EURORDIS Awards 2015 for Excellence in the Field of Rare Diseases

25 February 2015, Brussels - The European Organisation for Rare Diseases (EURORDIS) yesterday presented its 2015 Awards for excellence in the field of rare diseases at the EURORDIS *Black Pearl* Gala Dinner, held to mark Rare Disease Day 2015 which takes place on 28 February.

The EURORDIS Awards recognise outstanding patient advocacy groups, volunteers, scientists, companies, media and policy makers who have contributed to reducing the impact of rare diseases on people's lives.

HRH Princess Astrid of Belgium, who is sensitive to the rare disease cause, attended the Gala Dinner and was present for the awards ceremony, as was Rare Disease Day Ambassador Sean Hepburn Ferrer, the eldest son of the late Audrey Hepburn, who passed away from a rare cancer.

Yann Le Cam, Chief Executive Officer, EURORDIS, commented, "Rare Disease Day 2015 recognises the millions of families, friends and carers whose daily lives are impacted by rare diseases and who are living day-by-day, hand-in-hand with people living with a rare disease. We are honoured to have awarded this year's winners in recognition of the work they do as patient groups, researchers, rare disease patient advocates, volunteers and leaders to support anyone who is affected by or living with a rare disease."

Nominations for the EURORDIS Awards 2015, which were presented by Terkel Andersen, EURORDIS President, were submitted by the general public with the EURORDIS Board of Directors making the final decision.

The below awards were presented. Please click on the award title link or see further below to read full profiles of each winner and details of their commitment to the field of rare diseases. Photos of the award winners are available on the EURORDIS Flickr page.

European Rare Disease Leadership Award
Professor Josep Torrent-Farnell - Professor of Clinical Pharmacology and Therapeutics at the Autonomous University of Barcelona.

Policy Maker Award
Mrs Glenis Willmott - Labour Member of the European Parliament for the East Midlands.

Volunteer Award
Ms Rosa Sánchez de Vega - Dedicated rare disease patient advocate, Co-founder of the Spanish Alliance for Rare Diseases (FEDER) and President of the European Federation of Aniridia.

Media Award
Mr Peter O'Donnell - Associate Editor of European Voice.

Patient Organisation Award
Children with SMA, accepted by Mr Vitaliy Matyushenko - Children with SMA is a voluntary, non-profit foundation which supports those affected by or involved with Spinal Muscular Atrophy in Ukraine.

Scientific Award
Professor Kate Bushby - Professor of Neuromuscular Genetics and committed researcher in the field of rare diseases.

Company Award
Pfizer - One of the world's largest pharmaceutical companies, whose Rare Disease Research Unit is dedicated to developing new medicines across the spectrum of rare diseases.

Lifetime Achievement Award
Ms Abbey Meyers - Outstanding rare disease patient advocate and founder of the National Organization for Rare Disorders (NORD) in the USA.

###

About the EURORDIS *Black Pearl* Gala Dinner

The EURORDIS *Black Pearl* Gala Dinner is a fundraising event of European and high international standard held in support of people living with a rare disease and rare disease patient advocates. The net proceeds collected from the Gala will support community building initiatives to break the isolation of rare disease patients and their families in Europe and empower leaders of the rare disease community through training, capacity-building activities and exchange to stimulate their research. For more information, please visit the Gala Dinner website at galadinner.eurordis.org.

About Rare Disease Day

Rare Disease Day was launched by EURORDIS and its Council of National Alliances in 2008. Held on the last day of February each year, a rare day, it seeks to raise awareness of the impact that rare diseases have on the lives of patients and those who care for them. What began as a European event quickly became international in scope, with participants from more countries joining each year.

Since Rare Disease Day began, thousands of events have been held throughout the world, reaching hundreds of thousands of people. The political momentum resulting from the Day has also served advocacy purposes, contributing to the advancement of EU policies on rare diseases and the creation of national plans for rare diseases in a number of EU Member States and now in other countries. For more information visit RareDiseaseDay.org.

