Clinic for Special Children

Newsletter

ISSUE #46 SPRING 2019

At the Forefront of NPRL3
The Newswanger Family

When Mabel Newswanger initially heard about the Clinic for Special Children, she never thought that her own child would require the Clinic's services. In November 2016, the Newswanger's suddenly noticed that their son, Randy, was having irrational fits of laughter a couple of times per day. Unbeknownst to the Newswanger's at the time, Randy was actually having seizures.

Just several months later in January of 2017, Mabel Newswanger got a call from Randy's teacher that he had a seizure during the school day. Later that night Randy had over 20 seizures, leading to a restless and prayer-filled night for the entire family. In the morning, Mabel called the Clinic for Special Children and went into the Clinic for an urgent appointment. Upon arriving and after an initial examination, Dr. Vincent Carson, Pediatric Neurologist at the Clinic, recommended that the family take Randy straight to Lancaster General Hospital. The following days at the hospital included an EEG, which confirmed seizure activity in Randy's brain.

Earlier in 2016, the Clinic team discovered through whole exome sequencing that a variant in the gene, NPRL3, was the cause of epilepsy in a Mennonite family from Ohio. Dr. Carson and Dr. Kevin A. Strauss, Medical Director at the Clinic, collected blood from Randy for rapid targeted genetic testing, which confirmed that Randy had NPRL3. "We used knowledge of the community to develop a targeted genetic test which allowed for us to quickly get to the root of the problem", Dr. Carson explained.

Once diagnosed Randy continued to have seizures until he was placed on Topiramate. Since March 2017, Randy has remained seizure-free. Today he is doing well on Zonisamide.

Dr. Carson is working together with Dr. Peter Crino's team from the University of Maryland to learn more about NPRL3 and epilepsy. If your family has a history of seizure activity, please feel free to contact Dr. Carson at the Clinic at 717-687-9407 to learn more about this study.

SPECIAL DATES

2019 Auction Season
June-September
Find all auction details inside!

Clinic's 30th Anniversary
Thursday, April 4
Happy 30th Anniversary to the Clinic!

30th Anniversary Celebration
Tuesday, April 16
Clinic for Special Children
3 p.m.- 7 p.m.
An open house event to commemorate the Clinic's 30th Anniversary!

Good Friday - Office Closed
Friday, April 19
All Day

The Great Train Robbery
Saturday, May 4
Strasburg Rail Road
www.StrasburgRailRoad.com
4 p.m.- 5 p.m. (Event donations support CSC)

Memorial Day - Office Closed
Monday, May 27
All Day

Clinic for Special Children 5k
Saturday, September 21
Mark your calendars and save the date for our 2nd annual 5k run/jog/walk! Details will be announced when available.

Call the office, check our Facebook page, or our website for event specific information and updates.

Clinic for Special Children
CELEBRATING 30 YEARS

Our 30th Anniversary!

Thursday, April 4

On this day in 1989, in order to provide local and affordable services to those suffering from rare genetic disorders, the local Amish and Mennonite populations with Dr. D. Holmes Morton and his wife Caroline, founded the non-profit Clinic for Special Children.

A year later in 1990, the original post-and-beam style building was raised largely by volunteers from the Plain communities. Since our founding and over the past 30 years, the Clinic has endeavored to integrate advanced laboratory techniques into every day primary pediatric care. We thank all of the friends of the Clinic, near and far, for their support over the last 30 years!

30th Anniversary Celebration Event

Tuesday, April 16
3:00 p.m.-7:00 p.m.
at the Clinic for Special Children

Join us for a FREE drop-in event at the Clinic commemorating our 30th Anniversary! Enjoy walking through the Clinic and seeing mementos from our 30 year history. There will be free "Taste of Lancaster County" appetizers, desserts, and refreshments, giveaways, activities for children, and limited-edition 30th anniversary items for sale. We look forward to seeing you at the Clinic to celebrate this great milestone in our history!

If you are interested in providing any in-kind or monetary donations to cover the cost of this event, please contact Julia Martin at jmartin@clinicforspecialchildren.org or 717-286-4288.

