Over the next few months, Gaulton and the donor examined the network for the child’s particular illness within the landscape of orphan diseases as a whole. Each orphan disease (defined as affecting fewer than 200,000 U.S. patients) is unique, with specific causes, areas of research, and treatments. Each also has its own patchwork of investigators, clinical care providers, supporting foundations, and affected patients and families. Yet these diseases also have central elements in common, including the reliance on common research methodologies to determine the genetic basis and biochemical causes of disease and the need to rapidly advance research to clinical applications. Addressing these properties is essential for researchers to develop new therapies and cures. However, the vast majority of these disorders are under-funded by traditional government and pharmaceutical company sources because the potential benefits affect so few. These resources are also both expensive and technologically challenging, making it difficult for small research teams to progress on their own.

As the two men talked, the idea of creating a new kind of resource that could address not just one but simultaneously many orphan diseases began to take shape. Researchers could draw on the new program for state-of-the-art scientific support, including access to gene mapping and sequencing, drug screening, small and large animal models, as well as being able to conduct clinical trials and submit FDA applications. Foundation advocacy groups and families could draw on the program to organize scientific symposia and grant award programs, in essence cut-
Every family and every community knows the losses caused by behavioral disorders. From the severely depressed parent, to the college freshman struggling with substance abuse, to the traumatized returning veteran – these conditions harm individuals and families across the social spectrum and cause suffering on a massive scale. Many of these disorders are especially heartbreaking because they tend to affect people in the prime of life and represent a huge loss of productive potential. And while neurodegenerative diseases like Alzheimer’s generally occur toward the end of life, their effects on patients and families are no less tragic.

The members of one family made the decision to do all they could to combat this heavy personal and societal toll.

These donors were seeking nothing less than a transformation in the way we approach these areas – from research, to treatment, to outcome. They were long accustomed to turning to the best, and for them that meant Penn. Brian L. Strom, M.D., M.P.H., executive vice dean for institutional affairs and professor of biostatistics and epidemiology, became their guide on how a significant investment might make a real difference.

The discussions with Strom reinforced the donors’ view of Penn as the place for transformation. The presence of an already distinguished scientific and medical neuroscience community at Penn became the basis for their decision to create the Neuroscience of Behavior Initiative, which will focus on three areas: substance abuse, depressive disorders, and neurodegenerative diseases.

As a successful businessman, the head of the family had long believed that the best way to get results is to invest in people, so continuing discussions centered on creating a vehicle for bringing the very best minds in the field of behavioral neuroscience to Penn Medicine. It would be an initiative based on scientific stars – risk-takers who have the courage to face opposition when exploring bold new ideas.

“We want jaw-dropping names up front that will say to the world of neuroscience that something special is happening at

(continued on page 42)
A NEW CENTER TO ADDRESS ORPHAN DISEASES (CONTINUED)

ting through the mass of scientific data and approaches using a "Think Tank" concept. The impact of such a center would be huge – the donor’s gift could help thousands of families. For a committed philanthropist, this became the "Aha!" moment.

And so the Penn Center for Orphan Disease Research and Therapy was born. The Center, the only one of its kind in the world, is dedicated to supporting research programs working on rare diseases with the specific technologies that they need to hasten scientific breakthroughs and to advance discoveries rapidly to the clinic. The Center will bring together scientists, provide administrative resources, create partnerships between academic institutions, foundations, and industry and government agencies, and serve as a resource for patients and families.

This is truly a global initiative, and Penn has made it clear that the Center will support the best work wherever it is occurring. As the search for the Center director is under way, the first round of grants has been funded; recipients are not only at Penn but also at such institutions as Weill Cornell Medical School, the University of California San Diego, and the University of Minnesota.

The Center for Orphan Disease Research and Therapy is a model for the far-reaching, collaborative, philanthropic endeavor Penn has the power to create. It provides every family with a chance to directly join in the fight against rare diseases. All this would not have happened without the strong philanthropic instinct of a concerned grandfather. For the donor, and now patients and families worldwide, this Center offers a fresh basis for hope.

