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FDA Safety and Innovation Act Signed:
A Monumental Step Toward The Development of Safe and Effective Treatments for Millions of Americans With Rare Diseases

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

July 9, 2012, Washington DC----The *FDA Safety and Innovation Act,* signed by President Obama today, contains the most groundbreaking measures for rare disease patients and their families since the *Orphan Drug Act* of 1983, the National Organization for Rare Disorders (NORD) said.

NORD President and CEO Peter L. Saltonstall said he was pleased that NORD was able to provide successful leadership in developing the legislation with the FDA and in guiding its enactment by the Congress.

"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," Saltonstall said.

"This legislation represents true progress for people with rare diseases, who often struggle to access treatments for their disorders," Saltonstall said. "Our heritage, understanding, leadership and commitment to the community uniquely positioned us to identify and articulate the needs of people with rare diseases.

"Today's signing adds 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."

Established in 1983 by patient advocates who successfully advocated for the *Orphan Drug Act,* NORD has played a central role in the development of the legislation signed by President Obama today since 2010. The Act renews user fees to support 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 community, the Act provides the following:

- accelerated patient access to new medical treatments
- the development of Humanitarian Use Devices (medical devices for small patient populations)
- accelerated development of "breakthrough therapies" -- those that show early promise
- enhanced FDA consultation with rare disease medical experts
- a rare pediatric disease priority review voucher incentive program
- resolution of conflict-of-interest issues that kept voices of rare disease medical experts from being heard

"We are grateful for the hard work, leadership and collaboration provided over the past two years by our member organizations, policy partners, the Members of Congress, the FDA and the National Institutes of Health. NORD looks forward to continued partnership on this legislation and future opportunities to support the issues that deeply affect the rare disease community," said Saltonstall.

Read an op/ed piece by NORD's president on this topic on the BIO website.

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. Nearly 30 million Americans have rare diseases.

ABOUT NORD

Established in 1983, the National Organization for Rare Disorders (NORD) is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. A nonprofit organization, NORD represents the 30 million Americans with rare diseases, providing programs of education, advocacy, research and patient assistance.

For more information, please visit www.rarediseases.org.