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Honorees Announced for 2007 NORD Tribute Banquet

DANBURY, CT (April 30, 2007)-----The National Organization for Rare Disorders (NORD) will honor a National Institutes of Health researcher, two members of Congress, the European Organization for Rare Disorders, and four companies at its 2007 Tribute Banquet in Washington, DC, on Monday, May 21. This is the annual event at which NORD honors individuals and companies that have made significant contributions improving the lives of those affected by rare diseases.

The two members of Congress being honored are the co-sponsors of the *Genetic Information Non-discrimination Act (H.R. 493)*, which was passed by the U.S. House of Representatives on April 25. The bill, which now goes to the Senate, makes it illegal for group health plans and health insurers to deny coverage to a healthy individual, or to charge higher premiums on the basis of genetic predisposition to specific diseases. It also bars employers from using an individual's genetic information when making decisions related to hiring, firing, job placement or promotion.

The 2007 Tribute Banquet honorees are:

NORD Leadership Awards

- Representative Judy Biggert (R-IL), U.S. Congress
- Representative Louise Slaughter (D-NY), U.S. Congress

NORD Public Health Leadership Award

- William Gahl, MD, PhD, National Institutes of Health

NORD International Leadership Award

- The European Organization for Rare Disorders (EURORDIS)

NORD Corporate Leadership Awards

- Alexion Pharmaceuticals for the development of Soliris to treat individuals with paroxysmal nocturnal hemoglobinuria (PNH)
- Genzyme Corporation, for the development of Myozyme to treat Pompe disease
- Karl Storz Endoscopy-America, Inc., for the development of the Karl Storz Semi-Rigid TTTS Fetoscopy Instrument Set for twin-twin transfusion syndrome
- Shire Human Genetics, for the development of Elaprase to treat mucopolysaccharidosis II (Hunter syndrome)

Congresswoman Biggert

Representing a suburban Chicago district in the U.S. House of Representatives, Congresswoman Biggert is an attorney and elected official with intense interest in education, health, and women's issues. She and Congresswoman Louise Slaughter are being recognized by NORD for their co-sponsorship of the *Genetic Information Non-Discrimination Act*. Congresswoman Biggert has taken a leading role in women's health research, issues related to homelessness and domestic violence, and she helped to craft the *No Child Left Behind Act*.

Congresswoman Slaughter

Currently in her 11th term in the U.S. House of Representatives, Congresswoman Slaughter holds a bachelor's degree in microbiology and a master's degree in public health. She began her career as a bacteriologist for the Department of Health in Kentucky. With her scientific background, Congresswoman Slaughter has taken a leadership role on issues related to science, patient protections, and biomedical research in the U.S. Congress. She chairs the House Committee on Rules.

William Gahl, MD, PhD

Dr. Gahl is an internationally known expert on cystinosis, Hermansky-Pudlak syndrome, alkaptonuria and disorders of sialic acid metabolism. He is a dedicated public servant with a profound allegiance to the study of rare diseases. He currently serves as clinical director of the National Human Genome Research Institute (NHGRI), head of the Section on Human Biochemical Genetics in the Medical Genetics Branch of NIH, and director of the Intramural Research Program of the NIH Office of Rare Diseases. In particular, he is being honored for his support of the rare-disease community through his work with the Office of Rare Diseases at NIH and the National Human Genome Research Institute. Dr. Gahl serves on the medical advisory boards of the Cystinosis Foundation, the Cystinosis Research Network, and the HPS (Hermansky-Pudlak syndrome) Network.

EURORDIS

The European Organization for Rare Disorders (EURORDIS) is being honored on the occasion of its 10th anniversary. EURORDIS represents more than 260 rare disease organizations in 29 European countries, covering more than 1,000 rare disorders. The European Union defines a rare disease as one that affects fewer than one person in 2,000 European citizens. Therefore, EURORDIS is the voice for 30 million patients. It was initially modeled on NORD, and NORD's president, Abbey Meyers, has served as

honorary president of EURORDIS since its founding. Today, EURORDIS provides important advocacy for patients and families, and promotes awareness, research, and the development of new treatments.

Alexion Pharmaceuticals

Alexion is being honored for the development of Soliris, the first treatment to be approved by the U.S. Food and Drug Administration (FDA) for the rare blood disorder known as paroxysmal nocturnal hemoglobinuria (PNH). This disorder affects approximately one in a million people. It is characterized by abnormal development of red blood cells. Components of the body's immune system attack and destroy these abnormal cells, causing anemia and debilitating weakness. In severe cases, the disease may lead to life-threatening heart attacks and strokes.

Shire Human Genetics Therapies

Shire is being honored for the development of Elaprase, an enzyme replacement therapy approved by the U.S. Food and Drug Administration as a treatment for MPS II in 2006. MPS II (or Hunter syndrome) is a genetic disorder that primarily affects males. Signs and symptoms are generally not apparent at birth but may become noticeable during the first year of life. They include inguinal hernias, ear infections, and frequent colds. Later manifestations may include a decline in cardiac function, breathing problems, characteristic facial features, and joint stiffness.

Genzyme Pharmaceuticals

Genzyme is being honored for the development of Myozyme, an enzyme replacement therapy for Pompe disease. This disease occurs as the result of a deficiency or lack of an enzyme necessary for normal muscle development. There are different forms, including an infantile form and later-onset forms. Myozyme has been approved by FDA for treating the infantile form. It continues to be evaluated as a treatment for the other forms. About 40,000 people have Pompe disease, and the disease can result in life-threatening respiratory problems. The infantile form is the most serious.

Karl Storz Endoscopy-America

This company is being honored for the development of the Karl Storz Fetoscopy Instrument Sets, which are surgical tools used in the treatment of twin-to-twin transfusion syndrome (TTTS). This is a rare condition in which there is an imbalance in the amniotic fluid of some identical twins. The imbalance is caused by an uneven flow of blood between the twins through shared blood vessels that are present when the twins share one placenta. The fetoscope is a telescopic camera used to view a fetus. After it identifies the shared blood vessels, a laser is used to destroy the shared blood vessels with heat and normalize the flow of blood between the twins.