About Rare Diseases

The European Union considers a disease as rare when it affects fewer than 1 in 2,000 citizens. Over 6000 different rare diseases have been identified to date, affecting over 60 million people in Europe and the USA alone. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offering inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

About EURORDIS

EURORDIS, the European Organisation for Rare Diseases, is a non-governmental patient-driven alliance of patient organisations representing over 600 rare disease patient organisations in more than 60 countries. EURORDIS represents the voice of an estimated 30 million people living with a rare disease in Europe. Follow @eurordis or see the EURORDIS Facebook page.

Press Contact

Lara Chappell
Communications Director
EURORDIS
Plateforme Maladies Rares
96 rue Didot, 75014 Paris
Tel: +33 (0)1 56 53 52 61
lara.chappell@eurordis.org

Full details of award winners:

European Rare Disease Leadership Award **Professor Josep Torrent-Farnell**

Prof. Torrent-Farnell is a qualified pharmacist and also holds a degree in Medicine and Surgery from the University of Barcelona. He completed postgraduate courses in Pharmacology and Toxicology, Public Health and European Institutions and a doctorate in Clinical Pharmacology. Prof. Torrent-Farnell joined the newly established European Medicines Agency as Principal Scientific Administrator in 1995. In 1999, he became Executive Director of the Spanish Medicines Agency. He is currently Professor of Clinical Pharmacology and Therapeutics at the Autonomous University of Barcelona.

Prof. Torrent-Farnell is a strong advocate for the patient voice and has consistently demonstrated extraordinary leadership in the field of rare diseases. As the former Director General of the Fundació Doctor Robert, Advanced Centre of Services and Training for Health and Life Sciences, he was able to introduce a rare disease focus and impact the lives of many in Spain. He became the first Chair of the Committee for Orphan Medicinal Products (COMP) at the European Medicines Agency and with his Vice-Chair, Yann Le Cam, introduced many aspects of patient involvement in the EMA that remain today. Prof. Torrent-Farnell still serves as the COMP representative nominated by Spain. EURORDIS is delighted to present Prof. Torrent-Farnell with this Award as recognition for his pioneering leadership of the rare disease community. Prof.Torrent-Farnell continues to be a long-standing supporter of EURORDIS.

Policy Maker Award **Mrs Glenis Willmott, Member of the European Parliament**

Glenis Willmott, Labour Member of the European Parliament for the East Midlands since 2006 and three-time re-elected leader of the European Parliamentary Labour Party, has demonstrated outstanding dedication and commitment in addressing the needs of patients in the European Union.

Mrs Willmott is an active member of various committees including the Environment, Public Health and Food Safety Committee and the MEPs against Cancer Forum. She is so deserving of this award because of the instrumental role she has played in the passing of key legislation through her work as Rapporteur for the *Regulation on Clinical Trials on medicinal products for human use* and as Shadow Rapporteur for the Regulation establishing the *Health for Growth* Programme. These two pieces of EU legislation have a tremendous impact on the lives of people living with a rare disease in Europe.

Volunteer Award **Ms Rosa Sánchez de Vega**

As a rare disease patient herself, and the mother of a son with the same condition, Rosa Sánchez de Vega is a truly remarkable woman who has successfully managed to channel her difficulties with Aniridia into a positive force for change. Ms Sánchez de Vega first entered the world of rare diseases in 1996 when she founded the Spanish Aniridia Association, for which she served as president until 2008. In 1999, she went on to co-found the Spanish Alliance for Rare Diseases (FEDER), serving first as Vice President and then President until 2010.

Convinced that nothing could be achieved but at European level, Ms Sánchez de Vega joined the EURORDIS Board of Directors in 2003 and served as Vice President from 2006 until 2011. She has been recently appointed President of the European Federation of Aniridia, Aniridia Europe. Ms Sánchez de Vega is an incredibly deserving recipient of the Volunteer Award as she has worked tirelessly and selflessly, often putting the rare disease cause before her own needs, in order to shine a light on rare diseases and improve the lives of others.

