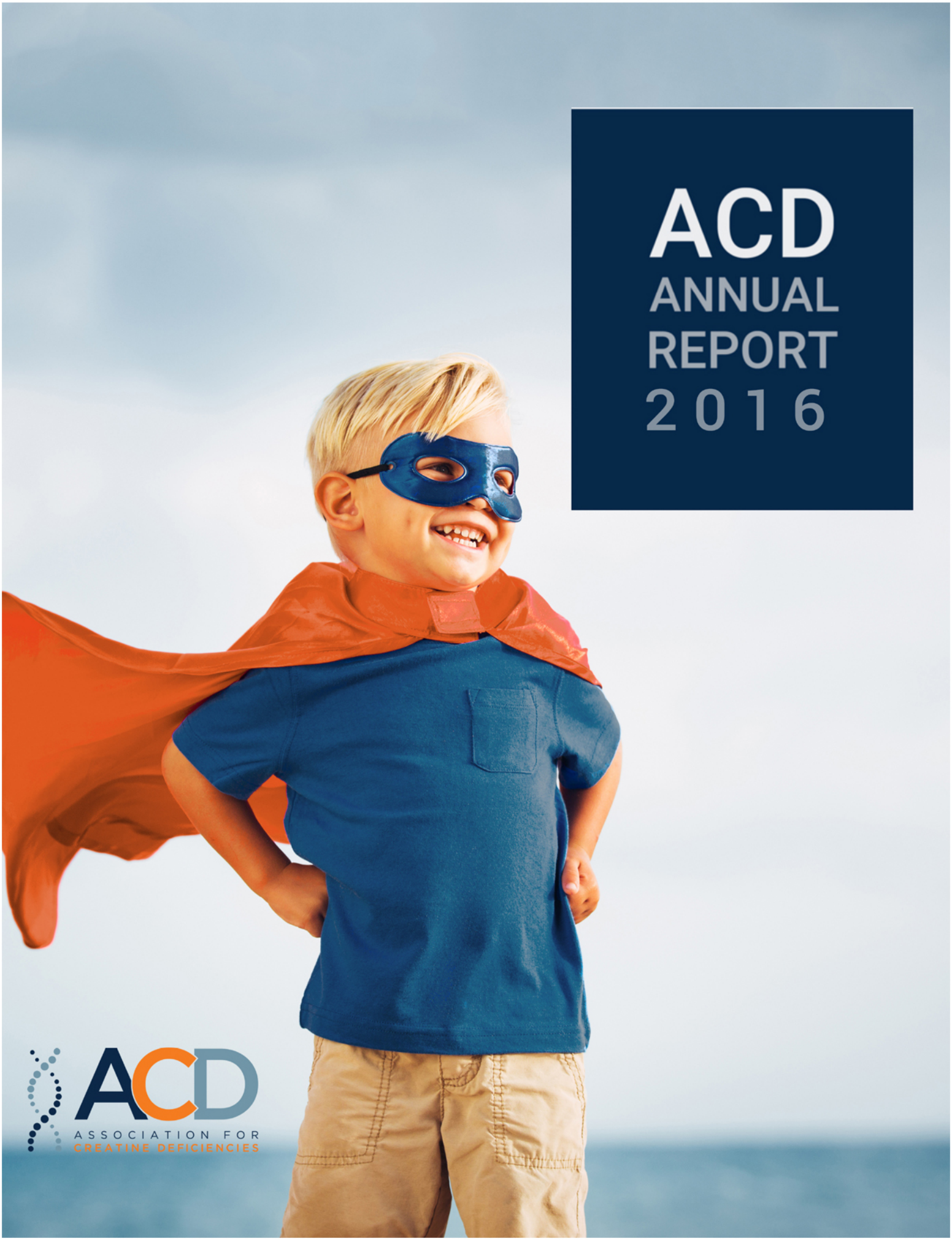


ACD ANNUAL REPORT 2016

ACD
ASSOCIATION FOR CREATINE DEFICIENCIES

Our Mission

The ACD is the only patient advocacy group that is devoted to Cerebral Creatine Deficiency Syndromes (CCDS).

We are committed to meet our goals, which are “to provide patient, family and public education, to advocate for early intervention through newborn screening, and to promote and fund medical research for treatments and cures for Cerebral Creatine Deficiency Syndromes.”

