--- PRESS RELEASE ---

EURORDIS Awards 2014

for outstanding accomplishments in the field of rare diseases presented in honour of Rare Disease Day in Brussels

Brussels, Belgium - 26 February 2014 - The European Organisation for Rare Diseases (EURORDIS) last night presented the EURORDIS Awards 2014 for excellence in the field of rare diseases during the EURORDIS *Black Pearl* Gala Dinnerheld on the occasion of Rare Disease Day.

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

"We are honoured to acknowledge the commitment and achievements of the recipients of the EURORDIS Awards on the occasion of Rare Disease Day 2014. This year,Rare Disease Day puts the spotlight on Care. Each of the 11 EURORDIS Award recipients is making a significant and unique contribution to improving access to the many different kinds of Care people living with a rare disease need and deserve. Working together, we are starting to make a real difference to the millions of patients and families throughout Europe who live with a rare disease," **- Yann Le Cam, Chief Executive Officer, EURORDIS**

The prestigious EURORDIS Awards 2014 were determined by process of vote by the EURORDIS Board of Directors from over 130 nominations received from the general public, EURORDIS members (rare disease patient advocacy groups from all over Europe and beyond), and EURORDIS volunteers and staff.

Pictures from the event will be available for use. Contact lara.chappell@eurordis.org for more information.

This year, the following awards were granted:

LIFETIME ACHIEVEMENT AWARD

Marlene Haffner, MD, MPH

For over 30 years, Dr. Marlene Haffner has had an immeasurable impact upon the development of orphan drug therapies. Dr. Haffner worked as the Director of the Office of Orphan Products Development at the United States of America Food and Drug Administration (FDA) for over twenty years. In this role she was responsible for the administration of the US Orphan Drug Act, the first act of this type in the world. Her influence in this role impacted far beyond the USA, as she applied her valuable knowledge and experience to assist the development of similar orphan drug programmes in Japan, Australia, and the EU, amongst other countries and regions. The EURORDIS Lifetime Achievement Award goes to Dr. Marlene Haffner in recognition of her strong, lifetime dedication and commitment to addressing the needs of people with rare diseases.

POLICY MAKER AWARD

Antonyia Parvanova, Member of European Parliament

Dr. Antonyia Parvanova has shown dedication and commitment in addressing the needs of people living with rare diseases as a Member of European Parliament, in her support of crucial amendments in EU legislation that have a positive impact on the rare disease community. Dr. Parvanova publically voices the importance of equality in access to health services, to provide affordable, high quality and safe medical care for all European citizens. Such relentless advocacy activity has allowed this topic to rise in the European political agenda leading to the adoption of the Directive on the application of Patients' Rights in Cross-border Healthcare. As Rapporteur of the EU Directive on Medicinal Products for Human Use: transparency of measures regulating the prices, Dr. Parvanova has shown substantial commitment to improving rare disease patients' lives.

EUROPEAN RARE DISEASE LEADERSHIP AWARDS

Professor Guido Rasi, became Executive Director of the European Medicines Agency (EMA) in 2011 and has been pivotal in increasing the transparency of the EMA's work. He has encouraged dialogue between patients, heads of scientific committees, and health technology assessment (HTA) bodies, and promotes access to clinical trial data. Professor Rasi's overarching leadership and drive for collaboration between all rare disease stakeholders has facilitated the road to the

authorisation of orphan products in Europe.

Paola Testori Coggi, biologist by education, was nominated as Director General for Health and Consumer Protection at the European Commission in 2010. In this position, she has facilitated European cooperation in the field of rare diseases through several important steps: the approval of the rare disease priority within the Health for Growth Programme; the adoption of a fundamental piece of legislation to facilitate patient mobility through the Directive on Patients' Rights in Cross-border Healthcare; responsibility for the European Medicines Agency within her Directorate General.

Professor Luca Pani undertook the role of Director General of the Italian Medicines' Agency (AIFA) in 2011. In this position he has emphasised the importance and urgency in creating orphan medicinal products, ensuring investment into rare disease research and granting the access to sustainable and successful treatments for those living with a rare disease. As a leader, Professor Pani has used initiative and experience to drive the equal access to cures for rare diseases, not only in Italy, but in Europe as a whole.

SCIENTIFIC AWARD

Professor Hans-Hilger Ropers

Hans-Hilger Ropers is Director at the Max-Planck-Institute for Molecular Genetics in Berlin and Professor of Human Genetics at the Humboldt University. A clinical geneticist, Professor Ropers has made many contributions to the molecular elucidation of monogenic disorders with a focus on eye diseases, deafness and particularly intellectual deficit. His department forms part of the European MRX Consortium which plays a central role in researching the molecular causes of X-linked intellectual deficit. It is in recognition of Professor Roper's scientific excellence and untiring dedication to put single gene disorders into focus worldwide that EURORDIS awards Professor Hans-Hilger Ropers the EURORDIS Scientific Award 2014.

