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# Exploring Gene Therapy

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# From the Dean



*As the crocuses and daffodils break the new Spring's crust, we as a school are showing our new colors.*

*The Markey Trust has provided us with a marvelous grant to support a new effort in molecular genetics at both the Veterinary and Medical Schools. The research supported by the grant will greatly enhance our understanding of the application of gene therapy to the benefit of animals and, hopefully, man.*

*At our New Bolton Center campus, the Connelly Intensive Care Unit/Graham French Neonatal Section is taking its final form and shortly we will be dedicating the Stubbs Laboratory, an important poultry research and diagnostic resource generously funded by the Commonwealth of Pennsylvania.*

*As the scaffolding on our historic quadrangle is dismantled, the traditional symbol of the school with its beautiful brick facade will shine anew.*

*We have a new fiscal plan, a new administrative organization, and new ideas for the School's direction. We have begun a strategic planning process to define our role in veterinary education. This effort, funded by the Pew Charitable Trusts, will help prepare us for the needs of veterinary medicine in the 21st century.*

*As my first year in the deanship comes to an end, I am thankful for the support of the faculty and staff, the continuing kindness of our friends, the confidence of our many agricultural constituencies, the enthusiasm of our students, and the commitment of the Commonwealth of Pennsylvania to our educational, research, and service mission.*

—Edwin J. Andrews, V.M.D., Ph.D.

## Exploring Gene Therapy

Under a \$3.8 million grant from the Lucille P. Markey Charitable Trusts, Penn will build up genetic research in the next five years in two health schools—Medicine, and Veterinary Medicine—with a view to treating genetic disease at the molecular level.

Dr. Mark I. Greene, professor of pathology in laboratory medicine and director of research immunology at the School of Medicine, and Dr. Donald F. Patterson, the Charlotte N. Sheppard Professor of Medicine at the School of Veterinary Medicine, are co-principals in the project.

The \$3.8 million grant is one of 11 given across the country. The Lucille P. Markey Charitable Trust commenced operation in November 1983 under the provisions of the will of the late Lucille P. Markey, who died in Miami in 1982. She directed that the Trust's assets be used exclusively for "supporting and encouraging basic medical research" and that all funds be spent by 1997. The recent round of awards, totalling \$75 million, is earmarked for biomedical science, where the foundation intends to "identify cutting edge

research and support those projects over a long period," the Trust's director for medical science, Robert J. Glaser, said.

At Penn, the grant will be used primarily to add young researchers and new equipment. Dr. Patterson, chief of the Veterinary School's Section of Medical Genetics which he started in 1970, heads an existing Gene Therapy Research Group which includes Dr. Roy Schmickel, professor and chairman of the department of human genetics at the School of Medicine, and colleagues from the School of Veterinary Medicine—Dr. Gustavo Aguirre, professor of ophthalmology and medical genetics; Dr. Ralph Brinster, the Richard King Mellon Professor of Reproductive Physiology; Dr. Mark E. Haskins, associate professor of pathology and medical genetics; Dr. Urs Giger, assistant professor of medicine in medical genetics; Dr. Vicki Meyers-Wallen, assistant professor of reproduction in medical genetics; and Dr. John H. Wolfe, assistant professor of pathology and medical genetics.

Research is aimed at correcting the gene functions

that have been lost in the cells of animals with genetic diseases. The investigators will isolate the normal gene and, through genetic engineering, construct a modified version of the gene that will assure its normal function in the defective cells. In initial studies, scientists will take cells from an animal patient and grow them in culture. Then, what scientists call "an engineered normal counterpart" to the defective gene will be transferred into the cells. The corrected cells will then be returned to the patient.

Since animals have many of the same genetic diseases as humans, the research is expected to lead the way to new approaches to therapy for a variety of human as well as animal genetic diseases. The first priority of the research will be a number of life-threatening or seriously debilitating genetic disorders for which no effective method or treatment now exists.

The group has been working for several years on treatment of diseases that involve enzyme deficiencies. One is mucopolysaccharidosis, a debilitating and sometimes fatal disease of both children and animals.