The CFIDS Association of America 2012 Annual Report

Many nonprofits are created to achieve a mission that would make their continued existence unnecessary. The CFIDS Association of America is one such organization. Founded in 1987 to conquer ME/CFS by pooling funds for research and information for patients, the Association is now the world's leading organization dedicated to making ME/CFS widely understood, diagnosable and treatable. It has also become the nation's largest source of ME/CFS research funds outside the federal government.

The organization's role has evolved over the years, but it has stayed true to roots planted 25 years ago. A tighter focus on research that began in 2007 with the addition of a full-time scientific director was sharpened even more in 2011. Acting on the guidance of a multidisciplinary Scientific Advisory Board, the Association's Board of Directors made the identification of disease-modifying treatment the organization's top priority. No other ME/CFS-related organization on the planet has set this target.

In this year-end report to our Catalyst Fund donors, we share progress on three fronts that represent unprecedented opportunity to advance the mission: solid science, opportune innovation and expanded engagement.

SOLID SCIENCE

ME/CFS is a complex condition that affects the brain and multiple body systems. Of the nearly 5,000 published research studies about ME/CFS, only a small fraction concentrate on treatment. A relative few of the hundreds of therapies used to manage symptoms have been studied in ME/CFS-specific clinical trials. This makes attempts to find even modest relief a frustrating game of "hide and seek." We are determined to change that by making discovery of effective treatment a strategic search and rescue mission for the millions of lives affected worldwide.

Here is how we are leveraging an ever-stronger base of basic research and new scientific opportunity to accomplish that bold objective.

SolveCFS BioBank

In a gap analysis completed in 2008 by our full-time scientific director, Dr. Suzanne Vernon, access to well-characterized ME/CFS patients was identified as a key barrier to engaging researchers in the study of ME/CFS. In 2010 the Association established the SolveCFS BioBank as the first patient-centered registry and repository of clinical data and biological specimens for ME/CFS. To date, we have enrolled 525 participants and completed our first proof-of-concept study with a major pharmaceutical partner. Results of this study are being prepared for publication.

The SolveCFS BioBank is at the hub of our newest research initiative, the Research Institute Without Walls, described below. Samples collected for the proof-of-concept study met the initial needs of RIWW projects and now we're able to offer partners a customized sample collection to fit particular study criteria and sample processing and storage protocols.

The SolveCFS BioBank enables participants to take an active role in research and has demonstrated value as a cost-effective resource for researchers who do not have access to clinical populations. We envision expanding capability to collect natural history information at regular intervals and to handle collection of other types of tissues. The BioBank can also be used as a clinical trials registry to facilitate treatment studies.

RESEARCH INSTITUTE WITHOUT WALLS

Turning Science Into Treatment for CFS

Research Institute Without Walls

Building on investments in ME/CFS research made by our organization, the National Institutes of Health (NIH) and other government agencies around the world, on February 23, 2012 we broke new ground by announcing our latest research initiative: the Research Institute Without Walls (RIWW). With the RIWW, the Association breaks out of the conventional non-profit role of simply sponsoring medical research and becomes a full partner with leading research institutions, putting patients at the core and center of tightly integrated projects that will advance objective diagnosis and effective treatment.

Our launch earlier this year announced five sponsored studies supported by grants and three new collaborations. All are under way and are proceeding according to the projects plans and performance milestones. Here's a closer look at those projects:

