Clinic *for*
Special Children

CELEBRATING 25 YEARS

Advancing medicine by serving children and families

Annual Report

2014

Our mission

Provide comprehensive local medical care, integrate science and clinical medicine, and share knowledge to improve the health of children who suffer from genetic disorders.

Dear Friends,

For 25 years, the Clinic for Special Children has been a leader in the application of genetics to medical care. By breaking down the barriers between primary clinical care and state-of-the-art laboratory methods, CSC has been able to provide the highest quality clinical, laboratory, and research services to a group of patients who would otherwise struggle to overcome the obstacles inherent to our modern healthcare system. CSC saves the Plain communities tens of millions of dollars annually by increasing diagnostic efficiency, reducing hospitalization rates, and preventing severe disabilities in children.

Our success depends on a network of many talented and generous friends and collaborators. We are grateful for contributions from diverse institutions such as Lancaster General Health, Franklin & Marshall College, Nemours Children's Health System, Children's Hospital of Pittsburgh, Regeneron Pharmaceuticals, Temple University, Columbia University, who have been instrumental in helping us to realize our clinical research mission.

We also thank the many individuals, foundations, and organizations that support the clinic. Support from five annual benefit auctions reflects the extraordinary generosity of the communities who depend on our services. Thousands of individuals donate their time, effort, and commitment to make the auctions successful year after year. We are moreover continually humbled by the outpouring of support from private donors, many of whom have supported the clinic since its inception.

More than two thirds of our annual revenue comes from charitable sources; those resources provide a vital foundation that supports exceptional services at a reasonable cost.

There are many challenges ahead. As the number of patients served by the clinic grows, we feel the strain on our budget and professional resources, but stand committed to respond to the increased need for CSC services. Salaries and benefits for CSC staff represent the largest annual expense, and we have started an initiative to build sustainable funds to insure that key staff positions are secure in perpetuity.

This is the first time the clinic has offered a publicly accessible annual report. We want to share our successes, challenges, and opportunities, while making our finances and needs transparent. We recognize *trust* is the bedrock of the clinic's future – Trust between patients and staff, trust between supporters and the institution, and trust between those who need care and those willing and able to offer it. This report, and ones to follow, is intended to cultivate that trust.

We look forward to a bright 2015 as we strive to continue to lead the effort to apply advances in medical knowledge into strategies that promote the health of children, families, and communities.

Kevin A. Strauss, MD
Medical Director

Adam D. Heaps, MS
Administrative Director

Our vision

We envision the Clinic for Special Children as a *medical home* for predominantly Amish and Mennonite children who are born with genetic predispositions to disability, chronic disease, or untimely death. We continually strive to integrate advanced scientific tools and concepts into clinical practice so that genetically vulnerable children have access to the most timely, affordable, and effective health care. The Clinic for Special Children represents an innovative and holistic approach to modern medical care that can inform the practice of genomic medicine in other settings. We seek opportunities for education and collaboration that promote the well-being of genetically disadvantaged, underserved individuals throughout the world, and are dedicated to training the young clinicians and scientists who will care for these individuals now and in the future.

Precepts

- Accurate and cost-effective diagnosis.
- Comprehensive, accessible, and affordable care.
- Core expertise and consultative capacity.
- Deep understanding of disease mechanisms.
- Integration of basic research and clinical practice.
- Preemptive diagnosis and disease prevention.
- Strategic collaborations to enhance patient care and research.
- Education and community empowerment.

