Morton Ruderman
An airline oxygen victory, and what is needed next

page 5
We are pleased to report that the Alpha-1 Foundation has reached a significant milestone in 2007-2008 in our quest to cure Alpha-1. Our investment in basic research to better understand the causes of Alpha-1 related lung and liver disease; our commitment to building the infrastructure to support the international research community; and our proven track record of engaging the community to participate in clinical research, have resulted in attracting several biotechnology companies to focus on therapeutic targets for Alpha-1.

Our research investment of more than $35 million to researchers in nearly 70 institutions in North America and Europe has been uniquely successful in delineating the pathways that lead from the abnormal gene to the clinical manifestations of Alpha-1, and has identified specific drug targets that can now be used to develop new drugs. Without your support, the Foundation would not have been able to reach this critical juncture.

2007-2008 was a banner year for our advocacy program. The President signed the Genetic Information Nondiscrimination Act (GINA) on May 21, 2008. The same day, the Foundation issued a press release celebrating the new law and announcing our commitment to evaluate the implications of the new law and explore how we can accelerate the detection of individuals with Alpha-1. We’re highlighting Mort Ruderman in this Annual Report and the impact that an individual donor can make on issues of importance to our community. The Department of Transportation’s airline oxygen ruling expands access to airline travel to millions on supplemental oxygen, including so many in our Alpha-1 community.

Our advocacy initiative for increased funding from Congress for the National Institutes of Health (NIH) has resulted in increased support for Alpha-1 research. The Foundation strongly endorsed the funding of the COPDGene study that will identify more genes that predispose individuals to develop lung disease, validating the importance of genetic risk factors such as Alpha-1.

The Alpha-1 Foundation ended its 2008 fiscal year in a strong and highly liquid financial position with over $1,267,000 in cash and equivalents and $4,118,000 in marketable securities. Total unrestricted and restricted net assets were $4,742,273 at fiscal year end. During the 12 months ended June 30, 2008, the Foundation realized $5,645,288 in restricted and unrestricted revenues and invested $4,617,695 in Alpha-1 research and related programs, including $2,778,615 in awards and peer-reviewed research grants.

Our ability to expand our research is no longer limited by basic knowledge of Alpha-1 or lack of interest in therapeutic development; it is now limited only by our ability to raise additional funds to finish the job. Our Celebration of Life raised more than $240,000 this year and more and more Alphas are participating in our Building Friends for a Cure Program, reaching over $500,000 in donations to date. Among them were Bettina and Ken Irvine and Joe Reidy, who organized a cocktail reception and a walk over the George Washington Bridge, respectively, which brought in close to $80,000 combined. Our Team Alpha-1 Escape to the Cape bike trek along Cape Cod raised over $20,000 and broke the record for the most participants from one team.

The Foundation’s continued strategic collaborations with the National Health Council, the NIH, professional societies and research coalitions have advanced the awareness of Alpha-1. The generous contribution from the John E. Kushner Family Fund that supported the production and dissemination of thousands of resource kits for the NIH Public Awareness Program Learn More Breathe Better has imbedded the Alpha-1 message in COPD forever.

Following the success of this campaign, the Kushner family spearheaded a tribute dinner to the late John Kushner sponsored by his good friend, Chef Emeril Lagasse, which again garnered awareness for Alpha-1.

Alphas from around the country, like Frank DeFord interviewing Len Geiger on HBO, John Kelley as an Ironman competitor, and so many others, have expanded awareness exponentially this year.

Thank you for your support in reaching this milestone and accelerating our ability to finish what we’ve started. On behalf of the Board of Directors of the Foundation and Alphas everywhere, we express our heartfelt appreciation for your continued support to cure Alpha-1 once and for all.

Wayne E. (Chip) Withers, Jr.
Chair

John W. Walsh
President & CEO
The Alpha-1 Foundation’s Grants and Awards Program continues to expand the scope, number and types of awards provided to qualified researchers.

Peer-Reviewed Research Portfolio for 2008

Our 2008 Peer Reviewed Principal Investigators are:

Jeffrey Brodsky, Ph.D.
- The Role of Add66/Clara5 in Alpha-1 Antitrypsin Biogenesis

Michael Campos, M.D.
- Alpha-1 Clinical Research Program

Tomás Carroll, Ph.D.
- Elucidation of a Role for Fasn2 in Alpha-1 Antitrypsin Deficiency

Robert Chatburn, RRT-NPS, FAARC
- Alpha-1 Educator Program for Allied Health Professionals

Marilyn Coors, Ph.D.
- Alpha-1 Genetic Testing at Risk Children and Adolescents

Harvey Coxson Ph.D.
- Quantification of Post-Transplant Bronchiolitis Obliterans Syndrome Using CT