Web Shop Now Open

We now have an online store on our website where you can shop for Clinic mugs, postcards, prints, books and more! All proceeds benefit the Clinic for Special Children. You can choose to ship directly to your home or pick up at the Clinic during our operating hours. To visit our shop, go to ClinicforSpecialChildren.org/Store.

30th Anniversary Items For Sale

To commemorate our 30th Anniversary, we will have several limited edition items for sale, starting at our 30th Anniversary Celebration event! View details of these exclusive items below.

30 Stories for 30 Years Book | Pre-sale

This hardcover book includes thirty first-hand experiences from patient families throughout the Clinic's 30 year history. This book serves as a history of the Clinic through stories of those who the Clinic has served.

We thank all the families who participated to make this book a reality! These will be available for purchase at Clinic events throughout 2019, at the Clinic, and our online web shop.

Our Story in Newsletters Book | Pre-sale

This book includes three decades of Clinic newsletters featuring forewords by Donald B. Kraybill, PhD and Alan Shuldiner, MD! Read about the Clinic from its very beginning through our most recent Fall newsletter. You can read about new innovations over the years, research projects, events and more! These will be available for purchase at Clinic events throughout 2019 as well as our online web shop.

30th Anniversary Mug | $10.00

Enjoy a cup of coffee, tea, or hot cocoa in this mug! Add to your Clinic mug collection with this camper-like design mug that features a unique arrow quilt design and our limited-time 30th Anniversary logo! These are now available for purchase at Clinic events throughout 2019, at the Clinic, and on our web shop.

Fraktur Prints & Notecards

For our 30th Anniversary, we are running limited 8.5 x 11 prints of this year's fraktur design! Framed prints of the 30th Anniversary Fraktur will be auctioned off at each of our 2019 benefit auctions. Join us for an auction this season to bid on this exclusive Fraktur print!

We will also be selling notecard sets of 10 featuring the Fraktur design on the front and a letter from the artist, Lynn Sommer, on the back. These will be for sale at Clinic events throughout 2019, at the Clinic, and on our web shop.

Patient Family Advisory Council

The Patient Family Advisory Council (PFAC) is interested in new members to join! The PFAC is a collaborative group of patients, family members, clinicians, and staff members that meet every other month with the purpose of positively influencing organizational policy, programs, and practices of CSC. Members provide patient and family insights to help CSC recognize organizational strengths and opportunities in addition to organizing Patient Fall Family Fun Day, which now plans to operate every other year (next in 2020).

If you or your family has a patient history with CSC and is interested in learning more about this opportunity, please contact Kelly Cullen, Communications Manager at kcullen@clinicforspecialchildren.org or 717-687-9407.

Save the Date!
2nd Annual Clinic for Special Children 5k Run/Jog/Walk

Saturday, September 21
9:00 a.m. - 11:00 a.m.
at the Clinic for Special Children

We are excited to announce our 2nd annual Clinic for Special Children 5k! Last year our first race saw over 280 runners and raised over $24,000 for CSC! Registration will open in the Spring for the race- applications will be available at the Clinic and on the Clinic's website.

If you would like flyers to distribute/advertise the race in your area, please contact Kelly Cullen, Communications Manager by calling 717-687-9407. Stay tuned to the 'Events' page on our website and the summer newsletter for more details about this fun event!

Leola Benefit Auction Volunteers

We are looking for volunteers to help us with a new food station being offered at this year's Leola Benefit Auction on Saturday, June 15, 2019! We plan to have steamed shrimp available for purchase throughout the day for auction attendees.

If you are interested in volunteering at this new food station, please contact Adam Heaps at aheaps@clinicforspecialchildren.org or 717-687-9407.

2019 Auction Season Kick Off!

You're invited to join us during our 2019 Auction Season! This year we have six benefit auctions throughout Pennsylvania, Ohio, and Missouri! Each auction features a day of handmade and unique items for sale, delicious food, fellowship, and more!

These benefit auctions make up about 30% of the Clinic's annual operating budget, which allows the Clinic to continue providing life-saving care to those with rare genetic disorders. Please visit our auctions website (www.ClinicAuctions.org) to view the most current auction information for the 2019 season.

Mark your Calendars for the 2019 Benefit Auctions!

