For Immediate Release

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International Collaboration Announced to Develop GaugeRx

WASHINGTON, DC – June 10, 2013 – The EspeRare Foundation of Geneva, Switzerland and Genetic Alliance of Washington, DC announced a collaboration to streamline the process of translating research into health outcomes by strengthening decision-making in drug development. The collaboration will result in an interactive, web-based analytics and assessment tool that integrates and translates the vast amount of knowledge about human health and disease to support drug repositioning and development decision-making for a broad range of healthcare stakeholders, from pharmaceutical companies to advocacy organizations.

Assessing the potential for drug development in different disease areas, particularly for rare and neglected diseases, is a complex and nuanced process, requiring the simultaneous consideration of multiple parameters and constraints. Today, without any open-source tool, these efforts are largely scattered and conducted in silos by different stakeholders. GaugeRx aims to bridge this gap by bringing together information and best practices to assist stakeholders in deciding how to most effectively leverage existing resources and prioritize programs with the highest probabilities of success to advance the research and development (R & D) portfolio for their disease of interest.

GaugeRx will build on Genetic Alliance's drug development model, Navigating the Ecosystem of Translational Science (NETS), which provides a network map of drug development that allows stakeholders to identify where they can collaborate and engage in the process. The tool will also draw on Genetic Alliance's Disease InfoSearch resource, an international, curated and crowdsourced compendium of data on genetic conditions. EspeRare will bring its knowledge management skills and pharmaceutical expertise to support the translation of data into actionable insights to advance therapeutic opportunities. Syapse, the leading clinical omics software company, will provide its semantic computing platform and application suite as the technology solution for data and knowledge management, data mining, and reporting.

Taking into account user input, GaugeRx will survey the R & D landscape for a particular disease and generate user-specific output that provides a comprehensive analysis of drug development and drug repurposing potential. Key features of the tool will include:

1. The option for stakeholders to weight input parameters and define constraints, allowing the tool to generate user-specific output
2. A database with disease-specific information drawn from internal sources like Disease InfoSearch as well as internationally recognized external sources

3. The generation of user-specific output describing drug development and drug repositioning potential, complemented with a series of recommended next steps

"New paradigms for evaluation of therapeutic interventions must be forged to address medical needs of underserved patients. With this collaborative and integrative tool, we are looking to bring together key participants and scientific knowledge to catalyze better health," commented Caroline Kant, Executive Director of EspeRare Foundation, adding, "For a foundation such as ours, which focuses on drug repositioning and early drug development for rare diseases, this will be a very important tool to discover and prioritize new therapeutic opportunities, we are confident that it will be true for others as well."

"Advocacy organizations are invited to update their listing in Disease InfoSearch," Sharon Terry, CEO of Genetic Alliance, explained, "so that all of the necessary information is available to GaugeRx to favorably score the particular condition they serve."
The team welcomes the input of collaborators from industry, advocacy, and academia to interactively refine the outputs of GaugeRx. The tool will be launched at the end of the year.

About Genetic Alliance

Genetic Alliance improves health through the authentic engagement of individuals, families, and communities. 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 Disease InfoSearch, visit http://www.diseaseinfosearch.org. For more information about Genetic Alliance, visit http://www.geneticalliance.org

About the EspeRare Foundation

EspeRare is a nonprofit private foundation established in Geneva, Switzerland in 2013. In collaboration with patient groups and other key stakeholders, the foundation strives to uncover the potential of existing drugs to address severe therapeutic unmet needs in rare diseases. Through the identification and translational validation of these therapeutic opportunities, EspeRare focuses on giving better chances to already existing drug to reach rare diseases patients. For more information, please visit www.esperare.org

About Syapse

Syapse is disrupting healthcare by bringing omics into routine medical use. Built on a powerful cloud-based semantic data platform, Syapse applications enable the generation and use of omics profiles in diagnosing and treating patients at diagnostic companies, research institutions, medical centers, and payers. Led by an experienced, multidisciplinary team of entrepreneurs, software developers, and scientists, Syapse was founded at Stanford University and is backed by The Social+Capital Partnership. For more information, please visit www.syapse.com

About Navigating the Ecosystem of Translational Science

Baxter, K, Horn, E, Gal-Edd, N, Zonno, K, O'Leary, J, Terry, PF, & Terry, SF. An end to the myth: there is no drug development pipeline. *Sci Transl Med*. 2013 Feb 6;5 (171):171cm1. PMID: 23390245.