Dawn DeFeo, M.D.
- MHC Loci and Epigenetic Factors as Modifiers of COPD

Terence Flotte, M.D.
- Spliceosomal-mediated RNA Trans-splicing Gene Repair of Pi*Z Mutant Alpha-1 Antitrypsin

Thomas Gildea, M.D.
- Post Lung Transplant FEV1 Change in AAT Deficiency

Kevin Glenn, M.D.
- Novel Ubiquitin Legases in AAT Degradation

Greg Grabowski, M.D.
- Nucleic Acid Therapy for Alpha-1 Antitrypsin Disease: Reinventing the Ribozyme

Catherine Greene, Ph.D.
- Defining the Role of Selenoprotein S in AAT Deficiency

Craig Hersh, M.D.
- Fine Mapping of a COPD Locus on Chromosome 19q

Kristen Holm, Ph.D.
- Illness Perceptions, the Social Environment, and AATD-Related Emphysema

Emer Kelly, M.D.
- Defining the Role of Selenoprotein S in Z Variant Alpha-1 Antitrypsin Deficiency (ZAATD) presented at American Thoracic Society, 2008

Darrell Kotton, M.D.
- Stem Cell-Based Therapy for Alpha-1 Antitrypsin Deficiency

Beena Krishnan, Ph.D.
- Folding/Nodefolding Landscape of Alpha-1 Antitrypsin

Chengwen Li, M.D., Ph.D.
- Develop Liver Specific AAV Vectors for AAT Deficiency

Chen Liu, M.D.
- Therapeutic Small Molecules for Alpha-1 Antitrypsin Deficiency

Margaret Lougee, A.S.
- Discovering Alpha-1 in COPD Patients Via Outpatient PFT

Noel G. McElvany, MB, BCh, BAO
- Clarification of the Risk of COPD in Pi MZ Individuals

Gillian McNab, Ph.D.
- Modification of Gene Expression and Increase in Alpha-1 Antitrypsin (A1AT) Secretion After Homologous Recombination in A1AT Deficient Monocytes presented at European Respiratory Society, 2007

Amar Deep Sharma, Ph.D.
- Hepatocyte-targeted Cell Fusion for the Correction of Alpha-1 Antitrypsin Deficiency

Richard Silers, Ph.D.
- Pi Z Polymer-Induced Re-Assignment of Degradation Route

Ronald Sokol, M.D.
- Sponsorship of the Clinical Research and Methodology for Rare Diseases: Opportunities, Challenges and Solutions Workshop

Martin Steffen, M.D., Ph.D.
- An Exploration of Proteasome Structure and Function in COPD

Rubin Tuder, M.D.
- Pathogenic Role of the Stress Response Gene RTP-801 in Cigarette Smoke Induced Alveolar Inflammation and Emphysema

Shivraj Tyagi, Ph.D.
- The Role of Brachio-Alveolar Stem Cells in Cigarette Smoke-Related Emphysema

Daniel Weiss, M.D., Ph.D.
- Sponsorship of the Stem Cells and Cellular Therapies in Lung Biology and Lung Diseases Workshop

Alice Wood, MBChB, MRCP PGCME
- Variation in the Vitamin D binding protein (GC) gene is associated with the development of bronchiectasis in Alpha-1 Antitrypsin Deficiency; Phenotypic characteristics of Alpha-1 Antitrypsin Deficient sibling pairs show discordance; Development of emphysema in Alpha-1 Antitrypsin Deficiency is more likely in urban areas; The tumor necrosis factor alpha (TNFalpha) gene influences clinical phenotype in Alpha-1 Antitrypsin Deficiency; Variation in the transforming growth factor beta (TGFβ) gene is associated with small airways disease in Alpha-1 Antitrypsin Deficiency presented at American Thoracic Society, 2008

Eun Jun Yun, Ph.D.
- The Regulation of Myofibroblast Development and Apoptosis

Program Grants Portfolio for 2008

Our 2008 Program Grants Principal Investigators are:

Mark L. Brantly, M.D.
- Alpha-1 Coded Testing Study
- Alpha-1 DNA and Tissue Bank
- Alpha-1 State of Florida Detection Program

James Curly, M.D.
- Implementation of a Statewide Screening and Detection Program for Alpha-1 Antitrypsin Deficiency

N. Tony Eissa, M.D.
- The Alpha-1 Research Program

Dawn McGee, M.S., C.G.C.
- Family Health History Project

Robert Sandhaus, M.D., Ph.D.
- Director, Alpha-1 Program, National Jewish Medical and Research Center

Ronald Sokol, M.D.
- Promoting Research of A1AT Deficiency Liver Disease through the Cholestatic Liver Disease Consortium (CLiC)

Charlie Strange, M.D.
- Alpha-1 Coded Testing Study
- Alpha-1 Research Registry

Adam Wanner, M.D.
- Scientific Director for the Alpha-1 Foundation

Ronald J. Sokol, M.D.

