EURORDIS

Black Pearl

AWARDS

ONLINE

TUESDAY, 8 FEBRUARY 2022

FROM 18:00 CET

CONTENTS

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We don’t expect patience from patients.

When you are affected by a rare disease, patience is not just a virtue, it’s a necessity. Waiting to receive the right diagnosis. Waiting to find the right treatment. Always waiting.

At Janssen, we are changing that. We are driven by the belief that ‘patients are waiting’ and there is no time to waste. We focus on areas of medicine where we can make the biggest difference, including rare diseases.

We’re advancing the science and chasing down breakthroughs in a variety of rare diseases such as pulmonary arterial hypertension (PAH), AL amyloidosis, myasthenia gravis, rare inherited retinal diseases, rare maternal-foetal diseases, and rare blood disorders and cancers.

At Janssen, we never stop working towards a future where disease is a thing of the past.

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EM-86780
January 2022

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FOREWORD

EURORDIS-Rare Diseases Europe is proud to be bringing this exceptional 11th edition of the EURORDIS Black Pearl Awards to you online! Whilst we are still navigating the effects of COVID-19, we are delighted to be able to gather virtually to spark new conversations and continue celebrating those making a real difference for people living with a rare disease, at one of the key annual events for our community.

Persons living with a rare disease are being recognised more and more as a priority worldwide. Rare diseases are featured on the agenda of the upcoming Trio of EU Council Presidencies, held by France, Czech Republic and Sweden between January 2022 and June 2023. On an international level, the United Nations' adoption of a Resolution on Addressing the Challenges of Persons Living with a Rare Disease in December 2021 also creates the enthusiasm and opportunity for new ambition in Europe and in countries around the world.

The fourth high-level meeting of the NGO Committee for Rare Diseases will be held at the 2022 World Expo in Dubai on Rare Disease Day (28/02) and will be the opportunity to discuss how key global intergovernmental commitments touching rare diseases are crucial to raise awareness and visibility, and must trickle down to the regional and national levels.

The 11th European Conference on Rare Diseases and Orphan Products (ECRD), taking place on 27 June - 1 July 2022, will be a critical opportunity for all stakeholders to consider how to transform the concluding recommendation of the Rare 2030 Foresight Study for a new European policy framework on rare diseases into a proposal of concrete actions, ultimately creating the ecosystem required to address the unmet needs and persisting inequalities across Europe.

This evening, we will hear about several inspiring stories of individuals, organisations and companies whose hard work has created significant advancements in the rare disease community. The brand new award category for Social Media has highlighted the inspiring commitment shown by our three incredible finalists to advocate for people living with a rare disease online, and has already gathered tens of thousands of votes worldwide.

For the first time in the event's journey, our Master of Ceremony will interact live with our Supporters Wall comprised of our amazing awardees, representatives of National Alliances, our Black Pearl Committee Members and our Corporate Donors, whom we sincerely thank for their vital support. We also take this opportunity to thank the event's Honorary Patrons for their commitment to the rare disease cause, as well as all our individual donors and attendees. Let's all come together to honour and celebrate those who are making our community proud!

Terkel Andersen
President, EURORDIS

Yann Le Cam
Chief Executive Officer, EURORDIS

WELCOME

EURORDIS-Rare Diseases Europe, our Honorary Patrons, and our Black Pearl Committee Members are pleased to welcome you to the eleventh EURORDIS Black Pearl Awards.

Terkel Andersen
EURORDIS President

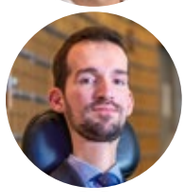
Yann Le Cam
EURORDIS Chief Executive Officer

HONORARY PATRONS

Frédérique Ries
Member of the European Parliament (Belgium)

Kateřina Konečná
Member of the European Parliament (Czech Republic)

Brando Benifei
Member of the European Parliament (Italy)



Stelios Kympouropoulos
Member of the European Parliament (Greece)

David Lega
Member of the European Parliament (Sweden)

Dr Véronique Trillet-Lenoir
Member of the European Parliament (France)

Philippe Leglise-Costa
Permanent Representative of France to the EU

H.E. Edita Hrdá
Ambassador Extraordinary and Plenipotentiary
Permanent Representative of the Czech Republic to the EU

H.E. Marcos Alonso Alonso
Ambassador Permanent Representative of Spain
to the European Union

H.E. Pavel Klucký
Ambassador of the Czech Republic to the Kingdom of Belgium

Michelle Muscat
Former First Lady of Malta

BLACK PEARL COMMITTEE

Black Pearl Committee Chair

Her Highness Princess Anne de Ligne

As Chair of the Black Pearl Awards Gala Committee, I am happy that we are able to come together, even if behind our screen.

We are proud of all our 2022 awardees and all that they do to help our community. I would also like to give thanks to awardees of previous years; it is thanks to them that we have such a strong network, allowing us to help each other.

I am also grateful to the EURORDIS team: their efforts to bring our big family together today.

Anders Olauson
Black Pearl Committee Vice-Chair & Chair of the NGO Committee for Rare Diseases

Paola Ricci
Black Pearl Committee Vice-Chair & Founder of The PHARI Fund of the Fondation Philanthropia

COMMITTEE MEMBERS

Alexis Arzimanoglou
Co-chair of the ERN
Coordinators Group

Nathalie Furrer
Director of Programmes
and Operations at Friends of Europe

Rodrigue Laurent
Press Officer

Simona Bellagambi
Member of the EURORDIS Board
of Directors & International Relations
Representative at UNIAMO

Marlene Haffner
Principal and Founder
of Haffner Associates, LLC

Mark Rothera
President & CEO
at Silence Therapeutics

Reem Boustany
Member of the French Brussels Bar

Claudia Hirawat
Executive Chair of VOZ Advisors

Bruno Sepodes
Vice-Chair of the Committee
of Medicinal Products for Human Use
at the European Medicines Agency

Robert Blum
President & CEO at Cytokinetics

Stéphanie Hoffmann-Gendebien
Senior Biotech Executive

Violeta Stoyanova-Beninska
Chair of the Committee for Orphan
Medicinal Products at the European
Medicines Agency

Hélène Dollfus
Chair of the ERN
Coordinators Group

Daria Julkowska
Scientific Coordinator of the
European Joint Programme
on Rare Diseases

Geske Wehr
General Secretary of the EURORDIS
Board of Directors & Chair
of the Board of Directors at ACHSE

MASTER OF CEREMONY

Dr Carrie Grant MBE (hc) is a BAFTA award-winning broadcaster, vocal coach and advocate. Her TV and music career has spanned over 35 years. She was awarded a MOBO award in 1998 and a BASCA in 2008 for her lifetime services to the music industry. She has been awarded an MBE in 2020 for services to Music, Media and Charity and also has two honorary doctorates (University of Bedfordshire 2018, Middlesex University 2020).