7:00 a.m. Breakfast | 8:30 a.m. Auctions Begin
Physician Remarks and Quilts to Follow

Union County Auction | 6.1.19

Flower Sale and Rib Dinner | 5.31 | 5-8 p.m.

Buffalo Valley Produce Auction
22 Violet Road
Mifflinburg, PA 17844
Contact: Leon Hoover | 570-966-2414

Lancaster County Auction | 6.15.19

Leola Produce Auction
135 Brethren Church Road
Leola, PA 17540
Contact: Mark Martin | 717-733-3070
NEW | Hot Air Balloon Rides!

Shippensburg Auction | 6.22.19

Cumberland Valley Produce Auction
101 Springfield Road
Shippensburg, PA 17257
Contact: Elvin Oberholtzer | 717-532-9088

Ohio Auction | 7.13.19

Blooming Grove Auction Inc.
1091 Free Road
Shiloh, OH 44878
Contact: Leon Newswanger | 419-896-3336

Missouri Auction | 8.17.19

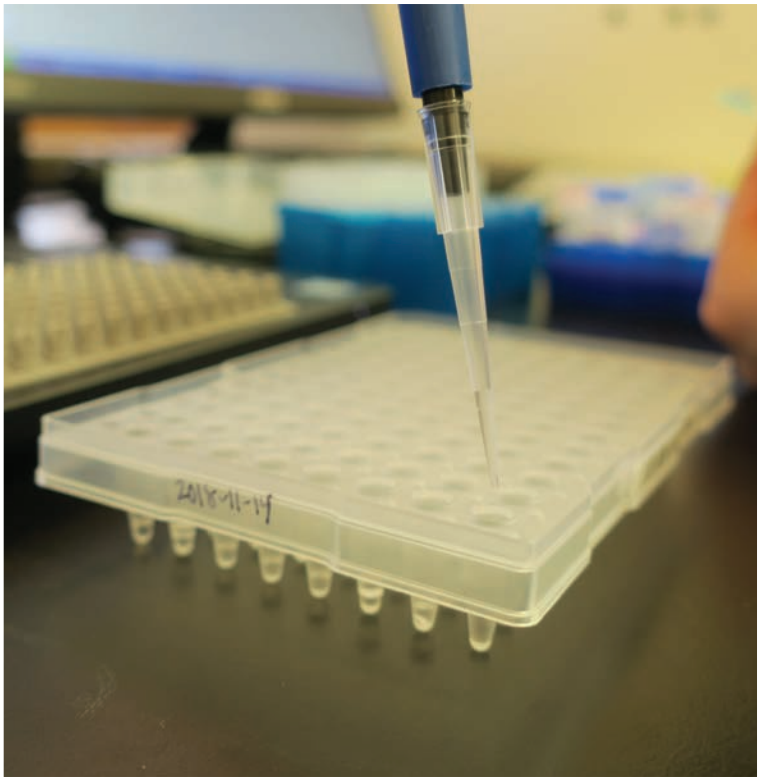
Ed's Machinery
17920 US Hwy 136
Memphis, MO 63555
Contact: Harlan Burkholder | 660-341-4113

Blair County Auction | 9.14.19

Morrison's Cove Produce
4826 Woodbury Pike
Roaring Spring, PA 16673
Contact: Mervin Z. Martin | 814-793-3529

"To me the Clinic has been more about an idea, than any individual. And it's a kind of idea that all of us can share and play an important part."

-Dr. Kevin A. Strauss, Medical Director



Plain Insight: Next Generation Sequencing Panel

New Laboratory Technology

Dr. Erik Puffenberger arrived at the Clinic for Special Children in Strasburg, PA, in January 1998 to run its analytical laboratory. Under Puffenberger's direction, more than 260 different gene mutations have been identified, enabling molecular diagnosis and therapy to begin the day a baby is born.

Along with a staff of two laboratory technicians, KaLynn Loeven and Ashlin Rodrigues, the Clinic runs thousands of genetic tests each year. While the Clinic's advanced capabilities include testing for specific genetic changes as well as larger scale testing like chromosomal microarray analysis, Dr. Puffenberger is currently focused on next generation sequencing: a new method for sequencing many genetic targets simultaneously at high speed and at low cost.

