



FOR IMMEDIATE RELEASE

Millions Around World To Observe Rare Disease Day

New York, NY--Liam's Lighthouse Foundation will be joining the National Organization for Rare Disorders (NORD) and others around the world in observing World Rare Disease Day on February 28, 2011. On this day, millions of patients and their families will share their stories to focus a spotlight on rare diseases as an important global public health concern.

“There are nearly 30 million Americans—and millions more around the world—affected by rare diseases,” said Peter L. Saltonstall, president and CEO of NORD. “Everyone knows someone with a rare disease. But, while many of these diseases are serious and lifelong, most have no treatment and many are not even being studied by researchers. This leaves patients and families without hope for a better future.”

A rare disease is one that affects fewer than 200,000 Americans. There are nearly 7,000 such diseases affecting nearly 30 million Americans.

World Rare Disease Day was launched in Europe four years ago and last year was observed in 46 nations. It is always observed on the last day of February. On that day, patients and patient organizations will post stories, videos and blogs online and host events to raise awareness of these diseases, which are often called “orphans”.

Liam's Lighthouse Foundation represents patients and families affected by Histiocytosis and is thrilled to announce their Histiocytosis Rare Disease Day Awareness Campaign which will take place at The Today Show in NYC on Rare Disease Day, February 28, 2011. Liam's Lighthouse Foundation is dedicated to raising awareness of all 6 types of Histiocytic diseases, providing support to families affected by these diseases, and funding research. It all began with a little boy named Liam.

Can you imagine hearing your 14 month old child has been diagnosed with a highly fatal blood disease after 10 months of multiple visits to the pediatrician, specialists, diagnostics, and misdiagnosis after

misdiagnosis only to find out that his only chance for survival is to undergo a bone marrow transplant?
How does a parent cope with that?

Liam Schulze was diagnosed with Hemophagocytic Lymphohistiocytosis (HLH). HLH is a life-threatening immunodeficiency. It affects people of all ages and ethnic groups. Common symptoms are fevers, enlarged spleen, low blood counts, liver abnormalities, and sometimes neurological involvement. The onset of HLH is usually early in life, and a permanent cure can only be achieved through a bone marrow transplant. In some ways, histiocytosis is similar to cancer, and has historically been treated by oncologists with chemotherapy and steroids to try and quiet the disease, ultimately needing a bone marrow transplant, but it is not cancer. It was once reported that HLH had a prevalence of 1 in a million. It has recently been reported to be 1 in 150,000, although this may be an underestimate. Unpublished observations are showing this to be more like 1 in every 50,000 live births due to better detection. Histiocytosis qualifies as an orphan or “rare” disease since there is no government funding for much needed research. “It continues to amaze me how many children are affected by this disease and it is still referred to as “rare”. I do not believe it is rare. I believe it is misdiagnosed or not diagnosed at all.” states Liam’s mom.

Liam lost his battle 41 days post bone marrow transplant for HLH on September 9, 2009. Liam’s mother, Michelle, was inspired to continue the fight he no longer could against this horrific disease. She founded Liam’s Lighthouse Foundation in her brave little boy’s memory shortly after he passed away. “If this disease is not diagnosed, it cannot be treated and if it’s not treated, it is fatal” states Michelle. It continues to bother her that physicians are still not able to identify this disease even though it has been diagnosed since at least 1985. Physicians are not diagnosing these children in time and then it's too late for treatment to be effective in more than one of these diseases. This is why Michelle founded Liam’s Lighthouse Foundation. The harsh reality is that most physicians are not familiar with HLH, many of which have never even heard of the disease. HLH is going undiagnosed or misdiagnosed, and many children have died before a diagnosis could be made due to its rapid, fatal nature. If Liam had been diagnosed at 4 months of age when his symptoms first became apparent, he may still be alive today. Educating the community, parents, and our primary care medical professionals is mandatory so a diagnosis can be made. Parents are their children's best advocates and are part of the team to provide their children the best possible chance for early detection and survival.

This year, supporters of Liam’s Lighthouse Foundation will again travel to New York City on this day. The plan is to fill the crowd outside with as many “Faces of Histo” as possible. There will be over 55 “Faces of Histo” in the crowd. Volunteers including Histo parents from all over the world, including the United Kingdom will be attending this event. As Michelle continues to emphasize, “There is power in numbers, and in numbers we can make a difference”.

ABOUT LIAM'S LIGHTHOUSE FOUNDATION: Liam's Lighthouse Foundation, a non-profit, tax-exempt organization, was established to create and provide educational material and awareness about Hemophagocytic Lymphohistiocytosis (HLH) including Histiocytic Disorders, and to distribute unbiased, factual information to physicians, hospitals, and the community regarding this disease. Our focus is also to bring families affected by Histiocytosis together and offer support through a variety of resources. We aim at raising much needed funds for continued education of physicians and research to develop safer and more effective treatment methods and ultimately a cure. LLF is also dedicated to bring awareness to the community about the importance of becoming a blood and bone marrow donor.

In the U.S., the coalition supporting Rare Disease Day includes patient organizations and advocacy groups, medial professionals and associations, government agencies, researchers, and companies developing treatments for rare diseases.

Rare Disease Day 2011 activities in the U.S. will include creating an online library of two-minute videos about specific rare diseases and how they affect patients' daily lives. Also, patients across the nation will help NORD create a database of physician experts. Patients will share their personal stories through a survey hosted by NORD and the Pew Research Center, and there will be a drive to enlist support for a new Rare and Neglected Diseases Congressional Caucus.

"More than half of the people who have rare diseases are children," Saltonstall said. "Challenges faced by patients and their families include delayed diagnosis, few treatment options, and difficulty finding medical experts. Many rare diseases have no approved treatment. Insurance may not cover treatments that aren't approved. Also, treatments for rare diseases tend to be more expensive than those for common diseases."

In 1983, the *Orphan Drug Act* was passed by Congress to create financial incentives for companies to develop treatments for rare diseases. Since then, more than 360 orphan drugs and biologics have been approved by the Food and Drug Administration (FDA). It is estimated that from 11 to 14 million Americans benefit from these products, but that still leaves more than 15 million Americans with diseases for which there is no approved treatment.

For more information about Rare Disease Day activities in the U.S., go to www.rarediseaseday.us. For information about global activities, go to www.rarediseaseday.org.

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