PRESS RELEASE

First EURORDIS RD Awards

First EURORDIS Awards for outstanding accomplishments in the field of rare diseases presented in Brussels on International Rare Disease Day

March 1, 2012 - The European Organisation for Rare Diseases (EURORDIS) last night presented the First EURORDIS Awards for excellence and leading work in the field of rare diseases, at its EURORDIS Gala Dinner on Rare Disease Day.

The EURORDIS Awards are designed to recognise the outstanding commitment and achievements of 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.

These prestigious awards are judged by the EURORDIS Board of Directors based on over 100 nominations received from EURORDIS members, volunteers and staff, with the aim of promoting leadership and the highest achievements in favour of people living with rare diseases.

The following awards were granted:

PATIENT ORGANISATION AWARD

French Muscular Dystrophy Association (AFM-Telethon)

In recognition of its unmatched support for the cause of rare diseases, rare disease patients' organisations throughout France and EURORDIS, as well as its outstanding success in increasing awareness, raising funds and widely supporting research.

VOLUNTEER AWARD

Michele Lipucci Di Paola, PhD

In recognition of his unshakable commitment to improving conditions for people affected by rare diseases and particular dedication in Italy and on the European level.

EUROPEAN RARE DISEASE ACHIEVEMENT AWARD

Kerstin Westermark, MD, PhD

In recognition of her expertise, dedication and more than a decade of support to those with rare diseases, as the Swedish delegate and Chairperson of the Committee for Orphan Medicinal Products at the European Medicines' Agency in London.

POLICY MAKER AWARD

Androulla Vassiliou, JD

In recognition of her invaluable contribution to the rare disease community as Commissioner for Health and Consumer Policy from February 2008 to end of 2009,

when she supported the adoption of the Commission Communication: "*Rare Diseases: Europe's Challenge*", and the Council Recommendation on Rare Diseases which has laid the groundwork to improve conditions for rare disease patients in all Member States of the European Union.

SCIENTIFIC AWARD

Professor Alain Fischer and Professor Maria Grazia Roncarolo

On behalf of the San Raffaele Telethon Institute for Gene Therapy, Italy and the Hôpital Necker - Enfants Malades, France

As an outstanding example of scientific research and European collaboration that has resulted in the successful development of the first gene therapy for rare diseases related to Severe Combined Immuno Deficiencies, which has set the stage for the extension of gene therapy to other genetic diseases with a high unmet medical need and for which there is currently no cure.

MEDIA AWARD

BBC

In recognition of more than three decades of support, raising awareness on rare diseases and covering rare disease issues.

COMPANY AWARD

CSL Behring

In recognition of the company's long-standing commitment to treatment of rare disease patients, and most recently for its advances to benefit people living with primary immunodeficiency diseases and secondary immune-deficiencies.

Sigma-Tau Pharmaceuticals

In recognition of being an early leader in developing medicines for rare diseases and for remaining an exemplary partner in the rare disease community.

Shire

In recognition of Shire's pioneering initiatives, particularly for patients with Hunter Syndrome and Gaucher Disease, and for the company's ongoing commitment to people living with rare diseases throughout Europe.

"On Rare Disease Day 2012, EURORDIS wishes to draw public attention to the cause of People Living with Rare Diseases and to honour, with the EURORDIS Awards, the leaders and achievements of individuals or organisations that are striving to improve conditions for patients and families", Terkel Andersen, President of EURORDIS' Board of Directors.

About EURORDIS Gala Dinner

The first EURORDIS *Black Pearl* Gala Dinner, held on Rare Disease Day, 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. It is expected to become the annual rendez-vous to celebrate and further encourage the values of Europe to benefit its people.

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 - 28th or 29th - 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 502 rare disease organisations in 46 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 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 cures exist. While the diseases are rare, collectively they affect more than 60 million people in Europe and the US alone. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

The European Union considers a disease as rare when it affects fewer than 1 in 2,000 citizens. 80% of rare diseases have a genetic origin. 5,000-7,000 rare diseases have been identified to date, affecting 30 million Europeans today.

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