2014 year in review

1,119 patients

16 staff members

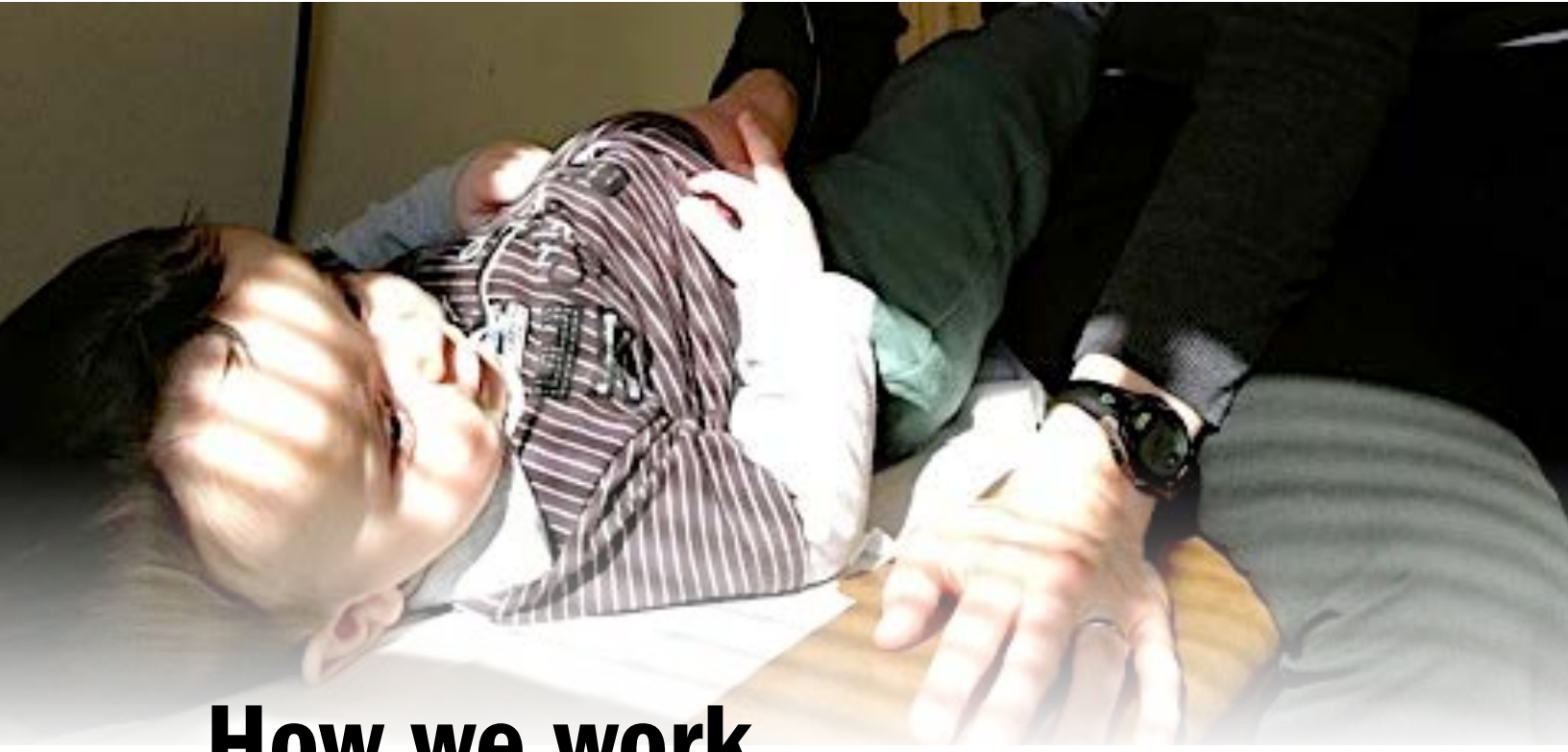
160 diseases managed

2,991 biochemical tests

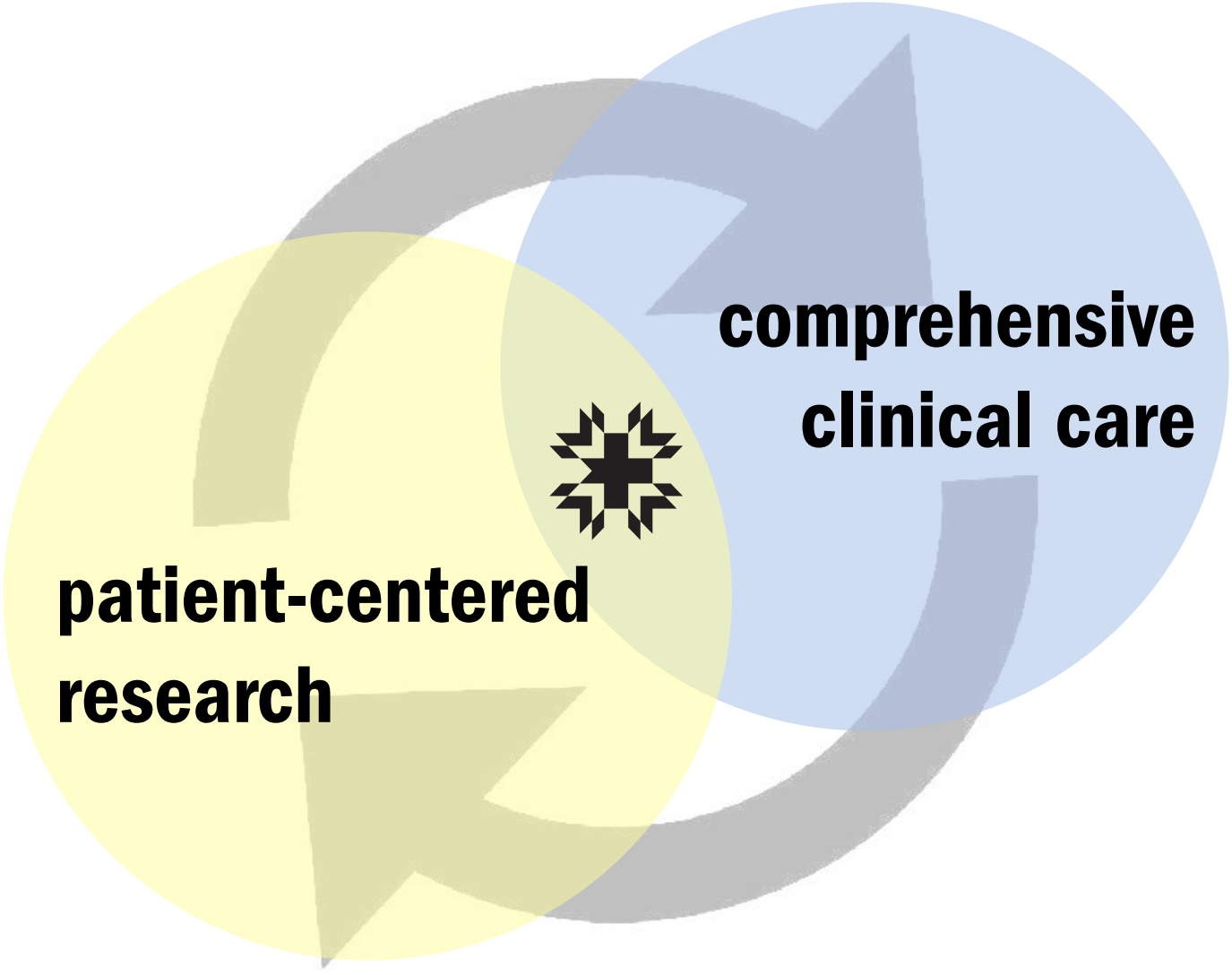
1,853 genetic tests

1,597 patient visits

4 peer-reviewed publications



How we work



Serving **34** states and **17** countries

CELEBRATING 25 YEARS

Looking back, measuring progress

	1989	2014
STAFF	3	16
ACTIVE PATIENTS	100	1,119
DISEASES MANAGED	2	160
OPERATING BUDGET	$163,000	$2,500,000
OFFICE VISIT FEE	$25	$50
AMINO ACID TEST	$45	$75

The clinic has grown in every way – staffing, services offered, patient population, diseases managed, collaborative relationships, and scientific research. Despite our growth and progress, our model for patient care that was established by Holmes and Caroline Morton has remained the same. We continue to provide medical home services to the most vulnerable children among us at an affordable cost.

What is wrong with our child?

clinical case study

Eight months ago, a family came to our clinic as a referral from a trusted friend and provider at a nearby children's hospital. Just as we have done for countless families for more than 25 years, the Clinic for Special Children sought to answer a deceptively simple question: "What is wrong with our child?"

The family lives just 50 minutes east by car but is a cultural ocean away from our typical Old Order Amish and Mennonite clientele. Arriving with a healthy dose of skepticism from the suburbs of Philadelphia, the family entered our post and beam, barn-like facility to find simple furniture, an Amish family waiting patiently, and an unnerving quietness. Is this really the state-of-the-art pediatric genetic facility that came so highly recommended?