Dr. Puffenberger explained there are about 110 known genetic mutations in the Old Order Mennonite population. "In the past if I wanted to test for these 110 mutations, I would have had to run 110 different tests," Dr. Puffenberger said. "In a perfect world, we would like to detect all known mutations in a single test. This would be especially useful for population-wide carrier screening to identify those couples at risk for having a child with a genetic disease."

Next generation sequencing (NGS) allows for massively parallel sequencing of millions of fragments of DNA in one test. The Clinic team identified a list of genes that they wanted to screen for mutations, and designed a panel to sequence all the regions of interest and identify unusual changes in the DNA. The technology permits panels of variants, mutations, and/or genes to be screened in a rapid and economical manner.

After a collaborative meeting at the Clinic about 2 years ago, Dr. Puffenberger teamed up with Drs. Erin Crowgey and Anders Kolb from Nemours Alfred I. duPont Hospital for Children and ArcherDX (Boulder, CO) to design an NGS panel to perform carrier testing in the Plain populations of Lancaster, PA. Recently, Dr. Puffenberger, with help from the members of the Plain Community Health Consortium, created a version which includes more than 1,300 mutations found in Plain populations across North America. In October, the Clinic purchased the MiniSeq DNA sequencer produced by Illumina (market value $50,000) to run the tests at its on-site laboratory in Strasburg. "Having this capability in-house will greatly reduce overhead and reagent costs, and allow us to tweak the protocol so that we can run the test more cheaply and rapidly, passing on the cost savings to our patients," Dr. Puffenberger said.

While the Clinic has purchased the machine, there are of ongoing reagent costs to run the testing. Each assay test costs about $400-$500 to run. When the test is offered clinically this summer, the test will be offered at $99 due to the financial support of generous local foundations.

Collaborator Spotlight: Wellspan Philhaven Behavioral Health

Dr. Jennifer Hailey and Dr. John Dolena

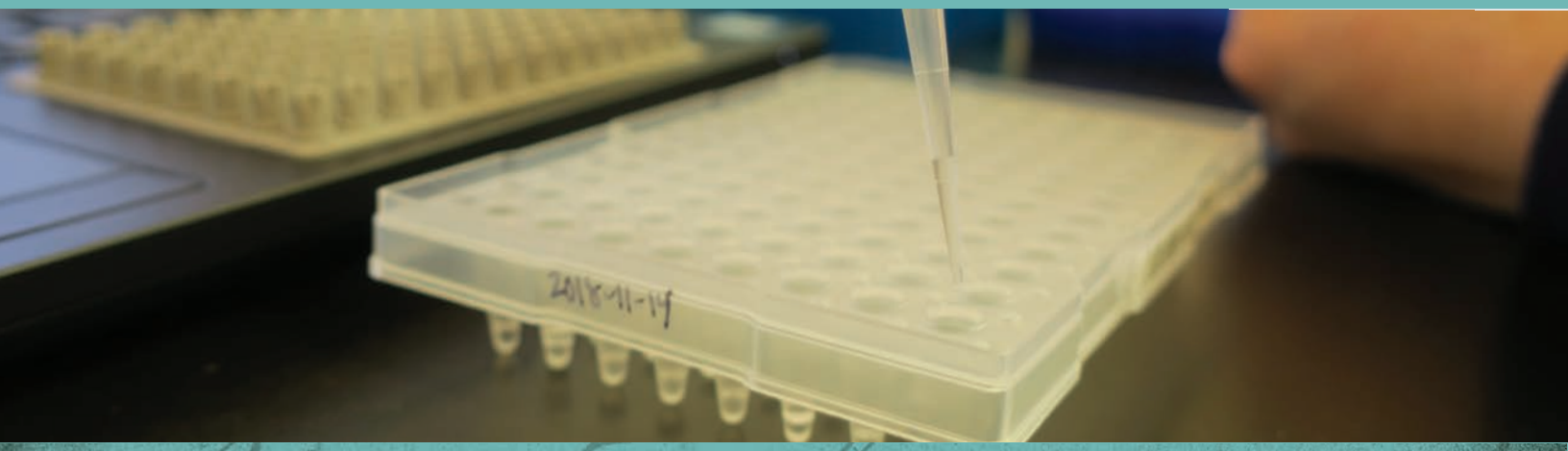
Dr. Jennifer Hailey clearly remembers the first time she heard about the Clinic for Special Children (CSC). In fact, it was her first day of work at Wellspan Philhaven Behavioral Health when Philhaven's Medical Director, Dr. Francis Sparrow, approached her about collaborating with CSC. Since that day in 2016, Dr. Hailey, along with her colleague Dr. John Dolena, visit CSC each month to deliver mental health care to our patients. Drs. Hailey and Dolena see patients for a wide variety of appointments at CSC including new evaluations, regular follow-ups, research testing, therapy sessions and more.