Terence R. Flotte, M.D.

2008 GRANT WINNERS
from the Alpha-1 Foundation included Ronald J. Sokol, M.D., of Children’s Hospital and the University of Colorado, and Terence R. Flotte, M.D., Dean of the University of Massachusetts Medical School. Sokol is principal investigator of the Cholestatic Liver Disease Consortium, which is investigating rare liver diseases in children, including Alpha-1. Flotte is investigating gene therapy as a potential treatment for Alpha-1.
## Financial Statements

### Statement of Financial Position

<table>
<thead>
<tr>
<th>YEAR ENDED JUNE 30,</th>
<th>2008</th>
<th>2007</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Assets</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Current Assets</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Cash (includes $1,033,116 and $1,085,336, respectively, which has been restricted by the donor)</td>
<td>$1,267,161</td>
<td>$1,297,065</td>
</tr>
<tr>
<td>Marketable securities</td>
<td>2,166,956</td>
<td>1,567,477</td>
</tr>
<tr>
<td>Accounts receivable</td>
<td>212,240</td>
<td>301,270</td>
</tr>
<tr>
<td>Due from COPD Foundation</td>
<td>—</td>
<td>99,999</td>
</tr>
<tr>
<td>Other assets</td>
<td>9,631</td>
<td>50,245</td>
</tr>
<tr>
<td><strong>Total current assets</strong></td>
<td>3,655,988</td>
<td>3,316,056</td>
</tr>
<tr>
<td>Marketable securities – non-current</td>
<td>1,951,765</td>
<td>2,202,601</td>
</tr>
<tr>
<td>Property and equipment, net</td>
<td>24,751</td>
<td>41,636</td>
</tr>
<tr>
<td>Other assets</td>
<td>19,379</td>
<td>19,379</td>
</tr>
<tr>
<td><strong>Total assets</strong></td>
<td>$5,651,883</td>
<td>$5,579,672</td>
</tr>
</tbody>
</table>

| **Liabilities and Net Assets** |               |               |
| **Current Liabilities**        |               |               |
| Accounts payable and accrued expenses | $717,704 | $710,259 |
| Deferred revenue                | 141,906 | 118,509 |
| **Total current liabilities**   | 859,610 | 828,768 |

| **Net Assets**                  |               |               |
| Unrestricted                    | 2,235,716 | 1,673,496 |
| Temporarily restricted           | 2,556,557 | 3,077,408 |
| **Total net assets**             | 4,792,273 | 4,750,904 |

| **Total liabilities and net assets** |               |               |
|                                      | $5,651,883 | $5,579,672 |

### Gross Revenues by Classification

**YEAR ENDED JUNE 30, 2008**

- **AlphaNet** $2,960,000 (53%)
- **Other Programs** $641,038 (11%)
- **Restricted Contributions** $1,248,769 (22%)
- **Unrestricted Contributions** $554,854 (10%)
- **Publication Income** $112,841 (2%)
- **Special Events** $112,841 (2%)
- **Investment Income** $127,786 (2%)

**TOTAL**: $5,645,288

### Functional Expenses

**YEAR ENDED JUNE 30, 2008**

- **Research Programs** $2,853,339 (51%)
- **Advocacy** $317,876 (5%)
- **Detection** $833,856 (15%)
- **Other Programs** $612,624 (11%)
- **Administration** $483,419 (9%)
- **Fund Raising** $502,805 (9%)

**TOTAL**: $5,603,919
# Statement of Activities and Changes in Net Assets

**YEAR ENDED JUNE 30,**

### Changes in Unrestricted Net Assets

#### Unrestricted Support and Revenue

<table>
<thead>
<tr>
<th>Description</th>
<th>2008</th>
<th>2007</th>
</tr>
</thead>
<tbody>
<tr>
<td>Contributions, bequests and grant revenues</td>
<td>$3,601,038</td>
<td>$3,468,818</td>
</tr>
<tr>
<td>Fund raising revenues</td>
<td>554,854</td>
<td>542,790</td>
</tr>
<tr>
<td>Investment income — net</td>
<td>127,786</td>
<td>156,761</td>
</tr>
<tr>
<td>Other income</td>
<td>112,841</td>
<td>48,000</td>
</tr>
<tr>
<td>Net assets released from restriction — satisfaction of program restrictions</td>
<td>1,769,620</td>
<td>1,052,139</td>
</tr>
<tr>
<td><strong>Total unrestricted support and revenue</strong></td>
<td>6,166,139</td>
<td>5,268,508</td>
</tr>
</tbody>
</table>