Sponsored Research

- **Dane Cook, Ph.D., at University of Wisconsin-Madison,** is linking information gathered from exercise testing, brain imaging and gene expression markers in the blood to understand post-exertional relapse, a hallmark feature of ME/CFS. This project will attempt to validate blood and brain markers independently identified in earlier studies. Dr. Cook has teamed up with Alan Light, Ph.D. of the University of Utah and Gordon Broderick, Ph.D. of the University of Alberta, leveraging two past Association grantees' findings to validate and expand their work. The complexity of this study and multiple institutions involved required a longer internal approval process, but it's started now and Dr. Cook and his colleagues are ramping up recruitment.
- **Spyros Deftereos, MD, of Biovista in Charlottesville, Va.**, is using a proprietary, large-scale drug repurposing platform to analyze the biomedical literature, patents, adverse event databases and other information sources to systematically identify non-obvious FDA-approved drug candidates to treat ME/CFS. The Biovista team has compiled an impressive synthesis of the ME/CFS literature and is prioritizing a large list of potential candidates for deeper exploration. Biovista is also helping develop tools to capture the "clinical intuition" of expert ME/CFS physicians to refine their search.
- **Patrick McGowan, Ph.D., at University of Toronto,** is building on evidence of environmental influences that affect immune system function in ME/CFS. Using blood samples collected through the SolveCFS BioBank, McGowan is looking for changes in patterns of gene expression that might be triggered by influences like nutrition, infection

and trauma. He has piloted an initial group of samples to test methods and those experiments were successful. We're in the process of collecting fresh samples from a tightly defined group of BioBank participants and he'll put his team to work on those samples soon. This is the first study to apply the "hot" new field of epigenetics to ME/CFS.

- **Marvin S. Medow, Ph.D., of New York Medical College**, will test three interventions in hopes that one or more will improve the "brain fog" that many patients experience after prolonged upright posture. With an earlier grant from the Association, Dr. Medow's team showed that the greater the orthostatic stress and the harder the cognitive challenge, the poorer the patient's performance on neurocognitive tests. In this study he will attempt to "tweak" the physiologic response to an upright tilt using three different interventions to identify patient subsets, understand mechanisms and potentially improve symptoms. Enrollment is proceeding.

- **Peter Rowe, M.D., of Johns Hopkins Children's Center**, has connected decades-old research linking rapid heartbeat, low blood pressure and abnormally sensitive nerves to ME/CFS. He hypothesizes that the link between simple movements and fatigue in ME/CFS is similar to fibromyalgia pain, where nerves become extra sensitive to stimulation, a process known as central sensitization. He and his colleagues will use a straight leg raise to provoke ME/CFS symptoms, comparing it to a sham test that doesn't engage nerves in the same way. Based on a small pilot study, Dr. Rowe expects results will identify a subset of patients who might benefit from a specialized form of physical therapy. Study enrollment is following plans laid out in his application.

Collaborations

- **Eric Delwart, Ph.D., at University of California at San Francisco,** is utilizing SolveCFS BioBank samples to screen for known and novel infectious agents with powerful sequencing tools. Dr. Delwart has completed the initial screen and will take a deeper dive based on initial results. He is among the first to apply metagenomics to the study of ME/CFS.
- **Dr. Leonard Jason, Ph.D., of DePaul University**, is using clinical and symptom information reported by SolveCFS BioBank participants to assess various case definitions used to classify ME/CFS patients. He is comparing this information to similar information collected from subjects in other study cohorts. Dr. Jason, one of the most prolific authors in the ME/CFS field, has several manuscripts in preparation and is involved with a group exploring case definition issues formed after the October 2012 meeting of the federal CFS Advisory Committee.
- **LogosOmix,** a small start-up company, is partnering with the Association to expand a knowledgebase of ME/CFS publications and related literature and refine text-mining tools to develop a "biomarker hit list" for ME/CFS. This work is based on the product of an earlier Association grant to New York University. It's a "big data" approach to pool vast information sources – like 200 million article abstracts in the National Library of

Medicine's database and catalogs of 100 million cellular proteins – made possible by advanced computational power that simply didn't exist just a few years ago.

2012 Meetings

We have convened two meetings this year to synchronize and synergize our RIWW investigators and other top minds focused on better ways to diagnose and treat ME/CFS. In March our grantees met in Baltimore to review and refine one another's approaches and to receive important information about linking their studies through the SolveCFS BioBank and a secure data-sharing platform called REDCap managed for us by Vanderbilt University. This condition of funding ensures that there is standardized data collection across studies and preservation of experimental data that survives the study period, important infrastructure not previously made available to ME/CFS researchers.