Dr. Hailey, a native of Houston, TX, originally planned to be an English Teacher and earned her Bachelor's Degree in English Literature from Baylor University in Waco, TX. It was during her training in education that she realized many of her students had needs related to their social, emotional, and family health that she couldn't address as a classroom teacher. She then decided to attend Wheaten College in Chicago and earned her Master's and Doctoral degrees in Psychology. When asked why she chose to study Psychology, Dr. Hailey explained, "I have always loved working with people, so even when I was a young girl I knew that I wanted to have a job someday that would allow me to help people. Throughout graduate school, I also realized that I enjoy thinking about the ways that medicine, psychology, and theology can all work together to help people."

Dr. Dolena has worked as a Psychiatrist at Wellspan Philhaven for the past 10 years. Originally from the Lehigh Valley, Dr. Dolena completed his Bachelor's Degree in Biology with a minor in French at La Salle University in Philadelphia, PA. After college, he worked several jobs, including at a group home for emotionally and behaviorally challenged boys in Phillipsburg, NJ. During this job, his interest in mental health was peaked. Dr. Dolena attended medical school at the Philadelphia College of Osteopathic Medicine and completed his two-year child and adolescent psychiatric fellowship at Penn State Hershey Medical Center.

Dr. Dolena explains that he typically has seen patients at CSC that "have been resistant to first line treatments offered by the CSC pediatricians or cases that seem to be particularly complex or challenging from a mental health standpoint." Common treatments include individual therapies (play, behavioral, or talk therapy) as well as family therapy, family and community supports, and when indicated, psychiatric medication. Dr. Hailey commonly sees CSC patients who have been referred to her by CSC physicians for concerns related to depression, anxiety, mood dysregulation, behavioral problems, difficulties with focus and attention, eating concerns, developmental delays, problems with communication or social skills, and/or difficulties adjusting to stressful life events. She explains, "I use different techniques to help children and parents understand each other better and to communicate with each other more effectively. When there are behavioral difficulties, I help parents understand the purposes of the behaviors and ways to increase desired behaviors. I also enjoy helping patients learn more about their emotions and the ways feelings are connected to our thoughts, actions and body."

Drs. Hailey and Dolena both express gratitude for the opportunity to work with CSC patients, medical providers, and researchers. Dr. Dolena also enjoys spreading awareness about mental health, "Mental illness, whether we like to admit it or not, still carries a lot of stigma. Coming to CSC offers me the opportunity to spread the word that it is OK to receive care for these struggles." Dr. Hailey shared that she enjoys meeting families, "The opportunity to be able to help families and develop relationships with special people through my work at CSC truly feels like a blessing to me." We send our gratitude to both Dr. Hailey and Dr. Dolena for their invaluable work with CSC patients and families!



What is Gene Replacement Therapy?

By Karlla W. Brigatti, MS, LCGC

In its nearly 30-year history, the Clinic for Special Children team has identified the genetic cause of many disorders that affect children and adults in many different ways. All people have around 20,000 genes, the individual recipes for making specific proteins necessary to carry out functions for proper growth and development. Most patients with genetic conditions have one of those genes either missing or not functioning properly, causing its specific protein to be made incorrectly, in too low an amount, or not at all. This imbalance gives rise to the disorder for which the patient is seeking medical care.

Understanding the genetic basis for these conditions has been a key element to finding new therapies and treatments to improve quality of life for these patients. Until recently, therapies targeted the symptoms or features of the disorders: for example, they replaced a missing vitamin, reduced intensity of symptoms, or prevented the buildup of certain toxic substances. However, a specific gene-based treatment that corrects the underlying genetic error has not been possible until recently.

