NEWS FROM NORD

AN UPDATE FOR OUR MEMBERS AND FRIENDS — SUMMER 2012

NORD HAILS FDA SAFETY AND INNOVATION ACT AS MONUMENTAL STEP FORWARD!

Approval Culminates Years of Advocacy, Education and Relationship Building Led by NORD

Signed by President Obama on July 9, 2012, *the Food and Drug Administration (FDA) Safety and Innovation Act* includes the most groundbreaking measures for rare disease patients since the *Orphan Drug Act* of 1983, NORD says. And, as the long-standing and recognized policy leader and advocate for the rare disease community, NORD was instrumental in developing and shaping this legislation, which includes provisions of primary importance to the entire rare disease community.

"We at NORD set very high goals when the legislative process began a few years ago, and I am delighted that the legislation includes provisions that will advance patient access to safe and effective therapies," said NORD President and CEO Peter L. Saltonstall.

"This legislation represents true progress for people with rare diseases, who often face many struggles accessing treatments for their disorders. NORD's heritage, understanding, leadership and commitment to the community uniquely positioned us to identify and articulate the needs of people with rare diseases. The approval of this bill by Congress and ultimate signing into law by the President add to NORD's 30-year history of driving landmark legislation, which would not have been possible without the sustained efforts of our members and policy partners," Saltonstall added.

Established in 1983 by patient advocates who successfully brought about the enactment of the *Orphan Drug Act*, NORD has been an integral part of the development of the *FDA Safety and Innovation Act* since 2010. The Act will provide more than $6 billion in industry user fees to the FDA over the next five years to fund a share of the agency's review of drugs and medical devices. Specific to the rare disease patient community, the Act provides the following:

- Accelerated patient access to new medical treatments
- Expanded development of Humanitarian Use Devices, or medical devices for small patient populations
- Accelerated development of "breakthrough therapies"– those that show early promise
- Enhanced consultation between FDA and rare disease medical experts
- A rare pediatric disease priority review voucher incentive program
- Resolution of conflict-of-interest issues that kept rare disease medical experts and patient advocates from participation on advisory committees

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The Honorable Kay Hagan

The Honorable Cliff Stearns

The Honorable Edolphus (Ed) Towns

NORD HONORS RARE DISEASE INNOVATORS

Celebration Highlights Progress in Advocacy, Awareness, Research and New Treatments

Before an audience of 500 members of the rare disease community, NORD honored members of the U.S. Congress, patient advocates, medical researchers, and companies that have brought new therapies to market on Tuesday, May 15, at Union Station in Washington, DC.

This took place at NORD's annual "Partners in Progress Celebration", an event that focuses on the ways in which stakeholders in the rare

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SAVE THE DATE:

2ND ANNUAL U.S. CONFERENCE ON RARE DISEASES AND ORPHAN PRODUCTS TO TAKE PLACE OCTOBER 22 - 24

This conference, which got rave reviews and drew together nearly 400 patient advocates, medical researchers, government officials, industry leaders, and investors last year, will take place in Washington, DC on October 22-24. This year's theme, "Shaping the Future Now", will emphasize the forward-thinking focus of the conference.

There will be both plenary sessions and small group sessions. The small group sessions will be organized according to three themes: policy, research and regulation, and special challenges.

Co-sponsor with NORD is DIA (the Drug Information Association). In addition, there will be major presence from NIH (National Institutes of Health), FDA (Food and Drug Administration), and Duke University School of Medicine.

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FDA SAFETY AND INNOVATION ACT:
A MAJOR SUCCESS FOR PATIENT ADVOCATES

(This op-ed piece was originally published on the editorial website hosted by BIO, the Biotechnology Industry Organization.)

The Food and Drug Administration Safety and Innovation Act (FDASIA) includes the most groundbreaking measures for rare disease patients and their families since the *Orphan Drug Act* of 1983. And, just as the rare disease patient community—with NORD as its unifying voice—played a major role in the *Orphan Drug Act*, NORD and rare disease patients have taken a leadership role in developing and shaping this new legislation and in securing its enactment in a timely manner.

