# Exemplar Genetics ExeGen® Cystic Fibrosis Research Model Utilized in Development of New Gene Therapy Treatments

*Publications in Journal of Clinical Investigation demonstrate potential of ExeGen® CFTR models*

**Sioux Center, IA, September 13, 2016** – Exemplar Genetics, a wholly owned subsidiary of Intrexon Corporation (NYSE: XON) committed to enabling the study of life-threatening human diseases, today announced two publications in the current issue of the Journal of Clinical Investigation (JCI) demonstrating the potential of ExeGen® CFTR miniswine research models to help define and develop effective gene therapies for cystic fibrosis.

"The foundational work by the excellent teams at the University of Iowa and the University of California, Berkeley, emphasizes the utility and importance of ExeGen® research models in the development of new treatment strategies for a devastating disease," said John R. Swart, Ph.D., President and Chief Executive Officer of Exemplar Genetics. "We are excited to provide the ExeGen® CFTR model to facilitate translational research in this field."

In the first JCI publication titled Lentiviral-mediated phenotypic correction of cystic fibrosis pigs, researchers utilizing Exemplar's research models demonstrated that delivery of gene therapy showed an increase in bacterial killing as well as partial physiological improvement in cystic fibrosis (CF), a genetic disorder resulting from mutations in the CF transmembrane conductance regulator (*CFTR*). This proof-of-concept study provided the first evidence of CFTR correction by a lentiviral vector in a large-animal model.

The second publication detailing a companion study titled CFTR gene transfer with AAV improves early cystic fibrosis pig phenotypes, describes an adeno-associated virus (AAV) gene therapy that revealed evidence of functional CFTR expression in the airways of the ExeGen® CFTR models.

As noted in the first JCI publication, "Progress for CF gene therapy has been hindered in part due to the lack of animal models that share similar lung disease phenotypes as humans…the CF pig spontaneously develops lung disease similarly to humans with CF. This allows us to measure CFTR correction in a large-animal model that recapitulates key features of CF in humans."

Historically, development of therapeutic interventions to treat symptoms and address the underlying causes of disease has been hindered by use of animal models that insufficiently replicate the human pathology. While genetically engineered mice have provided a foundation for biomedical research, their translational use has been restricted. Alternatively, use of Exemplar's engineered miniswine models offers a platform with greater anatomical, physiological, and genetic similarity to humans, mitigating the lack of translation with murine systems due to differences in size and metabolism.

In addition to its utilization for advancing gene therapy, the ExeGen® CFTR model has also been employed to investigate small molecules in the treatment of CF as detailed in another JCI publication from June 2016, Repurposing tromethamine as inhaled therapy to treat CF airway disease.

Exemplar has developed a broad pipeline of transgenic swine models for use in the evaluation of numerous human conditions, including heart disease, cancer, cardiac arrhythmia, neuromuscular disease and neurodegenerative disease. "We are pleased our models are attracting the attention of leading academic and commercial researchers by providing a powerful investigational platform, enabling better predictive efficacy in the generation of innovative therapies that translate from bench to clinic," added Dr. Swart.

**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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