In October the RIWW faculty met at the Banbury Center of Cold Spring Harbor Laboratory in New York to share updates and progress reports with our Scientific Advisory Board and a group of investigators from six countries representing academia, government and industry. The theme, "Decoding Clinical Trials for ME/CFS," shaped the agenda and presentations on an array of therapies that have been studied in ME/CFS. Three days and nights of intense interaction facilitated new insights and collaborations that will almost certainly shape the field moving forward. The dialogue helped focus the need for a defined regulatory strategy to shepherd drug development and approval for ME/CFS, a responsibility the Association is uniquely positioned to shoulder.

Enriched ROI

The most recent grants made prior to the launch of the RIWW were awarded to six teams of investigators in 2009-2010. We've reported the initial outcomes of those studies in our "SolveCFS" print publication and online at Research1st.org. Additional publications and partnerships are in the works to extend observations and validate findings and we'll keep you posted as those studies continue to advance our understanding of ME/CFS.

One important measure of impact is the amount of follow-on funding applied for and secured by those teams to foster larger studies. We're pleased to share an update that our 2009-2010 grantees have attracted a combined total of $7 million in awards from the NIH, Department of Defense, pharmaceutical companies and foundations. The most recent of these is still in the late stages of institutional sign-off but we look forward to announcing it through our on-line publications just as soon as possible!

OPPORTUNE INNOVATION

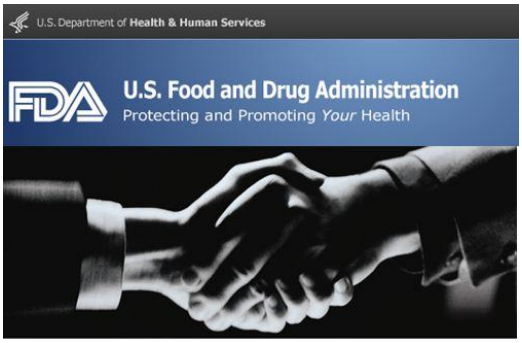
The search for effective treatment and cures is a key driver for many disease-specific organizations. With academic incentives focused on the "publish or perish" model of

advancement and pharmaceutical companies structured to satisfy investors, patients rely on nonprofits to inspire innovation and enforce accountability on their behalf. The patient-centered focus is important to organizations like *FasterCures* and Genetic Alliance that we work closely with. It's also taking hold with other institutions as well; the Institute of Medicine, Food and Drug Administration (FDA), Patient Centered Outcomes Research Institute, Society of Participatory Medicine and other organizations have helped give rise to a new patient-centric movement in health. We're riding that wave, learning from other organizations pioneering new models to short-cut the long, expensive pipeline for drug discovery.

Here are just a few of the initiatives that contribute to the convergence of opportunity for the Association to be a game-changer for people living with ME/CFS.

FDA Attention

In early 2011 the FDA announced that it would consolidate review of ME/CFS-related approvals in one division of its Center for Drug Evaluation and Research (CDER); prior to this change six different divisions had handled applications, depending on drug type. In February 2012 Dr. Suzanne Vernon was invited by FDA to give a grand rounds presentation to agency staff, helping deepen their understanding of ME/CFS and the impact it has on individuals, families and our nation. The Association used this and other opportunities to request that FDA convene another meeting to explore measures of treatment effectiveness or "endpoints" – the last one having been held in 1992. Our gap analysis had identified consistent endpoints and outcome measures as another barrier to progress.



Myalgic Encephalomyelitis and Chronic Fatigue Syndrome
Webinar: Working Together for Change

In response to a write-in campaign launched by a number of patient support organizations asking for a stakeholder meeting, CDER staff hosted a teleconference with 50 participants (including Dr. Vernon and CEO Kim McCleary) on September 13. During that call, FDA affirmed that the agency considers ME/CFS to be a "serious and life-threatening condition, a formal designation that speeds decision-making processes and provides other incentives to researchers and industry who want to test therapies. Sandra Kweder, MD, deputy director of FDA's Office of New Drugs, confirmed that the agency considers ME/CFS on a par with cancer, diabetes, epilepsy, heart failure and other serious diseases. FDA also announced plans to convene a scientific stakeholder meeting, open to the public, in spring 2013 to identify measurable outcomes that can be used in clinical trials to evaluate the effectiveness of therapies.

