



2017

ANNUAL REPORT



Clinic for
Special Children

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20. 2. ...
21. Wash

Our Mission

To serve children and adults who suffer from genetic and other complex medical disorders by providing

COMPREHENSIVE MEDICAL,

laboratory, and consultative services, and by

INCREASING AND DISSEMINATING

KNOWLEDGE of

SCIENCE AND MEDICINE.





Our Vision

We envision the Clinic for Special Children as a **MEDICAL HOME**

for predominately Amish and Mennonite children and adults 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 people have access to the most timely, affordable, and effective healthcare. 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 into the future.

GENERAL STATS

1,052
active patients

17 *staff*
members



Advancing Science and Medicine, One Child at a Time.

With deep gratitude, we reflect upon another year of providing a medical home for vulnerable children and adults, translating innovative research into practical solutions, and fulfilling the mission of the Clinic for Special Children. These accomplishments are only possible through the support of dedicated staff members, volunteers, and donors: people like you.

In the Clinic's 28 year history, 2017 might be the busiest and most exciting on record. We collaborated with Nemours to pioneer an innovative drug delivery method for patients with Spinal Muscular Atrophy (SMA) who would not have otherwise been able to receive the drug. We are serving as an important site for a new gene therapy clinical trial. Our leadership team held four public talks for Plain community members in an effort to share the Clinic's mission and services. Our Patient Family Advisory Council held its first social event, the Fall Family Fun Day, to honor and celebrate the families we serve. We continue to advocate on behalf

of our patients for affordable access to medications and advanced clinical services. All of these exciting projects are in addition to a significant increase in the number of patient visits and lab tests performed over the last year.

On behalf of the staff and board, we thank everyone who participated in an auction, shared our services with someone in need, participated in a project, or otherwise supported the Clinic for Special Children. We remain steadfast in our mission to provide the best quality care to patients with complex needs and develop innovative, practical solutions to the most important problems facing children and their families.

A handwritten signature in black ink that reads "Adam D. Heaps".

Adam D. Heaps, MS
Executive Director

A handwritten signature in black ink that reads "Kevin A. Strauss".

Kevin A. Strauss, MD
Medical Director



Patient Care

Delivering effective and

AFFORDABLE CARE *for* INDIVIDUALS

with genetic conditions.

PATIENT CARE STATS

3,914
*biochemical &
genetic tests*

1,606
patient visits

Manage **264**
*known variants
that cause disease*

42 *states &*
17 *countries*



“Your kindness and generosity inspire us to devote our lives to improving the care of the children and families we serve.”

- Dr. Vincent Carson, Pediatric Neurologist



Patient Focus

WHEN ASHTON WEAVER WAS ONE YEAR OLD, he could stand by himself and play with toys like any other children his age. It seemed that Ashton was healthy, but when his parents held him up, his legs began to hang limp, unable to support his weight.

Having heard about the Clinic for Special Children through several family members who used our services, Ashton's parents, Ken and Joanna Weaver, turned to the doctors at CSC for guidance. Through a diagnostic test in our laboratory, Ken and Joanna received news that no parents want to hear. At sixteen months old, their dear Ashton was diagnosed with Spinal Muscular Atrophy (SMA), a recessive genetic disorder that affects nerve cells that are housed in the spinal cord and the brain, leading to a progressive loss of muscle function.

Over the subsequent six years, Ken and Joanna experienced the joyful births of three more boys: Lincoln, Brenton, and Winston. With the birth of each new child, came another positive SMA diagnosis. The probability of all four children inheriting SMA, was 1/256 or less than half of one percent.

The Weavers were now parents to four boys living with a rare, incurable, and untreatable genetic condition that requires around-the-clock physical care. Each child presented new joys and challenges at different times, their stories unique. The Weavers turned to God for support. Joanna says, "God allowed them all to have SMA for a reason. For our own peace, we had to believe that and accept it, even when the future looked overwhelming!" The Weavers became actively involved in Early Intervention services for their children and remained up-to-date with all of the new research and medical findings about SMA through a group called Cure SMA. Day to day, they leaned on the support offered by their church, their family, and friends.