Gene replacement therapy (GRT) has the goal to deliver a new working copy of the missing or nonworking gene. This new gene is meant to produce the proper protein and therefore correct the disease; in some cases, it can potentially prevent any symptoms of the disease from even appearing if it is given early in the disease process before damage can occur. As a concept, GRT has existed for as long as the genetic basis of disease has been known. In practice, though, it has been in development for a few decades now, but recent advances have made it a safe and effective option for genetic disorders like those found in Clinic patients.

How does GRT work to deliver a functional gene to the parts of the body that need it? The working copy of the gene is made in a laboratory and placed in a delivery system called a vector. The vectors used in most GRT programs are created by changing or modifying a naturally occurring virus. All viruses have the ability to enter the cells of the body and replicate by delivering their own genetic material into the cell, causing the cell to make new copies of that virus. This can cause people to get sick. In GRT, the viral genetic material is removed and replaced with the working copy of the gene of interest, as well as a promoter that instructs the cell to make a lot of the protein coded by that gene, thereby correcting the imbalance of that protein that leads to disease. Since the viral material is removed, the patient does not get sick. The modified virus is given to the patient as a one-time infusion or injection, and as it enters the cells of the body, it introduces the corrected gene to the cell where it can work appropriately. The vector is then broken down and shed from the body. That new gene does not integrate into the person's genetic fabric but works a bit like a genetic prosthetic, restoring function without altering the existing genetic makeup of that person.

One type of virus now used in quite a few GRT programs is the adeno-associated virus (known as an AAV virus). The virus is small and is not known to make people sick. Different types of AAV viruses target different tissues in the body, so the type of AAV selected to be the vector for the GRT is based on where in the body that new gene needs to work best, such as the nervous system, liver, heart, and muscle.

Currently, GRT is being studied around the world for a number of rare disorders. In 2017 the FDA approved GRT for a rare type of blindness, and several studies in other genetic conditions have been completed. The Clinic for Special Children is currently one of only a few sites around the world for two clinical trials in GRT: the first for patients with Crigler-Najjar syndrome (CN1), and the second for young babies with spinal muscular atrophy (SMA), who receive the gene replacement before they reach six weeks of age and before they show signs of the disease. We also know of many other genetic conditions we believe can be corrected with GRT, and are working on a pipeline to develop GRT options for patients in a host of different conditions. These efforts are in concert with other academic and industry collaborators in rare disease research and would not be possible without the interest and partnership from our patients and their families. Gene replacement therapy is an exciting new frontier in personalized and targeted treatments for rare genetic conditions, and the Clinic for Special Children is excited to explore this therapeutic avenue.

RECENTLY PUBLISHED PAPERS

Bowser LE, Young M, Wenger OK, Ammous Z, Brigatti KW, Carson VJ, Moser T, Deline J, Aoki K, Morlet T, Scott EM, Puffenberger EG, Robinson DL, Hendrickson C, Salvin J, Gottlieb S, Heaps AD, Tiemeyer M, Strauss KA. **Recessive GM3 synthase deficiency: Natural history, biochemistry, and therapeutic frontier.** Mol Genet Metab. 2019 Jan 21. pii: S1096-7192(18) 30764-9. doi: 10.1016/j.ymgme.2019.01.013.

Marie Morimoto, Helen Waller-Evans, Zineb Ammous, Xiaofei Song, Kevin A. Strauss, Davut Pehlivan, Claudia Gonzaga-Jauregui, Erik G. Puffenberger, Charles R. Holst, Ender Karaca, Karlla W. Brigatti, Emily Maguire, Zeynep H. Coban-Akdemir, Akiko Amagata, C. Christopher Lau, Xenia Chepa-Lotrea, Ellen Macnamara, Tulay Tos, Sedat Isikay, Michele Nehrebecky, John D. Overton, Matthew Klein, Thomas C. Markello, Jennifer E. Posey, David R. Adams, Emyr Lloyd-Evans, James R. Lupski, William A. Gahl, May Christine V. Malicdan. **Bi-allelic CCDC47 Variants Cause a Disorder Characterized by Woolly Hair, Liver Dysfunction, Dysmorphic Features, and Global Development Delay.** American Journal of Human Genetics. October 25,2018. doi: 10.1016/j.ajhg.2018.09.014.