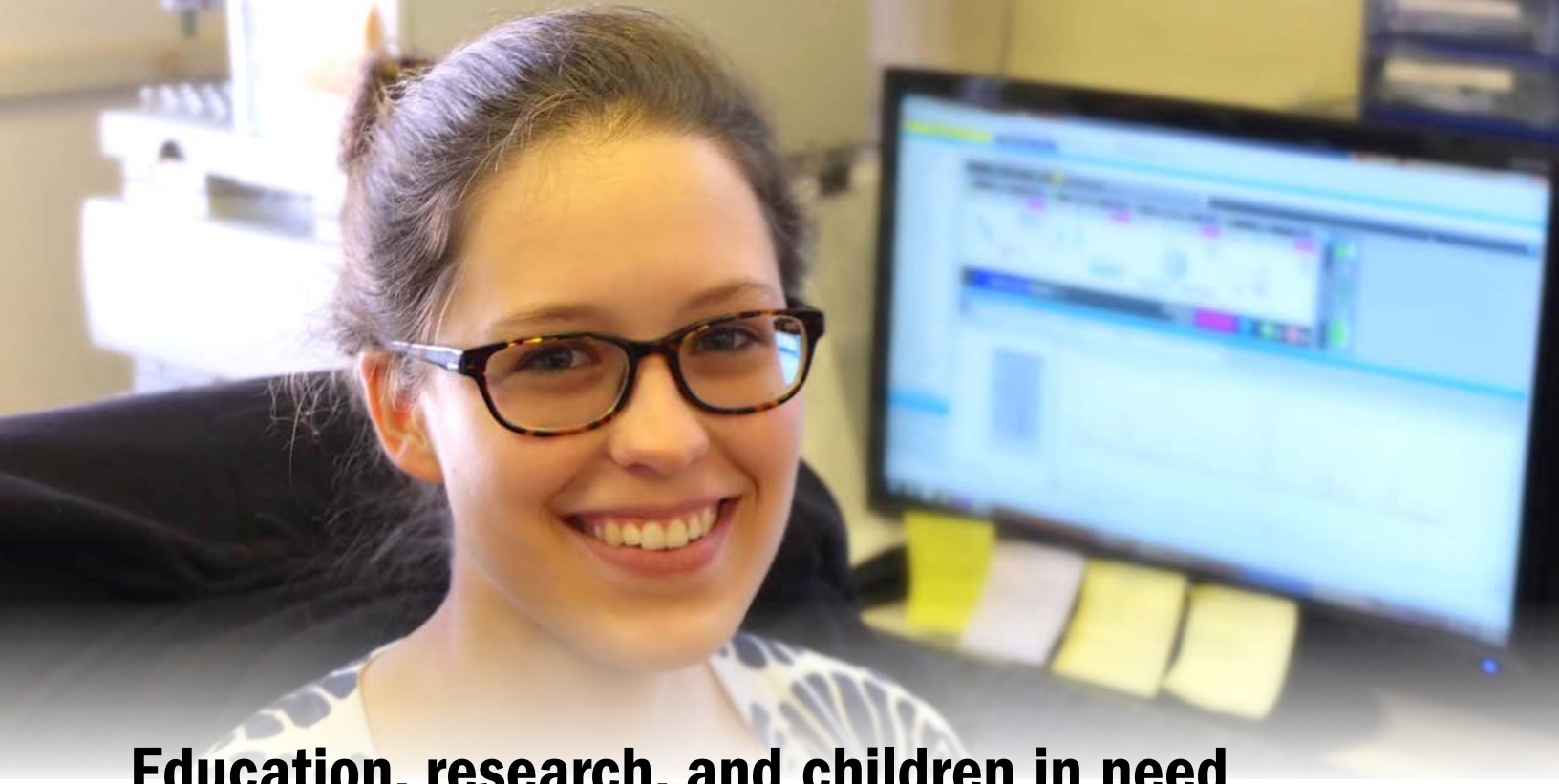
The family had already taken their child to see experts at all of the world-class centers in the surrounding area, but they were left without a diagnosis or a window of hope for treatment. Quality health insurance, financial means, and an expansive network of friends were also of little help when it came to their child's undiagnosed problem. It was clear the child was delayed, but special tutors and diagnostic testing did not solve the problem. After everything they had tried, this cornfield clinic in Strasburg, PA was worth a shot.