The ACD sets these goals to bring the CCDS community together as one strong voice, to be united in our efforts, to urge allied medical profession to test and to treat and promote scientific research.

It is through our mission that our vision for the future is clear: to eliminate the challenges of Cerebral Creatine Deficiency Syndromes.
It is with our integrity, community and hopes that we are “building strength and changing lives.”


IF YOU WANT
TO GO FAST,
GO ALONE.
IF YOU WANT
TO GO FAR,
GO TOGETHER.
UNKNOWN AUTHOR

President's Letter

Dear Friends,

In 2016, we continued our fundraising momentum, thanks to generous donors such as our community members, our families and friends, and corporations that all share our mission of one day having a cure for CCDS, and we are so thankful! With our fundraising events including our recent Holiday Heroes campaign, Shop for a Cause, and our first annual Walk for Strength, we raised over $57,000 this past fiscal year. These funds continue to allow us to operate and champion the cause of eliminating the challenges of CCDS through education, advocacy, and research. The ACD Board of Trustees meets weekly to review on-going strategic goals and their progress. Growth has always been a focus and will continue to be, and I know this past year will be one that we will always look back on with enormous pride.

My reflection this past year is centered around ACD's exponential growth. I think about the number of families that found us after their child (or children) were diagnosed with a CCDS, or the fundraising that allowed the ACD to attend more conferences, including international ones in Canada and Europe, and new programs that we have developed that will help highlight the need for more education and research on Creatine Deficiencies. Some of my personal favorite statistics from this past year would be that 65% of our website views are from new visitors and that the annual rate of growth is currently at an incredible 200%. Also, in just two years we have had an 87.5% growth rate in newsletter subscribers. So what does this mean for us as a community, our children, and their future? We believe that this continues to prove that we are making a huge difference in the medical community by helping raise awareness in getting a faster diagnosis, and that researchers will be more and more interested in CCDS. This growth will undoubtedly help improve the quality of life for those living with a Creatine Deficiency, and hopefully, one day find a cure.

Our international efforts this past year proved to be an outstanding opportunity for us to connect with other CCDS patients, physicians and researchers from around the world. Our Scientific Medical Advisory Board also continues to expand as we add those that are considered experts in the field of Creatine Deficiencies. With all of this growth, we plan to expand our Board of Trustees, and General Council, with an emphasis on bringing together respected leaders in the business and the medical communities.

On behalf of the ACD Board of Trustees, I am pleased to present the 2016 Annual Report. As the CCDS community comes together, we are committed to meeting their needs of bringing one voice together, because together we are building strength and changing lives.

Thank you for your continued support,

Kim Tuminello

President & Co-Founder

The Importance of Cerebral Creatine Deficiency Syndromes (CCDS)

There are three Cerebral Creatine Deficiency Syndromes with complicated medical names for some to remember. So, we refer to them by acronyms.

Arginine: Glycine Amidinotransferase Deficiency or AGAT

AGAT is involved in the first step of creatine formation. Mutations found in the AGAT gene impair the body's production of creatine. There are fewer than twenty AGAT cases reported, to date.

Guanidinoacetate Methyltransferase Deficiency or GAMT

GAMT is a mutation in the gene that makes the enzyme that creates creatine, resulting in a shortage of creatine. GAMT is the most severe of the three CCDS due to an elevation of guanidinoacetate (which is neurotoxic) in addition to creatine deficiency, and there are more than 100 cases reported in medical literature.

Creatine Transporter Defect or CTD

CTD is a mutation in the creatine transporter gene. This mutation results in a blockage in the transportation of creatine to the brain and muscles. CTD is the most common CCDS. It is estimated to account for about 2% of all unexplained X-linked intellectual disabilities.

Symptom(s)

The most common symptom of CCDS is a global delay. Speech delay may be particularly severe, and intellectual disability of variable severity is typically present in older children and adults. Other additional symptoms may include seizure disorders, muscle weakness, autistic-like behaviors, movement disorders, gastrointestinal problems and failure to thrive.

Treatment(s)

Treatment with oral supplementation is available and effective if initiated early for AGAT and GAMT patients. To date, this type of therapy has not shown to improve outcomes in individuals with CTD.

Education and Awareness

The ACD is the only non-profit patient advocacy group raising CCDS education and awareness.

Rare Disease Partners

As part of its commitment to patient and public education, the ACD has partnered with the National Organization for Rare Disease. This collaboration has helped to strengthen the ACD capacity to serve our patient population, to increase CCDS knowledge within the medical community and, in general, the public at large.