She has both the biggest selling vocal coaching book and online course in the world. She is currently a reporter for BBC's "The One Show," hosts a podcast for the elderly, "In Good Company" and co-hosts Radio London's Saturday Breakfast Show alongside her husband, David Grant MBE. Carrie is President of the Unite Union for Community Practitioners and Health Visitors (CPHVA) as well as being the Patient Lead for the College of Medicine and an Ambassador for Crohn's Disease; Colitis UK, The National Autistic Society and The Diana Award.

She is Mum to four children, three are birth children, one was adopted and all have special needs. Carrie is a keen campaigner and contributes to many forms of media on the subject of health and education.


1896.
That’s how long we’ve been improving lives.
A tradition of advancing science and medicine.
Then, now and in the future.
Roche

A special thanks to our

BLACK PEARL 2022 HONORARY CHAIRPERSON

Dr Ewa Kopacz

Vice-President of the European Parliament

Dr Ewa Kopacz was born in Skaryszew, Poland in 1956. Before her political career, Kopacz graduated from the Medical University of Lublin and worked as a pediatrician and general practitioner.

Kopacz became a member of the Civic Platform in 2001 which she chaired from 2014 to 2016. Kopacz was first elected to the Polish Parliament in 2005, where she became the head of the Health Committee. Since then she has held many positions of esteem such as being the first woman to serve as the Marshal of Sejm in 2011 and the second woman to serve as Prime Minister of Poland from 2014 to 2015. Kopacz has served as Vice-President of the European Parliament since her election in 2019.

Tribute to David Maria Sassoli 1956 - 2022

President of the European Parliament

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. During his mandate he helped steer EU legislature through landmark climate legislation, historic budget talks and the first years of the coronavirus pandemic.

Throughout these unprecedented times, we are particularly grateful for his repeated calls for cooperation and collaboration across countries, sharing knowledge and strengthening healthcare systems for the benefit of patients.

Sassoli has been a true friend of the rare disease community and more specifically of the Black Pearl Awards, taking part as Keynote Speaker at the event's 9th edition and serving as Honorary Chairperson at last year's ceremony.

Our thoughts are with Sassoli's wife, Alessandra, and their two children, Giulio and Livia.

PROGRAMME

18h00 - 19h30

Welcome by Carrie Grant

Welcome Speech
Yann Le Cam, *EURORDIS Chief Executive Officer*

Honorary Chair speech
Dr Ewa Kopacz, *Vice-President of the European Parliament*

EURORDIS Members Award
Childhood Cancer International-Europe

EURORDIS Volunteer Award
Graham Slater

Keynote Speech
Clément Beaune, Minister of State for European Affairs

European Rare Disease Leadership Award
Dr Anne-Sophie Lapointe

Performance by Chloe Temtchine

Policy Maker Award
Prof. Hans Georg Eichler
Association of Austrian Social Security Bodies

Rare Disease Day official video

Social Media Award
Awardee announced live

Media Award
Ewen Life

Performance by Suzie Birchwood

Company Award for Health Technology
Aparito

Young Patient Advocate Award
Awardee announced live

Company Award for Innovation
Lysogene

Scientific Award
Prof. Franz Schaefer, *Heidelberg University Hospital*

Lifetime Achievement Award
Dr William Gahl,
National Human Genome Research Institute

Company Award for Patient Engagement
European Alliance for Newborn Screening in SMA

Photo Award
Announcement

ABOUT THE BLACK PEARL AWARDS

The purpose of the EURORDIS Black Pearl Awards is to raise the profile of the rare disease cause in Europe and to recognise the work of some of the most inspirational people in the field of rare diseases. The Awards help to build awareness of the advancements and good practices made by and for the rare disease community. EURORDIS and the rare disease community are aware that we have much more to accomplish than we have achieved so far.
To this end, the EURORDIS Black Pearl Awards provides important support for EURORDIS' actions to:

BREAK THE ISOLATION OF RARE DISEASE PATIENTS AND THEIR FAMILIES through the development of national or European networking and international patient communities;

EMPOWER LEADERS OF RARE DISEASE COMMUNITIES through training, capacity-building activities and exchange to foster their research, therapeutic development and health policy activities;

INCREASE PUBLIC AWARENESS through information and our international communication campaign, Rare Disease Day.

THE IMPACT OF YOUR SUPPORT

The funds generated by the Black Pearl Awards are invested each year into work that directly benefits the rare disease community, and has a lasting impact for rare disease patients and their families.

The following examples highlight some of the **KEY PROGRAMMES** and priorities being advanced through the generous support of global donors and corporate members.

Coordination of the international Rare Disease Day campaign

The 15th edition of Rare Disease Day will take place on 28 February 2022.

Held on the last day of February each year, Rare Disease Day seeks to raise awareness of the impact that rare diseases have on the lives of patients and those who care for them. In the long-term, Rare Disease Day aspires to achieve equity for all people living with a rare disease and their families.

EURORDIS and its Council of National Alliances launched Rare Disease Day in 2008. What began as a European event quickly became international in scope, with participants from more countries joining each year. Today, Rare Disease Day is led by 67 national alliance patient organisation partners from around the world with thousands of international patient organisations, hospitals, research centres, companies, and policy makers participating in Rare Disease Day activities.

RARE DISEASE DAY®

www.rarediseaseday.org

ABOUT THE EURORDIS BLACK PEARL AWARDS

In 2022 we are again proud that the Chan Zuckerberg Initiative has offered a grant to EURORDIS, joining the European Commission and AFM-Téléthon among the funders of the Rare Disease Day campaign. With CZI's support we are continuing to scale up the global campaign to build, strengthen, and unify a movement to raise awareness of rare diseases across the globe.

