

Milestones SUMMER 2008

Update

News from the Cystic Fibrosis Foundation's
Major Giving Campaign

Dear Friends,

Several years ago, when we set the goal of the *Milestones to a Cure* campaign at \$175 million by 2010, we knew we were setting the bar high. Now, in the summer of 2008, we have reached the \$126 million mark, and I am confident that the goal is well within our reach.

The Foundation continues to make great progress in its scientific research efforts. In March, we achieved a historic milestone in a clinical trial of VX-770, an oral drug developed by Vertex Pharmaceuticals, Inc. that targets the basic defect in CF. In an ongoing Phase 2 study, a subset of CF patients who took the drug showed significant improvement in several CF indicators, including lung function, sweat chloride levels and nasal potential difference.

This is the first time that any potential therapy has improved the abnormal sweat chloride levels in a person with CF. VX-770 is particularly exciting because it addresses the basic defect in CF, rather than just treating the symptoms of the disease. The results are cause for great optimism and underline the importance the *Milestones* campaign plays in the development of new CF therapies. With your continued support, we will continue to explore other promising new treatments.

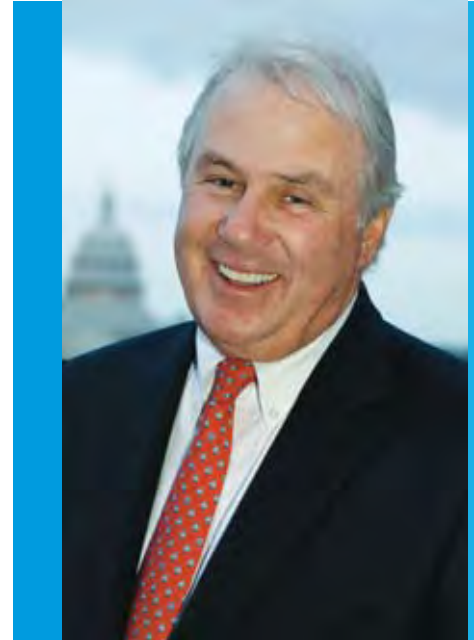
Meanwhile, the *Milestones to a Cure* campaign continues to gather momentum and to expand contributions across the country. Earlier this year, a group of key campaign leaders met in Menlo Park, Calif. to discuss strategies for marshalling new supporters in the Western region and accelerating the pace of major giving in potential new markets. We're excited by the many opportunities in previously untapped markets and encourage other regions to promote similar gatherings for discussion.

Your efforts, and those of your friends and colleagues in the CF community, are vital to the progress we are making in the battle to control and ultimately cure CF. Thank you, as always, for your generous support.

Sincerely,



Joe O'Donnell
Chair, *Milestones to a Cure*



Investments Into Action

Science Highlights

Support for *Milestones to a Cure* fuels the Foundation's Therapeutics Development Program, powering its many scientific investments and expanding new research opportunities. With more than 30 potential therapies currently in the pipeline—including the first oral drugs that address the basic defect in CF—the Foundation's research collaborations continue to yield groundbreaking results.

Treating the Basic Defect



Two compounds in the Foundation's drug development pipeline that address the basic defect in CF showed tremendous promise in clinical trials this spring. Early results from the Phase 2a trial of Vertex Pharmaceuticals, Inc.'s potential therapy VX-770 suggest that it improves the function of the faulty CFTR, the key protein associated with CF. VX-770 is the result of a decade-long collaboration with Vertex and a Foundation investment of \$76 million.

The interim data indicates that a subset of CF patients who took the drug over a 14-day period showed significant improvements in lung function, nasal potential difference and sweat chloride levels. This is the first time a therapy has improved sweat chloride levels in a person with CF and is the Foundation's most promising outcome from a Phase 2 trial to date.



Inspire Pharmaceuticals, Inc. also announced positive results from its first Phase 3 trial of denufosal, an inhaled CF therapy. Denufosal is designed to improve chloride transport in the airways and enhance mucus hydration and clearance. Results from the 24-week study showed significantly improved lung function in patients taking the drug.

Unlike VX-770, which aims to correct the faulty CF protein, denufosal seeks to treat this defect by using an alternative chloride channel to allow sodium and chloride to move in and out of the cell. Denufosal is the result of more than 17 years of Foundation-supported work. Inspire has begun a second Phase 3 clinical trial and is currently enrolling patient volunteers.

Advancing Other Strategies



The Foundation expanded its collaboration with EPIX Pharmaceuticals, Inc. following EPIX's successful development of a computerized 3-D model of the full-length CFTR protein. EPIX will receive up to an additional \$37.7 million to identify sites within the protein that may be good targets for treatment.



Albany Molecular Research, Inc. will receive up to \$23.7 million from the Foundation to explore natural products—substances taken from plants and bacteria—in an effort to increase the diversity of chemicals that may affect the faulty CFTR protein in CF.



GeneGo, Inc., the leading systems biology tools company, launched MetaMiner CF, a novel database designed to help advance CF drug discovery. This tool, developed with Foundation support, provides CF researchers with state-of-the-art methods for managing data, and increases the efficiency and pace of research.

...building a portfolio of progress



www.cff.org
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“With the CF Foundation on our side, I know we will win the battle against CF.”