Under provisions of new legislation that guides FDA's drug approval process, the agency is planning a series of 20 disease-focused meetings to explore ways to systematically include patient input in the review of new products. ME/CFS was listed by FDA on its preliminary topic list and at a public meeting held October 25, Association Board chairman Amy Squires gave testimony urging that it be retained in the final selection. The Association submitted written testimony as well and encouraged advocates to make their voices heard.

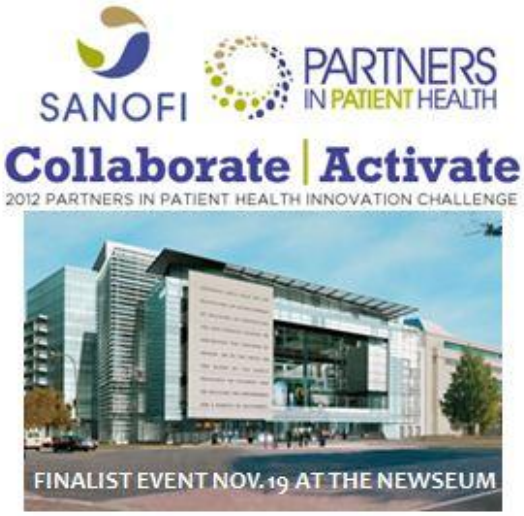
On November 15, FDA hosted a webinar titled, "Working Together for Change," showing how advocates can speed the development and approval of treatments through engagement of researchers and pharmaceutical companies. On December 20, the FDA's Arthritis Advisory

Committee will meet in open session to review the application from Hemispherx Biopharma to market its experimental immunomodulatory drug rintatolimod (Ampligen) as the first treatment for ME/CFS. The agency will render its decision by February 2, 2013.

After years of advocacy and effort to attract attention to the unmet need represented by ME/CFS, the recent actions by FDA are a powerful signal to biotechnology and pharmaceutical partners that investment is needed and welcomed by regulators. With our broad base of support, experienced leadership and solid scientific foundation, the Association is positioned to leverage the FDA's interest and industry investment to rapidly advance treatment-related research.

New Platforms for Partnership

Crowdsourcing solutions to complex problems has been popularized by groups like The X Prize Foundation that inspire "revolution through competition." In late August, the Association teamed with two sets of partners to submit ideas for engaging patients in their health to Sanofi's Collaborate | Activate Innovation Challenge. One hundred proposals submitted by 280 collaborating nonprofits were reviewed by a team of five high-profile healthcare judges. On September 17 we learned that both our submissions had made it into the final round of four. Finalist status came with $25,000 and help from two mentors appointed by Sanofi for each team – but just a few weeks to refine the proposals.



On October 29 we were scheduled to lead off the competition at the Newseum in Washington, D.C., with our "Partnering to End Pain" proposal and to close with our "Registries for All Diseases" pitch. Concerns about superstorm Sandy forced organizers to postpone the event and now we'll compete against "Flu Near You" and "21st Century Brain Trust" on November 19. Top prize is $300,000 and second prize is $100,000; both winning teams will meet with Sanofi executives in a roundtable session at the company's headquarters and they get access to non-financial resources to implement their solutions. We've developed two awesome pitches, but even if we're not among the final winners, exposure through the competition to staff at the world's fourth largest pharma company and other leaders in healthcare has been valuable. We look forward to sharing a positive outcome when the winners are announced in mid-December!

PARTNERING FORCURES
2012 Innovator Presentations

FasterCures is a nonprofit "action" tank that works across diseases to improve the effectiveness and efficiency of the medical research enterprise. Part of the Milken Institute, it has been highlighted by the *Wall Street Journal* and TIME magazine as an "impact amplifier." Association CEO Kim McCleary attended the Milken Institute's Global Conference in April and used the opportunity to meet with *FasterCures'* staff to share the Association's Research Institute Without Walls model. In July *FasterCures* featured the RIWW in its Innovator Spotlight. At *FasterCures'* invitation, Kim attended their September Celebration of Science in Washington, D.C., a three-day event attended by more than 1,000 health care

innovators, policymakers, advocates and academics. On November 29, Suzanne Vernon, PhD and Biovista president Aris Persidis, PhD, will be featured in one of the 30 "innovator" presentations at the Partnering for Cures conference in New York City, sponsored by *FasterCures*. The influential gathering gives us the opportunity to present to and meet personally with potential partners to advance promising drug repurposing candidates arising from our RIWW project with Biovista. We'll also learn from others presenting their innovative approaches to generating faster cures for people who need them.