We are also pleased and grateful that Fondation Ipsen, under the aegis of Fondation de France, has joined the community of Rare Disease Day funders by supporting two new initiatives: establishment of a Communications Campaign fund, to support national alliance partners in delivering the Rare Disease Day campaign in their countries/regions, and development of a Rare Disease Schools Toolkit, to help raise awareness among young people for rare diseases.

In 2021, 108 countries hosted events to raise awareness for the rare disease cause, including low and middle-income countries such as Nicaragua, Mali and Uzbekistan, which joined the campaign for the first time. The campaign's free and adaptable materials were translated into 33 languages and downloaded a record 55 000 times, helping patient communities around the world to build local awareness in their own languages.

RARE IS MANY
-
RARE IS STRONG
-
RARE IS PROUD

RARE DISEASE DAY®

www.rarediseaseday.org

SHARE YOUR COLOURS

Knowledge Exchange and Peer Support

EURORDIS' multilingual communications form an essential foundation to build and develop the international rare disease movement. Whether through training or digital media, EURORDIS helps to facilitate contact between people representing different diseases and cultural backgrounds, thereby creating communities of advocates who drive positive change further.

https://www.eurordis.org/enews

Rare Barometer

EURORDIS-Rare Diseases Europe regularly surveys the rare disease community via its Rare Barometer programme to identify patients' perspectives and needs on a number of issues in order to be their voice within European and international initiatives and policy developments. Rare Barometer brings together over 15,000 patients, carers and family members. Together, they represent more than 1500 diseases and over 80 countries, and contribute to raising awareness of the common challenges rare disease patients experience.

Rare Barometer

For more information visit eurordis.org/voices

EURORDIS Open Academy

The EURORDIS Open Academy plays a central role in ensuring patients have both the knowledge needed to bring their expertise to discussions on healthcare, research and medicines development, and the confidence to drive change at the local level.

In 2021, EURORDIS delivered four training programmes via the EURORDIS Open Academy: the Summer School on Medicines Research & Development (14th edition), the Winter School on Scientific Innovation & Translational Research (4th edition), the Leadership School (3rd edition) and the Digital School on Social & Digital Media. Due to the COVID-19 pandemic, all schools were converted into a full online delivery. Over 89 patient advocates from more than 27 countries, and 62 expert trainers were involved in the EURORDIS Open Academy programmes organised last year.

At the end of 2021, the e-learning platform of the EURORDIS Open Academy had over 2000 registered users from more than 155 countries. 5 new e-learning courses were added to the platform. In 2022, EURORDIS will further develop the plans for the future of the Open Academy programme, which will include a restructured training offer building on its existing programmes, additional e-learning courses, and regular outreach and communication with the alumni.

EURORDIS
OPEN ACADEMY

openacademy.eurordis.org

Better Health, Brighter Future

Takeda congratulates all EURORDIS Black Pearl Award 2022 winners who have demonstrated an outstanding commitment to make a difference for the rare disease community!

www.takeda.com

EURORDIS BLACK PEARL AWARDS 2021

We received hundreds of high-calibre nominations from people across the rare disease community, representing 38 different countries worldwide.

EURORDIS is delighted to present hereafter the 2022 awardees:

EURORDIS MEMBERS AWARD

Childhood Cancer International Europe

The EURORDIS Members Award is awarded to Childhood Cancer International Europe (CCI-E), the largest umbrella-organisation for childhood cancer in Europe, counting 67 patient-organisations in 34 countries.

The Award celebrates the organisation's outstanding advocacy of childhood cancers on a national and international level, as well as their active commitment to fulfilling their role as patient advocates in the European Reference Network for Paediatric Cancer and several other EU projects such as Harmony, PanCare and Accelerate.

Thanks to the work of CCI-E and their collaborations with local and international partners they have enhanced the ability of many medical professionals and health workers to recognise the early signs of childhood cancer enabling them to make accurate and early diagnoses. This award recognises that CCI-E is at the forefront of the establishment of an 'EU network of youth cancer survivors', as outlined in the Europe's Beating Cancer Plan. CCI-E has also been extremely supportive of EURORDIS, notably in relations to the BECA report, and stands shoulder to shoulder with the rare adult cancer patients' community.

EURORDIS VOLUNTEER AWARDS

Graham Slater

The EURORDIS Volunteer Award 2022 goes to Graham Slater, one of the first survivors of Esophageal Atresia (EA), for his outstanding contributions and dedication to the rare disease community and to EURORDIS.

The Award acknowledges Graham's incredible commitment to rare diseases and his work as Board Member of TOFS (UK EA support group), Chair of the Esophageal Atresia Global Support Group (EAT), partner of the UK National Health Service's Clinical Reference Group for specialised surgery in children, as well as lead ePAG at the European Reference Network for rare Inherited and Congenital (digestive and gastrointestinal) Anomalies (ERN ERNICA).

The award also recognises Graham's involvement in different EURORDIS working groups, long commitment to international collaboration in the rare disease domain and his leadership to ensure that all rare disease patients, regardless of where they live, can benefit from the sharing of good practices and the development of new surgical techniques and improved life-long after-care.

BLACK PEARL KEYNOTE SPEAKER 2022

Clément Beaune

Minister of State for European Affairs

Clément Beaune is a graduate of the Paris Institute of Political Studies (Sciences Po) and of the Bruges College of Europe ("Montesquieu" year). He is an alumnus of the French National School of Public Administration (ENA) ("Willy Brandt" year).

After leaving ENA in 2009, Clément Beaune joined the Budget Directorate, as Deputy Head of the Finance Acts Bureau.

In 2011, he became Deputy Head of the Research and Higher Education Bureau.

From 2013 to 2014, Clément Beaune was a technical adviser (budget) to the Prime Minister and from 2014 to 2016 he was European, International and Budgetary Affairs Adviser to the Minister of the Economy, Industry and the Digital Sector.

After serving a year as Deputy CEO of ADP Management, he was a Special Adviser within the Diplomatic Unit of the Presidency of the Republic from 2017 to 2020.