Crafted for the purpose of reauthorizing the *Prescription Drug User Fee Act* (PDUFA), the *FDA Safety and Innovation Act* includes far-ranging provisions of critical importance to rare disease patients. These reflect, in many cases, years of advocacy, education and relationship-building by NORD and its member organizations on behalf of patients.

Of particular importance to NORD and the 30 million Americans with rare diseases it represents are the provisions related to:

- Accelerated patient access to new medical treatments
- Resolution of conflict-of-interest provisions introduced in the previous PDUFA reauthorization
- Accelerated development of "breakthrough therapies" that show early promise
- Enhanced FDA consultation with rare disease medical experts
- A rare pediatric disease priority review voucher incentive program
- The development of Humanitarian Use Devices (medical devices for small patient populations)

PDUFA, as followers of this website know, provides essential funding for FDA by authorizing the agency to charge user fees to companies seeking to have products reviewed. Originally enacted in 1992, it must be reauthorized every five years. The deadline for the fifth reauthorization was September 2012.

Over the past two years, in anticipation of the 2012 reauthorization, NORD has been meeting with senior FDA officials to help them better understand the concerns and needs of patients, and the challenges of orphan product development.

At the same time, we and our advocacy partners have been working very closely with Members of Congress and their staff members, including the Chairs and Ranking Members of the Senate HELP Committee and House Energy & Commerce Committee, where the first Congressional debate related to the PDUFA reauthorization took place.

In past reauthorization cycles, the process was contentious at times, but this year it was bipartisan and collaborative in both the Senate and the House. We believe that happened, in part, because of the effective groundwork laid by many stakeholders and in particular by the patient community.

The stakes remain high for rare disease patients and patient organizations. Of the nearly 7,000 diseases considered rare ("rare" is defined as affecting fewer than 200,000 Americans), only about 250 have FDA-approved therapies. That is a huge challenge for the patient community, and there is a growing sense of urgency to accelerate the development of treatments.

At the same time, rare disease patients don't want to become second-class citizens with respect to treatments. They want the same reasonable expectation of safety and efficacy from medical treatments that other patients would have.

A Strong Commitment to Rare Disease Patients by FDA

Some very promising developments for rare disease patients to come out of this process were outlined in a document submitted by FDA Commissioner Margaret Hamburg, MD, to Congress in January 2012 (the PDUFA Authorization Performance Goals and Procedures for Fiscal Years 2013-2017). In that document, FDA committed to:

- Increased staffing and expansion of the Rare Disease Program in CDER (the Center for Drug Evaluation and Research) and CBER (the Center for Biologics Evaluation and Research)
- Increased FDA efforts to assure good communication among product reviewers, industry and patients
- Modernization and enhancements related to non-traditional clinical trial design for orphan drug development
- Enhanced staff training for FDA reviewers with regard to orphan product reviews
- Enhanced interaction between FDA's Rare Disease Program staff and review teams

In Congress, Several Important Bills Were Incorporated into FDASIA

In Congress, several bills that initially were introduced separately later were incorporated into the PDUFA reauthorization. Those of particular importance to rare disease patients and supported by NORD included:

The TREAT Act

Introduced by Senator Kay Hagan (NC), the *Transforming the Regulatory Environment to Accelerate Access to Treatments* (TREAT) *Act* included provisions to expand FDA's Accelerated Approval pathway; address the conflict-of-interest issue; provide greater clarity, consistency and transparency in the review process; and encourage innovation and adoption of modern scientific tools in regulatory science. NORD worked closely with Senator Hagan and her staff in developing and promoting this bill.