Finally, in 2017, the Weaver's hope was restored. CSC's doctors became aware of a treatment option for SMA, a new drug that is injected directly into the fluid around the spinal cord, intended to increase motor function and extend life expectancy for those living with SMA. The CSC clinicians recommended that the Weaver boys participate in a clinical trial for this new treatment. To ensure that families could receive this treatment with no barriers to care, the CSC clinical team helped to enroll each child in a philanthropic program that would allow each child to receive this medication, normally costing \$125,000 per dose at three doses per year, for free for life.

Joanna and Ken felt a new hope building inside of them. They shared the news with the boys who, sensing their parent's excitement, became excited themselves. The boys shared the news with their friends, dreaming of what they might someday be able to accomplish now that a treatment was available: things like getting a drink from faucet by themselves, moving around the house more freely, or even playing games with other children.

All four boys were accepted into the program and have received at least four doses of the new drug. Today, you wouldn't know by the boys' infectious smiles and positive spirit that they were living with a crippling genetic disorder. Each boy's journey through this treatment is unique and the ultimate results of their treatments are unknown, but they are all full of new life and a new hope.

"This experience made us turn to God in a deeper way than anything else ever had, as we looked to Him for strength. We have learned to want His will above ours, even if it is not strong physical bodies for our children.

If we could give a message to other families in similar situations, we encourage them to never give up on believing that treatment might be possible. Stay hopeful and do your own research to stay informed. The Clinic for Special Children is the most helpful place to go for the treatment of genetic disorders and we are very thankful for the caring and compassionate staff.

God will provide direction and make a way when it seems like there is none. You are not alone."



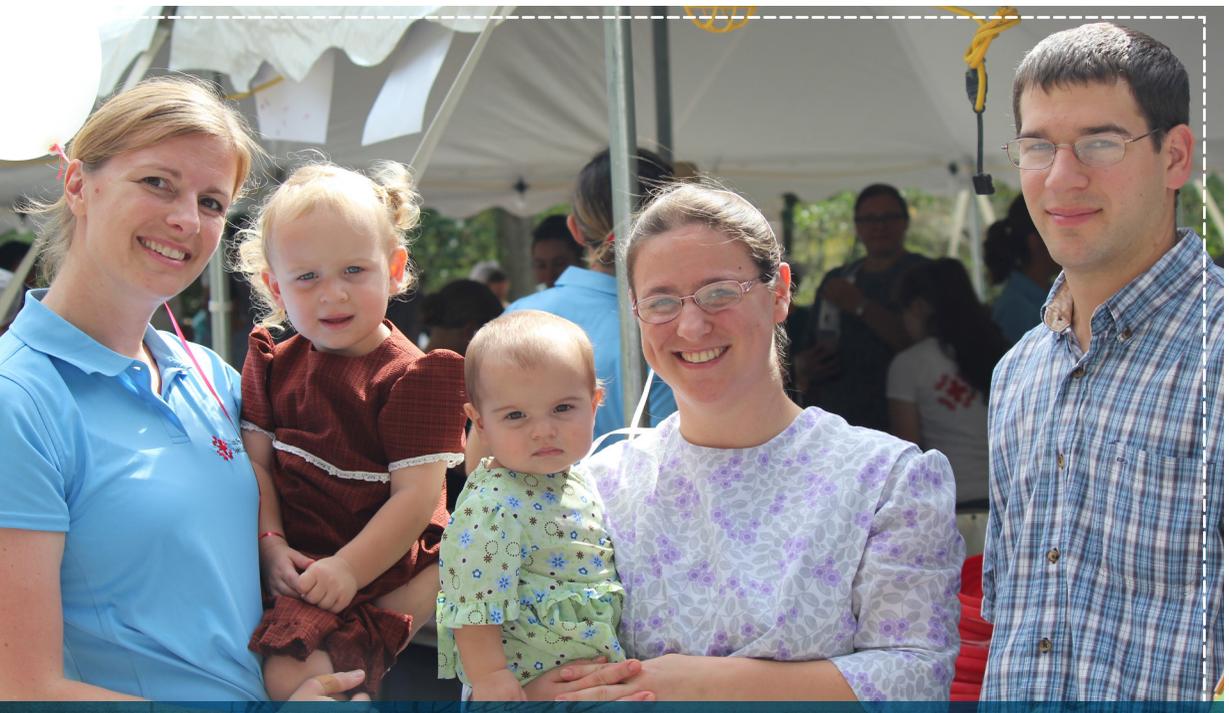
Front (L-R): Ashton, Joanna, Lincoln, and Brenton Weaver
Back (L-R): Kenneth and Winston Weaver