#### Unrestricted Expenses

<table>
<thead>
<tr>
<th>Description</th>
<th>2008</th>
<th>2007</th>
</tr>
</thead>
<tbody>
<tr>
<td>Program services</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Alpha-1 Research Network</td>
<td>2,519,282</td>
<td>1,802,799</td>
</tr>
<tr>
<td>Florida Detection Program</td>
<td>357,032</td>
<td>432,173</td>
</tr>
<tr>
<td>Alpha-1 Registry and ACT Trial</td>
<td>284,443</td>
<td>316,516</td>
</tr>
<tr>
<td>Alpha-1 Advocacy</td>
<td>317,876</td>
<td>272,403</td>
</tr>
<tr>
<td>National Targeted Detection Program</td>
<td>—</td>
<td>138,084</td>
</tr>
<tr>
<td>PFT Study</td>
<td>334,602</td>
<td>—</td>
</tr>
<tr>
<td>Alpha-1 Research Center</td>
<td>191,836</td>
<td>175,483</td>
</tr>
<tr>
<td>Other Alpha-1 Services</td>
<td>612,624</td>
<td>568,881</td>
</tr>
<tr>
<td><strong>Total program services</strong></td>
<td>4,617,695</td>
<td>3,706,339</td>
</tr>
<tr>
<td>Supporting services</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Administration and general</td>
<td>483,419</td>
<td>395,832</td>
</tr>
<tr>
<td>Fund raising</td>
<td>502,805</td>
<td>478,300</td>
</tr>
<tr>
<td><strong>Total supporting services</strong></td>
<td>986,224</td>
<td>874,132</td>
</tr>
<tr>
<td><strong>Total unrestricted expenses</strong></td>
<td>5,603,919</td>
<td>4,580,471</td>
</tr>
<tr>
<td><strong>Increase in unrestricted net assets</strong></td>
<td>562,220</td>
<td>688,037</td>
</tr>
</tbody>
</table>

### Changes in Temporarily Restricted Net Assets

<table>
<thead>
<tr>
<th>Description</th>
<th>2008</th>
<th>2007</th>
</tr>
</thead>
<tbody>
<tr>
<td>Contributions, bequest and grant revenues</td>
<td>1,248,769</td>
<td>2,805,513</td>
</tr>
<tr>
<td>Net assets released from restriction (Decrease) increase in temporarily restricted net assets</td>
<td>(1,769,620)</td>
<td>(1,052,139)</td>
</tr>
<tr>
<td><strong>Increase in net assets</strong></td>
<td>41,369</td>
<td>2,441,411</td>
</tr>
<tr>
<td><strong>Net assets, beginning of year</strong></td>
<td>4,750,904</td>
<td>2,309,493</td>
</tr>
<tr>
<td><strong>Net assets, end of year</strong></td>
<td>$4,792,273</td>
<td>$4,750,904</td>
</tr>
</tbody>
</table>

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"I am an Alpha and a proud supporter of the Alpha-1 Foundation. I heartily endorse the Foundation’s efforts to find better treatments and a cure for those of us with Alpha-1."

Lloyd Herlocker
Ruderman donation helps win victory on airline oxygen

In May, 2008, the US Department of Transportation issued a long-awaited ruling that airlines must accept portable oxygen concentrators carried aboard by passengers, as long as those concentrators are approved by the DOT.

The ruling, effective May 13, 2009, was long sought by the Alpha-1 Foundation and other patient advocacy groups. Many partnered with the Foundation as members of AOCA, the Airline Oxygen Council of America. AOCA spearheaded the effort to ease access to medical oxygen for airline passengers. The Alpha-1 Foundation coordinated the coalition’s advocacy and sponsored the AOCA website, www.airlineoxygen.org/ through a grant from the Ruderman Family Foundation.

“This will make air travel more accessible and convenient for those of us who need supplemental oxygen,” said Morton Ruderman of the Ruderman Family Foundation. “We have much more to do — patients using less expensive alternatives to the portable concentrators, such as liquid oxygen, deserve easier access to air travel as well — but we can all be pleased with this ruling.”

Ruderman said he is concerned about fairness and equal access for everyone who needs supplemental oxygen when they travel.

“Some people can afford to buy a five-thousand-dollar portable oxygen concentrator to bring aboard the plane. But most people cannot, so this ruling needs to be expanded. Many airlines still provide no oxygen at all on any flight. The DOT should require all airlines to provide medical oxygen for those who need it, and don’t have the wonderful luxury of a portable concentrator.”

Even so, Ruderman added, “This ruling is a great step forward.”

Honor Gallery

The Alpha-1 Foundation’s Honor Gallery salutes the individuals, corporations and foundations that share our commitment to advancing research, improving health, worldwide detection, and finding a cure for Alpha-1. The Foundation gratefully welcomes a number of new members who have supported our mission for the first time through their generous contributions.