The FAST Act

Introduced by Representatives Cliff Stearns (Fl-6) and Edolphus "Ed" Towns (NY-10), the *Faster Access to Specialized Treatments* (FAST) *Act* was aimed at accelerating the development of treatments for rare diseases while maintaining FDA's high standards for safety and efficacy. NORD and other patient advocates provided significant input to Representatives Stearns and Towns.

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By Peter L. Saltonstall,
President and CEO,
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(NORD)

SUMMER ISSUE – 2012

NORD
National Organization for Rare Disorders

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RARE DISEASE DAY: RE-CAP



As the national sponsor for Rare Disease Day, NORD extends sincere appreciation to all who supported the 2012 campaign. It was our most successful to date.

Here are a few highlights from Rare Disease Day 2012. Don't forget to follow this event throughout the year on the website (www.rarediseaseday.us) and on Facebook (facebook.com/RareDiseaseDay.us).

SUPPORT FROM MEMBERS OF CONGRESS

Several legislators issued press releases, videos and tweets in support of Rare Disease Day. Senators Sherrod Brown (OH) and John Barrasso (WY) sponsored a Senate resolution in honor of the day.

RARE DISEASE DAY PARTNERS

By the conclusion of this year's campaign, 710 patient organizations, companies, and government agencies had signed on as Rare Disease Day Partners on the Rare Disease Day US website hosted by NORD. This is the strongest support of any year to date.

SPECIAL EVENTS AT FDA AND NIH

FDA hosted its first-ever Patient Advocacy Day, with assistance from NORD and Genetic Alliance. The event, which took place at the White Oak Campus of FDA, drew a capacity crowd. It was the first time many patient organization leaders had visited FDA. NIH hosted a scientific symposium the day before the FDA event.

HANDPRINTS ACROSS AMERICA PHOTO GALLERY

More than 200 photos were submitted to the Rare Disease Day US website photo gallery, featuring the Rare Disease Day logo in many locales and settings. NORD will continue to display this gallery throughout the year.

MEDIA RESULTS

Stories to educate and raise awareness appeared everywhere, as the day drew closer. These included stories on the ABC and CBS News websites, in industry publications, on the *Wall Street Journal Radio Network* and on television. NORD's RN did 27 TV and radio interviews on Rare Disease Day.

RESOURCES FOR TEACHERS AND STUDENTS

A genetic counseling graduate student, working with NORD's genetic counselor, created a curriculum that could be downloaded free from the Rare Disease Day US website. One teacher responded, "This is a great way to bring awareness about rare diseases to our students and expand their worldview." Eighty-three teachers across the U.S. downloaded the curriculum.

SPECIAL EVENTS

Events that ranged from scientific symposia to a concert featuring performers who have rare diseases were posted on the Rare Disease Day US website. Many companies hosted awareness events for their employees.

WATCH FOR NEWS ABOUT THE 2013 CAMPAIGN

EURORDIS, NORD and others on the world Rare Disease Day planning committee have already begun to work on the theme and plans for 2013. Watch for more information, and visit RareDiseaseDay.us for news about the national campaign and RareDiseaseDay.org for news about what's happening around the world.

VISIT US THROUGHOUT THE YEAR AT: RareDiseaseDay.us

NORD HONORS RARE DISEASE ADVOCATES AND INNOVATORS

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disease community work together toward their shared goal of improving the lives of patients.

"This is NORD's opportunity to celebrate excellence and to say thank you on behalf of the patient community to those who have advanced the cause during the previous year," said Peter L. Saltonstall, NORD president and CEO. "We look forward to this event each year, and we truly appreciate the accomplishments it honors."

Actress Patricia Richardson of TV's *Home Improvement*, *Strong Medicine* and *The West Wing* and NORD Board Chair Michael Scott emceed the event. All members of the rare disease community were invited.

Sarah MacDonald of MassBIO and
Blair Van Brunt of Shwachman-Diamond Syndrome Foundation

Pfizer table

Alexion representatives with Ziegler family

Amgen award presentation

THE 2012 PARTNERS IN PROGRESS HONOREES WERE:

NATIONAL HEALTH LEADERSHIP AWARDS

These awards honor leadership in public policy to improve the lives of patients and families affected by rare diseases.

