



## RARE DISEASE DAY

### FOR IMMEDIATE RELEASE

#### **Rare Disease Advocates Commemorate Worldwide Awareness Day at the Utah State House**

**(Salt Lake City, Utah, 2/26/14)**----- Join rare disease patients, caregivers and other health care advocates as they share their stories on Friday, February 28, 2014, to observe Rare Disease Day in Utah. Rare Disease Day occurs each year on the last day of February, and on this day, millions of patients and their families will share their stories to focus a spotlight on rare diseases as a global public health concern. The day is observed in more than 70 nations.

Patient advocates have joined with the National Organization for Rare Disorders (NORD), the national sponsor of the day in the US, to organize this special event for legislators, legislative staff, the public and the media. The event will be held Utah State Capitol.

During this event attendees will have the opportunity to meet/ hear from: Steve Mikita, JD; Robert Selliah, PhD; Paul Melmeyer; Eliot Brinton, MD, FAHA, FNLA; Mike Spigarelli, MD, PhD; Sussie Wiet, MD

The purpose of this event is to raise awareness at the state level for the 1 in 10 individuals living with a rare disease and the challenges they face. Many important decisions related to rare diseases are made at the state level, and the implementation of the *Affordable Care Act* has highlighted the increasingly important role of state policies and programs in assuring that the healthcare needs of the American public are addressed.

Issues of importance to the rare disease community that may be decided at the state level including newborn screening, support services for families coping with complex medical needs, an environment that promotes innovative medical research and product development, and insurance practices that assure patient access to medically necessary therapies.

A rare disease is one that affects fewer than 200,000 Americans. There are nearly 7,000 such diseases affecting nearly 30 million Americans, according to the National Institutes of Health (NIH).

Two-thirds of those affected by rare diseases are children, and the diseases tend to be serious and lifelong. Even so, most rare diseases have no approved treatment, and many are not even being studied by medical researchers. Often, research on rare diseases is funded by the families and friends of patients or by patient organizations.

Participating Organizations Include:

- (1) **American MedChem Nonprofit Corporation (AMC)**, Salt Lake City, UT. AMC is a nonprofit drug discovery research organization with a commitment to discover, develop and provide safe and effective new medicines for childhood rare and neglected diseases.  
<http://americanmedchem.org>
- (2) **The Foundation for Children with Atypical HUS**, Roy, UT, dedicated to fighting aHUS through research, outreach, education and rare disease advocacy. [www.atypicalhus.net](http://www.atypicalhus.net)
- (3) **Familial Adenomatous Polyposis Foundation**, Park City, UT, serving the hereditary colon cancer community by connecting patients and their loved ones to educational and social resources, providing financial assistance for screening, treatment, counseling, and family planning, and by promoting requisite research and health care initiatives.  
[www.hcctakesguts.org](http://www.hcctakesguts.org)
- (4) **Pediatric Motor Disorders Research Program**, University of Utah, Dr. Kathy Swoboda, Director, focused on understanding and treating a group of disorders characterized by defects in motor function with onset in infancy or early childhood. Our dedicated team of clinical and research professionals sees patients from around the world and participates in cutting edge research and clinical trials.  
<http://medicine.utah.edu/neurology/research/pediatric-motor-disorders/index.php>.

Rare Disease Day was launched in Europe in 2008 by EURORDIS, the organization representing rare disease patients in Europe. It is now observed in more than 70 nations, and is sponsored in the U.S. by NORD.

For more information about Rare Disease Day in the U.S., go to [www.rarediseaseday.us](http://www.rarediseaseday.us). For information about global activities, go to [www.rarediseaseday.org](http://www.rarediseaseday.org).

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