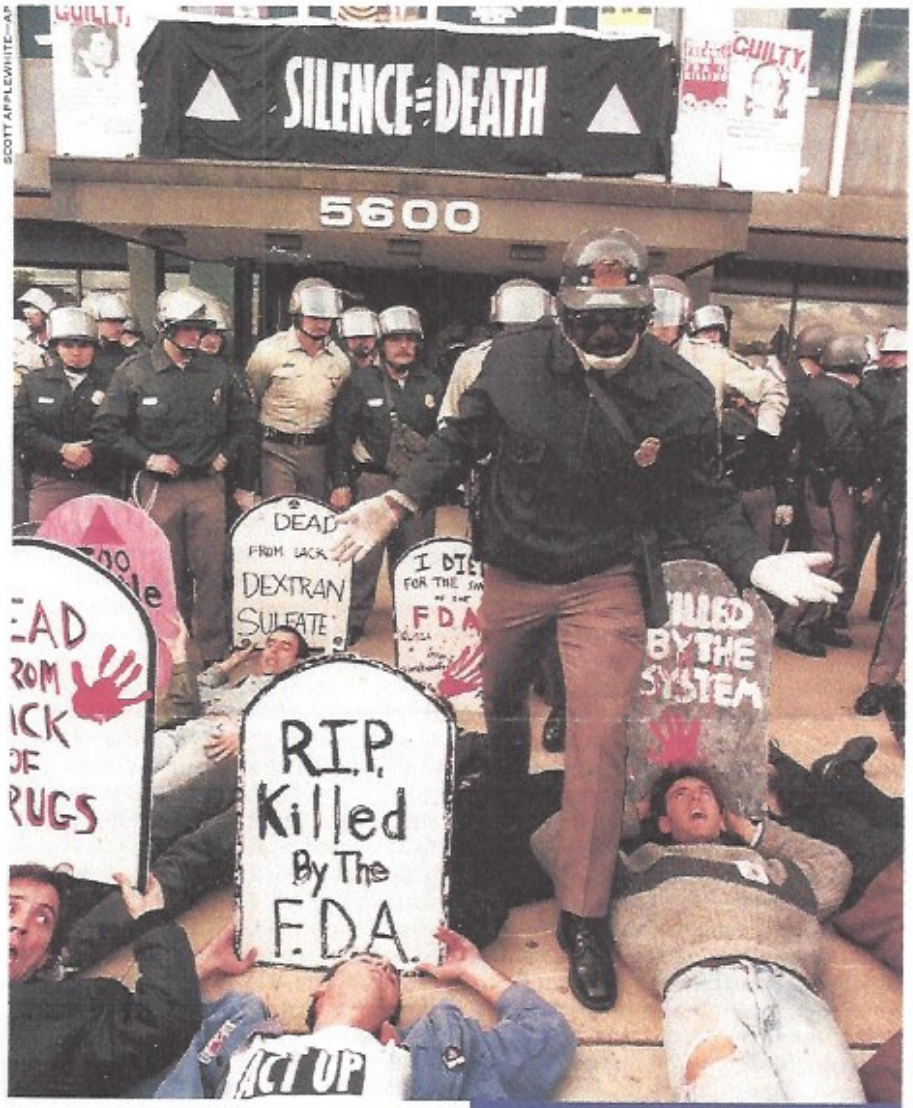
THEIR LIVES

Patient advocates are learning from AIDS activists how to work the system



SCOTT APPLEWHITE—AP

AIDS *When AIDS activists protested at FDA headquarters, other patient groups learned how effective confrontation can be*

The main target of the groups is a federal research enterprise perceived as being more interested in pursuing intriguing scientific questions than easing pain or finding the cures for afflicted patients. "The National Institutes of Health is a government institution that is supposed to be responding to the needs of its people," says Tom Sheridan, a lobbyist who began working with AIDS groups and now has other patient-advocacy clients, "and it hasn't always done that."

The AIDS lobby led the crusade to force the NIH to change its ways. Fully 10% of the NIH's $8.9 billion budget currently goes into AIDS research. The FDA created the Office of Special Populations just to handle the demands of the AIDS lobby. Activists have become so well informed that they now sit on many government advisory panels. Such recognition came only after years of confrontation: demonstrating outside the White House, shouting down the President at campaign stops and draping the home of conservative Senator Jesse Helms in a condom-shaped balloon. ACT UP members blockaded Wall Street and broke into the headquarters of Burroughs-Wellcome to demand, and get, a reduction in the price of the AIDS drug AZT.

Women's health groups have been especially effective in following the ACT UP model. They pressured the NIH to create an Office of Research on Women's Health and forced a re-examination of the practice of excluding women from clinical trials. Last spring breast-cancer activists deluged Congress with 650,000 letters from women demanding more research into the causes of the disease. Funding has jumped from $60.9 million five years ago to $132.7 today. "Women saw what could be done," says Fran Visco, a trial attorney in Philadelphia and president of the National Breast Cancer Coalition. "The more noise they made, the more successful they became. We decided we just couldn't be polite anymore."

Other groups of cancer patients have also broken out from the umbrella of the American Cancer Society. Lloyd Ney, a 73-year-old retired engineer, learned in 1984 that he was dying of prostate cancer. Told he had just months to live, Ney went to Canada in search of an experimental treatment—and found the hospital waiting room filled with Americans. From that encounter came not only the treatment that may have added years to his life but also a mailing list for PAACT, or Patient Advocates for Advanced Cancer Treatments. Eight years later, Ney continues to work out of his basement, communicating with a membership of 12,000 and managing a budget of $750,000. He is credited with helping get the once experimental treatment approved in the U.S., and now spends his time lobbying for new therapies and responding to the hundreds of inquiries he gets every week about prostate-cancer treatment.

