Transforming health

ANNUAL REPORT 2014

Genetic Alliance

Genetic Alliance engages individuals, families, and communities to transform health; we bring together diverse stakeholders to create novel partnerships in advocacy; we integrate individual, family, and community perspectives to improve health systems; we revolutionize access to information to enable translation of research into services and individualized decision-making.

ABOUT GENETIC ALLIANCE

Genetic Alliance, Inc. (Genetic Alliance) was incorporated as a non-stock, nonprofit organization on October 31, 1986 within the laws of the State of Maryland.

Genetic Alliance's network includes more than 1,200 disease-specific advocacy organizations as well as approximately ten thousand universities, private companies, government agencies, and public policy organizations. The network is a dynamic and growing open space for shared resources, creative tools and innovative programs.

IN THIS REPORT

DEAR GENETIC ALLIANCE FAMILY,

This has been an incredible year for Genetic Alliance. We share with you, in these pages, some of the amazing partnerships in which we have engaged. You will find that we are now in central roles in a number of national initiatives.

Take a look at the Community Engaged Network for All, Genetic Alliance's People-powered Research Network (PPRN), bringing together 10 organizations to work as part of the new National Clinical Research Network, PCORnet (page 4). Genetic Alliance is excited to join the other PPRN and create a more consumer focused research milieu.

Baby's First Test continues to grow in its service to the nation (page 6). Hundreds of thousands of parents have found it to be a terrific one stop resource for information on all of the states and all of the conditions for which they screen.

The National Consumer Genetics Education Network is a great example of working hand in hand with professionals to better understand the needs of families with regard to genetic services (page 8). We conducted a survey this year with an excellent response rate and saw some important themes emerge to help both us and the Health Resources Services Administration engage in prioritization of areas of need.

Finally, Genetic Alliance staff serve on just about every prominent national or international committee in genetics, health, and/or biomedical research with the goal of bringing the patient and participant voice into the discussion and policy making.

Thanks to all of our collaborators and supporters, without you we could not have achieved so much!

Sincerely,
Sharon F. Terry, President & CEO

BioTrust

People are the next blockbuster drug. Patients in the clinic and consumers of healthcare services alike are ready to have an impact on the world of biomedical research, particularly translational science. Genetic Alliance's BioTrust enterprise builds tools to empower individuals to be full participants in research that will transform health.

Genetic Alliance subscribes to the principle that, like other industries, biomedical research must be co-designed by the people who need it most. Other industries have long benefited from, and often profited from, the active participation of consumers. It is time for the consumer movement to advance research.

The tools that Genetic Alliance builds - some of which are described below - are the direct result of the voice and expressed needs of individuals, families, communities, research institutions, and industry. This broad audience can generate conflicting interests. Genetic Alliance seeks out that conflict and embraces it. We engage with our BioTrust Ethics Team and Institutional Review Board to face ethical and social issues head on. The tools and resources we create reflect the cutting edge of consumer involvement and the rise of participatory research. Our Ethics Team uses an implementation science approach in a "Lean Startup" methodology - we learn, we do, we fail, we learn, and we launch again in a generative cycle.

Platform for Engaging Everyone Responsibly (PEER)

2014 was all about BIG DATA. We know that big data is made up of little data – the data from each person where they live, work, and play. Several years ago, Genetic Alliance partnered with Private Access to build the Platform for Engaging Everyone Responsibly (PEER). PEER enables individuals to determine their data sharing, privacy, and access preferences as they share health data, and to change them over time. Individual researchers can query PEER and find information that has been properly consented for their access and use. They can also recruit individuals to clinical trial cohorts.

By providing affected individuals with information and access to connect directly with the research community, PEER will facilitate patient engagement in the research process, benefiting condition-specific as well as cross-disease clinical research endeavors.

This year we achieved a number of notable milestones.

- Genetic Alliance successfully competed with dozens of other groups to become part of PCORnet, the National Patient-Centered Clinical Research Network. Through this program, we received $1,000,000, were able to offer PEER-based registry services to eleven patient-powered clinical research networks, and began to create scalable and sustainable structures for disease advocacy organizations to conduct research.
- PEER was recognized in the Forbes Top 10 Best Business Models for Transforming Health.
- Genetic Alliance received funding from PhRMA to assist the FDA in the Patient Focused Drug Development effort they undertook as part of Prescription Drug User Fee Act V (PDUFA V).
- The Ethics Team, eight creative and energetic experts, worked to improve PEER and focused on realizing all of the Fair Information Practice Principles as engagement guidelines, rather than just relying on consent.