Outreach and Community Empowerment

COMMUNITY

This year, the Clinic's Patient Family Advisory Council (PFAC), hosted our first 'Family Fun Day' at CSC. More than 400 patients and family members gathered on the grounds of the Clinic for Special Children on a beautiful fall day to share fun and fellowship with Clinic staff and board members. The event, held on Saturday, October 7th, featured hayrides pulled by an antique tractor, games, an activities table, story time in the field, volleyball, tours of the Clinic, a variety of refreshments, and even a cotton candy machine. Foods were chosen with respect to the variety of dietary restrictions of many CSC patients, low in protein and gluten. The vision for the day was to create cross-cultural relationships between staff, practitioners, board members, patients, and family members who all shared at least one thing in common: their connection to a special child.

The Clinic's Patient Family Advisory Council (PFAC) was established in 2017 as a group intended to meet bimonthly to offer advice, feedback, and suggestions to improve patient and family experiences with CSC. The PFAC is a collaborative effort which partners patient families, CSC staff, and other stakeholders to advance the mission of CSC.





OUTREACH

Three thousand miles is a long way to travel to make house calls. But that's what Medical Director Dr. Kevin Strauss, Genetic Counselor Karlla Brigatti, and Board Chairman Herman Bontrager did last September when asked to provide medical attention to members of Old Colony Mennonites in Mexico's Yucatan region.

The request came from the Anabaptist Foundation, which helps support the work of Mexico Mennonite Aid (MMA). Richie Lauer, Foundation Officer for the Anabaptist Foundation, contacted the Clinic for Special Children. He explained, "We reached out to the Clinic for Special Children because of their work in treating rare genetic disorders among Amish and Mennonite children, and their understanding of plain community culture."

During their visit, Dr. Strauss and Karlla recommended treatment or follow-up analysis for 32 patients, both children and adults, for a wide range of conditions and identified ways to help others. They also provided guidance to MMA for the new maternity clinic being built for the colony.

Herman explained, "Our goal was to respond to Mexico Mennonite Aid's need to provide health assessments and determine what, if any, inherited genetic disorders are a factor," he said. "We also offered to share our knowledge and experience in supporting them in developing their own clinic."

EDUCATION & COMMUNITY STATS

10 family
days in 2017

4
research students

Research

This year, 8 Clinic staff members contributed to original research published in

GENETICS IN MEDICINE

In the peer reviewed publication, “Genomic diagnostics within a medically underserved population: efficacy and implications,” researchers investigated the efficacy of combining whole-exome sequencing (WES) and chromosomal microarray analysis (CMA) with a clinical work flow to elevate care for a medically underserved community. The project applied whole-exome sequencing analysis to select probands and family members who remained undiagnosed after biochemical and molecular investigations. In conclusion, “chromosomal microarray analysis and family-based whole exome sequencing streamline and economize diagnosis of rare genetic disorders, accelerate novel gene discovery, and create new opportunities for community-based screening and prevention in underserved populations.”



READ THE ARTICLE ONLINE AT:

<http://go.nature.com/zFnmPrc>

RESEARCH & DEVELOPMENT STATS

39 NEW
disease-causing
genetic variants

5

peer-reviewed
publications



Donor Spotlight

A Creative Approach to Advocacy

Esther Davies and John Thackrah beam with passion when they talk about the Clinic for Special Children. Through their creative approaches to education and advocacy for CSC over a combined 39 years, they have garnered invaluable support for the Clinic.

