PRESS RELEASE

EURORDIS Awards 2013
for outstanding accomplishments in the field of rare diseases
presented in honour of Rare Disease Day in Brussels

27 February 2013 - The European Organisation for Rare Diseases (EURORDIS) last night presented the EURORDIS Awards 2013 for excellence in the field of rare diseases during the EURORDIS Black Pearl Gala Dinner held 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 - directly or indirectly - to reducing the impact of rare diseases on people's lives.

"We are honoured to acknowledge the commitment and achievements of this year's recipients of the EURORDIS Awards on the occasion of Rare Disease Day 2013. Each of this year's ten awardees contributes in their own unique way toward fostering the goals of cooperation embodied in this year's Rare Disease Day slogan: "Rare Disorders without Borders". We are proud that the rare disease community is becoming a model for multi-stakeholder partnership and international collaboration as well as for solidarity and unity throughout Europe," ***-Yann Le Cam, Chief Executive Officer, EURORDIS***

These prestigious awards are voted by the EURORDIS Board of Directors based on over 100 nominations received from EURORDIS members (rare disease patient advocacy groups from all Europe), volunteers and staff, with the aim of promoting leadership and excellence in favour of people living with rare diseases.

This year, the following awards were granted:

LIFETIME ACHIEVEMENT AWARD

Eva Luise Köhler

The EURORDIS Lifetime Achievement Award recognises the lifelong dedication and commitment of German Former First Lady Eva Luise Köhler to addressing the needs of people living with a rare disease. Eva Luise Köhler has also contributed significantly to the promotion of the Rare Disease cause in Germany and her achievements in this field have inspired other public figures in various European

countries.

POLICY MAKER AWARD

MEP Françoise Grossetête

The EURORDIS Policy Maker Award recognises Member of European Parliament Françoise Grossetête's unique long-standing dedication and commitment in addressing the needs of people living with rare diseases.

As MEP and Rapporteur on several legislations, two essential EU Regulations have been adopted: the EU Regulation on Orphan Medicinal Products in 1999 and the EU Regulation on Medicines for Paediatric Use in 2006.

MEP Grossetête interventions in favour of the EU Regulations on Advanced Therapy Medicinal Products, and in the discussions around rare disease patients' needs within the negotiations on the Cross Border Healthcare Directive, have been instrumental in achieving the best possible outcomes for rare diseases patients.

EUROPEAN RARE DISEASE LEADERSHIP AWARD

Dr Ruxandra Draghia-Akli

The EURORDIS European Rare Disease Leadership Award recognises Dr Ruxandra Draghia-Akli's dedication and commitment in addressing the needs of people living with rare diseases as Director for Health Research at the European Commission,

Dr Draghia-Akli brought visionary leadership, when initiating the International Rare Diseases Research Consortium (IRDiRC) on which she is currently serving as Chairperson.

SCIENTIFIC AWARD

Dr Ségolène Aymé

The EURORDIS Scientific Award recognises Dr Ségolène Aymé for her overall scientific excellence, promotion of European and International collaboration, and support of the patient community via Orphanet, the world's leading reference portal for expert validated rare disease and orphan drug information. Dr Aymé's current roles as Chairperson of the European Committee of Experts on Rare Diseases (EUCERD), Scientific Secretariat of the International Rare Disease Research Consortium (IRDiRC), and Chair of the Topical Advisory Group for Rare Diseases for the revision of the International Classification of Diseases at the World Health Organisation, are also recognised with this award.

MEDIA AWARD

Andrew Jack, Financial Times

The EURORDIS Media Award recognises the contribution of journalist Andrew Jack to improve understanding of rare diseases and the issues people living with rare diseases face via his articles appearing in the *Financial Times* newspaper and other publications over the past several years.

COMPANY AWARD

Celgene Corporation

The EURORDIS Company Award recognises Celgene Corporation's established track record in the area of orphan diseases with several orphan designations and approved medicines addressing rare disease patient needs, in addition to the company's robust pipeline of products under development. This award also recognises the efforts of Celgene to engage in quality dialogue and partnership with

EURORDIS and patient groups in relevant disease areas. Celgene has three authorised orphan medicinal products on the market in Europe, two for multiple myeloma and one for myelodysplastic syndrome. In addition, Celgene has several orphan-designated products under development, primarily for rare cancers.

COMPANY AWARD

Prosensa

The EURORDIS Company Award recognises Prosensa's innovation and promise in developing innovative medicinal products for rare diseases. This award also recognises the efforts of Prosensa to engage in partnership with patient groups in relevant disease areas and is an encouragement to further the development of and access to orphan medicinal products in Europe. Prosensa has received orphan designation status in Europe and the USA for six different exon-skipping compounds for treating Duchenne muscular dystrophy.

COMPANY AWARD

Genzyme, a Sanofi Company

The EURORDIS Company Award recognises the pioneering achievements of Genzyme, a Sanofi Company, as well as the company's actions and initiatives undertaken to ensure patient access to life-saving products for rare diseases. This award also recognises Genzyme's long-standing support of patient organisations, including EURORDIS, as well as initiatives to increase patients' access to Genzyme treatments. Genzyme has four authorised orphan medicinal products in Europe for Fabry disease, mucopolysaccharidosis I, Pompe disease, and lymphoma/multiple myeloma. Genzyme currently has several products under development for other indications, including Gaucher disease.

PATIENT ORGANISATION AWARD

Alström Syndrome UK

The EURORDIS Patient Organisation Award recognises Alström Syndrome UK for its long-term commitment and outstanding achievements for Alström Syndrome patients. Kay Parkinson created this inspiring and exemplary patient organisation after losing her two children because of late diagnosis of Alström disease. Kay studied law in order to better defend her children's interests when she launched the patient organisation. One of the key achievements of Alström UK is the development of patient led, NHS funded multi-disciplinary clinics for Alström Syndrome. Alström UK is a also partner in the Euro-WABB project, an EU Rare Diseases Registry for Wolfram syndrome, Alström syndrome, Bardet-Biedl syndrome and other rare diabetes syndromes.

VOLUNTEER AWARD

Lesley Greene

The EURORDIS Volunteer Award recognises Lesley Greene as a true pioneer of the rare disease movement in Europe and for her outstanding and continued contribution as a volunteer for the rare disease community. Ms Greene's numerous appointments include the current position of Vice-Chair of the Committee for Orphan Medicinal Products at the European Medicines Agency. She previously served as EURORDIS President (2001-2003) and is Co-Founder and former Vice President of CLIMB, a national umbrella organisation working on behalf of children, young people and families affected by metabolic diseases in the UK.

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. For more information about Rare Disease Day, please visit the site: www.rarediseaseday.org.

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 561 rare disease organisations in 51 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.

About the EURORDIS Gala Dinner

The second EURORDIS Black Pearl Gala Dinner, celebrating Rare Disease Day 2013, 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 life threatening or chronically debilitating diseases. Due to the low prevalence of each disease, medical experts are rare, knowledge is scarce, care offering inadequate, and research limited. Very few treatments exist. 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.

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