In 2014 PEER began to partner with communities that are ready to manage their own health research – not only within disease-specific communities, but also organizations focused on health more broadly.

Community Engaged Network for All

Genetic Alliance received a substantial award from the Patient Centered Outcomes Research Institute (PCORI) to become a Patient Powered Research Network (PPRN) of the national clinical research network. Community Engaged Network for All (CENA) became this PPRN, one of 29 PCORnet projects, involving millions of Americans. CENA is a collaborative among two academic medical institutions, a company, and nine disease advocacy organizations:

University of California San Francisco University of California Davis Private Access Alström Syndrome International AXYS Dyskeratosis Congenita Outreach Inflammatory Breast Cancer Research Foundation Hepatitis Foundation International Joubert Syndrome and Related Disorders Foundation MLD Foundation National Gaucher Foundation PXE International

Each PEER registry is engaging community members in participant-centric biomedical research. Using PEER, advocacy organizations and academic partners are collaborating to align incentives, empower data sharing, and change the paradigm for clinical research. In the next phase, these communities will design and conduct participant-centric research.

Free the Data

Free the Data is Genetic Alliance's campaign to empower women and men who have been tested for BRCA1 and 2 to liberate their own health data. Instead of the BRCA mutation information being stored and accessed only by the testing companies, we use the PEER system to enable individuals to put their mutations in the public domain, through a partnership with the National Institutes of Health.

Patient Focused Drug Development Initiative

In 2014, Genetic Alliance was awarded an $180,000 grant from Pharmaceutical Research and Manufacturers of America (PhRMA) to support the use of PEER in a Food and Drug Administration (FDA) pilot program to advance patient preference science. The Patient Focused Drug Development (PFDD) initiative—mandated by the Prescription Drug User Fee Act V (PDUFA V)—seeks to elicit patient preferences. PEER is uniquely suited to this. More than 1,000 individuals in three disease areas participated over the course of this program.

Genetic Alliance Registry and BioBank

Founded in 2003, Genetic Alliance Registry and BioBank (GARB) was the first participant-powered biorepository and is the only lay-run, cross-disease biobank and registry. It includes more than 30,000 biological samples for eight conditions. Discussions began in 2014 to integrate the system into the PEER preference setting technology.

Expecting Health

The potential application of new genomic technologies in the prenatal, postpartum, and early childhood periods is an exciting prospect. However, there is still a gap in families' knowledge and awareness of their testing options and how to coordinate care moving forward.

This year, Genetic Alliance's Expecting Health enterprise focused on solidifying its current infrastructure and programs to enable growth in the near future. With tested and proven methods for engagement and education, we are able to expand into new content areas for which both healthcare providers and the public face a dearth of information and opportunities for dialogue.

Baby's First Test

In 2014, the nation's clearinghouse on newborn screening information, Baby's First Test, expanded its central website, BabysFirstTest.org, and planned for a site refresh. Over the past three years, use of the site has shifted dramatically from laptop/desktop to mobile devices. Mobile website access increased tenfold since the initial launch in 2011. Recognizing this shift enhanced our understanding of visitor needs and contributed to the plan for a site redesign that prioritizes quality presentation of the content independent of what type of device a user was using. This includes new functionality, such as responsive design.

The year brought almost 300,000 visits from more than 238,000 users. While October 2013 had about 15,000 sessions, September 2014 saw an increase to more than 41,000 sessions. We attribute this nearly 200% to a new site focus on sharing diverse content—on health, parenting, and child development—written by a range of expert partners.

Consumer Task Force on Newborn Screening

We continued our commitment to consumer training through the Consumer Task Force on Newborn Screening. This year, six people were selected to learn about national, state, and local-level newborn screening in addition to hot-topic issues, such as the retention of dried blood spots and how social media can help spread awareness. The group used this information to create outreach projects to educate and engage others in their community about newborn screening. These projects included creating books (now available on Amazon.com) to explain to siblings what it means to have a brother or sister with special healthcare needs, increasing support services for families with sickle cell disease and creating materials for those identified as carriers, and developing family-centered strategies around timeliness of newborn screening. The Task Force members also shared their perspectives through parent panels and public comments at national newborn screening conferences.

