of the "Orphanni Synytsi" foundation. I was born in Kyiv. I graduated from Borys Grinchenko Kyiv University. I

teach English at one of the capital's schools. For over 15 years I have combined my teaching career with public activism. It all started with my personal story, when my son Dmytro at 11 years old was diagnosed with a rare genetic disease - mucopolysaccharidosis. The diagnosis fell into the category of rare diseases, which was not treated in Ukraine at that time due to the high cost of lifelong treatment and the lack of medicines. Doctors predicted that my son would not live past 18, and then I decided to change the healthcare system. I communicated with patients with similar diagnoses from other countries, learned about the laws that work for them, and what documents need to be developed for Ukraine. For many years, I organized rallies under the Ministry of Health and the Cabinet of Ministers of Ukraine demanding attention to the problems of children with rare diseases. I initiated the creation of the NGO "Rare Diseases of Ukraine". Thanks to the wide publicity of activists and families where children with rare diseases were found, a law was passed in 2014, according to which orphan patients would be provided with medicines by the state for life. Also, a list of rare diseases registered in the country appeared in Ukraine, which includes about 300 positions. Currently, together with my team, I actively participate in the implementation of the Concept of Development of the Healthcare System for Patients with Rare (Orphan) Diseases for 2021-2026 approved by the government, which includes the introduction of expanded neonatal screening (already operational), the creation of a network of reference centers, and the integration of Orphanet into the Ukrainian eHealth system. As a mother of a child with a rare disease, I'm sincerely convinced that an incurable illness does not cancel life. The motto of the NGO, which I have been leading since 2014, is simultaneously our main goal: "Rare patients are the priority of the state." Our daily work is aimed precisely at this - to do everything in our power and even more so that the needs of patients with rare diseases are prioritized, and the patients themselves have access to effective treatment and a full life.

ALEXANDRE MEJAT, AFM-TÉLÉTHON, FRANCE

Personally, affected by a rare neuromuscular disease called Bethlem myopathy, I grew up in a family implicated in the French Telethon organization since 1987. For these reasons, it naturally made sense to dedicate my energy and career to rare diseases. I was previously member of the Board of Directors of the Institute for Stem cells research (I Stem, Evry, France. From 2005 to 2017) and AFM-Telethon (Evry, France. From 2010 to 2017).

In parallel, being a PhD in molecular and cellular biology by training, I have been working on neuromuscular junction defects and Emery Dreifuss muscular dystrophy in France and USA and I led a research group in Lyon during 8 years before joining AFM-Telethon in 2018 to manage International Scientific Networks. During the last 7 years, I have been involved in several international networks and consortia such as the European NeuroMuscular Center (ENMC), the ERN-EuroNMD, European Joint Program on Rare Diseases (EJP-RD) and the International Rare Diseases Research Consortium (IRDiRC) trying to secure a strong

environment of collaboration and innovation. I am also accompanying AFM-Telethon patient representatives in several neuromuscular disease federations such as World Duchenne Organization, SMA-Europe, FSHD Europe or Euro-DyMA; by providing training and support to develop their empowerment.

In May 2018, I was honored to be elected to the Board of Directors of EURORDIS. Since then, I have been more than impressed by the common efforts of staff and volunteers to promote collaboration, build a strong advocacy and empower rare disease patients. During my two mandates, I ensured that Patient rights were reinforced in the European Health Data Space legislation, promoted the genetic diagnosis and more particularly newborn screening by representing EURORDIS in Screen4Care and was involved in the definition of the future European Rare Diseases Research Alliance (ERDERA).

In the meantime, an ambitious strategy was defined to draw a vision changing the European and international rare diseases landscape by 2030. Thanks to your renewed trust, I really want to dedicate my enthusiasm and conviction to make this vision a reality.

STEFAN ŽIVKOVIĆ, NATIONAL ORGANISATION FOR RARE DISEASES OF SERBIA (NORBS), SERBIA

I am, Stefan Živković currently serving as the Project Coordinator at the National Organisation for Rare Diseases of Serbia (NORBS), and the director for Foundation NORBS Plus, a charity organisation dedicated to raising funds for procurement of much needed medical and technical devices for those living with rare diseases in Serbia.

With an educational background in Political Science and International Relations, coupled with extensive non-formal education and training, my career has spanned over various roles within civil society organisations in Serbia and Europe, before joining NORBS. I have also had the privilege of completing a traineeship at the European Parliament, which enhanced my understanding of policymaking at a European level.

My journey into the rare diseases is deeply personal, driven by my younger sister's battle with cystic fibrosis. This has not only fueled my commitment but also equipped me with firsthand insights into the challenges faced by families dealing with rare diseases. My involvement with EURORDIS has been extensive: from being a part of the Rare 2030 Young Citizens, attending Summer School on Medicines Research & Development, and engaging in the Open Academy School on Scientific Innovation & Translational Research, to participating in Training for patient representatives and advocates on leadership and communication skills. I have also represented our community at the Brussels Rare Disease Week, both online in 2021 and in-person in 2023, and

As a candidate for the EURORDIS Board of Directors, I aim to leverage my personal experiences and professional expertise to further the mission of EURORDIS. My goals are to empower young patient advocates, strengthen patient organisations, and ensure that the voices from non-EU countries, particularly from the Western Balkans, are heard and valued in shaping the future of rare disease policy and research in Europe.

Thank you for considering my nomination. I am eager to contribute more actively to EURORDIS and help improve the lives of those affected by rare diseases across Europe.