The Foundation acknowledges the continued support of the many long-time donors who have invested in our mission. It is with sincere gratitude and appreciation that we recognize our Honor Gallery. The following is a list of donors who contributed $500 or more between July 1, 2007 and June 30, 2008.

$1 Million or More
AlphaNet, Inc.

$100,000-$999,999
Centric Health Resources, Inc.
CSSL Behring, L.L.C.
State of Florida, Department of Health
telers Biotherapeutics, Inc.
Tampa Orlando Pinellas Jewish Foundation, Inc.

$20,000-$99,999
Accredo Therapeutics, Inc.
Baxter Healthcare Corporation
Becker Family Foundation
Ruth & Gordon E. Codwgan, Jr., Ph.D.

$5,000-$19,999
Alpha-Okies
American Lung Association of California
AmerisourceBergen Specialty Group
Amicus Therapeutics, Inc.
Arriva Pharmaceuticals, Inc.

Baltimore Community Foundation (Eric Becker)
BankUnited
Baptist Health South Florida
BP America, Inc. (Mike Szymanski)
Calder Race Course
Centurion
The Cobb Family Foundation, Inc.
Amelia & Ignacio De La Rocha
Lourdes & Alberto Dosal
Firstgiving, Inc., USA
Gus Machado Ford
Elizabeth K. Lutz
Cynthia A. & Michael R. McConnell, R.Ph.
Jill & Jeffrey Meltzer
Carole & Michael O’Mara

Ana & Jose A. Ortega
Cindy R. & Robert S. Paillet
The Paul J. DiMare Foundation
Pfizer, Inc.
Roche BioScience
Ruth & John Sawyer
Judy & Edward A. Schuck
Sharon King & Jerome Schwartz
Scotiabank
UBS Financial Services, Inc.
Diane & John W. Walsh, Co-Founder
Cindy & Robert N. “Rob” Williams, Esq.
Cindy & Wayne E. “Chip” Withers, Jr.
Dolores M. Worsham
Sharon S. & Bill E. Young