A NEUROSCIENCE INITIATIVE TO COMBAT A VAST SOCIETAL PROBLEM (CONTINUED)

Penn," says Strom, who has been named to head the Neuroscience of Behavior Initiative. Great people attract more great people. Four search committees – one crosscutting and one for each area of investigation – are at work. Already the word is out, and premier scientists are approaching Penn leaders such as eminent Alzheimer’s disease researchers John Q. Trojanowski, M.D., Ph.D., and Virginia M.-Y. Lee, Ph.D., M.B.A., about new possibilities. Drs. Trojanowski and Lee are the co-director and director of the Penn Center for Neurodegenerative Disease Research, respectively.

Highly visible meetings and symposia are also being designed to bring people to Penn and increase the reach of the work. With this gift, the donor and his family are confident that the brilliant minds at Penn will have the tools to generate meaningful changes in the fight against behavioral disorders.

Jean Bennett, M.D., Ph.D., with members of families who support her work on gene therapy for Leber’s congenital amaurosis (LCA). A rare genetic disorder that results in the progressive and finally complete loss of vision, LCA is one of the orphan diseases that Penn researchers have studied.

Virginia M.-Y. Lee, Ph.D., M.B.A., and John Q. Trojanowski, M.D., Ph.D., leading researchers in neurodegenerative disease. Dr. Trojanowski chairs the Neurodegenerative Disease Search Committee and is also part of the Behavioral Neuroscience Initiative Internal Advisory Committee.
Abramson Family Foundation continues its distinguished tradition of philanthropy to Penn Medicine with a recent gift of $4.42 million to benefit cancer research at the Abramson Family Cancer Research Institute.

Canon, Inc. donated an Adaptive Optics Scanning Laser Ophthalmoscope to the Scheie Eye Institute. Valued at $3 million, this powerful microscope yields real-time views of the living human retina with unprecedented optical quality, helping to reveal retinal disease and improve diagnosis.

M. Thomas Grumbacher and Nancy T. Grumbacher have generously made a first-time pledge of $1 million to create the Nancy Grumbacher Ovarian Cancer Research Fund, which supports the research on ovarian cancer by Dr. George Coukos in the Department of Obstetrics and Gynecology at Penn Medicine.

Mr. James S. Riepe and Mrs. Gail Petty Riepe pledged $1 million to endow the Arthur H. Rubenstein Endowed Scholarship Fund, honoring the School’s former dean. The fund will provide financial support to a medical student or students who would otherwise be unable to meet the cost of a medical education at the Perelman School.

Ralph and Brian Roberts pledged $1.5 million to establish the Roberts/Ende Department of Medicine Fund. This gift will support clinical, educational, administrative, and faculty development initiatives in the Department of Medicine at Penn Presbyterian Medical Center, including the creation of the Chief of Medicine Service, to be directed by Dr. Jack Ende.

Mr. Richard W. Vague, through a pledge of $5 million, funded the Richard W. Vague Endowed Professorship in Immunotherapy at the Perelman School of Medicine and established the Richard W. Vague Pancreatic Cancer Immunotherapy Research Fund to provide financial support for work in pancreatic cancer performed at the Abramson Cancer Center by the Associate Director for Translational Research, currently Dr. Robert Vonderheide.

Joseph R. Zebrowitz, M.D., and Lauren J. Wylonis, M.D., have pledged $1 million to establish a variety of funds that will benefit some key initiatives at the School, including medical student aid, child forensic psychology, enhanced patient care, and the new Medical Education Center.

For more information, please contact the Office of Development and Alumni Relations at 215-898-0578.

To make a gift, please mail your check made out to The Trustees of the University of Pennsylvania to: Penn Medicine Development and Alumni Relations 3535 Market Street, Suite 750 Philadelphia, PA 19104-3309

To make your gift online, please visit: www.med.upenn.edu/alumni/gifts

American Urological Association Reception
Monday, May 21, 6:00 p.m.
Marriot Marquis, Atlanta, Ga.

Red Carpet Premiere of Head Games
Thursday, June 7, 6:00 p.m.
Translational Research Center, Rubenstein Auditorium

Head Games is a revealing documentary about the concussion crisis in sports, from the acclaimed director of Hoop Dreams, Steve James.

Penn Medicine in Bar Harbor
Wednesday, August 8, 9:30 a.m.
Asticou Inn, Bar Harbor, Maine

For more information on these events, please e-mail PennMedicine@alumni.med.upenn.edu