MEDIA AWARD

Rick Guidotti

Successful fashion photographer Rick Guidotti launched the non-profit organisation, Positive Exposure, to change public perceptions of people living with genetic, physical and behavioural differences. The association runs educational and advocacy programs, organises exhibitions in public places and works with

other NGOs and medical societies to give “positive exposure” to the beauty of those living with rare diseases. Internationally recognised, his work raises public awareness for the beauty of difference. It is in the essence of his approach to changing the perception of beauty on a global scale for people with rare diseases that EURORDIS awards the EURORDIS Media Award 2014 to photographer Rick Guidotti.

COMPANY AWARD

Swedish Orphan

Swedish Orphan Biovitrum (Sobi), established in 2001, is an international healthcare company dedicated to rare diseases, focusing on developing innovative treatments across four key therapeutic areas: haemophilia, inflammation/autoimmune diseases, inherited metabolic diseases and oncology. Sobi has a robust portfolio of treatments and a well-established track record of creating successful dialogue with patient communities, demonstrating their objective for a transparent and progressive rare disease framework. This award recognises the excellence and consistency of Sobi’s work.

COMPANY AWARD

Orphan Europe

Orphan Europe, part of the Recordati group, has 25 years’ experience in bringing orphan medicinal products to the market. Seven orphan products produced by Orphan Europe have been authorised to date and this rich portfolio of successful treatments is supported by a robust pipeline of medicines in development. Orphan Europe’s support in the development of European Reference Networks has helped to provide European guidelines for best care and treatment of rare disease patients in specific areas. Strong alliances with patient organisations mark the central point of Orphan Europe’s platform for therapy development.

PATIENT ORGANISATION AWARD

Allianz Chronischer Seltener Erkrankungen (ACHSE)

The German National Alliance for Chronic Rare Diseases (ACHSE) has been instrumental in turning Germany into one of the most committed Member States in the European Union for rare diseases in the fields of research, information, healthcare organisation and drug development. As an umbrella organisation, it has strengthened the voice of rare diseases in Germany and acts as a role model for other Member States. This is especially true in recognising its outstanding

achievement to the progress of the German National Plan for Rare Diseases, which was presented to the German Health Minister in September 2013.

VOLUNTEER AWARD

Lise Murphy

As an individual affected by Marfan syndrome, Lise Murphy has used her experience and patient expertise generously for the benefit of all the rare disease community. In 2003 she became a member of the Board of Directors of the Swedish Marfan Organisation, (Svenska Marfanföreningen), and in 2004 became its Chairperson. Reaching beyond her own disease community, Lise Murphy has helped to catalyse the rare disease movement as a whole in Sweden. Lise Murphy has been instrumental in closing the gap between patients, health care professionals and pharmaceutical agencies, demonstrating the importance of patient dialogue. Her relentless dedication to EURORDIS and the rare disease community as a whole makes her a truly deserving recipient of the EURORDIS Volunteer Award 2014.

About Rare Disease Day

Rare Disease Day is an annual, awareness-raising event initiated and coordinated by EURORDIS at the international level and organised by National Alliances of Rare Disease Patient Organisations at the national level. Rare Disease Day is held on the last day of February each year - a rare day for rare people. The Rare Disease Day 2014 Ambassador is Sean Hepburn Ferrer, son of the actress and philanthropist Audrey Hepburn, who died from a rare cancer. For more information about Rare Disease Day, please visit the site: www.rarediseaseday.org.

About the EURORDIS Gala Dinner

The third EURORDIS Black Pearl Gala Dinner, celebrating Rare Disease Day 2014, is a fundraising event of European and high international standard in support of essential concrete actions to support patients and patient advocates as well as to raise public awareness. For more information, please visit the Gala Dinner website at galadinner.eurordis.org.

About Rare Diseases

Rare diseases are often life threatening or chronically debilitating diseases. They frequently affect children. Due to the low prevalence of each disease, medical experts are

About EURORDIS

EURORDIS is a leading International Non-Governmental Organisation (INGO) and is recognised as the largest European patient organisation in the field of rare diseases. **EURORDIS represents 614 rare disease organisations in 58 different countries**, covering more than 4,000 distinct rare diseases. EURORDIS is the voice of an estimated 30 million patients affected by rare diseases throughout Europe. For more information about EURORDIS and our activities, please visit our website at: www.eurordis.org.

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rare, knowledge is scarce, care and treatments are lacking, and research limited. The European Union considers a disease rare when it affects fewer than 1 in 2,000 citizens. Eighty per cent of rare diseases have a genetic origin. **Over 6,000 rare diseases** have been identified to date, **affecting approximately 30 million Europeans**.

While each disease is rare, collectively rare diseases affect more than 60 million people in Europe and the USA alone. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.