Continued
$1,000-$4,999
Alpha-1 Association
American Association for Respiratory Care
American Express Foundation, Matching Gifts Program
Amgen Foundation
The Anthony R. Abraham Foundation
Maria E. "Tete" & Agustin R. Arellano
Toni & Richard Bachmann
Maurine K. Bagwell
A. Edward Bain, Jr.
Dianne A. Baker
Katherine & Robert Barnard
Charlotte S. & Robert C. Barrett
Brian Becker
Vivian & Wayne Beckner
Eduardo Blanco
BNY Mellon
Bonnie Brae Ice Cream, Inc. ( Judy & Ken Simon and Cindy R. and Robert S. Paule) JoAnne & Edward "Ed" Brailey
Judy Faye Lew, M.D. & Mark L. Brantly, M.D.
David A. Brenner, M.D.
Ellen L. & Karl J. Breyer
William R. Brown
Maria & Alejandro Centurion, M.D.
Clar's Outpost, Inc.
Colorado Society for Respiratory Care
Cynthia M. & Hugh D. Cooley
Jennifer & Stephen Czech
Rosemary & Russell K. Davidson
Rori K. & Robert S. Denholtz
Michelle Hefley
Cheryl L. & Lawrence P. Glascott, III
Friends of Jack Reid
Maurine K. Bagwell
Toni & Richard Bachmann
Maria E. "Tete" & Agustin R. Arellano
The Anthony R. Abraham Foundation
American Express Foundation, Matching Gifts Program
$500-$999
James D. Ackerman
Albert A. Adams
Adrian Empire, Inc.
Kim & Jeff Alexander
AT&T United Way Employee Giving Campaign
Paul Balser
John C. Barrett
Timothy Berry
Jeanne Magowitz & Janice A. Bish
Jane G. & Alvin M. Blake
Susan & John M. Bonich
Frank Bonilla
Judy Xiao & Kenneth A. Bonnet, Ph.D.
Rabbi & Mrs. Terry Bookman
William C. Broadhurst, Esq.
Lea & Ray Brown
Marlene W. & Roger E. Buchanan
Sharon Minnock
Laine Moody
Tom L. Moran
Neighbor to Nation
Miriam O'Day
Dr. Wayne O'Hern
OmniTRAX
Alicia & Ramiro Ortiz
Out of the Blue Advertising (Sonia & Renato Tiradito)
Sonia & Eduardo Perez, Esq.
Thomas L. Petty, M.D.
Pfizer Foundation, Matching Gifts Program
Plainfield Asset Management
Christy & Earl Powell
Ana Martín-Lavielle & Víctor J. Pujals
Tui & Joseph M. "Joe" Reidy
Respiratory Health Association of Metropolitan Chicago
Respiromics, Inc.
RT Spiece Sales Company, Inc.
Linda & Raul Rodriguez
Mr. & Mrs. Charles Royce
Rudd Equipment Company
Maria Marin & Jose "Pepe" Sanchez
Maria Elena & Roberto Sanchez
Sloan Schauffer
Frederic & Jerome Shaw
Audrey & L.G. Shonka
Judy & Ken Simon
Erin & Timothy Smith
Sarah E. "Sally" Everett, Esq. & Gordon L. Snider, M.D.
Leland Sparks, Jr.
Stephen McConahay Family Foundation
Marolyn & James M. Stocks, M.D.
Diego Suarez
Lori Lee & Mike A. Szymanski
Gerald "Gerry" Treichel
Joel Walcott
Doris & Adam Wanner, M.D.
Washtenaw United Way, Michigan
The William L. Richter Family Foundation
Maria Elena & Robert Wollberg
Zinni Family Insurance SVS, Inc.
Cynthia & Bruce B. Zirinsky
Cindy & Joseph "Joe" Zuraw
Sandra L. Tosi & R. Paul Maidment
Karen A. & Peter J. Manzoni
The Marketing Research Bureau, Inc.
Pam J. Moriarty
Shari & William Murphy
Nola A. Musser
William Nitzel
Mr. & Mrs. James A. Offutt
William T. Oke, Jr.
Beverly & Peter Orthwein
Osip-Harris Insurance, Inc.
Ana & Ramon F. Oyarzun
PD. Interiors, Inc.
Peachtree Construction, Inc.
Yleana & Ismael Perez-Siam
Julia & John Perkins
PG&E Corporation Campaign for the Community
Sharon & Barret Phillips
Patricia & Julio Pito, M.D.
Richard Potvin
Guy Paul Proctor
Lorena & Ramon Quesada, M.D.
Myrion & Julio Ramirez
Lee Sevison & Kevin Ray
Riedy Gift Express, Il.L.C.
Marcia F. Ritchie
Manon Grau Rodriguez
Gary D. Sanseverino
Anna & Eduardo Sardina
The Schlang Foundation
Elaine P. & Michael M. Schreiber
Lyle Schute
Michelle E. & Richard M. Sher
Richard Sherman
Aimee & Michael J. Siegel
Bonnie & Lawrence Solomon
Sara W. Spidel
Radune Abramson & William Spier
Mr. & Mrs. James Stauffer
Terry & James K. Stoller, M.D., M.S.O.D.A.
Pamela & Charlie Strange, M.D.
Leonor M. & Oscar Suarez
Eleanor & John Sullivan
Donna Syrop
Cathy & Richard Tallmadge
Tanya & Raymond Tepper
Gayle Allison Tippett & Larry Tipper
Robin M. Tudor, M.D.
UBS Foundation
United Ways of Massachusetts Bay & Merrimac Valley
Vanguard Charitable Endowment Program
David D. Vanness
Sheri & David Walling
Linda J. Walsh
Waterford Ambulance Service, Inc.
Andrea Lou & Jack Weiner
West Flagler Associates Ltd.
Michael L. Wheeler
Althea I. Wilkins
Elana Del Rosal & Frederick Wilson, III
Lane Witten
Allison & Steven Wollowitz
Honor Gallery
In Honor Of
The Alpha-1 Foundation received donations in honor of the following individuals between July 1, 2007 and June 30, 2008.

Abearn Family
Suzanne & Philip Aird
Alpha-1 Foundation Staff
The Alpha Betties
AlphaNet Coordinators
Evert Alsbrook, M.D.
Charles Axness
Lauren Bacon
Barden/Roundtree Family
Vanessa Bettez
Janice A. Bishop
Edward "Ed" Brailey
David A. Brenner, M.D.
Victoria J. Joseph, Esq. & David Brown
Marlene W. Buchanan
Stacy Castillo
Chase Bank Employees (Bethel, OH)
Joshua Clouette
Sarah Colby
Dorothy B. Condon
Fred G. Crabb
Mark Croghan
Maurice Curry
Tom Dailey
Adrienne Danzeisen-Clor
Brent Alan Davis
Donald E. Davis
William J. Dawkins
Frederick J. de Serres, Ph.D.