In the case of the new family, there was no smoking gun. A 2-3 hour initial office visit did not provide any immediate answers. During the weekly patient review meeting where clinicians and scientists review cases together, the team agreed that the best hope for the child was exome sequencing, determining the genetic code for approximately 1% of the genome that encodes proteins. It was an educated guess, 25 years in the making, and had no guarantee of success.

Last week, the family came back for a meeting with the clinic's medical director and genetic counselor. The results were carefully explained, and it was made clear to all involved that their long journey to a cornfield clinic was really a new direction, not a conclusion. A promising genetic variant was found but the significance of the variant is currently unknown. Their child will be part of a study with a handful of other children identified throughout the country. There are no treatments available, and there is no specialist waiting to greet them back at the modern research hospital. But a genetic diagnosis is a lifeline – the first important answer to the question, "What is wrong with my child?"

The family's story is far from over. Who will help them to navigate their child's care? Who will follow through on routine care after the research has concluded and the papers are published? This is where the rubber meets the road for so many families in search of help, and it is where the Clinic for Special Children has thrived against all odds. This child is no different than the 17 Amish children diagnosed with GA1 25 years ago. Rather, this child only confirms the need for more primary care centers focused on personalized medicine.

"If you want to practice personalized, genomic medicine, you have to make it personal," says Medical Director, Kevin A. Strauss, MD. "The clinic strives to integrate powerful technological advances into the care of the most vulnerable among us. It is my hope that the Clinic for Special Children will help others find the humility to ask tough questions of our medical system, so that more communities have access to the best care. The technologies exist – we just have to learn how to close the implementation gap."



Education, research, and children in need

laboratory case study

Abby Benkert was no stranger to the Clinic for Special Children. As an undergrad at Franklin & Marshall College, Abby worked with Professor Rob Jinks on cellular studies, helping CSC to uncover fundamental principles of newly discovered genetic disease.

Her interest grew in the clinic, and she had her sights set on medical school.

A growing number of talented students like Abby decide to take a gap year between undergrad and medical school to bolster their education or embark on a unique experience. Fortunately, CSC offered Abby the perfect opportunity to do both.

Our Avery Fellowship provides talented students with a deep dive in the clinic's integrated model for primary care and research. Avery Fellows spend one full year at CSC as a salaried employee, including access to a flexible research budget.

Abby jumped right into an independent research project on Congenital Adrenal Hyperplasia (CAH). Dr. Strauss hypothesized that the current treatment could be improved, but he needed more data, a better diagnostic testing methodology, and a bright mind to tackle the project.

She spent many hours on back country roads in the wee hours of the morning to collect blood samples from patients. The project expanded, and Abby received generous backing to continue her work from fellow alumnus of F&M, Joan Fallon of Curemark, LLC.

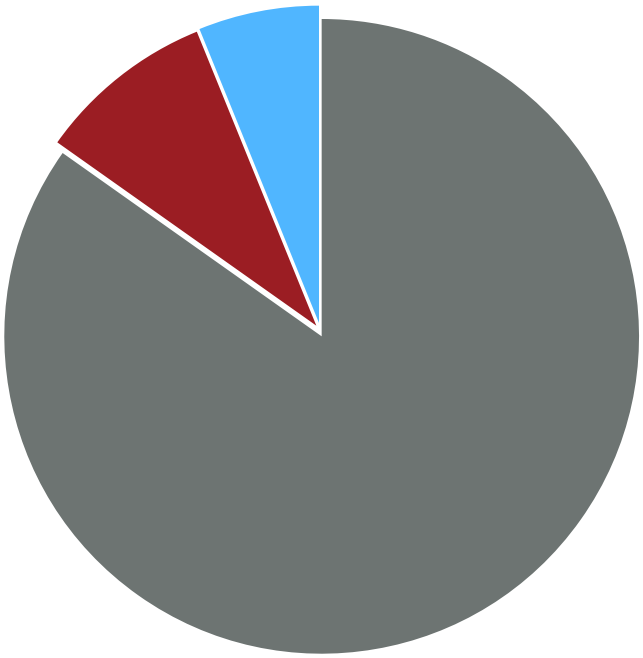
Abby also developed a new assay for monitoring dehydroepiandrosterone (DHEA) steroid levels that was fast, accurate, and affordable. Close contact with patients and families keeps research and diagnostics rooted in practical and accessible solutions for the communities we serve.

