"I work for a large pharmaceutical company, and I certainly recognize the need for critical mass in the very challenging job of making safe and effective drugs. The people I work with are extremely dedicated to this task. However, I also recognize that, with diseases such as ALS, a small but highly motivated and imaginative group of people can make a big difference, when they are absolutely single-minded about facilitating a cure and at the same time deeply involved in education and support of patients and their families. For me, involvement with ALS TDF is a perfect complement to my 'day job.'"

David Sears, Ph.D., Vice President and Director, Bioinformatics, Smithkline Beecham Pharmaceuticals
Dear Friends,

As I write this letter, ALS TDF is completing its fifth year of operation. In many ways, I feel that 2003 represents the completion of our first chapter. Today, we are a state-of-the-art biotechnology research center. Today, we have made significant advances in our understanding of ALS. Today, we are better positioned than ever to deliver on our mission to slow, arrest, and cure ALS.

My brother Stephen has had ALS for six years now. Although his body in large part has been destroyed by ALS and he now uses a ventilator, his mental strength and outlook continue to astound those who know him. Stephen is connected to the world through some amazing technology, and he is an active member of both our family and society. He spends time doing what he loves—being a good father to his three year old son Alex and continuing his craft as a custom builder.

Three years ago, we began a project to restore the carriage house behind my home that was virtually falling apart. Wood was rotting, the roof had huge holes and remnants from the previous owner were everywhere. Stephen knew that it was the perfect project for him—he could transform it into a work of art. Today, the carriage house has a solid structure, new three-inch-thick cedar doors, and windows overlooking the woods behind it. There is still much to do inside, but the first stage of construction is complete.

This is similar to ALS TDF. Our first few years laid the extensive groundwork for the program that exists today. We started with a vision and began to build an infrastructure to cure ALS. Today, we have made great strides and have many reasons to be proud. We remain focused on developing therapies for today’s patients and because of our progress, we now work from a much stronger foundation—with clear direction and new tools. We now have a controlled, systematic process for drug studies in ALS mice—the leading program in the world. Our translational research program collaborates with leaders in the biotech and pharmaceutical industries and our unique business model allows us to do so without conflict or delays. We have made significant scientific advances into understanding both the underlying mechanisms of the disease as well as potential treatments—exemplified by the start of our clinical trial at the University of California at San Francisco.

There is still a great deal for us to accomplish, but I know that today ALS TDF is more efficient, driven and focused than ever before. Each day we better equip ourselves to fight this disease, learning from both our successes and failures. Most importantly, we do this with the lives of ALS patients at the forefront of our minds.

Thank you.

James Allen Heywood
d’Arbeloff Founding Director

Your support makes ALS TDF a leader in ALS research and gives hope to my family and to thousands of others with ALS everyday. With your passion and leadership, we are writing the next chapter together—I have never been more excited by the prospect of what lies ahead.

Thank you.
The ALS Therapy Development Foundation and the Larry L. Hillblom Foundation, Inc. announced the initiation of the Ritonavir and Hydroxyurea clinical study in 2003, to be held at the University of California at San Francisco (UCSF). The study will enroll at The ALS Center at UCSF under the direction of ALS specialist Dr. Richard K. Olney. Ritonavir and Hydroxyurea will be tested separately; however, future studies may look toward combining the drugs.

Ritonavir and Hydroxyurea emerged from ALS TDF’s drug screening pipeline and are both FDA approved. ALS TDF strives to test approved drugs whenever possible, shortening the time between discovery and the clinic.

Ritonavir is an antiretroviral protease inhibitor used for the treatment of HIV infection. Hydroxyurea is approved for chronic myelogenous leukemia and sickle cell disease. Hydroxyurea is also used off label for malignant gliomas since it is very CNS penetrant. Each compound achieved at least two positive studies before being selected as clinical candidates. The effect of each compound exceeded the effect of Rilutek under identical test conditions. This study is jointly funded by ALS TDF and the Larry L. Hillblom Foundation, Inc.

Vanna Forrester, the mother of ALS TDF’s Vice President of Drug Development, Sean Scott, passed away in October 2003 after a difficult three-year battle with ALS. Vanna’s first symptoms appeared in early 2000, when she had difficulty breathing after climbing stairs. While her prognosis was grim, she chose to fight aggressively.