Williams KB, Brigatti KW, Puffenberger EG, Gonzaga-Jauregui C, Griffin LB, Martinez ED, Wenger OK, Yoder M, Kandula VVR, Fox MD, Demczko MM, Poskitt L, Furuya KN, Reid JG, Overton JD, Baras A, Miles L, Radhakrishnan K, Carson VJ, Antonellis A, Jinks RN, Strauss KA. **Homozygosity for a mutation affecting the catalytic domain of tyrosyl-tRNA synthetase (YARS) causes multisystem disease.** Human Molecular Genetics 2018 Oct 9. doi: 10.1093/hmg/ddy344.

SMA Prevention Readiness Program

Carrier Testing Volunteers

The Clinic's Spinal Muscular Atrophy (SMA) Prevention Readiness Program was launched in 2018 with the goal of identifying adult individuals and couples who are carriers for this condition, to present therapeutic options to at-risk couples should they have an affected child, and to ensure that any newborn with SMA in the Plain community is diagnosed within the first few days of life, before the onset of symptoms.

SMA is a progressive genetic condition affecting the motor nerve cells (neurons) in the spinal cord and brainstem that affect movement, breathing, and eating. It is the most common genetic cause of infant death worldwide. One in every 50 people worldwide is a carrier for SMA and we estimate that 1 in 25 Mennonites is a carrier for SMA, making it one of the more common conditions in the Mennonite community. We don't know the carrier frequency in the Amish, but several babies with SMA have been diagnosed within Amish communities across the country. In the past several years, recent targeted therapies have changed the course of this condition and improved the quality of life for affected individuals everywhere.

The SMA Prevention Readiness Program is tasked with conducting genetic testing for SMA (at no charge!) for adults from the Plain community. In addition to the Clinic's staff members on the SMA Prevention Readiness Program team (Karlla W. Brigatti, Millie Young, and Lauren Bowser), we have a number of volunteers helping conduct at-home or community blood draws for genetic testing. So far, the team and volunteers have tested hundreds of individuals in the Plain community from various states around the country. Since carriers don't often have a family history of SMA, it is of upmost importance for our team to conduct carrier testing on many individuals, even if they already have healthy children and no affected relatives.

If you have phlebotomy training and are interested in volunteering (especially Lebanon County & southern Lancaster County), would like to learn more about the SMA Prevention Readiness Program, learn about new SMA therapies or are interested in arranging carrier testing, please contact Karlla W. Brigatti, Millie Young, or Lauren E. Bowser at the Clinic at 717-687-9407.

Our Staff

Keturah Beiler, RN
Nurse

Lauren Bowser
Research Fellow

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

Vincent Carson, MD
Pediatric Neurologist

Kelly Cullen
Communications Manager

Adam D. Heaps, MS, MBA
Executive Director

Christine Hendrickson, RNC
Nurse

Candace Kendig
Medical Receptionist

Lavina King
Community Liaison

Yalonda L. Kosek
Office Coordinator

KaLynn Loeven
Laboratory Technician

Julia Martin
Development Assistant

Erik G. Puffenberger, PhD
Laboratory Director

Donna L. Robinson, CRNP
Nurse Practitioner

Ashlin Rodrigues
Laboratory Technician

Emily Seitz
Scientific Grant Writer

Kevin A. Strauss, MD
Medical Director

Millie Young, RNC
Research Nurse

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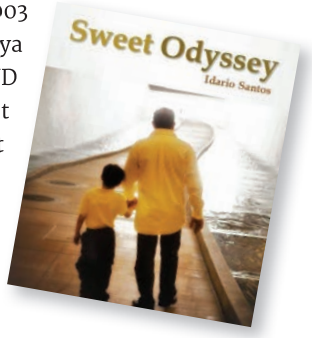
Donor Spotlight

Sweet Odyssey by Idario Santos

Idario Santos and Soraya Carvalho were overjoyed at the birth of their second son, Artur, in 2002. However, within several days of Artur's birth, his parents noticed that something was not right. After a brief stay at a local hospital, they moved Artur to a bigger city that had better medical resources. Thirty-one days and a couple misdiagnoses later, they found out their newborn son had Maple Syrup Urine Disease (MSUD).