Lisa Dilley
Parker Douglas
Julian Marc Du Bois
Harold Duckworth
W. David Engel
Marilina V. Leopoldo
Fernández and family
Nancy Feuerbach
Jessica Rouan & Michael Foley, Jr.
Mary Alice Glascott
Kathy Haduck
Sonja M. Hahn
James W. Hailey, Jr.
Jackson David Harader
Jenn & Matt Harader
Jonathan Hart
Jim Hawkins
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Greg J. Hules
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Michael Paul Williams, Jr.
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IRON MAN – John Kelley of Marshfield, MA, was a “Common Man” entry in the Ironman World Championship in Hawaii in October, 2007. He competed while wearing the logo of Team Alpha-1, a Foundation program, in honor of his son, who’s healthy now but was diagnosed with Alpha-1 at birth. Kelley raised both Alpha-1 awareness and $5,000 on his web page for Foundation research and programs. How common a man is he? Well, he ran a 26.2-mile marathon, swam 2.4 miles and then pedaled 112 miles on a bike — with no breaks in between. He finished the event in just under 14 hours, hand in hand with his wife, Krisann.
The Alpha-1 Foundation received donations in memory of the following individuals between July 1, 2007 and June 30, 2008.

Ten Thousand Miles on a bicycle built for two: Hap and Diane Eaton paused with their tandem bicycle amid the palm trees of Miami as they paid a visit to the Alpha-1 Foundation during their year-long trip pedaling around the United States. They created awareness of Alpha-1 in city after city, often getting stories in local newspapers and television, during their trip in honor of Diane’s brother-in-law, Dugan Reed, who has Alpha-1. The Eatons got back home to Circleville, Ohio, in May, 2008.
Research for a cure leads list of Alpha-1 Foundation’s

Research is at the heart of the Alpha-1 Foundation’s mission. From the Foundation’s beginning in 1995, the central goal has been research to improve the health of those with Alpha-1 Antitrypsin Deficiency and ultimately find a cure for Alpha-1.

The Foundation’s Grants and Awards Program provides grants for both basic science and clinical research.

A landmark clinical study that began in 2007 was the “PFT Study” which enrolls patients with chronic obstructive pulmonary disease (COPD) for testing to determine the prevalence of Alpha-1 in COPD patients referred for pulmonary function testing. This is a major cooperative effort between the Foundation and the American Association for Respiratory Care (AARC).

The study is also investigating what characteristics of lung function are likely to predict Alpha-1, and the age distribution of those tested for Alpha-1.

The principal investigator is Robert A. Sandhaus, M.D., Ph.D., the Foundation Clinical Director, who also directs the Alpha-1 program at National Jewish Health in Denver. “I believe the study will also demonstrate the effectiveness of using respiratory therapists to perform significant pulmonary clinical research,” Sandhaus said. The study is supported in part by an unrestricted charitable contribution from Talecris Biotherapeutics.

The Foundation also built and funds this extensive research infrastructure:

- The Alpha-1 Research Registry directed by Charlie Strange, MD, at the Medical University of South Carolina is a confidential database of Alphas and Alpha-1 carriers. It is a resource for investigators seeking individuals with Alpha-1 to participate in clinical trials, surveys, and other scientific and medical activities.

- The Alpha-1 Coded Testing (ACT) program provides free, confidential testing, administered through a research study which evaluates perceived risks and benefits of genetic testing.

- The Alpha-1 Foundation DNA & Tissue Bank at the University of Florida is the central storage site for DNA and tissue samples from Alphas and others who do not have the deficiency, but simply wish to donate their DNA or tissue. The Bank is an international resource for researchers.

- The Targeted Detection Program promotes worldwide awareness among medical professionals, the media and public and the identification of Alphas in population groups at risk. An important component of the Targeted Detection Program is the State of Florida Detection Program. This is a collaboration between the State of Florida Department of Health and Human Services, the Alpha-1 Foundation and the University of Florida College of Medicine.

- The Alpha-1 Clinical Resource Center Network has 57 centers throughout North America that specialize in patient care and education for those with Alpha-1. Alphas and their physicians are encouraged to contact their regional Clinical Resource Center for information and guidance.

- Scientific Meetings and Conferences – The Alpha-1 Foundation and the COPD Foundation sponsored a workshop of world experts on CT Scan technology for COPD research in April of 2008. The two-day workshop sought to make progress toward a set of recommendations for standardizing CT scanning in COPD research. This was the 10th in the Gordon L. Snider Critical Issues Workshop series, part of the Foundation’s ongoing sponsorship of scientific meetings and conferences.

- Publications – Alpha-1-to-

One magazine carried a cover story in the spring of 2008 on Frank Deford, sportswriter, author and correspondent for the popular HBO television show Real Sports with Bryant Gumbel. Deford, who has Alpha-1, did the emotional HBO interview of Len Geiger, whose life was saved when he received the transplant lungs of 14-year-old Korinne Shroyer in 2002. Korinne’s parents, Kevin and Kristie Shroyer were also interviewed on the show. Deford was the keynote speaker at the 2008 national conference of the Alpha-1 Association.