Challenge Awards

The 2014 Challenge Awards, totaling $80,000 in direct funds, support four community-based initiatives as they created and provided parent-oriented content through novel platforms. One program, out of Michigan, used parent narratives as tools to educate around the importance of newborn screening and the daily struggles of families affected by a condition. Another program used evaluation measures to better understand the needs of the Amish community. This is a critical group: the Amish population is expected to double by 2024, yet the community continues to face barriers in timely newborn screening. The next project focused on improving online education for families, distributed through the Association of Women's Health, Obstetrics, and Neonatal Nurses. The final project focused on working with Early Hearing Detection and Intervention Champions to disseminate Baby's First Test content on hearing loss—an important focus, since two to four percent of neonatal intensive care unit newborns will experience hearing loss.

Genes in Life

As science progresses, our understanding of the contribution of genetics and genomics to overall health accelerates, in turn greatly improving our understanding of health and disease. In addition, genetic services are maturing, including screening with large panels, pharmacogenomics, and even whole exome and genome testing.

Genetic Alliance offers a variety of tools and resources to help individuals identify and access quality information, services, and support. The launch of several of these high-quality tools and the advent of new and unique partnerships made this year particularly exciting and impactful. In addition, we proactively sought opportunities to further impact patient outcomes through public health genomics education and training.

National Genetics Education and Consumer Network (NGECN)

Genetic Alliance engaged individuals and families in a number of formal and informal dialogues to better understand what barriers and gaps exist for genetic services. We published national data—the first of its kind—concerning access to care and the care experience for individuals affected by genetic conditions (1,895 respondents). The **Survey on Genetic Services: Resources, Referral, and Collaboration** (96 organizational respondents) helped us understand the questions families ask disease advocacy and support organizations about genetic services and where they look for information on health and genetics. We led a collaborative effort with the American Academy of Pediatrics and the National Coordinating Center for the Regional Genetics Collaboratives to assess the needs and experiences of pediatric patients affected by genetic conditions. The common themes from interviewed parents [of children ages 0-18] were:

- Parents found peer support to be especially helpful for navigating services, but most respondents were not connected to that support through their provider.
- Parents commonly reported difficulty with the large financial burden and the time required to get care/services on their own.

Genetic Alliance worked closely with the NGECN Consumer Advisory Group, a group of eight passionate people from across the United States, to develop tools and trainings that amplify the voices of consumers and strengthen consumer participation in genetics programs. We continued to collaborate with the Regional Genetics and Newborn Screening Collaboratives, hosting in-person and virtual opportunities for family advocates across the regions and leaders from Parent to Parent and Family Voices to collaborate around what families need to better understand, access, and utilize services and support.

Tier I Public Health Genomics

Genetic Alliance partnered with the Centers for Disease Control and Prevention's Office of Public Health Genomics to champion multiple state- and consumer-focused initiatives during the year. We worked with advocates and experts to create several new, accessible education materials, including patient-friendly infographics on cascade screening for Lynch Syndrome and Hereditary Breast and Ovarian Cancer Syndrome. We also produced a video for institutions and providers, entitled *Tier I Genomic Applications and Pioneer/Trail Blazing States*, that highlights state public health pioneers who have successfully used genomics in a measurable way. These state leaders describe in detail the challenges and rewards of implementing evidence-based public health genomics applications to save lives. Actual examples and best practices are described and can be used by other states and partners to implement their own innovative programs.

