A crusader against rare diseases, Henri Termeer is credited with building the market for "orphan" drugs, which treat illnesses affecting only small numbers of people. His crowning achievements at Genzyme, a biotech group set up in 1981, were not just the development of new medicines for devastating genetic disorders, but also his ability to convince healthcare systems to pay high prices for the company's products.

By showing that it was possible to profit from diseases that Big Pharma had dismissed as uneconomical, Termeer helped spawn a new generation of biotech groups dedicated to illnesses that had largely been ignored. These include cystic fibrosis, which affects mostly the lungs; Pompe disease, which causes muscle weakness and difficulty breathing; and spinal muscular atrophy (SMA), involving progressive muscle wasting.

Until then, drugmakers had assumed they would not be able to recoup the costs of developing such niche medicines.

The Dutch executive, lauded as a crusader against rare diseases, was 71 when he died in May last year after collapsing at his home in Marblehead, Massachusetts.

During his tenure at Genzyme, Termeer turned the company from a tiny outfit that had 20 staff into one of the world's largest biotech groups with 12,500 employees.

In doing so, he played a major role in the emergence of the Boston-Cambridge cluster in Massachusetts as the epicentre of the world's life sciences industry.

Because Genzyme made medicines for such a small number of people, Termeer was able to take a different approach to drug development, which saw the company forge enduring personal relationships with patients that benefited from its products. That in turn gave rise to a patient advocacy movement for rare illnesses.

"His major accomplishment was he helped invent the whole area of rare disease drug development by keeping the focus on the patient — he was all about the patient," says Michael Rosenblatt, who served on several boards with Termeer, and who is the chief medical officer of Flagship Pioneering, a life sciences venture fund based in Cambridge.

Born in the Netherlands in 1946, Termeer studied economics at the Netherlands Economic Hogeschool, Erasmus University, before earning an MBA at the Darden School of Business in the US. He spent 10 years in sales and marketing at Baxter, a large US drugmaker, before taking a 50 per cent pay cut to become Genzyme's president in 1983.

Genzyme's first drug was an enzyme-replacement therapy for Gaucher's disease, a sometimes fatal condition caused by the body's inability to break down lipids. Unchecked, the fatty substances build up in the bone marrow, liver and spleen, causing swollen organs and confining sufferers to a lifetime of pain.

Although the drug, which was originally identified by the US National Institutes of Health, at first appeared promising, subsequent trial data were less clear-cut and many in the industry faulted Termeer as unrealistic when he tried to raise funds for fresh studies in 1987.

But Termeer persisted, emboldened by what Dr Rosenblatt describes as a "journey of enlightenment". Termeer had seen the "enormous impact" the medicine had on a four-year-old victim of Gaucher's and was determined to extend its benefit to others.

The subsequent trials were a success and, in 1991, Genzyme won regulatory approval for the medicine, which was marketed under the Ceredase brand. It was a first not just for people with Gaucher's, but also for the field of pharmacoeconomics, with a price tag of $150,000 per year.

To the surprise of some in the industry, Termeer convinced insurers and governments it was a price worth paying. Today, it is not uncommon for rare disease medicines to cost in excess of $300,000 annually.

In the following years, Genzyme would bring out medicines for other rare diseases such as Pompe.

Termeer had hoped to keep Genzyme independent, fearing its patient-focused approach would be stifled if it were acquired by a large pharmaceutical company. However, that dream began to unravel in 2009, when its main factory became contaminated with a virus, earning it a rebuke from patients, regulators and shareholders and forcing it to drastically scale back production.

The group's share price tumbled, creating an opening for activist investors, including Carl Icahn, who agitated for Genzyme to be sold.

After nine months of fending off advances from Sanofi, the French drugmaker, Termeer capitulated and agreed to the sale of the company for $20bn.

The takeover came as a blow to Termeer, who resigned as chairman and chief executive when the sale was completed in 2011.

Termeer remained active in biotech after his departure from Genzyme, becoming a director of several companies while joining the boards of various not-for-profit institutions.

Some of today's biotech executives say the sale of Genzyme in fact ended up reigniting the US biotech scene, because so many of his protégés left the company to start or join new outfits.

"What Henri did [while he was in charge] was he instilled so much passion that there was so little staff attrition at Genzyme," observes Stephane Bancel, chief executive of Moderna, a large private biotech company that counted Termeer as a director until he died.

Mr Bancel adds: "Then, when it was sold, there was a total implosion, and today there is almost no one left at Genzyme from Henri's days: and that ended up really seeding the biotech industry with new leaders in a big way."
New focus: Genzyme forged enduring relations with its patients — Getty Images

Henri Termeer: had seen the ‘enormous impact’ of treatment