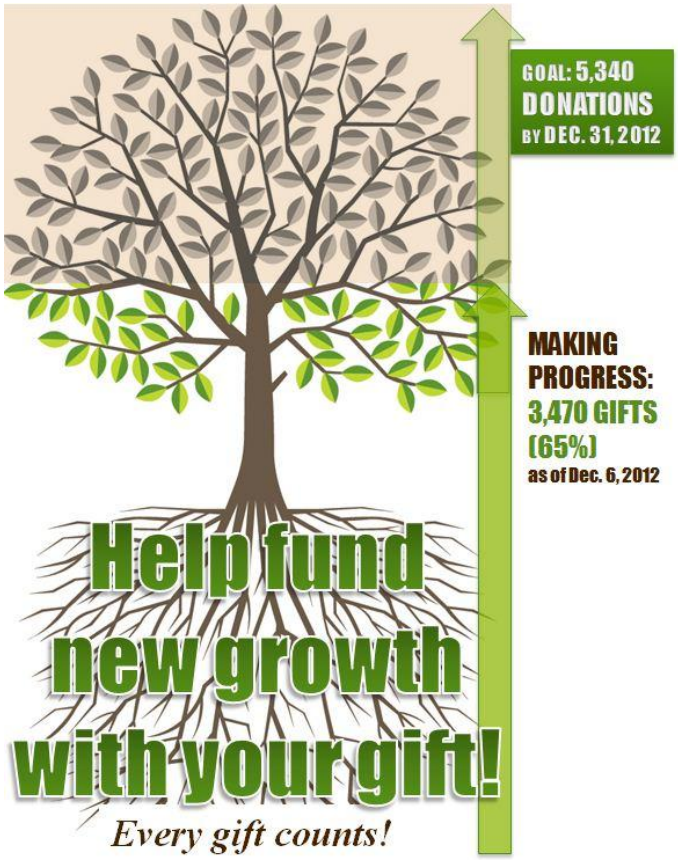
EXPANDED ENGAGEMENT

ME/CFS affects an estimated one million Americans and as many as 17 million people worldwide. Our patient-centered focus puts their needs at the forefront of our every action. ME/CFS also has a profound impact on family members and other loved ones around the individual. People living with ME/CFS and their families and friends form the core of our efforts and accelerating progress toward the mission depends on engaging them actively in the cause. We're taking important steps to expand our outreach within the community we serve and beyond it.

Repositioning

Over 25 years we have worked hard to make sure that our communications reflect our commitment to credibility, completeness and purpose. We recognize that they can sometimes be a bit "clinical" and "dispassionate" and that the array of different names and logos we use can be somewhat confusing, especially to people encountering us for the first time.

We aim to better convey the energizing and enterprising spirit with which we pursue the mission and to align our programs and information sources under a more cohesive "brand structure." To do that we have engaged expert communicators to help develop a new "look" for the Association, paired with fresh materials and clearer messages about the condition, the cause and the organization's work. You will be among the first to preview and give us feedback in the new year. Our intent is to invigorate the community of people we currently reach and to attract more people, resources and influence to the cause, translating to more treatment-focused research sooner.



Revenue Diversity

We are so grateful to individuals like you who provide the vast majority of our financial support each year. It's important to you – and to us – that we continue expanding our base of individual support with gifts small and large. This year we've launched an "Every Gift Counts" campaign with our annual fund appeal. We have asked people to help us achieve 25 percent growth in the

total number of gifts received, with a goal of 5,340 donations by December 31, 2012. As of November 11, we have reached 59% of that goal, or 3,134 gifts. Our revenue goal for 2012 is $1.65 million. Last year 56% of our total revenue was received in the last 26 days of the year, a statistic that underscores the importance of every year-end gift.