Making an Impact

In addition to consumer engagement, Genetic Alliance sought to promote expansion of evidence-based programs that increase access to genetic services. We funded seven Impact Awards in May 2014, with the following anticipated goals:

1. The **Center for Jewish Genetics** aims to create a replicable model for all states to educate and encourage screening for high-risk populations using proven outreach strategies and customizable educational materials.
2. The hearing-impaired community is at high risk for poor patient-provider communication, low health literacy, and low comprehension of health information. **Gallaudet University** will offer the first comprehensive genetic counseling resources in American Sign Language.
3. **A.I. duPont Hospital for Children-Nemours** will conduct a far-reaching outreach and dissemination campaign that will increase access to support for teens with genetic conditions and their families through the transition and family planning processes (data on family planning is frequently sought but not broadly accessible or available).
4. The goal of **The Hali Project Parent Partner Training** is to develop an effective model to support children with special healthcare needs and their families. The model will include evidence for cost effectiveness, increased patient and provider satisfaction, money and time saved, and reduction in emergency room visits.
5. **Boston University School of Public Health** will create a model and customizable resources that can be applied to other hospital settings to increase access to genetic services for individuals with autism and their families.
6. **Michigan Public Health Institute**'s project will result in a replicable, evidence-based model for improving access to a medical home for children with genetic conditions and their families.
7. **University of Wisconsin**'s focus is a low-cost training model to educate and improve access to newborn screening and follow-up services within insular communities.

Out and About

Each year, Genetic Alliance staff travel the country and the globe to present to our colleagues in the genetics, health, and advocacy community. We engage diverse stakeholders to bring about meaningful change in genetics and health policy and to advance the interests of individuals, families, and communities.

In 2014, Genetic Alliance staff delivered presentations in 12 states and on four continents and published 14 articles in books and peer-reviewed journals.

Public Policy

If an organization or entity works in the genetics, health, and advocacy space, they work with Genetic Alliance. In 2014, our staff worked with the following groups, among many others, on a variety of topics ranging from basic science to implementation science and service delivery.

Accelerating Medicines Partnership Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children, Education and Training Subcommittee American Association for the Advancement of Science American College of Medical Genetics American Public Health Association, Maternal and Child Health Section, Genetics and Bioethics Committee American Public Health Association, Genomics Forum American Society of Human Genetics American Society of Human Genetics, Social Issues Committee American Society of Matrix Biology Association of Public Health Laboratories, Newborn Screening and Genetics in Public Health Committee Association for Research in Vision and Ophthalmology Autosomal Recessive Polycystic Kidney Disease Alliance BabySeq, Genome Sequence-Based Screening for Childhood Risk and Newborn Illness Cellular, Tissue, and Gene Therapies Advisory Committee, FDA CANCERGEN External Stakeholders Advisory Group Coalition of Heritable Disorders of Connective Tissue Coalition for Genetic Fairness Coalition for 21st Century Medicine EspeRare Foundation Food and Drug Administration Genetics in Primary Care Institute (GPCI) Project Advisory Committee Genomic Applications in Practice and Prevention Network Genome Advisory Board GlobalAlliance House Appropriations Committee House Committee on Science and Technology House Committee on Oversight and Government Reform HHS ONC HIT Standards Committee HHS Secretary's Advisory Committee on Heritable Disorders in Newborns and Children Institute of Medicine Workshop, Assuring Integrity while Facilitating Innovation in Medical Research Institute of Medicine Roundtable on Translating Genomic-Based Research for Health Institute of Medicine Board on Health Science Policy Institute of Medicine Action Collaborative on Developing Guiding Principles for Integrating Genomic Information into the Electronic Health Record Ecosystem International Rare Disease Research Consortium Jackson Labs/ASHG Joint Advisory Committee for Cancer Genomics for PCPs Liaison to the National Advisory Council for Human Genome Research National Advisory Council for Human Genome Research Newborn Screening Translational Research Network, American College of Genetics and Genomics, BioEthics and Legal work group Committee MEDSeq, Integration of Whole Genome Sequencing into Clinical Medicine Non-Federal Working Group - U.S. Surgeon General's Family History Initiative NHGRI National Advisory Council For Human Genome Research Liaison PCORnet, Patient Centered Outcomes Research Institute PhenX: Consensus Measures for Phenotypes and eXposures Rosalind Franklin Society Board Senate HELP Committee Society of Investigative Dermatology Stakeholder Advisory Committee Health and Economic Outcomes for Newborn Screening White House Office of Health Information Technology

