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EDITOR'S PAGE

# Developing Breakthrough Drugs for Heart Failure

## Lessons Learned From the Cystic Fibrosis Experience

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In this issue, the second of our series on the U.S. Food and Drug Association (FDA), we describe innovation and opportunities at the FDA regarding drug development in heart failure. A team of scientists reports on changes at the FDA that include new programs such as biomarker qualification and clinical trial initiatives to provide new therapies for our patients. In the past 2 years, only 2 new significant drugs have been approved that enhance outcomes in heart failure. We obviously need to have more opportunities to benefit our patients, but looking at the history of the Cystic Fibrosis Foundation and its experience allows us to draw some important observations.

In 1989, the cystic fibrosis gene was discovered by a team of scientists funded by the foundation, which opened up a new channel for potential therapeutic opportunities. However, at that time, pharmaceutical companies had little interest in rare diseases and were focusing on the next large blockbuster drug, despite the devastating condition of cystic fibrosis (CF) with an average life expectancy of 32 years. The leadership of the Cystic Fibrosis Foundation developed a concept in which they would partner with the pharmaceutical industry in drug development. Through venture philanthropy, an unconventional approach was developed to obtain the resources necessary to tackle the continued problem of reduced outcomes in patients with CF.

The foundation developed a partnership with a pharmaceutical company. Using this partnership, the Cystic Fibrosis Foundation developed Cystic Fibrosis Foundation Therapeutics Inc., a nonprofit drug discovery and development affiliation partnering with the companies. Through this work, on January 31, 2012, the FDA approved the first drug to address the underlying causes of cystic fibrosis: ivacaftor/Kalydeco (Vertex Pharmaceuticals, Boston, Massachusetts). The foundation's target investment paid off, and it launched new and greater opportunities for patients with CF. Kalydeco is a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator indicated for the treatment of cystic fibrosis (CF) in patients age ≥2 years who have 1 of the following mutations in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R. Kalydeco is indicated for the treatment of CF in patients age ≥2 years who have an R117H mutation in the CFTR gene (1). As you can see from the labeled indication of this drug, it is a premier personalized medicine approach. The foundation sold the royalties to investors for $3.2 billion, allowing a significant amount of resources to continue to develop new drugs. This is an exciting model that the heart failure community must study and consider as a potent strategy.

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As the pressure continues on the government and industry to find manageable ways to conduct cardiovascular research for new drugs, the CF example may serve as a roadmap for HF investigators. Our patients deserve this innovative approach.

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