The clinic was fortunate to keep Abby for one extra year as a laboratory technician. Her scientific paper on CAH will soon be submitted for publication, but most importantly, patients suffering with CAH have a better treatment protocol and diagnostic tool as a result of Abby's work.

"**CAH children inspired me to look beyond established treatments so that each family has access to the best possible care and long-term outcome,"** says Benkert.

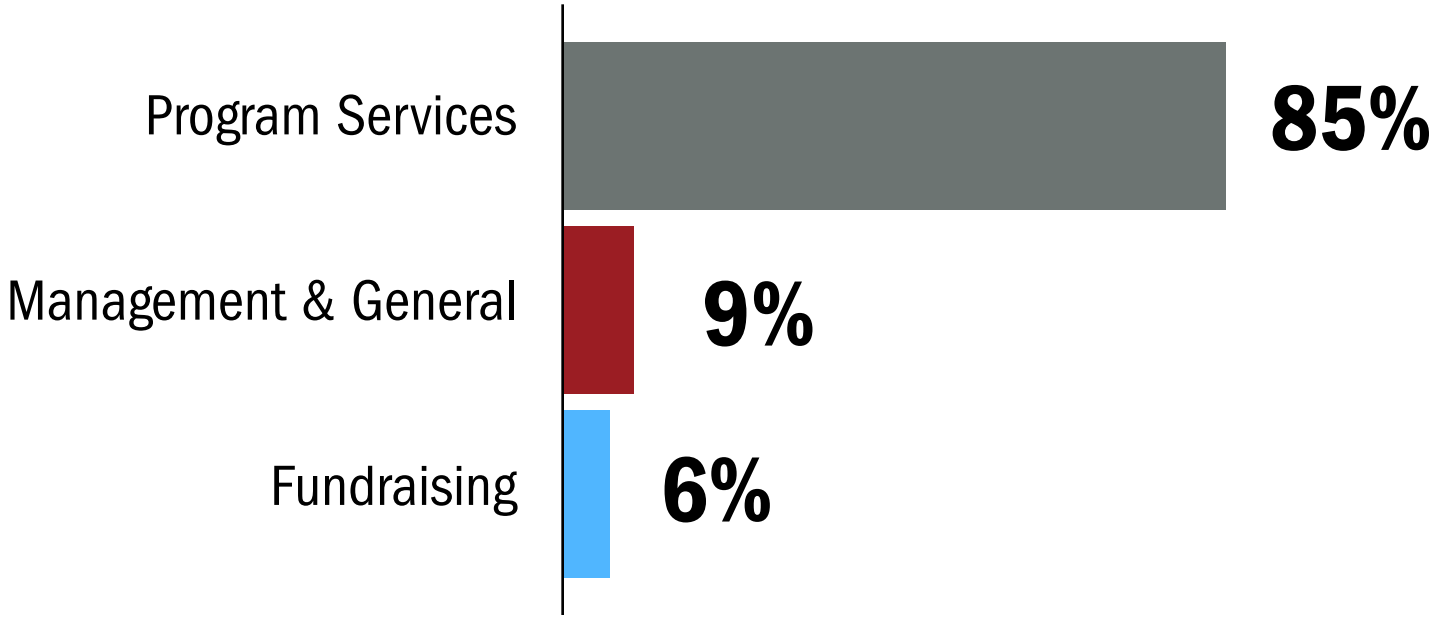
By design, it is difficult to separate the clinic's laboratory and clinical functions. Abby's work on Congenital Adrenal Hyperplasia is a perfect example of how patient care drives research, and research drives patient care. It is our hope that the clinic can foster Avery Fellows in perpetuity as the future clinician-scientists of tomorrow.

