2020

ANNUAL REPORT

Clinic *for* Special Children®

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

COMPREHENSIVE MEDICAL SERVICE

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*

31 *years serving the community*

26 *staff members*

Hope *in action*

Friends,

As we reflect on 2020, we feel immense gratitude for your overwhelming support for the Clinic for Special Children (CSC) in what was a challenging year for many. You allowed us to continue serving the children and adults who rely on our services, giving hope when we all needed it most. For that we are grateful.

Each year CSC relies on hundreds of volunteers to make our auction season a success. Many of these folks have given their time and talent for decades, helping CSC raise over one million dollars each year to support our mission of caring for children and adults living with rare genetic illnesses. This year the pandemic disrupted the auctions, yet the auction committees pulled together and raised the most money in their history. We are deeply grateful for this essential support – and yours – and we look forward to getting back to the auction block in 2021.

Our work to care for patients is only possible because of the support of our committees, volunteers, donors, and collaborators. Through your generous support, we were able continue our core mission while growing our services. For instance, Cherished Lives, our pediatric palliative care program, grew from 6 to 30 patients as word of the service spread. Likewise, we welcomed a family medicine physician to our staff to meet the growing demand for adult services. All the while we continue cutting-edge research and clinical trials that allow our clinicians and the medical community at large to better understand and care for those who live with the rare genetic diseases we specialize in treating.

As we look to the future and whatever challenges and opportunities lie ahead of us, we feel heartened by your continued support of CSC's vital work. Together, we will meet the future with hope and action, knowing that one is not possible without the other.

Herman Bontrager
Chair of the Board

Adam D. Heaps, MS, MBA
Executive Director

Kevin A. Strauss, MD
Medical Director

Expanding our Scope of Care

Growing Adult Services at the Clinic

MICHAEL'S STORY

Many of the children who received treatment at the Clinic for Special Children (CSC) over the past 32 years are now adults who continue to require complex medical care. It can be difficult to find physicians familiar with a patient's particular genetic condition - a situation Michael Fondacaro knows all too well.

Michael was diagnosed with Glutaric Acidemia Type 1 (GA-1) when he was only eight months old. The doctor who diagnosed Michael had never seen children with GA-1 live beyond the age of three. GA-1 is a rare and potentially devastating metabolic disorder caused by changes in the *GCDH* gene. It also happens to be a condition CSC has specialized in treating for over 30 years because of its prevalence in the Amish and Mennonite communities.

After their initial conversation with Michael's physician, the Fondacaro family did their own research and found CSC. With years of research and successful clinical treatment of GA-1, CSC physicians were able to provide a brighter outlook for Michael and his family.

Michael, now 23 years old, visits CSC once a year to monitor his health. He has also generously participated in studies initiated by CSC researchers and clinicians that will ultimately allow the medical community to better understand and treat GA-1.

The ability to continue his medical care at CSC is priceless for Michael. "I've been going to the Clinic since I was a baby," he says, "and they know everything about me and GA-1 with all of the great research they've done over the years."

In 2019 CSC took an important first step toward its goal of providing comprehensive care for those living with rare and other complex medical conditions by hiring Dr. Grace Loudon, a physician specializing in family medicine. With Dr. Loudon's arrival, CSC will be well positioned to care for patients across their lifespan.

Dr. Loudon's arrival couldn't be timelier. Approximately 25% of the Clinic's 1,153 active patients are over 21 years old. This number will only continue to grow as CSC continues to make cutting-edge therapeutic discoveries that enable children to live into adulthood.

Those living with rare disease recognize the difficulty that comes along with finding a clinician who specializes in their condition. "I trust the Clinic with everything I have," says Michael in a recent interview. Growing the CSC team to care for adult patients means that Michael can continue placing his trust in clinicians who know him as a person and a patient for years to come.

PATIENT CARE *STATS*

1,153 *active patients*

from **42** *states &* **17** *countries*

3,632 *laboratory tests to help identify & manage genetic illnesses*

1,310 *patient visits*

"It means I don't have to find another genetic specialist to go to, which truly puts my mind at ease"

LANDMARK STUDIES PUBLISHED

Clinic for Special Children (CSC) clinicians and researchers published two landmark longitudinal studies in 2020 that will help the global medical community better understand and treat Glutaric Acidemia Type 1 (GA-1) and Maple Syrup Urine Disease (MSUD), two rare and potentially lethal genetic illnesses. Both GA-1 and MSUD are prevalent in Plain communities and have been central to CSC's work over the last three decades. Information gathered from patients over the past 30 years informed both natural history studies. This research helps clinicians understand how the diseases progress over time and also from a historical baseline through which clinicians can compare the efficacy of new therapies.

GA-1 is a rare and potentially devastating metabolic disorder caused by changes in the *GCDH* gene. If left untreated, most children suffer permanent and severe brain injury, leaving them mute, wheelchair bound, and often fully disabled. GA-1 is now identified on the state newborn screen so that affected children can be diagnosed early and treated with dietary therapy developed by CSC clinicians.