Other radicalized patients arrived at their activism by similar routes. Most of them suffer from diseases that are rare or difficult to diagnose, which means that they were shuttled from specialist to specialist, leaving a trail of money and test results but no answers or cures. By banding together and sharing information, patients become informed consumers. Many groups have a Rolodex of scientists and physicians who can be called upon to educate a local doctor confronting his first case of a rare disease. Information campaigns help improve diagnosis, which in turn gives public health officials a more accurate sense of the impact that even rare diseases have.

Perhaps the most successful awareness campaign has been conducted by the Lyme Borreliosis Foundation. In 1988 Lyme disease was scarcely known even in the area around Lyme, Connecticut. Karen

Forschner, who had the Lyme-disease symptom of a bull's-eye rash during pregnancy, started the foundation with her husband Tom shortly after the birth of their only child, a son who was finally diagnosed with the disease. "Using the media is definitely a part of our strategy," says Tom Forschner. The husband-and-wife team has captured the attention of all the daytime talk shows and most newspapers and magazines. By 1990 a Gallup poll found that Lyme disease was recognized by 88% of the U.S. population. "We lived 40 miles away from Lyme, and Karen's physicians didn't recognize her rash," says Tom. Their son died last year of the disease. "Awareness would have made a huge difference."

Information campaigns are just the starting point. Medical researchers acknowledge that their work very often proceeds without anyone taking a broad view. "The approach to diseases in general has been sort of haphazard," says Donna Brogan, chairperson of the biostatistics division at Emory University's School of Public Health and a member of the research task force for the National Breast Cancer Coalition. By organizing their own scientific meetings, advocates help assess the state of research for a particular disease and look for areas that need strengthening. "That's unique to them," says NIH director Healy. "They are setting bold, far-reaching goals."

The drug industry has also felt the heat from new pressure groups. Abbey Meyers of the National Organization for Rare Disorders is acknowledged as the force behind creation of the Orphan Drug Act. This federal statute provides incentives for companies to develop drugs for rare diseases that might otherwise be overlooked by firms seeking more lucrative markets. Meyers is now back in Washington lobbying for a revision in the law. She wants to close a loophole that has allowed companies to reap windfall profits and leaves the orphan drugs so expensive that a new treatment can be out of the reach of most American families.

THE MOST PROFOUND IMPACT OF the advocacy movement has come within the Food and Drug Administration. The agency has for decades held to the rigid standard that new drugs must be unequivocally proved to be both safe and effective. But in the wake of intense lobbying, the FDA will now consider granting conditional approval to experimental treatments for terminal diseases for which there are few or no medical alternatives. This is a radical shift for the nation's pharmaceutical watchdog. Drugs conditionally approved will be closely monitored and withdrawn if they prove to be too toxic or ineffective. "We may be wrong," says FDA Commissioner David

ARMEN KACHATURIAN—GAMMA-LIAISON FOR TIME

LYME DISEASE

Karen Forschner, whose son died of Lyme disease, started a foundation to help raise public awareness

Kessler, "but with life-threatening illnesses, those risks are acceptable."

Some scientists are worried about the growing influence of patient groups, particularly their success in persuading Congress to micromanage research. In the past, elected officials were reluctant to give detailed directions to scientists. But for aggressive patient lobbyists, the lawmakers were an easy target. Activism on the front lines of medical research plays well to the hometown constituency. Now attached to every check from Congress is a growing list of what the NIH must do—even while the total allocation remains roughly the same. The only way for the NIH to follow Congress's orders is to eliminate existing programs and transfer the resources. This year's budget for the National Cancer Institute, for example, contains an order to increase by more than one-third spending on breast-, cervical- and prostate-cancer research. Yet congressional funding for the institute did not even keep pace with inflation.

Many NIH watchers are now concerned that responding to congressional whims will undermine America's biomedical-research structure. "We're seeing the pie being split up into smaller and smaller segments," says David Moore, an official with the Association of American Medical Colleges. "Some of these groups have to be cautious," says John Seffrin, CEO of the American Cancer Society. "They could advocate for major shifts in funding in ways that on the surface makes sense but in the long haul do great violence to the scientific effort. It raises the prospect that these precious resources can be wasted."

Researchers complain that junk science is interfering with real science and threatens to disrupt progress toward actual cures. Durland Fish, a Lyme-disease expert at New York Medical College, has been appalled at the studies put forward as science by clinicians funded by the groups. He recalls a recent meeting at which a doctor claimed evidence of transmission of Lyme disease through blood transfusion—without ever establishing that the recipient even had Lyme disease. The AIDS advocacy group Project Inform ran its own trial of an experimental drug called Compound Q, a purified protein derived from a cucumber-like Chinese plant. At least one of the test subjects, a man with AIDS, died from toxicity associated with the effort. "It's very sad and it's scary," says Fish. "These groups have a lot of political power now and a low appreciation of the scientific method." Agrees Peter Scardino, chairman of the urology department at Baylor College of Medicine: "These groups have a very dangerous side to them when they take the place of careful scientific investigations. They very deliberately say to their members, 'Don't let your doctor talk you into this or that.' It takes a long time for us to talk these people down and re-educate the patient."

It is too soon to judge how much junk science will be funded to appease these powerful groups. But the danger is that allocation of scarce research resources will devolve into a kind of political mud wrestling, with the spoils going to the most powerful and not the most needy. George Rehnquist knows one thing about the future. Two weeks ago, after he led Alzheimer's family members in a demonstration outside the offices of the FDA, Commissioner Kessler invited Rehnquist to come back this month for talks. It's another step toward the day when THA will be fully approved. ■