

Health Innovations

COLLABORATIVE
TRANSFORMATION IN PEDIATRIC
CANCER & BEYOND

by Emily Nikoo

The Nikoo Group, LLC | SEPTEMBER 2019

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Unnatural Hierarchy

*There is an unwritten hierarchy that exists here,
on the outdoor patio, in the recreation area,
in the hallways, on the elevators...*

*Its as if royalty has arrived.
The pathway clears, people stand aside,
offer their chairs, their shade,
their plug in the wall
for the IV pole to be connected.*

*An aura of reverence and respect takes over,
with a slight bow of everyone's head,
a hushed silence,
nervous energy building.*

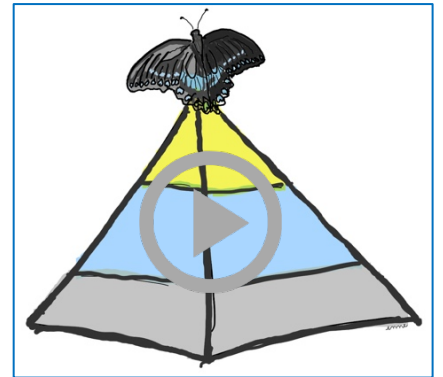
*But its not the nurses, or nurse practitioners, who have arrived.
Its not the doctors or surgeons that are causing such a stir.*

*It's a child,
hooked up to lifesaving IVs,
and their parent with exhausted eyes.*

*They are the absolute top of this unnatural pyramid-
the most feared; the most revered.
All of us would do anything we could to help at that moment,
to offer comfort and care,
but there's so little we can do.*

Or is there?

I wrote this sitting in Memorial Sloan Kettering Cancer Center, while my husband battled brain cancer. We spent months in the hospital, experiencing all forms of pain, and inspiration. Some of the hardest moments were seeing the children who were also battling the disease, with their family by their side. Pediatric cancer is heartbreaking. People shy away from it because it is so heartbreaking, feeling powerless to help. Surprisingly the government vastly underfunds it. But there are those who are helping, those on the front line of care in the clinic, researching in the lab and others raising money. All taking steps toward new ideas, new answers and new cures, making a difference and providing glimmers of hope for the future.



Unnatural Hierarchy

SEPTEMBER IS CHILDHOOD CANCER AWARENESS MONTH

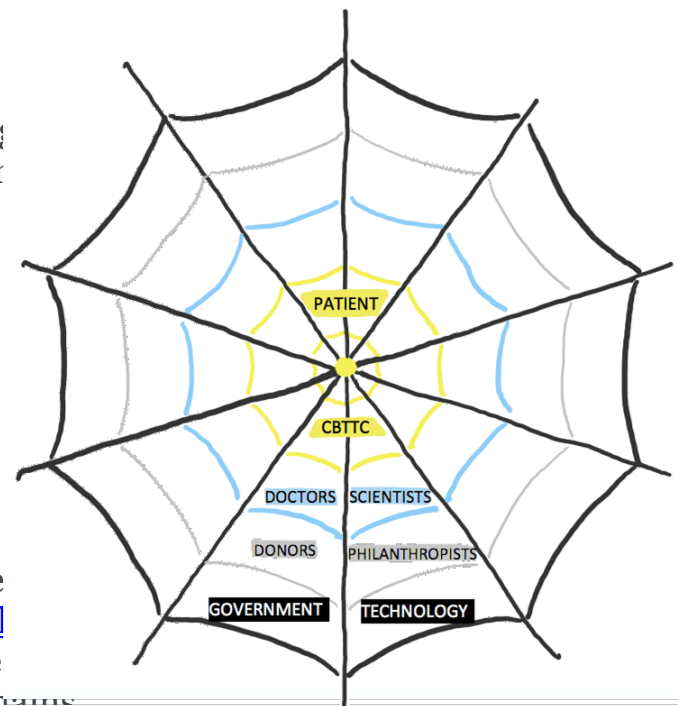
This is a success story, where philanthropy fueled a mission, creating a collaborative transformative approach in the heartbreaking world of pediatric cancer research:

- *Meet these amazing people with the vision who are making a difference- the Doctors, Scientists and Philanthropists*
- *Learn about their innovated methods and technology used to build a worldwide network accelerating breakthroughs*
- *Understand how their discoveries are providing time, and ultimately cures, not only in pediatric brain cancer treatments, but other cancers & for adults as well*

Making a Difference

I recently had the privilege to meet an amazing group of people who are doing just that, making a difference. They are an assembly of patients, parents, scientists, surgeons, doctors, philanthropists, technologists and ultimately the government. Each are a thread in the complicated web required to tackle pediatric cancer, all instrumental in their role.

