

Application summary

Application number:	ERMA200223
Application type:	Notified, GM Development, Containment
Applicant contact:	Dr Jimmy Suttie
Applicant:	AgResearch Limited Waikato Mail Centre Private Bag 3123 Hamilton 3240
Purpose:	To develop in containment genetically modified goats, sheep and cows to produce human therapeutic proteins, or with altered levels of endogenous proteins for the study of gene function, milk composition and disease resistance
Date formally received:	5 November 2009

Application summary prepared by AgResearch Limited

The activities proposed under this application provide the foundation for New Zealand to sit at the forefront of animal biotechnology to produce biopharmaceuticals and nutraceuticals. Biotech animals can be the reactors that provide a more versatile and cost-efficient means of producing therapeutic proteins compared to traditional cell culture systems. Large numbers of patients with serious diseases such as cancer have been treated with such therapeutics, for example Herceptin. The use of biotech animals as reactors ideally suits the production of these therapeutics.

This proposed research underpins New Zealand's efforts to create additional opportunities for the pastoral industry through the application of new biotechnologies. AgResearch as a Crown Research Institute (CRI) is charged with the responsibility of ensuring New Zealand's transformation to a high-value, knowledge-based economy. AgResearch plays a key role in achieving this objective and has done so through the changes over the last 30 years from the DSIR, MAF and to the current system. Today AgResearch adds value to the New Zealand pastoral industry through innovative technologies. The government sees CRIs as agents for transformation by creating the value that will create advantage for New Zealand. Through the Foundation for Research Science and Technology competitive funding model the Government has invested over \$12 million in this programme over the last decade. The success of this programme has attracted international partners and the Government has committed to extend its investment by another 8 million over the next five years.

The Government's investment is complimented by the strong interest that the major players in the biopharming sector in setting up operations in New Zealand in association with AgResearch. These players include GTC Biotherapeutics, GTC, USA; and Pharming NV, in the Netherlands. The Organisation for Economic Co-operation and Development (OECD) has estimated the potential contribution of the Bioeconomy to New Zealand GDP to be around \$182 billion by 2030 (OECD 2009). This requires New Zealand to capitalise on the scientific advances in the biotechnology sector and apply this in areas that complement primary production, The OECD report also suggests that New Zealand's economy is at risk and Government and Industry need to actively support innovation in biosciences.

Eleven years ago AgResearch obtained the first in a series of approvals from the Environmental Risk Management Authority (ERMA), to undertake specific transgenic work with cattle. In recent years AgResearch scientists have developed and bred small herds of transgenic cows. As a result of this work, AgResearch has developed a world-leading capability in transgenic livestock research and development. In addition, AgResearch has freedom to operate with all relevant transgenic technologies. New Zealand is free of certain animal diseases, such as transmissible spongiform encephalopathies and this positions AgResearch and New Zealand to take a leading international role in commercial applications of transgenic livestock.

Therapeutic monoclonal antibodies such as Herceptin used to treat breast cancer constitute some of the most attractive candidates for biopharming because they comprise a fast growing segment within the pharmaceutical sector and command high market values. Sales of the currently approved monoclonal antibodies were more than USD\$23B in 2006 (2012 est. USD\$36B). The impending patent expiry of many leading products has created a number of highly attractive market opportunities for companies that have the ability to produce 'biosimilars'; a market with high returns from sales into large well established markets. GTC has developed a transgenic production platform for the cost-efficient, large-scale production of recombinant therapeutic protein. The use of this platform has been validated through the approval of ATryn® (recombinant human antithrombin) by the European Medicines Agency in 2006 and for the US market.