Besides the magazine, Foundation publications include the Research Registry Update newsletter, and educational materials for Alphas, their families and healthcare providers.

- Building Friends for a Cure is a volunteer fundraising program for Alpha-1 research.

One “Building Friends” event organized for the first time in 2008 was the George Washington Bridge Walk, a cooperative effort by Alphas from New York and New Jersey and the James P. Mara Center for Lung Disease at St. Luke’s Hospital. The event raised more than $8,000 for Alpha-1 research and programs. Among the organizing committee members was Foundation Board member Joe Reidy.

The Bridge Walk was one of many events across the country in the Foundation’s Building Friends for a Cure Program, designed to make new friends and
nurture ongoing friendships in the Alpha-1 community. The goal is to increase awareness and raise funds for research and programs, ultimately leading to a cure for Alpha-1. Volunteers help Foundation fundraising efforts by becoming involved in special event planning, letter writing campaigns, potential donor introductions, and assisting in local, national and international social and sporting events.

For a free subscription to Alpha-1-to-One magazine or information on any Foundation program, contact the Foundation toll-free at 1-888-825-7421 or visit www.alphaone.org.

**Alpha-1 research draws interest from biotech companies**

One of the most exciting recent developments in Alpha-1 research is the interest of biotechnology companies in developing commercial therapies, especially for genetic conditions involving misfolded proteins, such as Alpha-1.

“Commercial research on Alpha-1 therapies is possible largely because of research done over the years by academic scientists,” says Adam Wanner, M.D., Scientific Director of the Alpha-1 Foundation. “Many of those researchers were working with grants from the Foundation, or had strong relationships with the Foundation.”

For 20 years, Rick Sifers, Ph.D., has been studying the reasons for Alpha-1 liver disease. He believed even two decades ago that the liver disease is caused at least partly by a defect in the body’s ability to break down the Alpha-1 protein (AAT). In some Alphas, the protein builds up in the liver and seems to cause liver disease.

Sifers, of Baylor College of Medicine in Houston, TX, says the evidence is mounting that Alpha-1 liver disease is accelerated by a mutation called a single nucleotide polymorphism (SNP, pronounced “snip”) in the gene for an enzyme called ER Mannosidase 1 (or ERMan 1).

Shujuan Pan, Ph.D., of Sifers’ research team at Baylor, has a Foundation research fellowship to study how the SNP blocks the breakdown of AAT in Alphas. One of Sifers’ own current projects is a Foundation-funded study of different ways AAT is broken down in the body. Sifers hopes this research will eventually find a way to predict which Alphas will develop liver disease, then lead to development of a cure.

Meanwhile, David Lomas, M.D., Ph.D., who chairs the Alpha-1 Foundation Grants Advisory Committee, has been studying Alpha-1 from a different direction.

Lomas, of the Cambridge Institute for Medical Research in the United Kingdom, is working on small molecules that can block polymerization of AAT. Polymerization is the process by which misfolded AAT molecules get stuck together and cluster in long chains. The Cambridge research team is hopeful that a small molecule that prevents the polymerization might prevent both Alpha-1 lung and liver disease. After screening 1.2 million compounds, the researchers are now focusing on a single molecular analog which was the most effective at blocking polymerization.

“I believe these small molecules are the first step to a cure for this condition,” Lomas said at the 2007 European Respiratory Society annual Congress.

Many biotechnology companies are exploring genetic conditions and therapies with similarities to Alpha-1. Amicus Therapeutics of Cranbury, NJ, is developing pharmacological chaperones to treat genetic diseases in which crucial proteins are misfolded. The company is specifically working on oral therapies that bind to and stabilize the affected proteins, restoring their shape and function.

FoldRx Pharmaceuticals Inc. of Cambridge, MA, is using yeast-based technology to discover small molecule modulators for diseases that involve protein misfolding, such as lung and liver disease caused by Alpha-1.

Proteostasis Therapeutics, also of Cambridge, MA, is a biotech just organized in 2008 to develop small molecule therapeutics designed to control the body’s protein homeostasis, or “proteostasis network.” Novel therapies to regulate the proteostasis network are designed to treat many genetic and neurodegenerative disorders, including COPD, Type II diabetes and Alzheimer’s disease.

Vertex Pharmaceuticals, a global firm which also has a US headquarters in Cambridge, MA, is another company developing small molecule drugs for serious diseases including Cystic Fibrosis.

“We’re pleased to see such widespread interest among biotech companies,” says Dr. Wanner. “We’ve been approached by several of these companies on possible collaborations. I believe this is a tribute to the credibility of the work done by Alpha-1 researchers.”
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*Denotes diagnosed Alpha-1 Antitrypsin deficient
+Denotes diagnosed family member