EUROPEAN RARE DISEASE LEADERSHIP AWARD

Dr Anne-Sophie Lapointe

The EURORDIS European Rare Disease Leadership Award 2022 is awarded to Dr Anne-Sophie Lapointe for the outstanding leadership and dedication shown to the rare disease community and the positive impact she has made in advancing rare disease policy and partnerships both at a national and international level.

Dr Lapointe's involvement in the rare disease field started in 1998 as a caregiver of two boys living with a rare disease. She then served as President for the French Lysosomal diseases association, Vaincre les Maladies Lysosomales, and as a board member at EURORDIS and the French national rare diseases alliance for 6 years.

Dr Lapointe holds a PhD in bioethics and she has worked for several years with geneticists at Necker Hospital and the ERN ITHACA with the French rare diseases network AnDDI-Rares. She developed an expertise in the genomic area, collaborating with human and social research teams around programmes linked to incidental findings and patient consent. She was also involved as a board member in the Inserm Ethics Committee (IEC) with a constant dialogue between the scientific and medical research community and society.

In 2016, alongside Ana Rath from Orphanet, she drafted the third national plan for rare diseases. Her domain was "information, training and e-health". The third plan, launched the 4th of July 2018, is the result of the concerted efforts of all the stakeholders committed to promoting an ambitious health and research policy designed to help patients with rare diseases and their families. In October 2018, she joined the French Ministry of Health as project manager of the rare diseases mission and with her team she works closely with the Ministry of Research to undertake the third national plan with its 55 actions.

This award celebrates her determination and engagement, as well as her active involvement in advocating for rare diseases.

PERFORMER

CHLOE TEMTCHINE

Chloe Temtchine is an award-winning singer, songwriter, speaker. Chloe was diagnosed with Pulmonary Hypertension/PVOD and survived on oxygen for years despite the fact that she was given little time left to live. Twelve years later, Chloe went into cardiac arrest and she ended up in a coma for four days, on life support, when she received a life-saving double-lung transplant. Chloe has always been a motivating force to anyone who has known her; struggling to stay alive for 12 years only sharpened her perspective, understanding, and compassion. She started Super Brave Kids as a platform that would allow her to empower children to see beyond their obstacles and to in turn use their own stories and the lessons they've learned in an effort to inspire others. In all of her capacities - as a singer, songwriter, and speaker - Chloe does the same thing: she gives people their power back. She founded The Chloe Temtchine Foundation (TCTF), a non-profit organisation dedicated to inspiring and empowering people living with Pulmonary Arterial Hypertension (PAH) and other life-threatening illnesses, through entertainment. Fluent in French, Spanish, and English, Chloe, who sings with a paralysed vocal cord, has always been a consummate communicator.

Chloe is on a mission to use her music and story to help those in need of finding hope, motivation, and inspiration and to continue to bring awareness to Pulmonary Hypertension and the importance of organ donation. Chloe believes that a combination of perspective, gratitude, trust, never losing one's sense of humour, and putting in the actual work is a magical formula when it comes to taking one's power back. Learn more about Chloe here.

POLICY MAKER AWARD

Professor Hans-Georg Eichler

The EURORDIS Policy Maker Award is awarded to Prof. Hans-Georg Eichler MD, MS for his dedication to making a real difference in the rare disease community through his academia, institution and regulatory experience.

Hans-Georg is currently the Consulting Physician of the Association of Austrian Social Security Bodies. Before holding this position, he was the Senior Medical Officer of the European Medicines Agency for more than 14 years, and Professor and Chair of Clinical Pharmacology at the Medical University of Vienna. He held a range of other full-time and honorary positions in academia, industry and government.

This award acknowledges the role he played at the forefront of the campaign for clinical research to be more innovative in order to reduce the time and money it takes for a drug to come to the market. In his role as Senior Medical Officer of the European Medicines Agency he showed incredible leadership advocating for drugs licensing.

The award also recognises his active engagement with the Clinical Trials Transformative Initiative (CTTI), his commitment to making clinical research more global and bringing information to those who need it most and the active role he has played within EURORDIS by co-chairing the 32nd ERTC workshop and being a Programme Committee member for ECRD 2022.

In loving memory of Dr Jordi Llinares Garcia

The Black Pearl Awards are an opportunity to celebrate individuals who have committed their lives and careers to the rare disease cause. Therefore, whilst we celebrate the incredible awardees tonight, it is also important to think of those who cannot be with us and to honour their achievements.

The past year has brought many lows as well as the highs we are celebrating. One such event which holds intense sadness was the tragic and sudden passing of Dr Jordi Llinares Garcia in July 2021. Jordi was the Head of Research & Innovation at the European Medicines Agency (EMA), an agency he served with patient-centric dedication for almost 20 years until his unexpected death. We would like to commemorate Jordi for his lifelong dedication to the rare disease community and his unbridled devotion to his work. Jordi was a compassionate and dedicated individual who devoted his career to the rare disease cause, and he is greatly missed by his family, friends and colleagues.

It is impossible to mention Jordi without making reference to his wife, Maria, and four children, Alberto, Oscar, Irene and Elena. He was so proud of his family and talked about them frequently. Colleagues, delegates, friends will all miss his sense of humour, his easy smile and laugh and easy-going nature. He brought warmth to any discussion along with scientific rigour.

In loving memory of Dr Jordi Llinares Garcia

Jordi's journey with rare diseases began with his studies of Medicine at the University of Barcelona, Spain, after which he went on to the London School of Hygiene & Tropical Medicine, gaining a Masters in Epidemiology. Jordi practised as a Clinical Pharmacologist at the Hospital de la Santa Creu i Sant Pau in Barcelona, Spain, for over 5 years before joining the European Medicines Agency in the early 2000s. He started as a Scientific Administrator, focused very strongly on orphan medicinal products and rare diseases.

Jordi was head of the Orphan team at the EMA and he also participated in COMP meetings where he strongly supported the voice of patients in the evaluations. He was also responsible for the EMA's making reference to Rare Disease Day on their homepage each year. Jordi also participated and contributed extensively to the EURORDIS Summer School both as a speaker and in helping prepare documents for the mini-COMP breakout sessions.