Before the advent of effective therapy and the inclusion of GA-1 on state newborn screening, 90% of children born with the disease would be fully disabled by a young age. Today, only 7% of children born with the genetic condition will experience severe disability if cared for with effective protocol created by the Clinic for Special Children.

Like GA-1, MSUD is also a rare and potentially life-threatening metabolic disorder caused by changes in one of three genes: *BCKDHA*, *BCKDHB*, *DBT*. Left untreated, one in three children born with MSUD will die from neurological complications of the disease before the age of 10. A majority of those who survive are permanently disabled. Again, like GA-1, MSUD now appears on state newborn screening panels so infants can be diagnosed and treated as quickly as possible. Through dietary protocols developed at CSC, survival rates for children living with MSUD have increased from 63% to 95% while hospitalization rates have lowered from 7 to just 0.25 hospital days per patient per year.

Both natural history studies appeared in the journal *Molecular Genetics and Metabolism* and feature data collected over a thirty-year study period. CSC clinicians and researchers are grateful to the 184 people with MSUD and the 168 people with GA-1 who willingly shared their time and experiences for these landmark studies. Because of them, clinicians caring for patients living with these rare genetic conditions around the world now have a resource for establishing safe and highly effective standard-of-care treatments.

READ OUR PUBLISHED PAPERS ONLINE AT CLINICFORSPECIALCHILDREN.ORG

Find 'Published Papers' under the "What We Do" tab

RESEARCH *STATS*

25 **NEW** *disease-causing genetic variants identified*

9 *peer-reviewed publications*

Philanthropy at the Clinic

The Clinic for Special Children has experienced tremendous growth over the past five years as we expand to meet the needs of our patients. Like many nonprofits, CSC was scared when the pandemic started because we rely on support from our community, particularly through our summer auction events. Aside from the financial support they bring to CSC, the summer auction season is a time for all of us to gather and enjoy one another's company. It was sad to think about missing this opportunity for fellowship.

This was truly a moment for hope and action. The CSC auction committees and staff sprang to work and came up with the idea of launching a direct mail campaign to offset the lost auction revenue. With the help of a mailing list from a local business publication, the auction committees were able to completely recoup the lost revenue from the cancelled events while introducing 2,379 new donors to CSC's work.

Our fall 5k committee was also forced to rethink their event this year. While it was disappointing that we couldn't gather at CSC for our annual race, going "virtual" meant we had runners from across the globe join us. Racers in Brazil, Hawaii, and Puerto Rico joined virtually with those in Lancaster County on September 19, 2020 to support CSC's mission.

We could not do this work without YOU. To our new donors: thank you, and welcome! We look forward to getting to know you better. To our long-time supporters: we're grateful for your unwavering support this past year. Thank you for standing with us. Your support helps us support those who need it most.

You are

HOPE IN ACTION

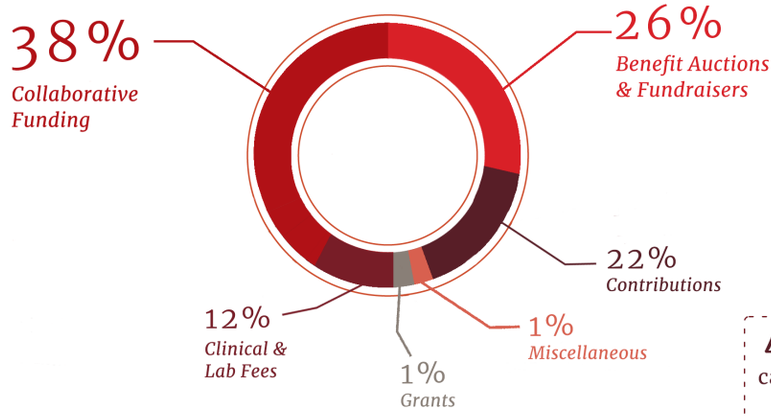
PHILANTHROPY *STATS*

2,379
NEW *donors in the 2020 fiscal year*

$400,000
increase in giving over previous fiscal year

FINANCIALS OVERVIEW

2020 Revenue Snapshot



49% of CSC's revenue came from philanthropic sources like benefit auctions, fundraisers, contributions, and grants.

The Clinic is funded from four major sources of revenue—annual benefit auctions and fundraisers, philanthropic donations, collaborative relationships, and patient fees.

Your support enables us to continue providing patients with timely, affordable, and effective care! We work hard to keep clinic fees to a minimum and effectively utilize funds to fulfill our mission.

2020 Expenses Snapshot

Community Engagement

2020 RARE IMPACT AWARD | NORD®

While 2020 was a year full of challenges, a bright moment for the Clinic was receiving a Rare Impact Award from the National Organization for Rare Disorders (NORD). Each year the Rare Impact Awards "celebrate the individuals, groups and companies making extraordinary contributions to the lives of rare disease patients and caregivers."

