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Transgenic Animals for Preclinical Drug Development

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1. INTRODUCTION

The means to transform a physiologic pathway from one genotype to that of another is termed transgenic technology. In the realm of drug development, transgenic animals typically carry human physiologic pathways for efficacy and metabolism. This technology carries tremendous potential to elucidate potential human outcomes early in the lead optimization and preclinical development stages. Transgenic technology is a maturing area of science and, although it already provides considerable value, it is anticipated that its true value is yet to be realized.

Transgenic animals are produced in the laboratory by molecular manipulation of their genomes. The methods used to produce these animals have become routine practice in biomedical sciences over the past two decades. Although transgenic technology was first developed to address fundamental problems in biology, this technology has evolved rapidly over recent years and is now able to accommodate a wide variety of needs in different research areas, including basic biological sciences and application-oriented research such as drug development. Preclinical studies in drug development are intended to define the pharmacologic and toxicologic effects predictive of human responses. Animal models are essential tools for these phases of study; however, suitable animal models may not always be available.