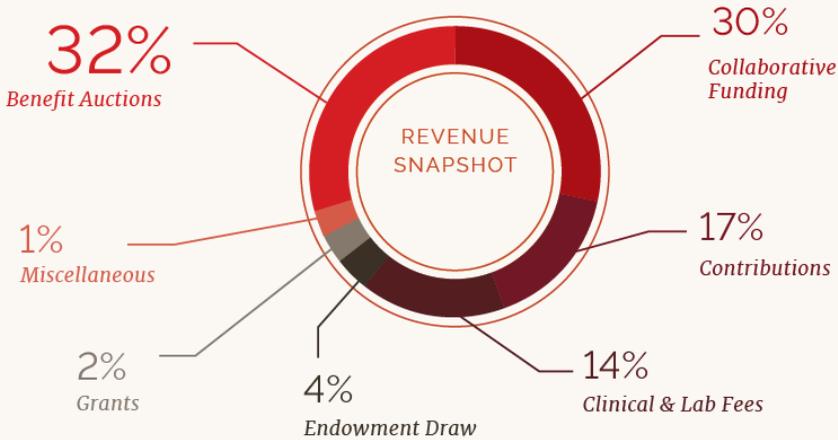
John has been an artist for as long as he can remember. His second painting from 1951 is centered over his couch pictured above. Over the past 40 years, John has used nature, wildlife, buildings, and landmarks from across several states and countries as inspiration for his artwork.

Esther's support of CSC's mission stems from her very close ties to the Amish community for over 35 years. John and Esther met almost nine years ago when John and his wife, Doris, moved into Willow Valley Communities. They formed a friendship immediately. Esther invited John to tour CSC and when John saw the Clinic firsthand, he knew he had to do something to help.

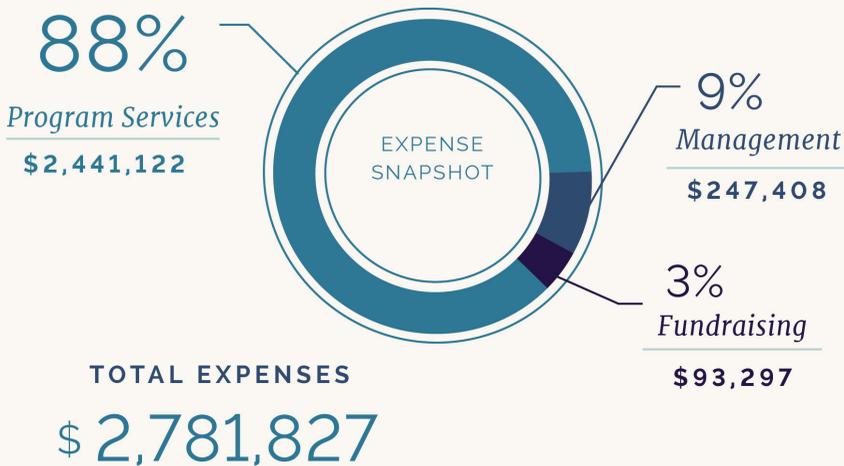
John and Esther arranged a creative awareness campaign for CSC. John converted his pen and ink artwork depicting native and iconic landmarks from around Lancaster County into greeting cards and matted prints. Proceeds raised from the sales of John's cards and prints benefit CSC. Esther and John work with volunteers from Willow Valley to box the cards and pictures for sale.

Through card sales alone, John, Esther, and volunteers from Willow Valley have raised over \$3,000 for CSC. Additionally, their public support through advocacy and education of others in Lancaster County about the Clinic's work and mission has led to invaluable support for CSC. The Clinic for Special Children welcomes and is grateful for creative ideas that support the Clinic and increase awareness of the CSC's work.

2017 | FINANCIALS



The Clinic is funded from four major sources of revenue—annual auctions, donations from caring people just like you, collaborative relationships, and fees for services paid by patient families. Your support enables CSC to continue to provide patient with timely, affordable, and effective care! We work hard to keep clinic fees to a minimum and effectively utilize funds to fulfill CSC’s mission.