Medical and Scientific Conferences

With this as a major focus, the ACD has been active and instrumental in urging the medical professionals to seek out and test patients for CCDS. By attending and exhibiting at conferences, such as the Child Neurology Society and the American College of Medical Genetics and Genomics, we are helping to advance CCDS knowledge and training.

Patient and Family Symposiums

Along with attending and exhibiting at medical and scientific conferences, the ACD raises awareness within the public sector by exhibiting and participating in Autism Walk(s) and Talk About Autism (TACA) venues.

Website

Within this scope, the ACD is continually updated with current research summaries, patient resources, facts related to CCDS, our Insider eNewsletter, the CCDS Blog Post, and listed opportunities to participate in Natural History Studies, Observational Studies, and Clinical Trials, plus much more.

Social Media

We have increased our social media presence on Facebook, Twitter, and Instagram to keep our message and educational interest visible.

Online Video Publications

Our awareness video called “A Short Story About Creatine Deficiencies” has been distributed worldwide, through our Social Media output and YouTube, to raise disease awareness Nationally and Internationally. And at year-end, we added a whiteboard video called “The Power Of The CCDS Patient” to our library. This whiteboard presentation was introduced in Paris, France to CTD researchers, from all over the world, to help in the understanding of patient hopes and fears for CTD trials.

FDA Patient Network

The ACD is pleased to announce the board’s involvement with the FDA Patient Network (PN) for inborn errors of metabolism. The FDA Patient Network is a comprehensive program that works to sustain communications with patients and their community. The PN also helps educate patients, patient advocacy groups, and health care professionals about medical product regulations.

Other Opportunities

The ACD continues to review and publish detailed, clinical diagnostic booklets, binders, and brochures to aid in our educational efforts.

Advocacy

Along with education and awareness, the ACD is passionate about advocating the rights of individuals with CCDS.

Newborn Screening

The ACD aims for CCDS to be placed on newborn blood screens nationwide. Newborn screening identifies conditions that can affect a child's long-term health or survival. CCDS early detection, diagnosis, and disease-specific treatment can prevent disabilities and will enable a child to reach their fullest potential.

We are currently pursuing this initiative. We have been paving the way for a favorable future outcome for GAMT newborns. With a current pilot study in Utah, we are hopeful that catching a positive GAMT patient from newborn bloodspot will prove the effectiveness of GAMT newborn screening and will encourage nationwide following.

We believe our involvement within the CCDS medical community, our continued activity with the Advisory Committee on Heritable Disorders in Newborns and Children and our productive collaborative partners, we will see GAMT on the Recommended Uniform Screening Panel and have every baby tested for this disease.

Collaborative Partners

The ACD is partnered with NORD and Global Genes. This year we worked with and initiated a united effort with other non-profits, such as, Autism Speaks, Talk About Autism, Everylife Foundation, and Child Neurology Foundation.

Our partners have been effective in our training, communication, and growth for the future representation of CCDS patients.

> Approximately 50% of rare diseases do not have a disease specific foundation supporting or researching their rare disease.
>
> Global Genes

UNITING FOR A SINGLE CAUSE TO SHOW OUR SUPPORT AND STRENGTH AS WE BATTLE CCDS.

HEIDI WALLIS

Medical and Scientific Research

The ACD assists approved non-drug research studies for CCDS by promoting volunteer participation in Natural History Studies, Observational Studies, and Clinical Trials.

Patient Registry

This year the ACD is excited to announce our partnership with AltaVoice, formally Patient Crossroads, to house our Patient Registry database.

One of the most important aspects of research is a patient registry. The ACD's International Patient Registry collects critical information to understand the history and progression of CCDS, to make it easier for researchers to study, and for the patient and family to learn about treatments, and for the ACD to speak on behalf of the CCDS community.

Alta Voice collects and safeguards the ACD's data to protect participants' privacy.

With our patient registry data, the ACD has been actively reaching out to the National Institute of Health (NIH), the Center for Disease Control (CDC) and establishing relationships within the CCDS medical, scientific and pharmaceuticals to promote research.

We have been leading the way to secure more interest in CCDS studies, with establishing and promoting a CCDS patient registry.

Patient and Family Supportive Services

We listen to the needs of our community and take every means to produce helpful and beneficial information. The ACD's website is an essential tool for CCDS information.