Jordi's role as a leader at the EMA meant that he participated in many forums nationally and internationally. He worked very closely with the team members at the EMA responsible for ensuring that the patient voice is represented in the work of the Agency. He also worked very closely with EURORDIS and its member organisations to ensure that he was connected with the real experiences and concerns of the rare disease patient community. Where the patients were, Jordi was – ready to listen and to do what he could to move things forward in both his formal role as well as by encouraging others in all settings; and in bringing them along with him.

Jordi will be greatly missed by everyone that knew him.

SOCIAL MEDIA AWARD FINALISTS

Vote for your favourite one!

Established in 2022, the new Social Media Award is presented to an individual or organisation that has created innovative and meaningful social media content, advocating for people living with a rare disease and bringing the reality of their daily lives into the public arena.

Communication, awareness raising, creativity and ability to reach people outside the rare disease community are the qualities we value most. The Award honours the best advocates on social media platforms such as Twitter, Facebook, YouTube, Instagram, and TikTok.

The inspiring commitment and dedication to patient advocacy shown by our three finalists have enabled the needs of people living with a rare disease to be broadcasted beyond the rare disease community to an even wider audience.

SOCIAL MEDIA AWARD FINALISTS

Vote for your favourite one!

FINALIST 1

Francesco Cannadoro

Francesco Cannadoro (Italy), father of a child born with an undiagnosed disease, created "Diario di un padre fortunato" ("Diary of a lucky dad") in 2016 as a request for help and the blog soon became a page of reference in Italy for a new disability narrative online.

Over the years, the relaxed tone of the diary, together with the total absence of pitifulness and spectacularisation of sorrows and suffering, has attracted all those people who would have never otherwise approached other types of disability-related content. The diary shows them a portrait that is quite far from their expectations, a life with challenges but full of positivity, well-represented in the author's words: "Disability sucks.
But your life, despite disability, sucks only if you let it".

Francesco's goal is to become the voice of a different narrative around disability and do what he can, story after story, to push towards a real change in society, to make it become more inclusive through the sharing of knowledge and experience.

Learn more about Francesco here

SOCIAL MEDIA AWARD FINALISTS

Vote for your favourite one!

FINALIST 2

Milica and Noa

Milica (Serbia) is the mother of a little hero and an amazing girl called Noa. Noa was born prematurely at 26 weeks, weighing only 700g. Due to the complications after her birth, she had 3 brain bleeds, and they developed into an extreme Hydrocephalus that left 80% of her brain damaged. Up to now, Noa has had 18 brain surgeries, and she had some other diagnoses due to hydrocephalus, like severe epilepsy and cerebral palsy, and about 10 more diagnoses. But Noa never let her rare condition dictate her life. She is now 9 years old, almost walking, she still has to learn to talk but she knows gestures and understands a lot now, even though the diagnoses meant she had a chance of survival of less than 1%, she is making more out of her 1% than most would have thought possible.

Through social media (mainly Instagram) Milica tries to educate and bring people closer to the life of a child with special needs. There is no room for negativity on their social media: you can only find beautiful and inspirational things like therapies, cute stories and Noa's everyday life. Noa's story provides a source of inspiration for anyone reading it via Milica who's "just her voice until she finds her own and continues to inspire with her own words".

Learn more about Milica and Noa here

SOCIAL MEDIA AWARD FINALISTS

Vote for your favourite one!

FINALIST 3

Noah Higon Bellver

Noah Higon Bellver (Spain), is a young patient advocate born on August 4th, 1998 in Valencia, who is living with 7 different rare diseases. She holds a double degree in Law and Political Science and Public Administration from the University of Valencia.

Over the past years she has participated in several project supporting people living with a rare disease, including documentaries, collaborations with TV, newspapers and radios. She is also the author of two books: "De qué dolor son tus ojos" and "De esperanza marchita".

She has been using her social media channels to increase visibility of rare disease and has represented people living with a rare disease by giving presentations at several events, including conferences and the European Youth Event. Noah collaborates on a regular basis with FEDER, the FE hospital research institute, and she is an ambassador for the NGO "Proyectos Juntos" and "Muévete por los que no pueden".

Learn more about Noah here

MEDIA AWARD

Ewenflix

The EURORDIS Media Award is awarded to the EwenLife on-demand video platform for offering all people living with a rare disease and their families free and accessible online tools to break through the sense of isolation frequently going hand in hand with rare diseases.

The platform hosts videos where patients, parents and caregivers talk about how rare diseases affect their everyday life - and share some personal tips or advice. What is also noteworthy about the project is the abundant sense of optimism which is prevalent in the interviews, seminars/web series and filmed testimonies which the platform hosts.

The award also recognises the needs of people living with a rare disease and brings them to the attention of a large audience as well as simultaneously forging bonds of solidarity among the rare disease community.

PERFORMER

SUZIE BIRCHWOOD

Suzie Birchwood is a Dancer and Dance Educator specialising in diversity and equality in dance.

At 16 Suzie achieved her dream of training to be a professional ballet dancer with a full scholarship at the London Studio Centre. Weeks before completing her first year, Suzie developed generalised dystonia, a profoundly disabling neurological condition. She lost the movement in her legs and experienced painful and incapacitating spasms throughout her body.

Over the following years, using her wheelchair, Suzie carved out a different method of dancing, pioneering a new approach to classical repertoire. She worked as a guest artist with The Scottish Ballet, Ballet Cymru, Candoco, StopGap and GDance, as well as creating and performing her own works, including a double bill at Sadlers Wells.

Through continued research and discovery into her own unique physiology, she was not only able to continue working in the form she loved, but also to regain mobility and better management of her condition. Further medical intervention and neuroplastic brain retraining has brought Suzie to a near-impossible recovery. She has started a family, is able to walk with crutches and live an independent and fulfilling life.

Driven by her experiences as a disabled dancer, Suzie's choreography is defined by the discovery and nurture of individuals and their unique movement personalities. The stories that she tells through dance express the deep link between human bodies and human experiences. Suzie now works to spread a universal approach to dance to the widest possible market through coaching and mentoring disabled artists, teachers and choreographers.