The Honorable Kay Hagan, U.S. Senator (NC)
The Honorable Clifford Stearns, U.S. Representative (FL-6)
The Honorable Edolphus (Ed) Towns, U.S. Representative (NY-10)

POWER OF PARTNERSHIP AWARD

This award honors individuals and/or organizations for initiatives undertaken voluntarily on behalf of the rare disease patient community.

Blair Van Brunt of the Shwachman-Diamond Syndrome Foundation and the Massachusetts Biotechnology Council (MassBIO)

ABBEY S. MEYERS LEADERSHIP AWARD

Established in honor of NORD's founding president, this award honors a NORD Member Organization for strong and effective leadership on behalf of its members.

The Pulmonary Hypertension Association

PARTNERS IN PROGRESS CORPORATE AWARDS

These awards honor companies that have brought important and innovative treatments to market within the previous year.

Alexion Pharmaceuticals, Inc., for Soliris to treat patients with atypical Hemolytic Uremic Syndrome (aHUS)

Amgen, Inc., for Sensipar for patients with primary, severe parathyroidism who are unable to undergo surgical resection

Genentech, Inc./Biogen Idec, for Rituxan to treat, in combination with glucocorticoids, patients with Wegener's granulomatosis and microscopic polyangiitis

Pfizer, Inc., for Xalkori, approved with a companion diagnostic test to treat certain patients with late-stage, non-small cell lung cancers who express the abnormal ALK gene

Rare Disease Therapeutics, Inc., for Anascorp for treatment of patients for scorpion stings

PARTNERS IN PROGRESS AWARDS

This award honors patient/researcher partnerships driving progress through collaboration.

NIH Coalition of Patient Advocacy Groups (CPAG) and Rare Diseases Clinical Research Network (RDCRN)

Cystic Fibrosis Foundation and Vertex Pharmaceuticals, Inc.

Genentech award presentation

Cystic Fibrosis Foundation and Vertex representatives

Rare Disease Therapeutics and the Bray family

Rino Aldrighetti (center) of the Pulmonary Hypertension Association

NIH Office of Rare Diseases Research, CPAG, and NIH RDCRN

NORD AWARDS FIVE RESEARCH GRANTS

In May, NORD awarded five one-year grants for studies of rare diseases. The patient organizations that funded the grants (when applicable), investigators, their institutions, and their projects are as follows:

Congenital Skeletal Abnormalities Associated with Cat Eye Syndrome or Other Rare Chromosomal Disorders

Two One-Year Awards:

Kerby C. Oberg, MD, PhD
Associate Professor
Divisions of Human Anatomy and Pediatric Pathology
Loma Linda University, California
Characterization of the Lmx1b Regulatome During Limb Abnormalities

Francesa Mariani, PhD
Assistant Professor
Broad Center for Regenerative Medicine and Stem Cell Research, Department of Cell and Neurobiology
University of Southern California
Molecular Basis of Split Hand-Foot Malformations (SHFMs)

Primary Orthostatic Tremor (funded by the group OT Resource)

Two One-Year Awards:

Sabine Meunier, MD, PhD
Neurologist, Senior Researcher
Movement Disorders Clinic, Pitié-Salpêtrié Hospital
Paris, France
Is Cerebellum a Potential Therapeutic Target for Primary Orthostatic Tremor?