Patient**Strong**™

The Patient Strong Scholarship was reconfigured to help assist families with a CCDS diagnosis, by providing travel funds to attending ACD's CCDS bi-annual educational conference. Applications are available on-line and up to three scholarship will be awarded the first quarter of conference year, ranging in $500-$1,000.

Ask The Expert

This year our Scientific Medical Advisory Committee is engaged in the patient participation of CCDS related questions. Our "Ask The Expert" series, featured in our quarterly eNewsletter, helps to answer questions our community members may have.

Patient Resource Guide

The ACD has developed a resource guide for CCDS patients to use as a tool, to aid in supportive services that may be reviewed, and to complement patient's knowledge and programs. We promote this tool on our website for easier accessibility.

Creatine Community Blog

The ACD has asked the CCDS community to submit their personal stories, to share with others that may be dealing with similar issues. We feature regular posts from contributors on our Creatine Community Blog. We are honored to be entrusted with such personal stories.

Fundraising

The ACD is a 501(c)(3) charitable non-profit organization which can provide valuable services to the CCDS community only because of the generosity of our contributors.

Special Events and Corporate giving are essential to raising CCDS awareness and funds needed to continue the ACD's mission.

The following is a list of areas of sources, which provided funding for 2016:

Contributors

Corporate:

United Way
Microsoft
Mode-ology
Smith-Cooper International

Matching Gift Funds:

Enterprise Rent A Car

Mode-ology

Annual Signature Events:

Walk for Strength

Holiday Heroes

Supportive Grant(s):

Lumos Pharma

NORD

DIA

Individual Donors(s):

150 + individual donors in 2016

Individual Events:

Shop for a Cause:

Armadillo Crafts
ATAP Trio
Essential Bodywear
Joyce Stephens
Rodan + Fields
Silpada
Younique

We gratefully acknowledge these generous donors, each playing a vital role, in carrying out our mission to serve CCDS patients and families. And to all our donors, sponsors and volunteers across the county who helped our various events, it is with sincere gratitude, we thank you. Thank you for your continued support and generosity

Our Board

The ACD was founded in 2012 by parents with children diagnosed with a CCDS. The ACD was established to raise awareness and education of CCDS among the medical community, and the general public, and to advocate on behalf of the families and patients living with CCDS.

2016 Executive Directors:

Kim Tuminello
President, Co-Founder

Whitnie Strauss
Vice President

Linda Cooper
Director of Operations, Co-Founder

Melissa Parker
Director of Finance

Heidi Wallis
Administrative Director

Board Trustees:

Missy Klor
Co-Founder

Laura Martin

2016 ACD Scientific Medical Advisory Board:

Dr. Nicola Longo, M.D., Ph.D.
Chief, Division of Medical Genetics
University of Utah Health Care
Pediatrics Medical Genetics

Dr. Bruce Barshop, M.D., Ph.D.
Metabolic and Mitochondrial Medicine
Pediatrics, Rady Children's Specialty
University of California San Diego

Dr. Sarah Young, Ph.D.
Assistant Professor
Department of Pediatrics - Medical Genetics
Duke University School of Medicine
North Carolina

Dr. Denise Morita, M.D.
Pediatric Neurologist
Granger Medical Clinic
Riverton, Utah

Volunteer Representatives:

Beth Robinson

Jerry Robinson

Tina Strauss

Nathan Vandenberg

Celine Wheaton

Blog Contributors:

Janet Grigoratos

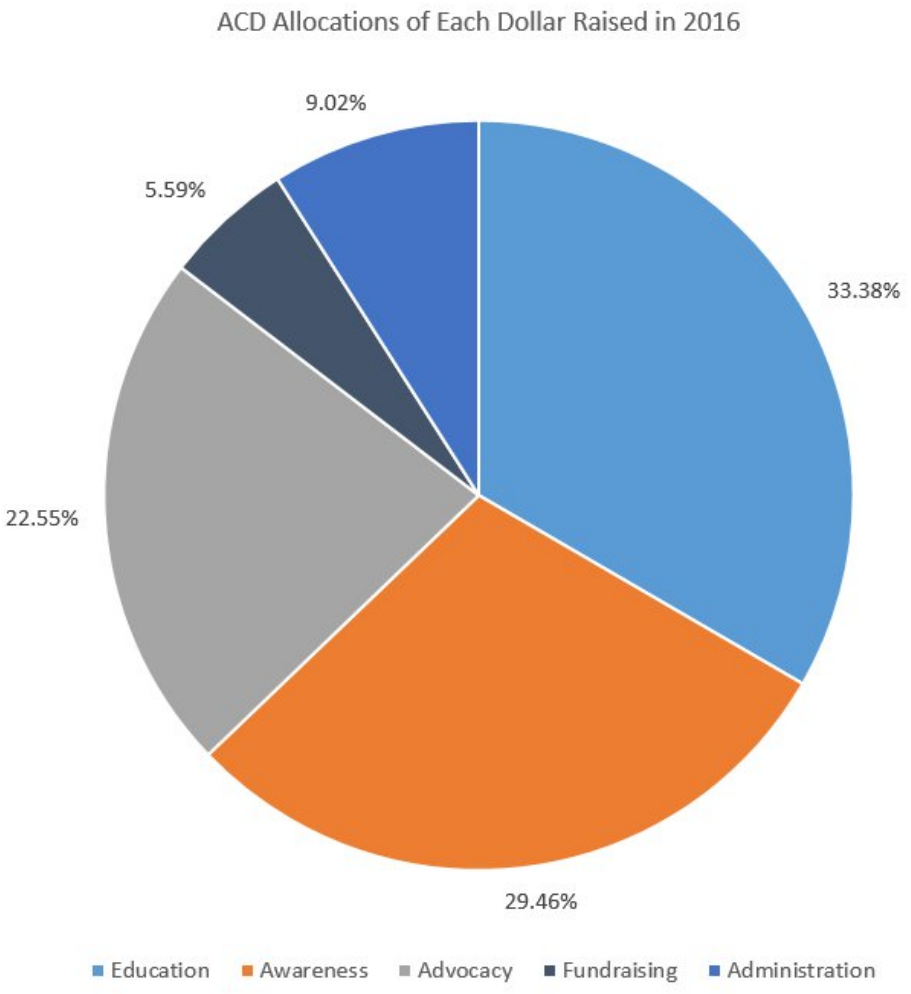
Mikelle Law

Sarah Rose

Laura Ward

ACD Allocations of Each Dollar Raised in 2016

Every dollar donated to the ACD is allocated to education, awareness, advocacy, fundraising and administrative expenses.



Below shows approximate funds given for every dollar donated to the ACD.

Education

.33 cents from every dollar donated is used towards CCDS educational efforts, including but not limited to, conference exhibiting and attendance, website and social media outreach, educational material and video publications.

Awareness

.29 cents of every dollar is used to promote awareness of CCDS. These funds help reach families, allied medical professions and the general public at large through awareness walks and other non-profit partnerships and networking.

Advocacy

.23 cents of every dollar donated goes directly to advocacy efforts. These funds are crucial in helping to advance CCDS newborn screening and collaborate with other non-profits to communicate and grow the future representation of CCDS patients.

Fundraising

.06 cents of every dollar is used to help raise funds for ACD's mission of Education, Awareness, and Advocacy to eliminate the challenges of people affected by a CCDS diagnosis.

Administration

.9 cents from every dollar is allocated for operational supplies, equipment, fees and charges that are necessary throughout the year.

ACD 2016 Financial Accounting

Statement of Financials
January – December 2016
Balance

Assets

Current Assets	$70,095.02

Liabilities & Equity

Equity

Opening Balance Equity	$50,177.88
Net Income	$19,917.14
Total Equity	**$70.095.02**

Total Liability & Equity

As of December 31, 2016	$70,095.02

Statement of Profit and Loss
January – December 2016

Ordinary Income/Expense

Income	
Donations	$51,611.62
Grants	$5,700.00
Total Income:	**$57,311.62**

Expense

Accounting & Tax	$360.00
Advertising	$4,313.10
Conference(s)	$2,981.10
Dues & Subscript	$1,925.00
Fees & Charges	$691.28
Fundraising	$2,091.18
Insurance	$1,195.00
License & Permits	$25.00
Office Equipment & Supplies	$1,195.00
Postage, Delivery	$1,858.67
Printing, Copies	$2,462.59
Travel, Meetings	$14,591.53
Web & Social Management	$2,960.00
Total Expense	$37,394.48
Net Income	**$19,917.14**

Independent Monthly Data: Bookworks,
Costa Mesa, California

Independent Annual Accounting: Titanium Financials,
Irvine, California