Shortly after Artur's MSUD diagnosis, Idario met Dr. Strauss in 2003 in Natal, Rio Grande Do Norte, Brazil. The next year Idario and Soraya visited the USA to attend a MSUD support group and biannual MSUD symposium. Idario was shocked at seeing the children with MSUD that were Artur's age and how they were thriving. It was in that moment that Idario decided to meet with Dr. Strauss again at the Clinic for Special Children. After consulting with Dr. Strauss, the family decided the best solution for Artur would be a liver transplant at the Children's Hospital of Pittsburgh. The family moved to Pittsburgh from Brazil permanently in 2005. With his liver transplant, Artur became the first Brazilian to be cured of MSUD.

Since his son's treatment, Idario has been dedicated to supporting the Clinic. He's written a book about his journey entitled, A Sweet Odyssey. The book is available for purchase on Amazon and 50% of the proceeds benefit the Clinic. When asked why he's passionate about the Clinic, Idario says, "I'm passionate about the meaningful work that you're giving a chance to a family like mine. It's important for me to keep the work up and try to be a great supporter and advocate for the Clinic." Since writing the book, Idario and his eldest son, Vinicius, have spoken at events all over the world including a symposium in Finland hosted by Perkin Elmer. Numerous games were held throughout the event in Finland and all proceeds were donated to the Clinic at Idario's request.

Today Artur is doing great and is in 10th grade. He speaks three languages: English, Portuguese and Spanish. When asked if he'd like to share anything with our newsletter readers, Idario said, "I really wish that our story would become a movie because it would bring awareness to the whole world. Most people in the USA have no clue that people are dying for no reason." Idario recently wrote a bill in Artur's name to establish proper newborn screening, protocols and proper treatments in Brazil. The bill will be going to vote in the House on August 8th.

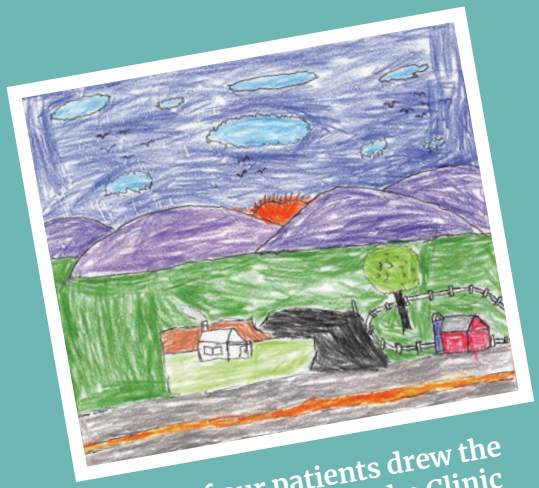
All of us at the Clinic thank Idario and his family for their tireless work in support of the Clinic for Special Children.

What's going on at the Clinic?

Dr. Kevin A. Strauss delivered a Keynote Address at the Hot Topics in Neonatology Conference in Washington, D.C.

Emily, a Clinical Scientist trainee from the UK, visited us for several weeks in January!

Dr. Erik G. Puffenberger gave a talk at the Rotary Club of Lancaster's first meeting of 2019!



One of our patients drew the landscape around the Clinic which features an Amish schoolhouse and farm!

The Spinal Muscular Atrophy (SMA) Prevention Readiness Team visited Ohio to conduct carrier testing on over 100 people.

The Clinic team's annual staff photo
(Not pictured: Lavina King and Donna Robinson)

Clinic *for* Special Children

535 Bunker Hill Road
PO Box 128
Strasburg, PA 17579

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

ClinicforSpecialChildren.org

2019 Benefit Auctions
for the Clinic for Special Children

June

1 Union County, PA

15 Lancaster County, PA

22 Shippensburg, PA

July

13 Shiloh, OH

August

17 Memphis, MO

September

14 Blair County, PA

The Clinic's Mission

"To serve children and adults who suffer from genetic and other complex medical disorders by providing comprehensive medical, laboratory, and consultive services, and by increasing and disseminating knowledge of science and medicine."