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Obama Signs FDA User Fee Legislation Bringing Hope to Rare Disease Patients

EveryLife Foundation for Rare Diseases Applauds Congress for Including Provision to Empower the FDA to Accelerate Approval of Lifesaving Treatments

July 10, 2012, Novato, CA – Yesterday, President Obama signed into law *The Food and Drug Administration Safety and Innovation Act (FDASIA), S. 3187*, bipartisan legislation that will spur the development of lifesaving treatments for 30 million Americans suffering from rare diseases.

"We are thrilled the language to improve access to the FDA's Accelerated Approval pathway for rare diseases has been included in *FDASIA*," said Emil Kakkis, MD, President, **EveryLife Foundation for Rare Diseases**. "We wish to thank Representatives Cliff Stearns (R-FL) and Ed Towns (D-NY) for being champions for the rare disease community."

Stearns and Towns first introduced *Unlocking Lifesaving Treatments for Rare Diseases Act (ULTRA)* to empower FDA to use all the science available for allowing surrogate endpoints in clinical trials for rare diseases to determine whether a drug is working, significantly decreasing the development time and cost. Stearns and Towns later introduced *Faster Access to Specialized Treatments (FAST) Act* that improved Accelerated Approval for life-threatening diseases while maintaining high safety and efficacy standards.

FDA's Accelerated Approval has been successful in getting treatments approved for cancer and AIDS patients, but has been essentially unavailable for rare disease treatments. There are currently fewer than 400 FDA-approved treatments for nearly 7000 rare diseases. Investment and interest in development will surge for rare diseases if there is access to the Accelerated Approval pathway.

"We would not have been successful if it were not for the great work of Energy and Commerce Chairman Fred Upton (R-MI), Biotechnology Industry Organization (BIO), and more than 300 patient organizations that advocated for improving the FDA's regulatory process," added Kakkis.

FDASIA is the culmination of more than a year of negotiations between industry and FDA and includes the reauthorization of the drug and device user fees.

The Foundation will host its fourth Rare Disease Workshop on "Developing Guidance and Policy Recommendations for Accelerated Approval in Rare Diseases" on November 15th in Washington, D.C. FDA, NIH, industry and academic scientists are invited to participate.

The EveryLife Foundation for Rare Diseases, headquartered in Novato, CA, is dedicated to accelerating biotech innovation for rare disease treatments through science-driven public policy. We can do more with the science we already have and bring life saving treatments to millions of people suffering from rare diseases.

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