With determined assistance from neurologists Rick Olney and Josh Novic, and information from ALS TDF’s research program, Vanna bravely tried many experimental treatments. Her experimentations directly caused the discovery of Hydroxyurea for ALS. ALS TDF tested Hydroxyurea after her first anecdotal reports of benefit and found that the drug showed a positive effect in multiple animal studies.

Vanna leaves as her legacy a clinical trial for Hydroxyurea, which is in its early stages at UCSF. She wanted nothing more than to see others benefit from her efforts and to avoid the horrors she faced. She was a hero to all who knew her or knew of her efforts. Sean joined ALS TDF in 2000 in an effort to help her, setting an aggressive pace for the research program and bringing an appropriate sense of immediacy to the decisions we make.
Dr. Harvey Lodish
Chair of ALS TDF’s Scientific Advisory Board

ALS TDF proudly announced the addition of a Scientific Advisory Board in 2003, with Dr. Harvey Lodish presiding as Chair. Lodish, a renowned leader in the field of membrane biology and his results have important implications for the treatment of cancer, diabetes, heart disease, and obesity.

“I’ve been very impressed by the way ALS TDF has uncovered promising drugs by testing a mouse model of ALS. My hope is that over the next year, the Scientific Advisory Board, working together with ALS TDF scientists, can devise new assays that are more rapid and less expensive and that will allow us to screen many more compounds for their potential as ALS TDF therapeutics.”

Lodish, a founding member of the Whitehead Institute for Biomedical Research, and Professor of Biology and Bioengineering, MIT, joined the MIT faculty in 1968 and has been a professor of biology since 1976. He earned his Ph.D. at Rockefeller University in 1966 and was elected a Fellow of the American Association for the Advancement of Science in 1986, a member of the National Academy of Sciences in 1987, and a fellow of the American Academy of Arts and Sciences in 1999. Lodish serves on advisory boards for the Cleveland Clinic Research Institute and the Division of Biology at CalTech. Currently, he is the President of the American Society for Cell Biology.

Harvey Lodish Photo: Sam Ogden
Our Funding

ALS TDF leads the way in drug discovery today due to a dedicated group of individuals and families that have banded together to help us reach our goal to slow, arrest, and cure ALS – thank you. Together, we have raised more than $14 million since 1999.

Jack Orchard ALS Foundation

Jack Orchard got involved with ALS TDF in the summer of 2002 after his friend told him about our unique approach. After visiting ALS TDF later that year, Jack was most intrigued by the drug screening model that couples the scientific approach with a real sense of urgency.

“At ALS TDF, where the focus remains on screening existing compounds known to be effective in treating conditions that share a biochemical pathway with ALS, hope accompanies every assay,” says Jack.

Betty Luke

Betty Luke went to school with Peggy Heywood, Stephen and Jamie’s mother, in the 1960’s at the Simmons College School of Social Work. Over the years, she, Peggy, and a few of their classmates would still get together for luncheon and kept in touch. When Betty heard that Stephen was diagnosed with ALS, she wanted to help and support the family and his friends when they founded ALS TDF.

“I’m fond of Peggy, and I had met John and her sons. I felt compassion and admiration for Stephen and for this very fine cause to find a cure or at least slow this disease. The story of this important undertaking is compelling and inspirational,” said Betty.

Betty has contributed generously to ALS TDF’s research and lives in North Andover, Massachusetts with her dog Gracie, a Welsh Corgie, and is a psychotherapist with a private practice in Andover.

Ride for Life

Ride for Life’s annual event draws scores of patients that ride their electric wheelchairs from Manhattan to the tip of Long Island to raise money to fund research for a cure along with public awareness about ALS.

“Basic biological research will someday unravel the mystery of ALS,” says Christopher Pendergast, President and Founder of Ride for Life. “Most patients living with ALS will die while waiting for this type of research to bear fruit. On the other hand, the type of research that is the main focus at ALS TDF is identifying currently available agents that will slow or stop the progression. Much like how aspirin was accidentally discovered to be a powerful treatment for cardiovascular disease.”

Ride for Life donated nearly $50,000 to ALS TDF’s laboratory in 2003, and more than $150,000 since ALS TDF’s inception.

