

Transposagen Biopharmaceuticals, Inc.

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National Institutes of Health Commercialization Assistance Program (NIH-CAP)

# **Company Profile**

Industry Sector: Research Tools - Genetically Modified Research Animal Models

**Company Overview:** Transposagen is the worldwide leader in the creation of unique knockout rat lines called TKO™ Knockout Rats. These models are laboratory rats with a single gene disruption that mimic a human disease. TKO™ Knockout Rats will enable a greater scrutiny of candidate drugs, will facilitate more favorable clinical testing in humans saving millions in development dollars, and will create new drug targets for the treatment of diseases.

While mice have proven to be a useful rodent model, a knockout rat is more relevant for many human pathologies, including cardiovascular disease, diabetes, arthritis, and many autoimmune and behavioral disorders.

Target Market(s): Large Pharmaceutical Companies
Academic Research Institutions

## **Key Value Drivers**

**Technology\*:** Transposagen has exclusive worldwide rights to create TKO<sup>TM</sup> Knockout Rat Models and associated TKO<sup>TM</sup> products using a variety of mobile DNA technologies. The company's platform technology has the advantages of patent protection and the freedom to operate with no dominating intellectual property. Transposagen is also working with several academic collaborators to further develop technologies that allow the creation of site-specific gene knockouts in rats.

Competitive Advantage: Rats are superior to current mouse models in these areas:

Disease Categories	cardiovascular, diabetes, arthritis, neurodegenerative, and
	many autoimmune and behavioral disorders
Larger Size	Instrumentation, blood sampling, studying nerve conduction or
	performing surgery is much easier.
Physiology	More similar to humans, e.g. heart beat - 80 beats/minute
Toxicity	Detoxifying enzymes very similar to those in humans
	Larger Size Physiology

Plan & Strategy: Seeking a strategic partner for global distribution.

\*Technology funded by the NCRR and being commercialized under the NIH-CAP

# Management

#### Leadership:

Eric Ostertag, M.D., Ph.D— President and CEO Joseph Ruiz, Ph.D—VP, Research Richard Stump, M.B.A.—VP, Operations George Ward— VP, Business Development

### Scientific Advisory Board and Key Collaborators:

John K. Critser, Ph.D., Rat Resource and Research Center (RRRC) Howard Jacob, Ph.D., Medical College of Wisconsin Aron Geurts, Ph.D., Medical College of Wisconsin Nancy Craig, Ph.D., Johns Hopkins University, HHMI David Largaespada, Ph.D., University of Minnesota Haig H. Kazazian, M.D., University of Pennsylvania

## **Product Development**

Transposagen's initial products will be TKO<sup>™</sup> Knockout Rat Models, the TKO<sup>™</sup> Germ Line Repository containing cryopreserved sperm, and the TKO<sup>™</sup> Tissue Bank containing blood, CSF, heart, lung, brain, and other tissue created from each knockout rat line. As thousands of rat models are added to the TKO<sup>™</sup> Tissue Bank, meaningful proteomic and metabolomic studies can be preformed to discover new drug targets.

Over 100 unique TKO™ Knockout Rat Lines were created by the end of 2008. More than 25% of these are in the hands of academic researchers for characterization, phenotyping, and eventual publication in scientific journals. 1,200 more knockout rat lines are expected by the end of 2009 and an additional 5,000 are expected to be available each year thereafter until a model for each gene in the rat genome is available.

The public database of available knockout rat models can be found at the Knock Out Rat Consortium (KORC) website – <a href="https://www.knockoutrat.org">www.knockoutrat.org</a>.