COMPANY AWARD FOR HEALTH TECHNOLOGY

Aparito

The EURORDIS Company Award for Health Technology recognises companies developing technologies with significant positive impact on the daily lives of people living with a rare disease. EURORDIS commends Aparito as a company at the forefront of innovative technologies that report patient experiences accurately. By placing patient needs at the heart of the solution, Aparito's expertise facilitates medicines development and contributes to addressing complex regulatory challenges. Aparito's collaborative approach, working across rare diseases and alongside patient organisations, clinicians as well as sponsors, also deserves recognition. With the ever-increasing pace of developments in science and technology, Aparito's remarkable journey in the field of remote patient monitoring is testament to the potential of digital solutions to profoundly impact the delivery of healthcare for people living with a rare disease.

Award selection made in Partnership with **Medtech Europe**

YOUNG PATIENT ADVOCATE AWARD

Danielle Drachmann

Danielle Drachmann founded Ketotic Hypoglycemia International (KHI) - the world's largest patient organisation for patients with idiopathic (unexplained) ketotic hypoglycemia - after failing to get a diagnosis, despite extensive clinical and genetic investigations, on her two children's dangerously low blood glucose (sugar) and high ketone levels. This international patient association works in close collaboration with leading medical experts from all over the world and has initiated patient-driven research projects later disseminated in scientific publications. The organisation is also very active on its social media platforms, where it strives to unite the families in an online community, so they can support, guide and help each other navigate the life living in a ketotic hypoglycemia rollercoaster.

Drachmann also recently entered the European Health Parliament and the European Medicines Agency as a Patient Representative for patients with rare hypoglycemia diseases. She is working at the Center for Research with Patients and Relatives at Odense University Hospital, Denmark, while also sitting in the Research Committee at H.C Andersen's Children's Hospital, Odense University Hospital, Denmark, as a patient and relative representative.

Danielle's outstanding resilience in the face of the medical mystery, idiopathic ketotic hypoglycemia (IKH), combined with her perseverance in initiating patient-driven research with leading medical experts from different fields of medicine, has been an extraordinary example of how we envision the future of rare disease research: **Passionate, patient-driven and co-created.**

COMPANY AWARD FOR INNOVATION

Lysogene

The EURORDIS Company Award for Innovation acknowledges companies undertaking ground-breaking activities to advance rare disease research and medicines development. EURORDIS recognises Lysogene as a pioneering gene therapy company which has established itself among major players driving European-led innovation, in just over ten years. We are particularly encouraged by Lysogene's promising advances towards delivering novel treatments in neuro degenerative and neuro developmental disease areas with high unmet medical needs. Lysogene's continued readiness to advocate for early diagnosis and engage patients and caretakers as equal decision makers in the product development lifecycle also merits acknowledgement. The award also celebrates the remarkable personal journey of the company founder, Karen Pignet-Aiach, as a mother, patient advocate and entrepreneur determined to change the landscape for severe rare diseases affecting children's central nervous systems.

SCIENTIFIC AWARD

Prof. Franz Schaefer

The Scientific Award is awarded to Prof. Franz Schaefer for his scientific excellence, outstanding dedication to the rare disease community and the positive impact he has made in rare disease research and patient communities on an international level.

Prof. Schaefer is Professor of Pediatrics and Chief of the Pediatric Nephrology Division at Heidelberg University Hospital. He received his M.D. in 1986 at Würzburg University Medical School. He performed research scholarships at the Institute of Child Health, London, the University of Virginia and Stanford University and he has also served on the boards and councils of numerous medical societies. He is the current President-Elect of the International Pediatric Nephrology Association.

Prof. Schaefer has a special interest in rare kidney disease research. In 2009 he established the eRare-funded PodoNet Project for Research in hereditary and immune mediated steroid resistant nephrotic syndrome. The PodoNet Registry has become the world's largest database for this group of rare glomerulopathies and has led to the identification of new genetic disease entities and prognostic biomarkers. Since 2021 Prof. Schaefer has contributed his expertise in clinical data management by contributing to the development of a rare disease data ecosystem as Pillar co-lead in the European Joint Programme for Rare Diseases (EJP RD) and since 2021 by leading the data integration workpackage of ERICA, the ERNs' research coordination programme. Prof. Schaefer's publication record encompasses 650 scientific articles and book chapters.

We would like to acknowledge his leadership and outstanding work in the ERN Coordinators Group and in several of the cross-ERNs Working Groups, as well as his prominent role in rare disease Registries and his innovative approach in the use of health outcome measures to drive improvements in care and his work within the European Joint Programme on Rare Diseases. It is thanks to his scientific leadership, research and collaborative spirit that key achievements have been made in the field of rare kidney diseases, paediatric nephrology and hypertension. His dedication, engagement, collaboration with scientists, clinicians and patients cannot be overstated, and we believe him to be a truly deserving recipient of this award.

LIFETIME ACHIEVEMENT AWARD

Dr William Gahl

The EURORDIS Lifetime Achievement Award goes to Dr William Gahl for the lifelong dedication he has shown in addressing the needs of people living with rare and undiagnosed diseases. Dr William Gahl graduated from the Massachusetts Institute of Technology and earned his M.D. and Ph.D. from the University of Wisconsin. He served as paediatric resident and chief resident at the University of Wisconsin hospitals and completed clinical genetics and clinical biochemical genetics fellowships at the NIH. Dr Gahl elucidated the basic defects in cystinosis and Salla disease and helped bring cysteamine to new drug approval by the Food and Drug Administration as the treatment for cystinosis. He has published over 530 peer-reviewed papers, trained 42 biochemical geneticists and cultivated international experts for dozens of rare diseases, syndromes and disorders. In 2008, he established the NIH Undiagnosed Diseases Program (UDP), which has made more than 300 rare disease diagnoses and discovered 30 new genetic diseases. Dr Gahl expanded the UDP to a national Undiagnosed Diseases Network and a worldwide Undiagnosed Diseases Network International. He established the American Board of Medical Specialties certification for medical biochemical genetics. Dr Gahl received the Dr Nathan Davis Award for Outstanding Government Service from the AMA, the Service to America Medal in Science and the Environment, and numerous other awards. In 2019, he was elected to the National Academy of Medicine.