Ronya Kozmetsky

Ronya Kozmetsky committed a $1 Million gift in 2003 to ALS TDF, representing her and her family’s commitment and desire to have the greatest possible impact on the advancement of a cure for ALS. The Kozmetsky family was introduced to ALS TDF in the fall of 2002. The patriarch of the family, Dr. George Kozmetsky, had ALS, and they decided to get involved in the ALS research arena. Dr. Kozmetsky, who passed away in April 2003, was the co-founder of Teledyne and the former Dean of the McCombs School of Business and Graduate School of Business at the University of Texas, Austin.

“I hope that Jamie can beat the odds for his brother,” says Ronya Kozmetsky. “Dr. Kozmetsky really appreciated and applauded the efforts of the ALS Therapy Development Foundation on his behalf and on behalf of all those living with ALS.”

“My husband died one year ago from ALS — it’s a horrible, horrible disease. He and my son Greg got involved first with ALS TDF, and now it’s important to me for George’s sake to help eliminate this disease so that no one else has to suffer.”

— Ronya Kozmetsky —
Andy Nodaros & Family
Andy Nodaros originally found ALS TDF through his friend Dave Etschlager. Both patients, they came together to ALS 101 in 2002 and met the ALS TDF team. From then on Andy was determined to help support our research. A charismatic and charming personality, full of wit, Andy convinced reporters, local businesses, and friends and family to support ALS research in a variety of ways. Sadly, Andy passed away in 2003 but continues to be a catalyst for change, even now.

Today, Dave continues this work in his memory, along with a group of people in the Pittsburgh area now connected through ALS. The families and friends of Walt Knopp, Jon Russell, Scott Furner, Andy, and Dave continue their work toward a cure. Collectively, this group of ALS TDF supporters raised nearly $60,000 toward the research in our laboratory in 2003.

Paul Gerbick & Family

The Nodaros Family
He later decided to auction off his beloved classic red Thunderbird to benefit ALS TDF’s research as well.

The Gerbick Family
After his death, the Gerbick family raised more than $60,000 in support of ALS TDF’s research program.

The Douglass Family
Tony Tucker was diagnosed with ALS nine years ago, and continues to live at home, cared for by his loving wife, friends and family. Though he encounters the daily struggles with ALS, he manages to find beauty and enjoys being surrounded by a large community of family and friends.

The Tucker & Haley Families
When Tony was diagnosed, his wife’s family and his own gathered around him in support. Today, Tony’s brother, Walt Tucker, heads up Golf 4 ALS, an annual golf tournament to support the laboratory at ALS TDF. The Haley family, Tony’s in-laws, also started an annual event nine years ago. Together, the two events raised more than $40,000 in 2003—honoring the life of Tony and funding critical research at ALS TDF.

>> Driving 4 Life

On June 16th, 2003, professional golfer Tom Watson and his caddy Bruce Edwards along with his wife Marsha announced the Driving 4 Life campaign to support ALS TDF’s research on the Today show. Bruce was diagnosed with ALS in 2001, and had been Tom’s caddy for 27 years. Just days before, Tom Watson had stunned the golf community with a 5-under par 65 in the opening round of the U.S. Open.

Another ALS patient and former PGA professional, Jeff Julian, along with his wife Kim, also led the way for Driving 4 Life, and held an event to support ALS TDF. By the end of the year, the campaign had raised nearly $1 Million to fund ALS TDF’s laboratory, and fans around the country banded together in support of Bruce Edwards and Jeff Julian through individual donations, sponsorships, golf tournaments, and other creative ways of raising money for ALS TDF. Bill Denney, Darren McBratney, and Mark Burrow formed Driving 4 Bruce—a team that drove a golf cart from their hometown in Kansas City to Pebble Beach, California in honor of Bruce, recruiting sponsors and donations in their honor. ALS TDF and Driving 4 Life thank all those who have invested in our research and in today’s ALS patients.

Driving 4 Life

“Doctors tell us (a cure) may be out there, but money is needed for critical research. That’s where Driving 4 Life will help.”

Tom Watson

“Over the past two years we have searched for and gone after every possible lead. That search led us to ALS TDF. We have not met a more aggressive, determined organization than the team at ALS TDF.”