In addition to the generous support we receive from individuals, we recognize the need to tap into foundations, corporate philanthropy programs and other revenue sources that will fuel and sustain healthy growth. To that end we sought counsel from Patton McDowell & Associates, a regional development consulting firm based in Charlotte. They helped us develop a three-year plan to meet our funding needs for the transformative research program we intend to cultivate. They also led a national search for a highly qualified director of development to implement that plan. We are pleased that Mark A. Stone joined our staff in that role on October 15.
Read more about Mark and his record of achievement, described on the "Tradition + Transformation" enclosure. His experience as chief operating officer of the Polycystic Kidney Disease Foundation, where he led resource development to catalyze new therapeutic approaches for people with PKD, will be invaluable to us.

We've benefited this year from several independent fundraising events, including Jennifer Williams' first marathon run that generated nearly $5,000 through more than 100 donations from family members and friends in support of her husband, Mark's, 20-year battle with ME/CFS. Koerner Gray Buchta walked the width of the state of Michigan, covering a few miles at a time over a two-month period to raise awareness and $2,650 (so far) for research. The girls' softball team at Deerfield High School in Deerfield, Ill., raised $2,145 in support of the Association at a spring "Strike Out CFIDS" benefit game, held to honor the memory of one team member's aunt who lost her battle to ME/CFS earlier this year. Like the Michael J. Fox Foundation has done through its successful Team Fox program, we intend to foster more of these kinds of events that enable individuals to amplify their own impact, raise visibility and elevate the cause. We are in the process of recruiting an Engagement Manager to help us provide this support, as well as to enhance our other outreach efforts.

THE CFIDS ASSOCIATION OF AMERICA, INC.

STATEMENT OF FINANCIAL POSITION

DECEMBER 31, 2012

(WITH COMPARATIVE TOTALS FOR DECEMBER 31, 2011)

	December 31, 2012				
	Unrestricted	Temporarily Restricted	Permanently Restricted	Totals	December 31, 2011
ASSETS					
Current Assets:					
Cash and cash equivalents	$ 874,000	$ 98,541	$ -	$ 972,541	$ 1,025,010
Contributions receivable, net	6,473	5,000	-	11,473	45,930
Other receivables	937	-	-	937	1,893
Inventories	515	-	-	515	706
Prepaid expenses	8,012	-	-	8,012	8,096
Total Current Assets	889,937	103,541	-	993,478	1,081,635
Property and equipment					
Office furniture and fixtures	77,292	-	-	77,292	77,642
Computers and related equipment	69,806	-	-	69,806	100,393
Leasehold improvements	29,259	-	-	29,259	25,872
	176,357	-	-	176,357	203,907
Less accumulated depreciation	(159,390)		-	(159,390)	(177,134)
Net property and equipment	16,967	-	-	16,967	26,773
Other Assets:					
Beneficial interest in assets held by others	-	7,946	5,400	13,346	12,031
Accumulated policy value of life insurance	-	12,500	-	12,500	13,149
Total Other Assets	-	20,446	5,400	25,846	25,180
Total Assets	$ 906,904	$ 123,987	$ 5,400	$ 1,036,291	$ 1,133,588
LIABILITIES AND NET ASSETS					
Current Liabilities:					
Accounts payable	$ 52,412	$ -	$ -	$ 52,412	$ 7,260
Accrued payroll expenses	23,436	-	-	23,436	21,438
Deferred revenue	3,162	-	-	3,152	-
Capital lease obligation - current	4,102	-	-	4,102	3,642
Total Current Liabilities	83,112	-	-	83,112	32,340
Other Liabilities:					
Capital lease obligation - noncurrent	17,256	-	-	17,256	21,358
Total Liabilities	100,368	-	-	100,368	53,698
Net Assets:					
Unrestricted					
Undesignated	620,705	-	-	620,705	811,675
Board designated	185,831	-	-	185,831	185,518
Total unrestricted	806,536	-	-	806,536	997,193
Temporarily restricted	-	123,987	-	123,987	77,297
Permanently restricted	-	-	5,400	5,400	5,400
Total Net Assets	806,536	123,987	5,400	935,923	1,079,890
Total Liabilities and Net Assets	$ 906,904	$ 123,987	$ 5,400	$ 1,036,291	$ 1,133,588

THE CFIDS ASSOCIATION OF AMERICA, INC.

