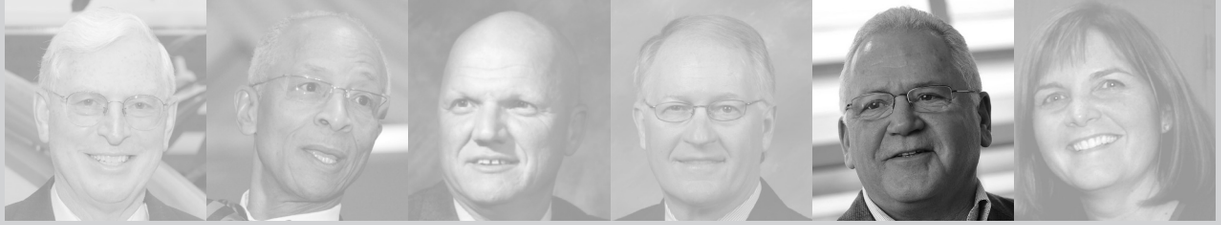

# DISTINGUISHED ALUMNI AWARDS

## Norman W. Barton '74g, '76g Med

**To Norman W. Barton for researching, developing, and making widely available innovative therapies that improve the lives of people with rare, life-threatening disorders.**

As a student at the Penn State College of Medicine in the 1970s, Norman Barton studied the use of enzyme replacement to treat a rare disorder that typically causes sufferers to face a painful life and early death. The work was hard and often tedious. Barton's novel approach meant there were many doubters. But at Penn State and later at the National Institutes of Health (NIH) Barton and his colleagues persevered.

"Our problem was to direct an enzyme to a specific location in a specific cell," he said. "We did that by modifying the structure of the sugars on the enzyme so that the enzyme would bind to the appropriate receptor and eventually move to the lysosome where the undesirable material had accumulated. It took an incredible amount of work to figure out the engineering, and further to show that what we had done was beneficial to the patient, but it was—100 percent of the time."

Barton can remember when his focus on rare diseases, as opposed to better-known afflictions like cancer or heart disease, was considered eccentric. Today, though, the early study of those orphan disorders is widely recognized as the foundation of one of the most exciting frontiers in medicine, the development of treatments that target personalized genetic makeup.

Barton earned his doctorate in biochemistry and molecular biology from the Penn State College of Medicine in 1974 and his M.D. in 1976. He is the author or co-author of more than 90 papers on subjects including enzyme replacement therapy, which has become the effective standard of treatment for a half dozen genetic storage disorders. Recently, Barton had the opportunity to attend a reunion of patients who participated in his early clinical trials.

"Once, these were children with a grim prognosis. Now they have grown into handsome young men and women with spouses and families and careers," Barton said. "Wow! That's exciting."

Barton comes from a long line of medical professionals on his Canadian father's side of the family. He pursued undergraduate studies in biology at the University of Toledo where a charismatic instructor kindled his interest in protein chemistry and metabolism. When in 1968 he interviewed at the Penn State College of Medicine, he found "an innovative, pioneering spirit committed to a new way of doing things."

Among Barton's mentors at Penn State was doctor Abe Rosenberg, who introduced him to Roscoe Brady at the National Institute of Health. Brady was an early pioneer of the concept of enzyme replacement therapy, and he and Barton had a very productive collaboration that extended from the early 1980s until 1996. Today, they remain in close contact. Another influence was Graham Jeffries, who served as the chair of the department of medicine for more than 20 years—and who is being inducted as an honorary Penn State alumnus this year.

In 1996, Barton earned the meritorious service medal of the U.S. Public Health Service. Since then, he has held a succession of increasingly responsible posts with forward-looking biotechnology companies, currently serving as head of global medical affairs, senior fellow, and disease expert at Shire Human Genetics Therapies.

At Shire, his primary focus is developing therapies to treat the brain manifestations of storage disorders such as metachromatic leukodystrophy by administering proteins directly to spinal fluid, an approach that may lead to a variety of new treatments for previously intractable conditions.

Barton credits his Penn State experience with launching a career committed both to science and service.

"The path has been extraordinarily exciting and fulfilling," Barton said. "I've had the opportunity to improve people's lives and to see my work extended by corporations that have the wherewithal to do good. I've been fortunate to have the opportunity to improve people's lives in a global way."

Barton and his wife, Ellen, a healthcare attorney, live outside of Baltimore. Their family includes four sons and six grandchildren.