Jeff Julian
### Financial Report 2003

#### Assets
- Cash: $375,845.00
- Accounts Receivable: $1,100,000.00
- Investments: $754,260.00
- Fixed Assets, Net: $130,209.00
- Other Assets, Net: $63,802.00

#### Total Assets: $1,569,856.00

#### Liabilities & Net Assets
- Net Assets: $1,569,856.00
- Liabilities: $2,031,199.00

#### Total Liabilities & Net Assets: $2,031,199.00

#### Contributions & Revenue
- Support & Services: $2,524,562.00
- Fundraising Events: $1,669,856.00
- In-kind Donations: $251,287.00

#### Total Income: $4,390,771.00

#### Expenses
- Research & Development: $3,649,068.00
- Patient Services: $1,041,873.00
- Fundraising: $2,031,199.00
- Other: $1,669,856.00

#### Total Expenses: $2,982,895.00

#### Net Assets: $408,066.00

#### Financial Statement for the Year Ended December 31, 2003

#### Donors

**Leadership Circles**

As a member of one of the Leadership Circles, you demonstrate your leadership through annual gifts of $1,000 or greater. You provide support for ALS TDF’s laboratory operations, patient services, and clinical research into potential treatments for ALS. Thank you to all members of our Leadership Circles who continue to enable ALS TDF to make significant strides toward slowing, arresting, and curing ALS.

### Family & Friends Community Partners

The Family and Friends Community is a key part of ALS TDF’s fight to develop treatments and a cure for ALS.

This fast-growing group of family and friends has increased our motivation and strength to find a cure for ALS and together raised approximately $2 million in 2003. Thank you to each and every person that made the following efforts possible, and exhibited ALS TDF’s values of Courage and Love.

#### Founders’ Circle

$100,000 plus annually or $31 million or more cumulative giving

- ALS Hope--The Chris Holaber/James Maritz Foundation
- Anonymous
- Alex & Brit d’Arbeloff
- Dee Dee Forengo Fund
- Driving 4 Life Effort
- Fran Delaney Fund
- William Eckhardt
- George & Daphne Hatiosoulos
- Ronya Kazemetsky
- Mary Lou Krauseneck Fund
- Stephen Milne Society

### Financial Highlights

- **Total Assets:** $1,569,856.00
- **Liabilities:** $2,031,199.00
- **Net Assets:** $408,066.00

### Additional Information

**Governors’ Circle**

- ALS Family Charitable Foundation, Inc.
- Anthony Tucker Effort
- Steven Brozann
- The Carter Family ALS Research Fund
- Connecticut Friends for ALS Research
- David Reinstein Fund
- Elliott Martin Effort
- HHS Host
- The Jack & Carol Oratz ALS Foundation
- Jean Angell Fund
- Matt Doff Fund
- McDonald’s Corp
- Paul Geltich Fund
- PGA TOUR, Inc.
- Pittsburgh Pirates Efforts
- Ride For Life
- Chetny Sloan/ The ALS Race for Research

**Directors’ Circle**

- $10,000 plus annually
- 94-Lumber Classic of Pennsylvania
- Adcock & Walla Ray Wilson Foundation
- David Gross Effort
- The Noel David Carpenter Foundation
- DeeDee Fornengo Fund
- Driving 4 Life Effort
- The Dunphy/Sister’s Fund
- Peter Hecht
- Hoss Foundation, Inc.

**Associates’ Circle**

- $5,000 plus annually
- Allen Lebovitz Co.
- Apex Foundation
- Bruce & Linda Nelson
- Ryan & Mark Allan Fund
- Harrell & Phillips Charitable Foundation
- Caddie Master Enterprise, Inc.
- Elizabeth Courtenay
- Gary & Donna Dake
- DGA Builders, Inc.
- Richard & Vivian Douglas
- Bruno & Victoria Fazio
- Foxwoods Resort Casino
- Knights of Columbus
- Joseph & Suzanne Anton
- John & Diane Appleton

**Benefactors’ Circle**

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- American Insurance Outlet, Inc.
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Memorials
ALS TDF acknowledges the following individuals whose gifts were received in memory during 2003. Our staff extends our deepest memory during 2003. Our...