NORD News for November - The Voice of the Community

Published Online: *Wednesday, Nov 16, 2016*

News from NORD and its Member Organizations

NORD Ready to Address New Challenges with Changes in Federal Government

NORD President and CEO Peter L. Saltonstall issued a statement of continued commitment to patients as preparations begin for the new administration and Congress. "The long election season has ended and we have a new President, a new Congress, and a new set of challenges for NORD and the rare disease community," he said. In particular, Saltonstall noted that NORD would be monitoring proposed changes to the Affordable Care Act to ensure that key provisions would not be lost.

NORD, Global Genes and EveryLife Foundation Issue Joint Call for Support for 21st Century Cures

Calling it a "Day of Action", NORD, Global Genes and the EveryLife Foundation joined together in a call for support for 21st Century Cures on November 15th, urging their members and social media followers to call their legislators.

Fibrous Dysplasia Foundation and NORD Launch Study of Fibrous Dysplasia/McCune Albright Syndrome

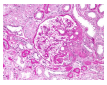
This new study – the FD/MAS Patient Registry – creates a platform for patients around the world to share information about FD/MAS. The intent is to build an international resource to be used by scientists in future research. The registry will be used to gather information about the patient experience and disease progression to better understand FD/MAS.

NORD Patient/Caregiver Speakers Get Enthusiastic Welcome From Students at Northeastern University

Genetic counseling and pharmacy students at Northeastern University turned out in large numbers to hear two speakers from NORD's Patient/Caregiver

Latest Articles

FDA Approves Darzalex for Multiple Myeloma



Phase 2 Study Focal Segmental Glomerulosclerosis Shows Positive Results

Sobi's Elocta for the Treatment of Hemophilia A Approved in Kuwait

Rare Disease Doesn't Stop Model from Shining

Tweets by @RareDR

Rare Disease Report
@RareDR

Phase 2 Study Focal Segmental Glomerulosclerosis Shows Positive Results

raredr.com/news/phase-2-s…

5h

Rare Disease Report
@RareDR

Sobi's Elocta for the Treatment of Hemophilia A Approved in Kuwait

raredr.com/news/treatment…

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Speakers Bureau share their experiences with two rare diseases – myocarditis and congenital central hypoventilation syndrome. To schedule a speaker at your university or community event, write to education@rarediseases.org.

Nominations are Open for NORD's Rare Impact Awards

January 13th is the deadline for submitting nominations for 2017 NORD Rare Impact Awards. These awards honor individuals and organizations for notable work in rare disease advocacy, science, patient care, ethics, research and public policy. The 2017 Rare Impact Awards celebration will take place in the amphitheater of the Ronald Reagan Building and International Trade Center, the largest structure in Washington DC.

NEWS FROM NORD MEMBER ORGANIZATIONS

Deadline is December 1 to Sign Up for Certification Exam in Rare Neuroimmunologic Disorders

The Consortium of Multiple Sclerosis Centers has announced that the next Certification Examination in Rare Neuroimmunologic Disorders will take place in January. Dec. 1 is the deadline to register. This certification is intended to validate quality of care for licensed health professionals providing care for patients with rare disorders such as transverse myelitis, neuromyelitis optica spectrum disorder, optic neuritis and more.

PNH Research and Support Foundation to Merge with Aplastic Anemia & MDS International Foundation

These organizations, strategic partners since 2013, have announced a merger that will become effective on January 1, 2017. Activities that have defined the PNH Foundation, such as online chat, the PNH Walk in New York City, and a travel assistance fund, will continue.

New Guidelines Published for Diagnosis and Management of Cystathionine Beta-Synthase Deficiency

The new guidelines have been published in the Journal of Inherited Metabolic Disease. The HCU Network Australia will host an online webinar featuring Doctor Andrew Morris, the primary author of the new guidelines, on November 29 at 8 pm Australian time. The webinar will be streamed live from the HCU Network website. Use the time converter to check for local times.

PSC Partners Seeking a Cure Launch Campaign to Educate About Difference Between PSC and PBC

The disease formerly known as primary biliary cirrhosis is now called primary biliary cholangitis and PSC Partners Seeking a Cure is taking this opportunity to promote awareness of the difference between that condition and primary sclerosing cholangitis. Despite sharing certain similar characteristics, these two diseases are not the same and require different treatments and monitoring. Learn more on the PSC Partners website.

Patients Are Being Recruited for LPL-Deficiency Study

Adult patients with lipoprotein lipase deficiency (LPLD) in the US and Canada may be eligible for a study of alipogene tiparvovec. LPLD is a rare, autosomal recessive disorder of lipid metabolism. Additional information can be found on clinicaltrials.gov under study ID NCT02904772.

Scleroderma Research Foundation to Host Evening of Comedy and Cuisine

On Monday, Dec. 12, Scleroderma Research Foundation Board Member Bob Saget will host an evening of food, wine and comedy with guests Andy Cohen,

John Oliver, Jeff Ross and Chefs Susan Feniger and Mary Sue Milliken. Cool Comedy/Hot Cuisine.

Transverse Myelitis Association to Host Podcasts

Upcoming podcasts offered by the Transverse Myelitis Association are: Contraception, Pregnancy, Delivery and Childcare After a Diagnosis of ADEM, NMOSD, or TM, November 21; and Quality of Life in a Wheelchair, December 19.

Lipoprotein(a) Foundation Highlights Studies

The Lipoprotein(a) Foundation has highlighted several studies presented at the recent American Heart Association's scientific session in New Orleans.

CHOPS Surgeon-in-Chief Recognized by Congenital Hyperinsulinism International

N. Scott Adzick, MD, of Children's Hospital of Philadelphia, was honored at the third annual Sugar Soiree of Congenital Hyperinsulinism International. Dr. Adzick is one of the founders of the CHI Center at CHOP.

CDLS Foundation Announces Research Grants

The Cornelia de Lange Syndrome Foundation has announced research grants totaling $45,000.