Julian Rodrigues, MBBS, FRACP
Australian Neuromuscular Research Institute
Perth, Western Australia
Pregabalin for the Treatment of Primary Orthostatic Tremor

Tarlov Cyst Disease

(Funded by the Tarlov Cyst Disease Foundation)

One One-Year Award:

Kieran Murphy, MB, BCh, FRCPC, FSIR
Principal Investigator
Professor & Vice Chair, Medical Imaging
Deputy Chief, Medical Imaging, University Health Network, Mt Sinai Women's' Hospital
Director, International Medical Affairs, University Health Network, Toronto Western Hospital
University of Toronto, Ontario, Canada
A Genetic Analysis of Patients with Tarlov Cyst Disease

Co-Investigators:

Chantal Morel, MD, FRCPC, FCCMG
Clinical and Metabolic Geneticist Assistant Professor
University Health Network/ Mount Sinai Hospital
Toronto, Ontario, Canada

Jordan Lerner-Ellis, PhD, FACMG
Director, Laboratory for Advanced Molecular Diagnostics, Mount Sinai Hospital
Assistant Professor, University of Toronto
Department of Laboratory Medicine and Pathobiology
Toronto, Ontario, Canada

Aaron Goldman, PhD
Director, Research Project Portfolio
Clinical Genomics Center – Gene Profiling Facility
Samuel Lunenfeld Research Institute
Mount Sinai Hospital
Toronto, Ontario, Canada

NORD's Medical Advisory Committee is in the process of reviewing responses to five additional Requests for Proposals to be awarded in 2012. Details are posted on NORD's website (www.rarediseases.org).

NORD LAUNCHES NEW ONLINE PHYSICIAN GUIDES

New platform will provide the latest information on specific rare diseases to help patients get earlier diagnosis and treatment

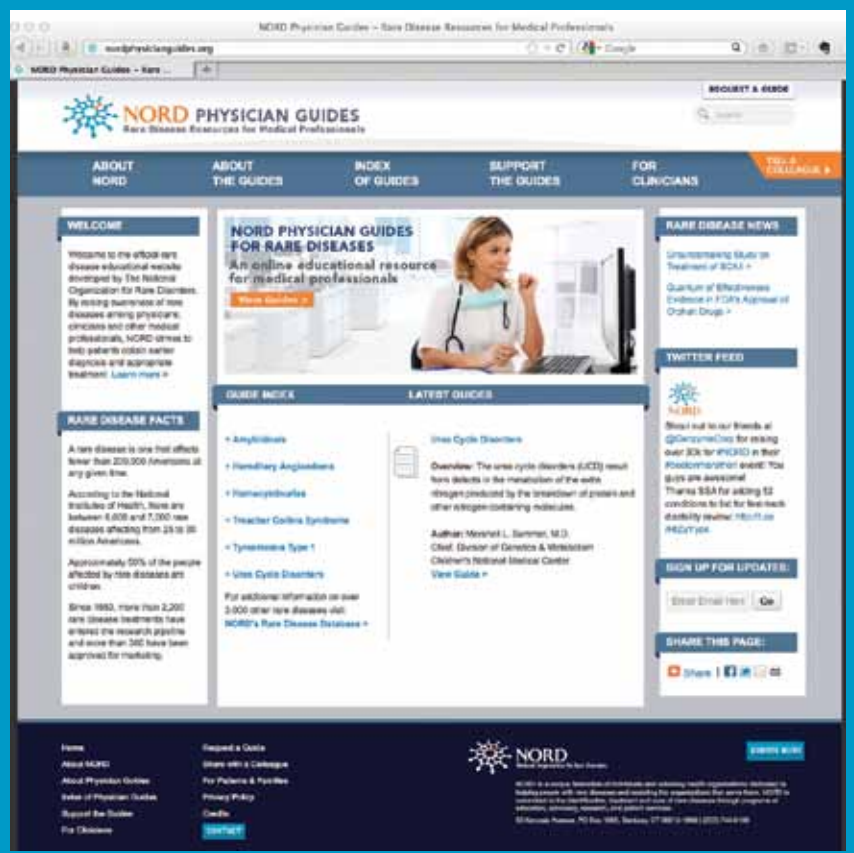
NORD has established a new online platform to provide information from medical experts on specific rare diseases to clinicians. The purpose is to encourage earlier diagnosis and appropriate treatment for patients.