Tina Allen, mother of Drew (left) who has CF

Giving A Weinberg Family Tradition



Jean Weinberg Rose and Joe O'Donnell

“Families like the Weinbergs are integral to the great strides we are making to achieve our goal—a cure for CF.”

Robert J. Beall, Ph.D.
President and CEO
Cystic Fibrosis Foundation

For the Weinberg family, the spirit of giving is a longstanding tradition. Cystic fibrosis has touched this family across generations and, unflagging in their desire to combat the disease, the Weinbergs have made multimillion dollar contributions to the Cystic Fibrosis Foundation.

Most recently, members of the Weinberg family have made a combined commitment of \$15 million to the *Milestones to a Cure* campaign. Over the past several years, John S. Weinberg and his wife Amy, mother Sue Ann, and sister Jean Weinberg Rose and husband Jeff have not only made generous commitments, but have also actively expanded support, encouraging friends, relatives, and business colleagues to join the Foundation's efforts. In addition, John and Amy serve on the *Milestones* Campaign Executive Committee, and Amy is a valued trustee of the Foundation.

At the heart of the Weinbergs' generosity is the family's determination to help accelerate therapeutics development and research. Thanks to their dedicated support, enormous progress is being made in the quest for a cure. Amy Weinberg calls the Foundation, “a visionary, best-in-class in every way.” She notes, “The CF Foundation has driven extraordinary advancements in scientific research, most recently, in addressing the basic defect in the CF gene. We believe that new progress is being made every day, and we have every reason to be encouraged by these efforts.”

“Families like the Weinbergs are integral to the great strides we are making in learning how to control and eventually cure this disease,” says Robert J. Beall, Ph.D., president and chief executive officer of the CF Foundation. “They have been with us steadfastly for years, as both friends and supporters, participating earlier in our *Bridges* campaign and now in our *Milestones* campaign since its inception. Their friendship and commitment are invaluable; we are enormously grateful for their support.”

Steadfast Support

of Generous Friends

Jim and Mary Bush's relationship with the Cystic Fibrosis Foundation began unexpectedly. Their daughter Jessica played on the same middle school basketball team as Jane Detrich, whose younger sister Lo has CF. Over time, the two families became friends, and the Bush's awareness of CF grew stronger. "Until we met Lo, Jim and I had no clue about cystic fibrosis and what a devastating disease it is," Mary says. "After meeting and becoming inspired by this brave and beautiful young girl, we simply had to get involved in the search for a cure."

Jim and Mary joined the CF effort when their friends, John and Stephanie Cole, encouraged them to get involved with the **Sooner Chapter – Tulsa Office**. They became active fund-raisers for the Foundation in 2000, and they chaired the chapter's biggest event of the year, the *All Sports Ball*. Jim, who became **Sooner Chapter – Tulsa Office** Board President in 2002, also served as chair for the *CF Golf Classic* in 2005 and was selected as honorary chair for the event in 2007. In 2003, the **Sooner Chapter – Tulsa Office** awarded the couple its highest honor, the Henry Zarrow Award for Outstanding Persons. Most recently, the Bushes made a generous \$250,000 commitment to *Milestones to a Cure*.

Jim, an independent businessman, respects and admires the Foundation's venture philanthropy business model. "The Foundation's strategy of investing funds for initial research and then for building on promising therapies is extremely efficient," Jim says. "We are confident that the dollars are spent where they can do the most good." He and Mary also recognize that individuals with CF are living longer, healthier lives as a direct result of therapies developed by the Foundation.

Today, the Bushes remain steadfast in their support of the Foundation, and their close friendship with Lo Detrich, now 24, continues to flourish. "Lo's courage and determination remind us every day of the importance of fighting the battle against CF," says Mary. "We're going to do all we can to help make sure Lo—and all people with CF—triumph over this disease."



Jim and Mary Bush

Accelerating Scientific Progress

CF Foundation Supporters Expand the Campaign

Founded in 1951 by Herman and Margaret Root Brown and George R. and Alice Pratt Brown, the Brown Foundation, Inc. has awarded more than \$1.1 billion in grants to support programs in education, the arts and community service, as well as in civic and public affairs, human services, and medicine and science.

To support the Cystic Fibrosis Foundation's mission of developing the means to cure and control CF and improving the quality of life for those with the disease, the Brown Foundation, Inc. recently made a generous commitment of \$500,000 to the *Milestones* campaign.

The Brown Foundation awards 80 percent of its grants within the state of Texas. The CF Foundation has invested \$3.6 million in research projects over the past five years in Texas. In addition, nearly 1,400 patients seek medical care from 11 CF Foundation-accredited care centers in that state.

With the Brown Foundation's support, the CF Foundation will continue to play an essential role in developing therapies that are now a routine part of treatment regimens for many with CF, and in extending the lives—and improving the quality of life—for those with the disease.



Jack Fiedor and Bob Feldman



Angela Kinney and Chris Palmer

Donors Gather

The logo for "Milestones to a Cure" features a stylized yellow sun with rays above the text "Milestones to a Cure" in blue.

Key volunteers and campaign leaders came together on March 7, 2008, to discuss new ways to rally support for the *Milestones to a Cure* campaign.



Marie and Tom Hennessey



Beau Guyette and Gina Schewe



Kirk Ogren