With this award, we would like to acknowledge the key role Dr Galh has played in the creation of the National Institutes of Health (NIH) Undiagnosed Diseases Network (UDN) and its essential role in discovering many new genetic disorders. His unwavering support of the rare and undiagnosed disease cause and his patient-centred approach is demonstrated by the leadership role he played in the development of the International Network on Undiagnosed Diseases (UDNI). This notable achievement is bolstered by the work being done by the UDNI which he spearheaded as the Chair; the programme provides an admirable example of an international institution which brings together the undiagnosed community at the global level. UDNI has provided a structure and forum to exchange on undiagnosed cases for clinicians working around the globe.

The importance of all his efforts in the field of rare disease research cannot be overstated, and we believe him to be a truly deserving recipient of this award.

COMPANY AWARD FOR PATIENT ENGAGEMENT

Spinal Muscular Atrophy (SMA)-NBS Alliance

The EURORDIS Company Award for Patient Engagement recognises the collaborative effort of the companies involved in the European Alliance for Newborn Screening in SMA to ensure that newborn screening programmes in all European countries diagnose all newborn children with SMA. This close collaboration reinforces the demand of the rare disease community for a harmonized approach to newborn screening in Europe. The achievements of the Alliance, which is succeeding in creating much-needed awareness and momentum towards an accelerated implementation of newborn screening for SMA in an ever-growing number of European countries, merits acknowledgement. The Award also recognises how the Alliance has set a model example which has the potential to encourage similar initiatives across other rare diseases. EURORDIS truly appreciates the companies' mutual engagement with patient advocates and multiple stakeholders around a single cause, which further reflects their commitment for and solidarity with the rare disease community.

SMA NBS Alliance

EURORDIS PHOTO AWARD

Dalmatian(s)

Throughout my life I have often been compared with Dalmatians because of my skin condition, so I took a photo with one of them!

Congenital Melancoytic Nevus

Photographer: Lucía Lamata, Spain

I climbed Mount Kosciuszko!

Francesca at 14 years old with the support of family and friends climbed Mount Kosciuszko, tallest mountain in Australia at 2,228 meters above sea level.

ADCY5-related dyskinesia

Photographer: Angelina Canturi, Australia

EURORDIS PHOTO AWARD

Girlfriends for Life

Despite their different religions, cultures and handicaps, these two young women are best friends and enjoy life to the full.

MS

Photographer: Josef Hinterleitner, Austria

Jazz the two of us

This photo was taken during one of the outdoor music concerts and illustrates our typical way of experiencing music, mainly jazz concerts, by Borys and myself. Unexpectedly jazz became our shared passion and emotional glue building up the strong bond of a Father and son's love and joy.

Metrachromatic Leukodystrophy

Photographer: Rafal Mroziński, Poland

EURORDIS PHOTO AWARD

The world of hope

This boy has been affected by Hereditary hypophosphatemic rickets since his early childhood. Still he is happy with his elder sister and his world of hope.

Hereditary hypophosphatemic rickets

Photographer: Pranab Basak, India

Smiling underwater

Pedro is an ANGEL and is always smiling. Pedro loves water and even underwater he keeps his contagious smiles.

Angelman Syndrome

Photographer: Catarina Costa Duarte, Portugal

EURORDIS PHOTO AWARD

Federico

Trisomy 9 Mosaic is a rare chromosomal disorder for which the chromosome 9 appears 3 times (trisomy) rather than 2. The term 'mosaic' indicates that the chromosome is not present in all the cells but only in some of them. Associated symptoms and results may change substantially according to the percentage of cells with the extra chromosome. However, the common features include slow growth before birth, mental retardation, heart structural malformations and distinctive features of the skull and face like a sloping forehead, and malformed ears. Individuals with trisomy 9 mosaicism can vary very much one from another. He is Federico: six years old, blind. He speaks through body language, just like a dancer.

Trisomy 9 Mosaic

Photographer: Emiliano Cribari, Italy

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PREVIOUS BLACK PEARL AWARDEES

EURORDIS Members Award

AKU Society UK – 2021

ALAN Maladies Rares Luxembourg - 2020

ALS Liga Belgium – 2019

Pulmonary Hypertension Association Europe – 2018

Dravet Europe – 2017

UNIQUE – 2016

Children with SMA – 2015

Allianz Chronischer Seltener Erkrankungen – 2014

Alström Syndrome UK – 2013

Association Française des Myopathies – 2012

EURORDIS Volunteer Award

Ingunn Westerheim, Rebecca T Skarberg – 2021

Claudia Crocione – 2020

Richard West, Russell Wheeler – 2019

Chris Sotirelis, Helene and Mikk Cederroth – 2018

Elizabeth Vroom – 2017

Tsveta Schyns – Liharska – 2016

Rosa Sánchez de Vega – 2015

Lise Murphy – 2014

Lesley Greene – 2013

Michele Lipucci Di Paola, PhD – 2012

Policy Maker Award

Kateřina Konečná – 2021

Dr Vytenis Andriukaitis – 2020

Dr Edmund Jessop – 2019

Elena Gentile – 2018

Frédérique Ries – 2017

Christian - Silviu Buşoi – 2016

Glenis Willmott – 2015

Antonyia Parvanova – 2014

Françoise Grossetête – 2013

Andrea Vassiliou, JD – 2012

Scientific Award

Alain Hovnanian – 2021

Prof. Annemieke Aartsma-Rus – 2020

Professor Philip Van Damme – 2019

Professor Michele De Luca and Doctor Tobias Hirsch – 2018

Dr Lucia Monaco – 2017

Professor Dr Peter N. Robinson – 2016

Professor Kate Bushby – 2015

Professor Hans-Hilger Ropers – 2014

Dr Ségolène Aymé – 2013

Professor Alain Fisher and Professor Maria Grazia Roncarolo – 2012

Media Awards

Ana Ilic (Written) – 2021

Noémie Desquiotz-Sunnen (Visual and Media) – 2021

Tomasz Śliwiński & Magda Hueckel
(Visual and Audio) – 2020

Anne-Dauphine Julliand (Visual and Audio) – 2019

Bojana Mirosavljević (Written) - 2019

Serge Braun, Christopher Ulmer – 2018

Aldo Soligno – 2017

France Télévisions - AFM – Télethon – 2016

Peter O'Donnell – 2015

Rick Guidotti – 2014

Andrew Jack – 2013

BBC – 2012

European Rare Disease Leadership Award

Dr Enrique Terol, Victoria Hedley, Dr Birutė Tumienė – 2021

Dr Daria Julkowska – 2020

Professor Till Voigtländer – 2019

Professor Bruno Sepodes – 2018

Antoni Montserrat Moliner, Jarek Waligóra,
Michael Hübel – 2016

Professor Josep Torrent-Farnell – 2015

Paola Testori Coggi, Professor Guido Rasi,
Professor Luca Pani – 2014

Dr Ruxandra Draghia – Akli – 2013

Kerstin Westermark, MD, PhD – 2012

Company Awards

Orchard Therapeutics (Innovation Award), Takeda (Patient Engagement Award),
Epihunter (Health Technology Award) – 2021