2014 expense snapshot

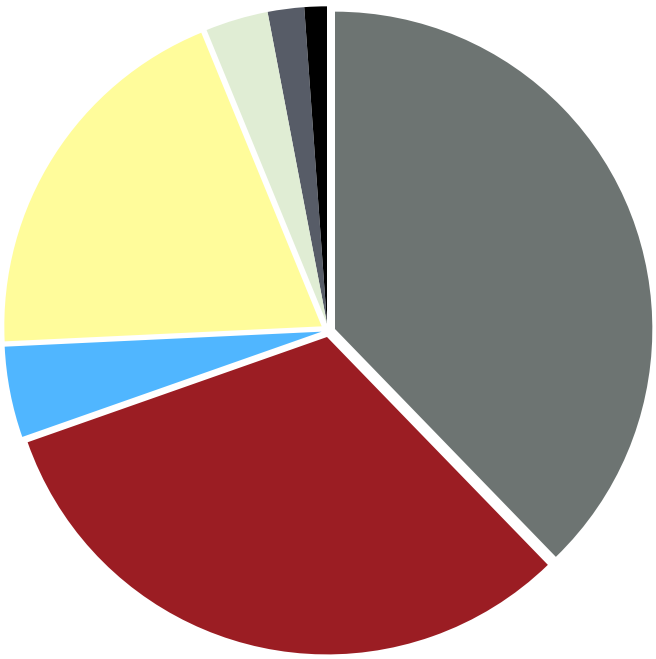


Total budget

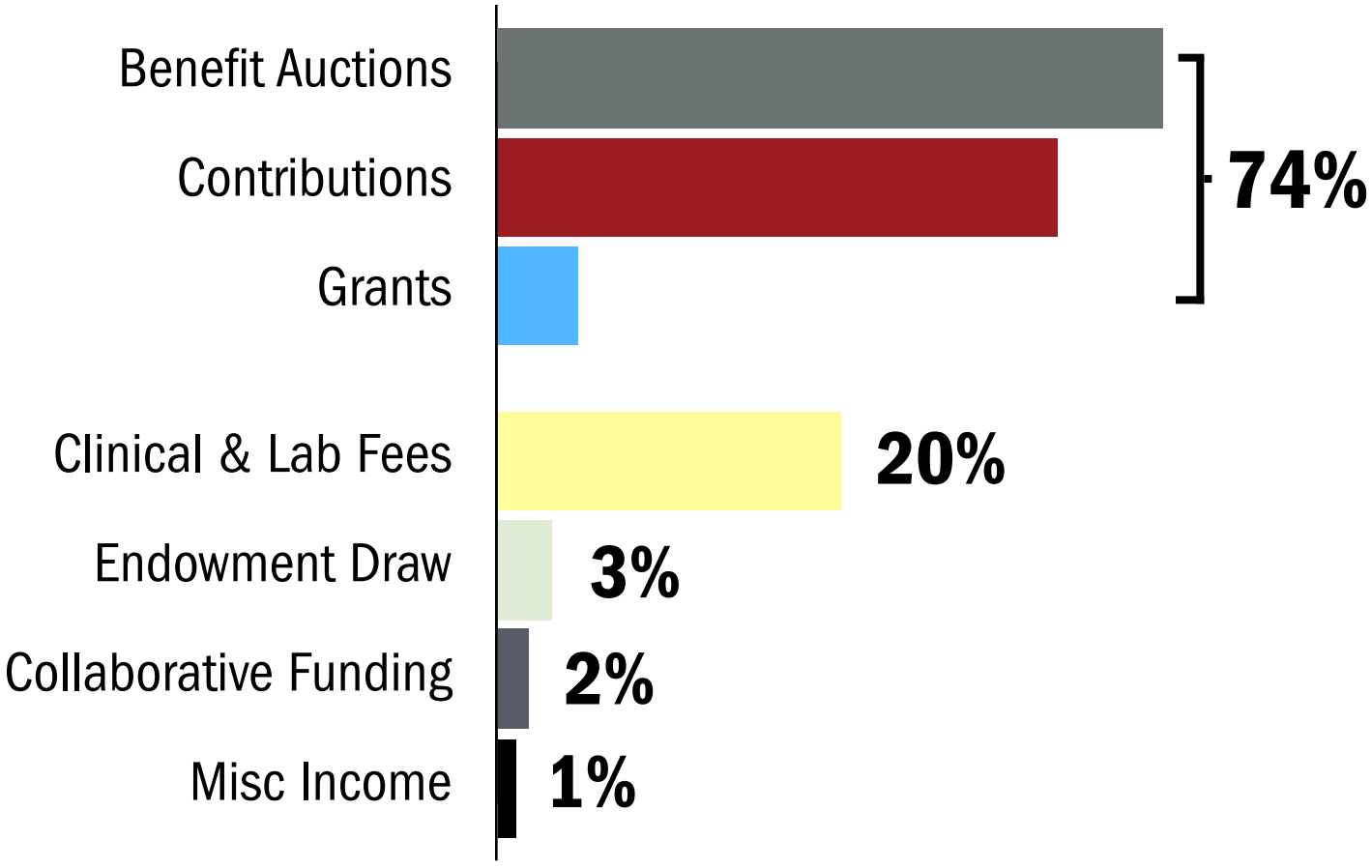
$2,531,645



2014 revenue snapshot



Almost **3/4** of all revenue comes from fundraising



Detailed financials

Statement of Financial Position

Assets	2014
Cash and Cash Equivalents	$698,200
Accounts Receivable	$107,209
Pledges	$75,000
Prepaid Expenses	$20,311
Property and Equipment	$710,475
Investments	$2,453,010
Investments held for Endowment	$689,492
Total Assets	**$4,753,697**
Liabilities and Net Assets	
Liabilities:	
Accounts Payable	$143,959
Accrued Expenses	$21,029
Deferred Revenue	$9,289
Total Liabilities	**$174,277**
Net Assets:	
Unrestricted	$3,761,319
Temporarily Restricted	$538,701
Permanently Restricted	$279,400
Total Net Assets	**$4,579,420**
Total Liabilities and Net Assets	**$4,753,697**

Statement of Activities

Revenue	2014
Contributions	$748,518
Grants	$108,700
F&M Collaboration Funds	$43,625
Benefit Auctions	$886,543
Clinic Fees	$311,135
Investment Income	$374,440
Laboratory Fees	$148,228
Material Aid Donated In-kind	$132,008
Miscellaneous Income	$26,844
Total Revenue	**$2,780,041**
Expenses	
Program Services	$2,146,881
Management and General	$229,151
Fundraising	$155,613
Total Expenses	**$2,531,645**