Located at www.nordphysicianguides.org, the new resource is an online version of the printed booklets for physicians that NORD has published over the past several years.

Studies have shown that getting an accurate diagnosis remains one of the greatest challenges for people with rare diseases. As a result, NORD is increasing its outreach to physicians and other medical professionals on behalf of patients and their families.

The first topic to be posted on the new platform is "Urea Cycle Disorders". This guide was written by Marshall Summar, MD, Chief of Genetics and Metabolism at Washington National Medical Center. This guide was also prepared in collaboration with the Urea Cycle Disorders Foundation, which is a member of NORD.

Guides will be funded with educational grants from patient organizations, foundations, and corporations. NORD will be responsible for all content and will arrange for physicians with expertise on the various topics to author the guides.



For more information, contact us at: NORDPhysicianGuides@rarediseases.org

CORPORATE COUNCIL SPEAKERS HIGHLIGHT FDA UPDATES

At the most recent NORD Corporate Council meeting, Anne Pariser, MD, Associate Director for Rare Diseases in the FDA Center for Drug Evaluation and Research (CDER), presented results from a recent comparison of product approvals showing that the approval rate for rare disease therapies was actually slightly higher than the rate for common disease treatments.

She also said that, contrary to what many people believe, orphan designations and approvals are highest in diseases of low prevalence on the rare disease spectrum.

FDA Forming Rare Disease Council

Another FDA official, Gayatri Rao, MD, JD, Director of FDA's Office of Orphan Products Development, said FDA is forming a new Rare Disease Council. The purpose is to improve communications related to rare diseases among CDER; CBER (the Center for Biologics Evaluation and Research); CDRH (the Center for Devices and Radiological Health); CFSAN (the Center for Food Safety and Nutrition); and the Office of Special Health Issues.

Other speakers at the meeting included two policy analysts, Steven Grossman, a regulatory consultant, and Paul Kim, a partner in the Foley Hoag law firm, who presented an update on the PDUFA reauthorization process.

Corporate Council Membership

The Corporate Council provides a neutral forum for discussion of challenges and opportunities related to orphan product development. Membership is open to all companies involved in developing orphan products. Information is available on NORD's website. Inquiries may be sent to aashley@rarediseases.org

SAVE THE DATE

Continued from Cover

The emphasis will be on gaining a common understanding of current and emerging challenges and opportunities, and developing strategies for working together effectively to shape a better future for rare disease patients and their families.

Registration is open now. You can read about this conference on the NORD website (www.rarediseases.org), where there is also a link to the online registration page. A limited number of scholarships are available for patient organizations. For information about scholarships, write to amvallarino@rarediseases.org.

To learn more, go to

www.rarediseases.org/news-events/news/nord-dia-2012

NORD HAILS FDA ACT

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"We are grateful for the hard work and input our member organizations and policy partners have contributed over the past two years – raising awareness among Members of Congress, the FDA and the National Institutes of Health. NORD looks forward to continued collaboration to support the issues that deeply affect the rare disease community," said Saltonstall.

About Rare Diseases

A rare disease is any disease affecting fewer than 200,000 Americans. There are nearly 7,000 such diseases – of which only about 250 have FDA-approved treatments – affecting nearly 30 million Americans.

NORD: SETTING THE STANDARD FOR PATIENT ASSISTANCE PROGRAMS

Since 1987, NORD has helped more than 1.3 million patients obtain life-saving or life-sustaining medication through Patient Assistance Programs (PAPs). NORD was the first organization to offer PAPs, which provide free drug, co-pay assistance and other help to obtain needed medications. Over the past two decades, NORD has administered nearly 400 PAPs.

All of NORD's programs and services are patient-focused, and its PAPs reflect this commitment. NORD provides patient education and a welcoming, supportive environment, in addition to state-of-the-art services, to ensure patient access to medications.