Our leadership team (Karlla Brigatti, MS, CGC, Research Operations Director; Vincent Carson, MD, Clinical Operations Director; Adam Heaps, MS, MBA, Executive Director; Erik Puffenberger, PhD, Laboratory Director; Emily Seitz, Development Director; Kevin Strauss, MD, Medical Director) was nominated for their accomplishments in leading our organization in fighting rare genetic illnesses with cutting-edge research and compassion everyday. The Clinic's work on the Plain Insight Panel™ (PIP) was highlighted as a recent, ground-breaking accomplishment. The PIP is an expanded carrier test that is able to determine carrier status for a wide variety of genetic conditions found in the Plain communities of North America.

The 2020 Rare Impact Award ceremony was originally scheduled for May 2020 at the Rock and Roll Hall of Fame in Cleveland, Ohio. After required COVID-19 event changes, our 2020 Rare Impact Award was accepted during a well-organized virtual ceremony in October 2020. Our Executive Director, Adam Heaps, MS, MBA, accepted the award on behalf of the Clinic's leadership team. As a dedicated team of pediatricians, clinicians, nurses, scientific researchers, administrative staff, and patient advocates, we share NORD's mission and focus on the identification, treatment, and cure of rare diseases. We are so grateful to the patients and supporters of the Clinic and to NORD for their invaluable work in the rare disease community. We were humbled to have received a 2020 Rare Impact Award!

2021 | FINANCIALS

STATEMENT OF FINANCIAL POSITION
As of 9/30/2020

ASSETS

Cash and Equivalents	$2,283,765
Accounts Receivable	$121,184
Promises to Give	$62,559
Prepaid Expenses	$61,375
Property and Equipment	$714,023
Investments	$4,364,900
Loans Receivable	$125,000
Investments Restricted	$1,116,380
Total Assets	$8,849,186

LIABILITIES & NET ASSETS

LIABILITIES	
Accounts Payable	$184,321
Accrued Expenses	$2,975
Accrued Wages	$75,561
Deferred Revenue	$5,000
Total Liabilities	$267,857
NET ASSETS	
Without donor restrictions	
Undesignated	$3,097,295
Board Designated	$4,336,260
With donor restrictions	$1,147,774
Total Net Assets	$8,581,329
Total Liabilities and Net Assets	$8,849,186

STATEMENT OF ACTIVITIES
10/1/2019 - 9/30/2020

REVENUE

Contributions	$839,376
Grants	$61,200
Collaboration Funds	$1,456,099
Special Events	$983,109
Clinic Fees	$257,666
Investment Income	$395,487
Laboratory Fees	$184,600
Government Grant	$403,227
Miscellaneous income	$27,548
Total Revenue	$4,608,312

EXPENSES & PROGRAM INVESTMENTS

Program Services	$3,288,298
Management	$449,621
Fundraising	$307,039
Total Expenses	$4,044,958

Clinic *for* Special Children®

Our Staff

Clinical Services

Keturah Beiler, RN
Nurse & Cherished Lives Program Manager

Taylor Brown, RN, CPN
Nurse

Vincent Carson, MD
Clinical Operations Director

Christine Hendrickson, RNC
Nurse

Caitlin Lavin, MS, CGC, MPH
Genetic Counselor

Grace Loudon, MD
Family Medicine Physician

Laura Poskitt, DO
Pediatrician

Stephen D. Ratcliffe, MD, MSPH
Senior Consulting Physician

Donna L. Robinson, CRNP
Nurse Practitioner

Kevin A. Strauss, MD
Medical Director

Research Operations

Karlla W. Brigatti, MS, CGC
Research Operations Director

Mariah Everett
Research Associate

Millie Young, RNC
Research Nurse

Laboratory

KaLynn Loeven
Laboratory Scientist

Erik G. Puffenberger, PhD
Laboratory Director

Ashlin Rodrigues
Laboratory Scientist

Development

Julia Martin
Development Associate

Emily Seitz
Development Director

Administration

Kelly Cullen
Communications Manager

Jennifer Giacoio
Medical Receptionist

Adam D. Heaps, MS, MBA
Executive Director

Candace Kendig
Office Manager

Lavina King
Community Liaison

William Van Ess, MS, CFE
Accounting Manager

Dawn Wade
Medical Receptionist

Board of Directors

Cindy Bo, MBA
Chair- Charity Committee
Secretary

Herman Bontrager
Chair of the Board

Peter Crino, MD, PhD

Elam Esh

Leon Hoover
Vice Chair

Leonard Hurst

Mark Martin
Treasurer

Jacob Petersheim

Stephen Tifft, MD

Glen Zimmerman
Chair- Development Committee

Jacob Zook

535 Bunker Hill Road
PO Box 128
Strasburg, PA 17579

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

ClinicforSpecialChildren.org