2017 | FINANCIALS

STATEMENT OF FINANCIAL POSITION As of 9/30/2017

| ASSETS | |
|------------------------|--------------------|
| Cash and Equivalents | \$922,560 |
| Accounts Receivable | \$83,840 |
| Promises to Give | \$12,500 |
| Prepaid Expenses | \$23,473 |
| Property and Equipment | \$625,472 |
| Investments | \$3,444,800 |
| Investments Restricted | \$879,425 |
| Total Assets | \$5,992,070 |

LIABILITIES & NET ASSETS

| LIABILITIES | |
|--------------------------|------------------|
| Accounts Payable | \$158,597 |
| Accrued Expenses | \$25,205 |
| Accrued Wages | \$27,082 |
| Deferred Revenue | \$5,062 |
| Total Liabilities | \$215,946 |

| NET ASSETS | |
|-------------------------|--------------------|
| Undesignated | \$1,391,461 |
| Board Designated | \$3,422,149 |
| Temporarily Restricted | \$683,004 |
| Permanently Restricted | \$279,510 |
| Total Net Assets | \$5,776,124 |

| | |
|-------------------------------------|-------------|
| Total Liabilities and Net Assets | \$5,992,070 |
|-------------------------------------|-------------|

STATEMENT OF ACTIVITIES 10/1/2016-9/30/2017

| REVENUE | |
|----------------------|--------------------|
| Contributions | \$472,986 |
| Grants | \$46,480 |
| Collaboration Funds | \$835,059 |
| Special Events | \$910,555 |
| Clinic Fees | \$243,344 |
| Investment Income | \$532,703 |
| Laboratory Fees | \$160,846 |
| In-Kind Donations | \$139,367 |
| Miscellaneous income | \$26,328 |
| Total Revenue | \$3,367,668 |

| EXPENSES & PROGRAM INVESTMENTS | |
|-----------------------------------|--------------------|
| Program Services | \$2,441,122 |
| Management | \$247,408 |
| Fundraising | \$93,297 |
| Total Expenses | \$2,781,827 |



Front (L-R): Ashlin Rodrigues, KaLynn Loeven, Kevin Strauss, Karlla Brigatti, Candace Kendig, Kim Calderwood
 Back (L-R): Erik Puffenberger, Christine Hendrickson, Keturah Beiler, Millie Young, Vincent Carson, Katie Williams, Donna Robinson, Yalonda Kosek, Teresa Rineer, Adam Heaps
 Not Pictured: Lavina King



Clinic for Special Children

Our Staff

Keturah Beiler, RN
 Nurse

Karlla Brigatti, MS, LCGC
 Research Operations Director and
 Genetic Counselor

Kim Calderwood, MA
 Communications Manager

Vincent Carson, MD
 Pediatric Neurologist

Adam D. Heaps, MS
 Executive Director

Christine Hendrickson, RNC
 Nurse

Candace Kendig
 Medical Receptionist

Lavina King
 Community Liaison

Yalonda L. Kosek
 Office Coordinator

KaLynn Loeven
 Laboratory Technician

Erik G. Puffenberger, PhD
 Laboratory Director

Teresa Rineer
 Development Director

Donna L. Robinson, CRNP
 Nurse Practitioner

Ashlin Rodrigues
 Laboratory Technician

Kevin A. Strauss, MD
 Medical Director

Katie B. Williams, MD, PhD
 Pediatrician

Millie Young, RNC
 Research Nurse

Board of Directors

Cindy Bo, MBA
 Chair-Charity Committee

Herman Bontrager
 Chairman

Richard Fluck, PhD
 Secretary, Chair-Development
 Committee

Leon Hoover

Leonard Hurst

Mark Martin
 Treasurer

Jacob Petersheim

Stephen D. Ratcliffe, MD, MSPH

Jacob Zook
 Vice-Chairman

535 Bunker Hill Road
 PO Box 128
 Strasburg, PA 17579

tel (717) 687-9407
 fax (717) 687-9237

clinicforspecialchildren.org