To date, NORD has administered more than $60 million in free drug and co-pay assistance programs. It works closely with its industry partners to develop innovative programs and customized services.

NORD'S PAPS INCLUDE:

- Medication Assistance Programs
- Premium and Co-Pay Assistance Programs
- Travel and Lodging Assistance for Clinical Trials
- Expanded Access/Random Selection Program
- Emergency or Limited Access Programs
- Ancillary Access Programs

Companies interested in exploring the possibility of establishing a Patient Assistance Program with NORD should contact Pamela Gavin (pgavin@rarediseases.org). Patients and/or family members seeking medication assistance may visit NORD's website (www.rarediseases.org) to see whether NORD currently has a program for the rare disease affecting them.

NORD APPLAUDS CONGRESS FOR BIPARTISAN ACTION ON NEW LAW

Many Members of Congress played key roles in shaping the *FDA Safety and Innovation Act* (see cover story). NORD has written to thank them, and we encourage our members also to do so. You can do this easily by clicking on "Take Action Now" under the Advocacy button on our website. If your Representatives in Congress supported FDASIA, let them know you appreciate their support.

In particular, NORD would like to recognize the following, who worked closely with us on several important provisions:

Leadership of the Senate HELP (Health, Education, Labor and Pensions) and House Energy & Commerce Committees:

- HELP Committee Chairman: Sen. Tom Harkin (D-IA)
- Ranking Member: Sen. Mike Enzi (R-WY)
- Energy & Commerce Committee: Rep. Fred Upton (R-MI)
- Ranking Member: Henry Waxman (D-CA)

For Leadership Related to Drug Approval and Patient Access Provisions

Enhancement of Accelerated Patient Access to New Medical Treatments

- Senator Kay Hagan (D-NC) and Representatives Cliff Stearns (R-FL) and Edolphus (Ed) Towns (D-NY)

Breakthrough Therapies

- Senators Michael Bennet (D-CO), Orin Hatch (R-UT), and Richard Burr (R-NC), as well as Representatives Brian Bilbray (R-CA) and Diana DeGette (D-CO)

Consultation with External Experts on Rare Diseases, Targeted Therapies and Genetic Targeting of Treatments

- Senator Sheldon Whitehouse (D-RI) and Representatives Ed Markey (D-MA), Thomas Marino (R-PA) and Cliff Stearns (R-FL)

Rare Disease Pediatric Disease Priority Review Voucher Incentive Program

- Senators Robert Casey (D-PA), Scott Brown (R-MA), Sherrod Brown (D-OH), Johnny Isakson (R-GA) and Al Franken (D-MN), as well as Representatives Michael McCaul (R-TX) and G.K. Butterfield (D-NC)

For Leadership Related to Medical Device Regulatory Improvements

Humanitarian Device Exemptions

- Senators Al Franken (D-MN) and Lamar Alexander (R-TN), as well as Representative Charles Bass (R-NH)

For Leadership Related to Other Provisions

Conflict of Interest

- Senators Amy Klobuchar (D-MN), Richard Burr (R-NC), and Michael Bennet (D-CO), as well as Representative Michael Burgess (R-TX)

FDA SAFETY AND INNOVATION ACT

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The Breakthrough Therapies Act

Promoted by the Friends of Cancer Research, the *Advancing Breakthrough Therapies for Patients Act* addressed the need to provide expedited development and evaluation of potential therapies that show promise early in the research process.

The EXPERRT Act

Championed by the Cystic Fibrosis Foundation, the *Expanding and Promoting Expertise in Review of Rare Treatments (EXPERRT) Act* was designed to expand cooperation between FDA and outside rare disease experts and patient advocates.

The Creating Hope Act

Promoted by Kids v Cancer and the Congressional Childhood Cancer Coalition, this Act would expand a priority review voucher program to incentivize the development of new drugs for rare pediatric diseases, including childhood cancers.