STATEMENT OF ACTIVITIES

YEAR ENDED DECEMBER 31, 2012

(WITH COMPARATIVE TOTALS FOR DECEMBER 31, 2011)

	Year Ended December 31, 2012				Year Ended December 31, 2011
	Unrestricted	Temporarily Restricted	Permanently Restricted	Totals	
Support and Revenues:					
Public support:					
Contributions and grants	$ 942,848	$ 145,498	$ -	$ 1,088,346	$ 1,196,911
Revenues:					
Research subcontracts	12,357	-	-	12,357	14,671
Pharmaceutical contracts	-	-	-	-	5,605
Educational material sales	851	-	-	851	1,449
Interest and other	2,615	-	-	2,615	1,275
	15,823	-	-	15,823	23,000
Net assets released from restrictions	98,808	(98,808)	-	-	-
Total Support and Revenues	1,057,479	46,690	-	1,104,169	1,219,911
Expenses:					
Program services:					
Research	868,414	-	-	868,414	759,958
Communications	189,854	-	-	189,854	169,791
Supporting services:					
Management and general	45,201	-	-	45,201	58,276
Fund raising and development	144,667	-	-	144,667	131,620
Total Expenses	1,248,136	-	-	1,248,136	1,119,645
Loss on disposal of assets	-	-	-	-	2,401
Change in Net Assets	(190,657)	46,690	-	(143,967)	97,865
Net assets, beginning of year	997,193	77,297	5,400	1,079,890	982,025
Net assets, end of year	$ 806,536	$ 123,987	$ 5,400	$ 935,923	$ 1,079,890

THE CFIDS ASSOCIATION OF AMERICA, INC.

STATEMENT OF FUNCTIONAL EXPENSES

YEAR ENDED DECEMBER 31, 2012

(WITH COMPARATIVE TOTALS FOR DECEMBER 31, 2011)

	Year Ended December 31, 2012					
	Program Services		Supporting Services			
	Research	Communications	Management and General	Fundraising and Development	Totals	Year Ended December 31, 2011
Contract services	$ 43,410	$ 76,428	$ 4,878	$ 50,907	$ 175,623	$ 217,823
Salaries and benefits	396,440	69,204	27,160	44,168	536,972	616,065
Payroll taxes	24,429	8,294	3,255	5,295	41,273	46,587
Direct grants	235,121	-	-	-	235,121	13,003
Printing and postage	1,424	6,677	120	13,426	21,647	23,209
Repairs and maintenance	1,224	416	163	265	2,068	3,014
Supplies	2,077	626	246	443	3,392	4,595
Educational materials/ cost of sales	-	279	-	-	279	546
Travel expenses	55,350	5,032	645	13,137	74,164	46,300
Event expenses	17,040	-	-	640	17,680	10,415
SolveCFS BioBank costs	24,481	-	-	-	24,481	28,874
Insurance	4,410	1,497	588	956	7,451	6,295
Telephone	4,023	1,177	292	730	6,222	6,066
Occupancy costs	38,681	13,134	5,155	8,382	65,352	61,871
Depreciation	7,808	2,651	1,041	1,692	13,192	11,731
Miscellaneous	12,496	4,439	1,658	4,626	23,219	23,251
Total program and supporting services expenses	$ 868,414	$ 189,854	$ 45,201	$ 144,667	$ 1,248,136	$ 1,119,645
Management and general expenses					$ 45,201	$ 58,276
Fundraising and development expenses					144,667	131,620
Total management and general, and fundraising and development expenses					$ 189,868	$ 189,896
Total support and revenue					$ 1,104,169	$ 1,219,911
Supporting services ratio					17.20%	15.57%