

Neufeld Steps Down as Department Chair with Big Plans for Her Future

By Elaine Schmidt

Twenty years of leadership as chair of UCLA's Department of Biological Chemistry would appear a crowning achievement to most careers—particularly when it caps off a 21-year tenure at the National Institutes of Health. But Elizabeth Neufeld, Ph.D., has no intention of resting on her laurels.

"I plan to devote the next several years to my research," says Neufeld, 76. "I haven't had the chance to proceed as fast as I'd like, and I have definite goals for the future."

The children's faces pictured in her office reflect the urgency behind Neufeld's drive. Posters, news clippings and plaques describe the young lives made possible by her research on a group of inherited diseases called mucopolysaccharidoses (MPS), which include Hurler and Sanfilippo syndromes.

MPS affects lysosomes, the cell's "recycling plants" that use enzymes to break down leftover molecules for reuse. If a lysosome has a missing or defective enzyme, the undigested material accumulates, leading to misshapen cells and enlarged organs. The progressive MPS disorders often lead to neurological deterioration, abnormality of the bone structure, mental retardation and childhood death.

Neufeld's research at NIH identified the enzyme deficiencies that cause MPS. Her findings led to tests that enabled physicians to accurately diagnose the syndromes and counsel families, and paved the way for enzyme replacement therapy.

Leonard Rome, Ph.D., senior associate dean for research at the David Geffen School of Medicine at UCLA, vividly recalls his formative years as a postdoctoral researcher in Neufeld's lab at NIH in the late 1970s.

"We worked elbow-to-elbow with Liz in a crowded lab," describes Rome. "The atmosphere was so charged and stimulating that we never wanted to leave for fear we might miss the next big finding. We were driven by Liz's brilliant insights and instincts for helping us interpret often puzzling results. It was a very exciting time."

The findings from Neufeld's laboratory also laid the foundation for enzyme-replacement therapies, a breakthrough that has allowed many MPS children to live longer, more normal lives.

Dr. Emil Kakkis, who received his Ph.D. from the Department of Biological Chemistry, later worked in Neufeld's UCLA lab as a medical genetics fellow, where he produced recombinant enzyme for the treatment of Hurler syndrome.

"Liz Neufeld was the scientists' chairman," Kakkis says. "She took care of new department recruits and set the pace for transforming a department that was in need of renovation to the high rank it holds today."

Now a senior vice president at BioMarin Pharmaceutical, Kakkis built on his work with Neufeld to bring the therapeutic enzyme for Hurler's syndrome to clinical testing and approval by the Food and Drug Administration in 1983.

"That was the 'Big Event,'" recalls Neufeld. "My lab had conducted the basic science for the drug and proposed the treatment in the late 1960s. Emil led the clinical testing on the enzyme and made it into a pharmaceutical drug. I'm very happy about that."



With characteristic determination, however, Neufeld always has her eye on the next research challenge.

"The enzyme used in MPS therapy isn't able to penetrate the blood-brain barrier," she explains. "As a result, current replacement therapy cannot help children with Sanfilippo syndrome, which leads to profound mental retardation. I'm working with Dr. Betty Chen in my lab to address this. We believe that our findings will apply to other neurological diseases."

Neufeld's contributions have earned her many of the highest honors in science, including the 1994 National Medal of Science, the 1990 California Scientist of the Year Award, the 1988 Wolf Prize in Medicine and the 1982 Albert Lasker Clinical Medical Research Award. She is an elected member of the National Academy of Science, the American Academy of Arts and Science and the Institute of Medicine.

"There are few scientists that have trained their enthusiasm and focus on a rare disease for so long," notes Kakkis. "Liz's career is all the more compelling because her work resulted in new therapies for patients with MPS disorders."

Despite her prestigious accolades and rare talents, Neufeld is quick to deflect attention from herself and aim the spotlight at those around her.

"The exciting aspect of my job has been the opportunity to watch young scientists grow," she says. "I'm most proud of recruiting some wonderful faculty. Biological Chemistry has been a fertile ground for cultivating senior leaders, such as associate deans and center directors like Lenny Rome and Judy Gasson. Our department has contributed a remarkable share of scientific leadership to the medical school."

Neufeld is a modest and down-to-earth person. It doesn't occur to her that these faculty members are following her leadership example. She's too busy thinking about where she'll take her research next. ❖