Healx (Innovation Award), Boehringer Ingelheim
(Patient Engagement Award), Tobea (Health Technology Award) – 2020

Chiesi (Innovation Award), The HERCULES Project
(Patient Engagement Award), Air Liquide Medical Systems
(Health Technology Award) - 2019

Novartis (Innovation Award), EFPIA, Bayer, UCB and MSD
(Patient Engagement Award) – 2018

GSK – 2017

Actelion – 2016

Pfizer – 2015

Sobi, Orphan Europe – 2014

Celgene Corporation, Prosensa, Genzyme – 2013

CSL Behring, Shire, Sigma-Tau Pharmaceuticals – 2012

Lifetime Achievement Award

Milan Macek – 2021

Michael Griffith - 2019

Alastair Kent – 2018

Anders Olauson – 2017

Renza Barbon Galuppi – 2016

Abbey Meyers – 2015

Marlene Haffner - 2014

Eva Luise Köhler - 2013

Young Patient Advocate Award

Rachele Somaschini – 2021

Jana Popova – 2020

Laëtitia Ouillade – 2019

Sammy Basso – 2018

Here is what some of our Patrons and Commitee members are saying about EURORDIS, Rare Diseases and the EURORDIS Black Pearl Awards:

“I would like to express my gratitude that I can serve as the Honorary Patron for the 11th edition of the EURORDIS Black Pearl Awards. The awards recognise those who transform great ideas, hopes, and the needs of patients into practice and thus improve their lives. Recognising the leaders and advocates who can go the needed extra mile, who demonstrate their hard work and commitment to the cause is absolutely necessary for our efforts to improve the lives of people living with a rare disease. To get a Black Pearl Award is an honor and privilege and it can truly make the difference.”

Kateřina Konečná

Member of the European Parliament (Czech Republic)

“I want to express my gratitude and admiration for the EURORDIS Black Pearl Awards.

Many people go the extra mile to make a real difference in our society. Recognising their achievements and hard work in supporting the rare disease community is essential. Recognition along with raising awareness can promote medical research and production of orphan drugs and thus ensure that everyone has access to the medical advances and the specific care they need.”

Stelios Kympouropoulos

Member of the European Parliament (Greece)

“C’est avec grand plaisir que j’avais parrainé les “Black Pearl Awards”. Je serai ravi de parrainer à nouveau cet évènement et de réaffirmer la mobilisation des autorités françaises sur la question des maladies rares.”

Philippe Leglise-Costa

Permanent Representative of France to the EU

I am pleased to continue my Honorary Patronage and to serve as one of the Honorary European Patrons for the eleventh edition of the EURORDIS Black Pearl Awards in 2022.

I am proud to be a part of the international effort to increase awareness about lives of people living with a rare disease.

H.E. Mr. Pavel Klucký

Ambassador of the Czech Republic to the Kingdom of Belgium

To be a patron of the EURORDIS Black Pearl Awards 2022 is a commitment and an encouragement to continue working for the benefit of patients with rare diseases and their families.

The first element for judging quality of life is health and its loss has a big impact on sick persons and on those close to them. The challenges for patients with rare diseases go far beyond the loss of health. It is the difficulty of having a diagnosis or accessing an effective treatment. Even more, for many years social stigma has accompanied people with rare diseases reflecting a social failure that only in the last years is changing thanks to the many actions launched in scientific and social issues.

Spain is committed and involved in all the actions directed to supporting research, families and carers, the associative movement, and to accompanying the sick persons to improve their life conditions.
You can count on my personal commitment and that of the Permanent Representation to contribute fostering all these efforts

H.E. Marcos Alonso Alonso

Ambassador Permanent Representative of Spain to the EU

I feel very much honored to be one of the Honorary Patrons for the eleventh edition of the EURORDIS Black Pearl Awards 2022.

To be a part of such a great community of dedicated people is an obligation for the future. I can only say that the topic of rare diseases was already important during the first Czech Presidency in 2009 and can reassure you that the topic will also be highlighted during the Czech Presidency in the second half of this year.

Having said this, let me express my firm support for your work and my congratulations to all individuals, organisations, and companies who dedicate their lives to making a difference for the rare disease community.

Enjoy the evening!

H.E. Edita Hrdá

Ambassador Extraordinary and Plenipotentiary
Permanent Representative of the Czech Republic to the EU

Quote from one of our Committee members:

The Black Pearl Awards are a great opportunity to give visibility to rare diseases and to highlight people who are committed to them day after day. Thank you EURORDIS for this beautiful initiative!

Prof. Hélène Dollfus

Chair of Network Coordinators Group

SPECIAL THANKS TO OUR DONORS

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ERN EpiCARE • Épilepsies Rares et Complexes

ERN EuroBloodNet • Rare Hematological Diseases

ERN eUROGEN • Urogenital Diseases

ERN EURO-NMD • Rare Neuromuscular Diseases

ERN EYE • Eye Diseases

ERN GUARD-HEART • Rare and Complex Heart Diseases

ERN ITHACA • Intellectual disability, TeleHealth, and Congenital Anomalies

ERN PaedCan • Paediatric Oncology

ERN RARE-LIVER • Heptological Diseases

ERN ReCONNET • Connective Tissue and Musculoskeletal Diseases

ERNICA • Inherited and Congenital Anomalies

ERN-RND • Rare Neurological Diseases

VASCERN • Vascular Diseases

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EURORDIS *Black Pearl* AWARDS

Thank you for joining us at the Black Pearl Awards 2022.

To see the highlights of this evening,
please visit blackpearl.eurordis.org

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