Humanitarian Use Devices

NORD worked closely with Senator Al Franken (MN), Representative Charles Bass (R-NH) and other Members of Congress to bring about much-needed reforms related to Humanitarian Use Devices (HUDS). These are medical devices developed for small patient populations through the FDA Humanitarian Device Exemption Program. This program encourages the development of medical devices for patient populations of fewer than 4,000 people. Provisions included in the *FDA Safety and Innovation Act* related to this will encourage the development of devices for both pediatric and adult patients and also expand the existing pediatric device incentive to adult HUDS.

Rare Disease Patient Voices Were Heard

NORD is very grateful to FDA, industry and Congress for listening to the concerns of rare disease patients articulated over the past two years and being responsive so that this critically important legislation could be enacted quickly. We thank President Obama for signing the *FDA Safety and Innovation Act* so that all involved can begin to implement these desperately needed measures.

Most of all, we thank our members, supporters and policy partners for understanding that effective change begins with education, advocacy and relationship-building, and for working with us to make that happen.

WE'RE GROWING!

NORD welcomes the following new Member Organizations. We encourage all our friends and constituents to become familiar with the following organizations, which have joined NORD this year.

Our Member Organizations play a vital role in NORD's advocacy and educational programs. The entire list of members may be viewed on our website: www.rarediseases.org/patient-orgs/current

Acromegaly Community, Inc.
Guthy Jackson Charitable Foundation
International Association for Food Protein Entercolitis
LAL Solace
Liam's Land Organization, Inc.
MitoAction
MEBO Research, Inc.
Myotonic Dystrophy Foundation
National Brain Tumor Society
NBIA Disorders Association
NOMID Alliance
Parent Project Muscular Dystrophy
Short Bowel Syndrome Foundation, Inc.
Soft Bones

THE BENEFITS OF MEMBERSHIP

KEEPING OUR MEMBERS INFORMED WITH MONTHLY POLICY CALLS

NORD is committed to providing our member organizations with information and resources to help them advocate more effectively on behalf of their members and the entire rare disease community. With this goal in mind, we have launched a series of monthly policy calls for our member organizations.

ENGAGING OUR MEMBERS IN ADVOCACY

NORD is seeking a deeper level of engagement with its members, particularly in the area of advocacy. One example of how this is happening is the Risk Tolerance Initiative.

Over the past year and a half, NORD and its members have played a key role in bringing this issue to the forefront at the FDA. "Risk tolerance" refers to the fact that people with serious rare or chronic diseases who have few or no treatment options may view risk very differently from those who have common diseases with many options.

In January 2011, NORD and its partners convened a Risk Tolerance Working Group and began a process that resulted in patient representatives being able to meet with senior staff of FDA's CDER, CBER and CDRH. Later, NORD and more than 30 of our advocacy partners sent follow-up letters to key FDA officials seeking more frequent and regular opportunities for patient input on risk/benefit decisions. These efforts are bearing fruit and represent one of the ways in which NORD and its members can partner effectively on an issue of importance to all.

CONNECTING OUR MEMBERS AT REGIONAL MEETINGS

In an effort to connect the patient organizations that are members of NORD, three regional meetings occurred in the last year in Chicago, New York, and San Francisco. Now, additional meetings are being planned.

Member organizations can join us in Philadelphia on September 20-21, in Atlanta on December 5-6, and on March 27-28 in a soon-to-be determined location in the Midwest.

NORD values these meetings and the opportunities they provide to reconnect, share news about how to become more involved in advocacy and educational initiatives, and learn from each other. These regional meetings provide wonderful opportunities for our members to share their experiences with each other to further enhance the value of being part of the NORD coalition.

For more information on membership, contact:
amvallarino@rarediseases.org

NORD
National Organization for Rare Disorders

NORD is a unique federation of individuals and voluntary health organizations dedicated to helping people with rare diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment and cure of rare diseases through programs of education, advocacy, research, and patient services.

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