Game-Changing Animal Research Models Offer Superior Translational Research and Better Predictive Efficacy

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First Genetically Engineered Miniswine Research Model Cleared by FDA for Commercial Use

SIOUX CENTER, Iowa, April 27, 2016 /PRNewswire/ -- Exemplar Genetics, a wholly owned subsidiary of Intrexon Corporation (NYSE: XON) committed to enabling the study of life-threatening human diseases, today announced the U.S. Food & Drug Administration (FDA) has exercised enforcement discretion in regard to the ExeGen® low-density lipoprotein receptor (LDLR) miniswine clearing it for commercial use as a research model.

As the first genetically engineered (GE) miniswine model reviewed and cleared by the FDA, Exemplar's powerful investigational platform can now be offered to researchers and drug developers helping forge a more reliable, consistent path from pre-clinical testing through human studies. The ExeGen® LDLR model is a game-changer that enables superior translational research and better predictive efficacy in the generation of novel gene- and cell-based therapies, small molecules, as well as biologics for cardiac disease.

"Current animal research models are useful yet not predictive, and the results they generate pre-clinically often do not translate into the clinic," said John R. Swart, Ph.D., President and Chief Executive Officer of Exemplar Genetics. "We thank the FDA for their comprehensive four-year review and decision which empowers researchers and drug developers focused on cardiovascular disease with the potential for greater drug and device discovery. This is an important milestone in the drug development industry."

Animal models that fail to sufficiently represent human pathologies create a significant barrier to understanding disease mechanisms, as well as hinder the development of efficacious therapeutics and novel diagnostics and devices. Since the late 1980's GE mice have been a mainstay of biomedical research and today represent the fastest growing segment within the $1 billion mice model market. However, these mouse models often fail to recreate the pathology of human disease limiting their translational research utility. Failure rates for drug development are greater than 80% today; thus better, more predictive research tools for pre-clinical studies are necessary to alleviate the risk of costly clinical trial failures.

Exemplar's GE miniswine research models are much more anatomically, physiologically, and genetically similar to humans, mitigating many of the limitations of GE mice systems, including lack of translation and differences in size and metabolism. Their utility as a superior research model may hold particularly true in therapeutic development for genetic diseases and orphan indications, where there is currently no clear path for evaluation given insufficient models and limited patient populations. Exemplar's research models will allow scientists to focus on a specific genetic disease among the estimated 7,000 monogenic diseases to develop a personalized approach to therapeutic discovery, opening the door to solutions for many human diseases that today are considered untreatable.

Dale Mais, Ph.D., Director of Metabolism and Endocrinology at MPI Research, stated, "The pioneering ExeGen® LDLR research model opens the door to a powerful investigational platform to elucidate human pathologies for use in the generation of effective therapeutics. The FDA's decision bodes well for Exemplar's processes and pipeline, which we believe to be the furthest advanced and broadest platform of miniswine research models under development."

Through its suite of proprietary technologies, Exemplar has developed a broad pipeline of transgenic swine models for use in the evaluation of several human health conditions for drug and device discovery, including heart disease, cancer, cystic fibrosis, cardiac arrhythmia, and neuromuscular/neurodegenerative disorders. The ability of Exemplar's models, often custom-built for leading academic and commercial researchers, to more effectively mimic human disease has been well documented through studies conducted by contract research organizations as well as throughout more than 30 peer reviewed publications including:

- *ExeGen® Cardiovascular Model: Targeted disruption of LDLR causes hypercholesterolemia and atherosclerosis in Yucatan miniature pigs*
- *ExeGen® Rare Genetic Disease Model for Ataxia Telangiectasia: A novel porcine model of ataxia telangiectasia reproduces neurological features and motor deficits of human disease.*
- *ExeGen® Cardiac Arrhythmia Model: Genetically engineered SCN5A mutant pig hearts exhibit conduction defects and arrhythmias*
- *ExeGen® Rare Genetic Disease Model for Huntington's disease: Mind the gap: models in multiple species needed for therapeutic development in Huntington's disease.*
- *ExeGen® Cystic Fibrosis Model: Adenoviral gene transfer corrects the ion transport defect in the sinus epithelia of a porcine CF model.*
- *ExeGen® Cancer Model: Development and translational imaging of a TP53 porcine tumorigenesis model*

About Exemplar Genetics
Exemplar Genetics, a wholly owned subsidiary of Intrexon Corporation (NYSE: XON), enables discovery by providing models and services that aid scientists in the development of next-generation procedures, devices and therapeutics. Through its innovative models and AAALAC-certified facilities,

Exemplar Genetics assists researchers in making advances in the discovery of human disease mechanisms, the optimization of novel diagnostics, and the development of new treatments. For more information, visit www.exemplargenetics.com.

Trademarks
ExeGen is a registered trademark of Exemplar Genetics. Other names may be trademarks of their respective owners.

Safe Harbor Statement
Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon our current expectations and projections about future events and generally relate to our plans, objectives and expectations for the development of our business. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and uncertainties and actual future results may be materially different from the plans, objectives and expectations expressed in this press release.

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