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Genetic Alliance & PhRMA Announce Pilot Initiative to Advance Patient-Focused Drug Development

Washington, D.C. (November 4, 2013) — Today, Genetic Alliance and the Pharmaceutical Research and Manufacturers of America (PhRMA) announced an [initiative](#) to explore the use of a technology-enabled, crowd-sourcing approach to patient engagement as a complement to ongoing patient-focused drug development efforts under the Prescription Drug User Fee Act (PDUFA V).

As part of the reauthorization of PDUFA, the U.S. Food and Drug Administration (FDA) committed to gain the patient perspective on 20 disease areas in public meetings to be held between 2012 and 2017.

“PhRMA is fully supportive of FDA’s efforts to advance patient-focused drug development and seeks to contribute additional patient-driven data that reflect a range of patient perspectives on issues such as severity of conditions and unmet medical needs over time,” stated Sascha Haverfield, PhD, Vice President of Scientific and Regulatory Affairs at PhRMA.

After issuing a Request for Proposals, Genetic Alliance chose advocacy organizations representing three disease areas that will be the focus of FDA patient-focused drug development public meetings in 2014 and 2015. The patient communities in these three disease areas will pilot a crowd-sourcing, technology-enabled approach to gathering input from a diverse set of patients on key benefit-risk questions.

“Using the Platform for Engaging Everyone Responsibly (PEER), there is an opportunity to demonstrate the power of a secure, crowd-sourced approach to provide additional insight into patients’ experience with a disease or condition,” stated Sharon Terry, President and CEO of Genetic Alliance. “The organizations we selected are expert at broad and diverse engagement from the very people that have a vested interest in patient-focused drug development. We are excited to engage these communities.”

The following organizations were selected to engage the sickle cell disease community:

- [North Alabama Sickle Cell Foundation, Inc.](#)
- [Sickle Cell Disease Association of America, Southern Connecticut Inc.](#)
- [Sickle Cell Warriors](#)
- [The William E. Proudfoot Sickle Cell Fund, Inc.](#)

The [Coalition for Pulmonary Fibrosis](#) was chosen to engage the idiopathic pulmonary fibrosis community.

The [Gastroparesis Patient Association for Cures and Treatments, Inc.](#) and [Share and Care Cockayne Syndrome Network](#) were selected to engage the irritable bowel syndrome, gastroparesis and gastroesophageal reflux community.

Genetic Alliance will provide the participating organizations with a customized version of the PEER system, through which they will engage in a systematic collection of affected individuals' perspectives on a condition's severity and current unmet medical needs.

[Private Access](#) is Genetic Alliance's technology partner for this project. Private Access is a pioneer in developing participant-centric access controls and privacy management systems. Private Access empowers each individual to set meaningful sharing and privacy preferences that reflect his or her needs and interests.

For more information about this initiative, visit <http://www.geneticalliance.org/pfdd>.

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About Genetic Alliance

Genetic Alliance is the world's leading nonprofit health advocacy organization committed to transforming health through genetics and works to connect consumers to the smart services they need to make informed decisions about their health and healthcare. Genetic Alliance's network includes more than 1,200 disease-specific advocacy organizations, as well as thousands of universities, private companies, government agencies, and public policy organizations. For more information about Genetic Alliance, visit www.geneticalliance.org.

About PhRMA

The Pharmaceutical Research and Manufacturers of America (PhRMA) represents the country's leading innovative biopharmaceutical research and biotechnology companies, which are devoted to discovering and developing medicines that enable patients to live longer, healthier, and more productive lives. Since 2000, PhRMA member companies have invested approximately \$550 billion in the search for new treatments and cures, including an estimated \$48.5 billion in 2012 alone. For more information, please visit <http://www.phrma.org>.