Media Award **Mr Peter O'Donnell**

Peter O'Donnell is a prominent writer and editor in the rare disease field currently working as Associate Editor of European Voice. His impressive career has spanned over twenty years and has included working for The Financial Times, The Sunday Times, Reuters, and the Economist Intelligence Unit. He has worked as an editor and speechwriter for numerous clients in the corporate, political and academic world and has frequently chaired EU-level policy debates on EU affairs. This has made him very well placed to be able to write and report forthrightly on the various complicated issues surrounding rare diseases. Peter continues to become increasingly active in the rare disease community, demonstrating commitment and passion. Just recently he spoke at the Lunch Debate on Data Protection at the European Parliament.

Patient Organisation Award **Children with SMA, accepted by Mr Vitaliy Matyushenko**

Children with SMA is a non-profit foundation which has undertaken the incredibly difficult mission of supporting those affected by, or involved with, Spinal Muscular Atrophy (SMA) in Ukraine. SMA is a motor neuron disease characterised by the progressive degeneration of nerve cells in the spinal cord and brainstem, leading to muscle weakness, muscle atrophy, and respiratory complications. Children with SMA has worked tirelessly on behalf of those who suffer from SMA, promoting knowledge around the disease and encouraging dialogue between legislators, researchers and patients.

This award serves to recognise the efforts and successes of Children with SMA in Ukraine, including its contribution to the adoption of the law for Rare Diseases in Ukraine and the foundation of the National Alliance.

Scientific Award **Professor Kate Bushby**

Prof. Kate Bushby (MD FRCP) is a Professor of Neuromuscular Genetics and currently holds joint appointments between Newcastle University and the NHS. Her commitment to research in rare diseases has been evidenced through her impressive publication list, clinical activities and involvement in policy actions.

Prof. Bushby is actively involved in many European projects including being a founding co-ordinator of the TREAT-NMD Network of Excellence. She has played a leading role in the European and national rare disease policy area, acting as vice Chair on the European Union Committee of Experts on Rare Diseases from 2010 to 2013 and still acts in the capacity of invited expert on the new Commission Expert Group on Rare Diseases.

The EURORDIS Scientific Award recognises her outstanding achievements in inherited neuromuscular diseases research and her commitment to patients. Her impressive body of work has seen her become a leader in the rare disease community at the forefront of developments in the field of translational medicine. This award serves to recognise her long-standing contributions that have shaped rare neuromuscular disease research and will continue to fuel the future of rare disease research as a whole.

Company Award **Pfizer, Inc.**

Pfizer is one of the world's premier pharmaceutical companies, and has demonstrated commitment to, and passion for, the rare disease cause - with 22 approved products to treat rare diseases worldwide including 4 in Europe. In 2010, Pfizer established its own Rare Disease Research Unit (RDRU), with the objective of taking an innovative and collaborative approach to the development of new medicines across the spectrum of rare diseases. The current pipeline includes clinical and pre-clinical programmes in several rare diseases. Pfizer has been an active participant in the EURORDIS Round Table of Companies (ERTC) since 2007 and has joined other companies in fostering the empowerment of rare disease patient organisations through support of EURORDIS.

Through this award, EURORDIS recognises the role major pharmaceutical companies can play in the development of, and ensuring access to, innovative treatments for rare disease patients. The award also encourages companies to maintain a high level of corporate social responsibility by reassessing the value of medicines today so as to ensure that unmet medical needs are covered in the development of new treatments. Through their actions, companies should work to cooperate with and support the actions of patient advocacy groups.

Lifetime Achievement Award **Ms Abbey Meyers**

Abbey Meyers is an extraordinary woman: once a housewife and mother from Connecticut, USA, Ms Meyers was drawn into the world of political advocacy, fundraising and organisation development when it became painfully apparent through her experience as the mother of a child with Tourette syndrome, that patients with rare diseases were being neglected in favour of more common diseases that affected larger patient populations.

Ms Meyers founded the National Organization for Rare Disorders (NORD) in the USA. 32 years later and NORD is an incredible organisation dedicated to helping people with rare diseases. NORD was the precursor and inspiration for the creation of EURORDIS. This Lifetime Achievement Award serves to honour all that Ms Meyers has done for the rare disease community in the USA and throughout the world, her personal and tireless dedication to the cause and her instrumental role in the passage of landmark policies such as the Orphan Drug Act of 1983. Although retired now, Ms Meyers continues to be an inspiration to rare disease patient advocates.