



Amicus CEO Represents BIO on Rare Diseases at U.S. Senate Committee on Health, Education, Labor and Pensions Bipartisan Hearing

John F. Crowley provides perspectives and recommendations to Committee on treating rare and neglected pediatric diseases and promoting the development of new treatments-

CRANBURY, N.J., July 21, 2010 /PRNewswire via COMTEX News Network/ -- Amicus Therapeutics (Nasdaq: FOLD) announced today that the Company's Chairman and CEO, John F. Crowley, testified on behalf of the Biotechnology Industry Organization (BIO) at the Senate Committee on Health, Education, Labor and Pensions bipartisan, full committee hearing focused on rare and neglected diseases held on Wednesday, July 21 on Capitol Hill. Mr. Crowley, a member of the Board of Directors of BIO, provided background and perspectives on the current state of drug development for orphan products. He also made a series of recommendations to the Committee regarding the establishment of additional incentives for companies to develop more treatments for rare and neglected diseases.

During his testimony, Mr. Crowley discussed the unprecedented success of the Orphan Drug Act (ODA) of 1983 and its market-based incentives for biotechnology and pharmaceutical companies to develop products for rare diseases. Crowley made several recommendations on new policies for the Committee's consideration to accelerate the development of treatments for rare and neglected diseases that will complement and advance the objectives of the ODA and facilitate the availability of the next generation of orphan products for children.

These recommendations included:

- Improving the U.S. Food and Drug Administration (FDA) regulatory environment for pediatric rare diseases;
- Establishing a separate Division of Genetic and Metabolic Disorders at FDA;
- Urging FDA to publish further guidance regarding increased use of surrogate endpoints for product approval, either for full approval or accelerated approval purposes;
- Improving standards for demonstrating safety and efficacy of rare disease products;
- Improving communications processes for rare disease stakeholders;
- Extending the Qualifying Therapeutic Discovery Project Tax Credit; and
- Fully funding the Cures Acceleration Network (CAN).

John F. Crowley commented in his testimony, "We have come very far in the last quarter of a century but we have much further to go. The change brought about by the Orphan Drug Act improved millions of lives in this country and abroad, helped launch an industry and established the global rare disease advocacy movement. It does not come easily for every family that struggles with illnesses and then receives a life-altering diagnosis of a rare disease with no treatment or cure. However, each of us committed to orphan drug development, including the FDA and those responsible for seeing the Agency is appropriately funded, owes those families a more-than-fighting chance that their medical needs will be met."

"Through his compelling personal story and his professional passion, John Crowley truly understands the importance of the Orphan Drug Act and the need for additional incentives to spur the development of breakthrough treatments for rare and orphan diseases," said BIO President and CEO Jim Greenwood. "The recommendations John made in his testimony today reflect the concerns and interests of our member companies who are actively researching the next generation of therapies and the patients and their families who so desperately need them."

About Amicus Therapeutics

Amicus Therapeutics is a biopharmaceutical company focused on developing treatments for rare diseases. The Company is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and CNS diseases. Amicus' lead program is in Phase 3 for the treatment of Fabry disease.

About Biotechnology Industry Organization (BIO)

BIO represents more than 1,200 biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO members are involved in the research and

development of innovative healthcare, agricultural, industrial and environmental biotechnology products. BIO also produces the BIO International Convention, the world's largest gathering of the biotechnology industry, along with industry-leading investor and partnering meetings held around the world.

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