Back, L-R: Adam D. Heaps, Erica Sue Eisenbise, Millie Young, Katie B. Williams, Kevin A. Strauss, Erik G. Puffenberger, Yalonda Kosek, Matthew M. Sware
Front, L-R: Rebecca Willert, Donna L. Robinson, D. Holmes Morton, Caroline S. Morton, Christine Hendrickson, Abigail R. Benkert
Not pictured: Karlla Brigatti, Mindy Kuebler, Aarti P. Rao

Our staff

Abigail R. Benkert
Laboratory Technician

Karlla Brigatti, MS
Genetic Counselor

Erica Sue Eisenbise
Office Manager

Adam D. Heaps, MS*
Administrative Director

Christine Hendrickson, RNC
Nurse

Yalonda L. Kosek
Receptionist

Mindy Kuebler, MS
Laboratory Technician

Caroline S. Morton, EdM
Co-founder

D. Holmes Morton, MD
Co-founder, Pediatrician

Erik G. Puffenberger, PhD*
Laboratory Director

Aarti P. Rao
Avery Fellow

Donna L. Robinson, CRNP
Nurse Practitioner

Kevin A. Strauss, MD*
Medical Director

Matthew M. Sware*
Development Director

Katie B. Williams, MD, PhD
Pediatrician

Millie Young, RNC
Nurse

* Leadership Team

Board of directors

Herman Bontrager – Chairman | Enos Hoover | Leonard Hurst | Mark Martin—Treasurer | Caroline Morton, EdM—Secretary
D. Holmes Morton, MD | Stephen D. Ratcliffe, MD, MSPH | Kevin A. Strauss, MD—Ex officio | Jacob Zook

To study the phenomena of disease without books is to sail an uncharted sea, while to study books without patients is not to go to sea at all.

William Osler