Financials

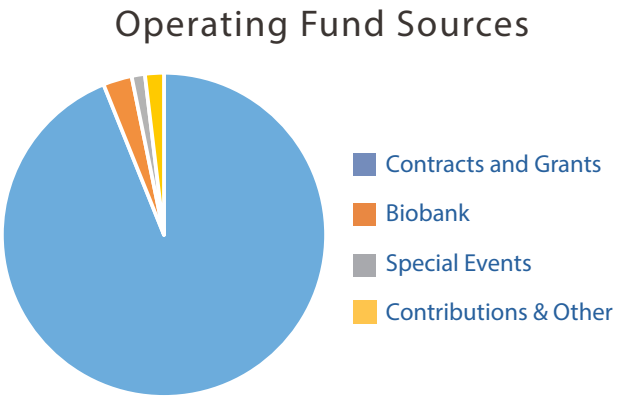
Genetic Alliance's work is supported by a blend of funding from government grants and contracts, industry and corporate support, individual donations, and fees generated by services and events.

We continually work to diversify our funding sources. A key part of this diversification plan is to secure strategic, fee-for-service partnerships that leverage our expertise, serve the needs of the field and community and bring greater financial sustainability to the organization.

Statement of Activities and Changes in Net Assets

as of September 30th of each year

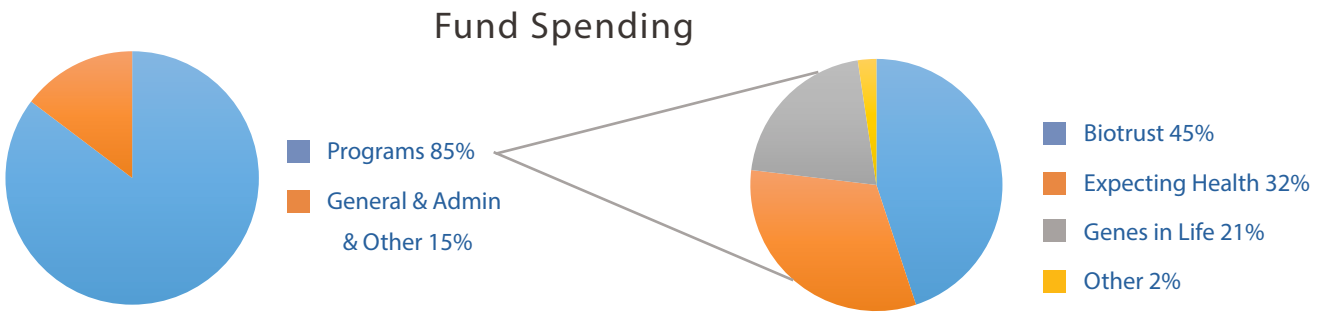
	2014 Total	2013 Total
Revenue and Other Support		
Government Contracts and Grants	$2,469,580	$1,1955,242
Biobank Program Revenue	75,598	289,753
Special Events	34,750	115,718
Contributions	13,631	22,218
Listserv Income	0	300
Interest Income	21	41
Other Revenue	35,955	15,027
Total Revenue and Other Support	$2,629,535	$2,392,580
Expenses		
Program Services		
General Programs	$2,708,552	$2,185,073
Supporting Services		
General and Administrative	415,968	498,359
Fundraising	2,419	59,387
Total Expenses	$3,126,939	$2,742,819
Net Increase (Decrease) in Net Assets	(497,404)	(350,239)
Net Assets, Beginning of Year	$ 931,046	$1,281,285
Net Assets, End of Year	$ 433,642	$ 931,046



Statement of Financial Position

as of September 30th of each year

Assets	2014	2013
Cash and Cash Equivalents	$86,911	$219,313
Accounts Receivable	-	157,816
Grants Receivable	261,378	220,601
Prepaid Expenses	32,934	28,760
Deposits	6,595	6,595
Furniture & Equipment	222,760	553,307
Total Assets	$610,578	$1,186,392
Current Liabilities		
Accounts Payable and Accrued Expense	$140,521	$207,802
Long-term Liabilities		
Deferred Rent	36,421	47,544
Total Current Liabilities	-	255,346
Net Assets		
Unrestricted	433,636	931,046
Temporarily Restricted	-	-
Total Net Assets	433,636	931,046
Total Liabilities and Net Assets	$610,578	$1,186,392



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