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Novel Gene Therapy Yields Funding Support

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Little did Eric Kmiec, PhD, Associate Professor of Pharmacology, suspect when he was addressing an NIH-sponsored meeting in April, 1994 that he was auditioning to become the focus of a new biotechnology start-up company. In the audience at that meeting on emerging gene therapies was Mark Fisher of the investment banking firm of Alex. Brown & Sons. Mr. Fisher travels to such meetings seeking likely candidates for start-up opportunities. He was very interested in Dr. Kmiec's novel approach to gene therapy, which involves the use of a chimeric vector based on the activity of the Rec 2 gene product invented by Dr. Kmiec to repair defective genes. Current gene therapy for inherited diseases having specific gene mutations, such as sickle cell anemia and thalessemia, involves extraction of the patient's bone marrow, correction of the genome, then reintroduction of the marrow into the patient. To be successful, the progenitor cells introduced into the marrow must populate the body. Dr. Kmiec's approach is radically different, involving gene targeting and in vivo repair of the mutant gene which caused the diseased condition. Normal genes would then repopulate the body after which the body will no longer be subject to the disabilities caused by the defective gene. (Jefferson is the owner of the patents covering this invention.)

Negotiations were opened among Dr. Kmiec, Mr. Fisher and Jefferson's offices of Technology Transfer and Research Administration, culminating in the execution of several research and licensing agreements. At the same time a new company, Kimeragen, Inc., was established, with control held by Fisher and his co-investors, and significant minority interests held by Dr. Kmiec and Jefferson. A funding program was agreed upon and the project was formally implemented September 1, 1994. Kimeragen, Inc., is committed to raising a minimum of $400,000 in its first year to support Dr. Kmiec's research, which is budgeted at $370,070 in the initial 12-month period and $1,160,000 for its first three years. This project is the sole venture to which Kimeragen, Inc. is committed.

Though several pharmaceutical firms were interested in acquiring or funding Dr. Kmiec's project, he has found the Kimeragen approach much better for his purposes. Foremost, Dr. Kmiec retains full scientific control of the work. The external funding has enabled him to expand his laboratory and hire an additional faculty-level scientist and an additional technician. While continuing to develop his chimeric vector, Dr. Kmiec remains free to pursue other grant opportunities (he has several other research grants currently), and he retains full editorial control over any articles he may submit for publication. Finally, should the chimeric vector prove to be as effective on a large scale as it has been in confines of the laboratory, all parties involved with Kimeragen could receive substantial monetary rewards.

About the Author

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