



September 11, 2013

Amicus Therapeutics' CEO John F. Crowley to Advocate for Rare and Orphan Disease Community in Washington, D.C. This Week

Speaking at Research!America National Health Research Forum with FDA Commissioner Hamburg

Moderating Panel Discussion on Capitol Hill at Rare Disease Congressional Caucus Briefing

Joining Rep. Leonard Lance and Rep. Joe Crowley to Reintroduce "MODDERN CURES ACT"

CRANBURY, N.J., Sept. 11, 2013 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) today announced that John F. Crowley, Chairman and Chief Executive Officer, will advocate for the rare and orphan disease community at several upcoming events in Washington, D.C tomorrow on September 12, 2013:

[Rare Disease Congressional Caucus Briefing](#)

Date: Thursday, September 12, 2013

Time: 10:00 a.m. ET

Place: Rayburn House Building

Mr. Crowley will moderate a panel discussion at a briefing of the Congressional Rare Disease Caucus entitled, "In Jeopardy: Programs that Accelerate the Development of Rare Disease Treatments." The Rare Disease Congressional Caucus, co-chaired by Representatives Leonard Lance (R-NJ) and Joe Crowley (D-NY), is a forum for members of Congress to voice constituent concerns, share ideas, and build support for legislation that will improve the lives of people with rare diseases. During the hearing, Mr. Crowley and the panelists will discuss:

- The timely implementation of the Food and Drug Administration Safety and Innovation Act (FDASIA) in giving people with rare diseases access to Accelerated Approval
- Regulatory challenges that prevent access for U.S. patients to participate in clinical trials for rare disease treatments in development
- The potential elimination of the Orphan Drug Tax Credit
- The Food and Drug Administration Safety Over Sequestration Act of 2013, H.R. 2725

In conjunction with the Caucus Briefing, Mr. Crowley will join Representatives Lance and Crowley to support the reintroduction of the bipartisan "Modernizing Our Drug and Diagnostics Evaluation and Regulatory Network (MODDERN) Cures Act."

"We are fortunate to have the support of John Crowley, a nationally recognized rare disease advocate, as we reintroduce The MODDERN Cures Act," stated Congressman Lance. "This legislation seeks to promote the development of meaningful cures for the rarest diseases that have few or no options by removing outdated barriers and creating new invention and innovation opportunities."

[Research!America 2013 National Health Research Forum: Straight Talk about the Future of Health Research](#)

Date: Thursday, September 12, 2013

Time: 12:00 p.m. ET

Place: Newseum Knight Conference Center

Mr. Crowley will participate in a panel, "Biomedical Research & Development in America: Will it flourish or flounder?" moderated by Eleanor Clift, Contributing Editor for *Newsweek* and contributor to *The Daily Beast*. Co-panelists include Margaret Hamburg, MD, Commissioner, FDA; William Hait, MD, PhD, Global Head R&D, Janssen; Bart Peterson, JD, Senior Vice President, Corporate Affairs and Communications, Eli Lilly and Company; and E. Albert Reece, MD, PhD, Dean, School of Medicine,

University of Maryland. For more information please visit www.researchamerica.org/forums.

"We are pleased to have John Crowley as a panelist at this year's National Health Research Forum: Straight Talk, a lively and informative discussion about the future of medical and health research featuring key leaders of our nation's research enterprise," said Mary Woolley, President of Research!America. "John is a tireless advocate for the patient community, as well as the recipient of Research!America's 2013 Gordon and Llura Gund Leadership Award for his outstanding efforts to accelerate new treatments for Pompe disease and other genetic diseases."

About Amicus Therapeutics

[Amicus Therapeutics](#) (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of therapies for rare and orphan diseases. The Company is developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering new benefits to individuals with lysosomal storage diseases. Amicus' lead programs include the small molecule pharmacological chaperones [migalastat HCl](#) as a monotherapy and in combination with enzyme replacement therapy (ERT) for Fabry disease; and [AT2220 \(duvoglustat HCl\)](#) in combination with ERT for Pompe disease.

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