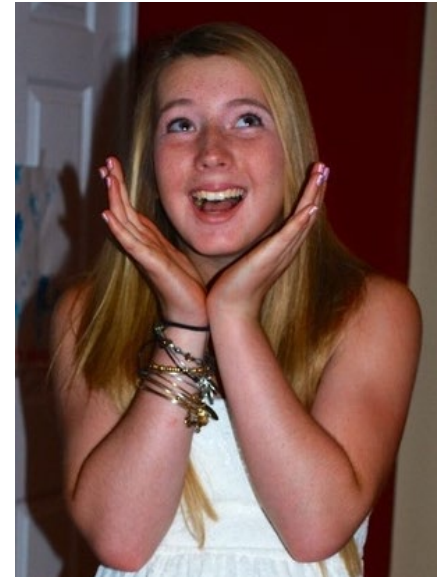
The group met to discuss the Children's Brain Tumor Tissue Consortium ([CBTTC](#)) operated by the Children's Hospital of Philadelphia (CHOP). The consortium, supported by [Dr. P. \(Jay\) Storm](#) and [Dr. Adam Resnick](#), started years ago¹ in 2009 when collaboration between domains of expertise (such as surgeons/oncologists and scientists) was limited, partnerships among health care organizations were uncommon, cloud computing was in its infancy and pediatric cancer funding from the government was scarce.



Pediatric Cancer Complicated Web

¹ Listen to [“BREAKING THROUGH with Madeline Bell”](#) Brain Tumor Awareness Month with Dr. Storm and Dr. Resnick

Day-to-day Dr. Storm and his team face patients and families who have little hope in the most critical of situations. He can remove every known speck of cancer, and still not be able save some of his little heroes. As he speaks of their challenges, his voice catches, his eyes moisten, and his bright face darkens. Sue Funck's daughter, [Hannah](#), was one of those heroes; a bright 14-year-old, an amazing soccer player with a keen wit and a big heart. This is a labor of love, and devastating pain.



Hannah Duffy

Pediatric brain cancer is rare, which is one reason why its difficult to attract government funding. Accordingly, no one hospital alone sees enough cases at a time to gather sufficient data. In addition, no one institution on their own could afford to build the infrastructure required to quickly process the tissues and their data. In order to accelerate new discoveries, they had to work together and they had to find the money to do so.

Working Together

The CHOP team began by funding a research scientist to work in collaboration with the clinical domain, that is when Dr. Resnick joined Dr. Storm. The team reached out to other hospitals with similar challenges and began the task to convince them to participate in a collaborative consortium.



Children's Hospital of Philadelphia

The consortium was created solely to benefit patients, to transform the research and accelerate discovery. All members are at equal parity of ownership and responsibility where every institution benefits in the same way, no one benefits more than the others. The goal was to “look for cures rather than credit”. But participation required institutional resources, each hospital would need funds to manage the effort. Therefore, the CHOP team tapped into the same resources that funded Dr. Resnick's work- **PHILANTHROPY**.

Believing in the Mission

When no other resources were available, there were those who believed. For the first 10 years of the consortium, over 50 [philanthropy organizations](#) like Sue Funck's [Hannah Duffy Foundation](#) entirely funded the vision. It grew from 4 to 18 institutions.

In the meantime, the philanthropic partnership became far more than writing a check. The families gained purpose and were invaluable to the mission and direction of the consortium's efforts. Even today the consortium is at least one-third completely philanthropic. Without these funds, the consortium would not be able to participate to this degree, translating efforts into new discoveries.

That is why we met that day, for Sue to present her annual donation (\$15,000) and for Andrea Verdone Gorsenger and Jim McCaffrey of [Infinite Love for Kids Fighting Cancer](#) to join in their efforts in memory of Hannah, donating \$25,000.

Collaboration extends to every facet of this complex solution.



CBTTC Biorepository

Phillip "Jay" Storm, MD, CHOP Chief of the Division of Neurosurgery,
Andrea Verdone Gorsenger, Infinite Love President/Founder,
and Jim McCaffrey Infinite Love Vice President

Network of Discovery

In parallel to the consortium's collaboration and scientific discoveries, technology advancements became an important piece of the puzzle². Cloud-based technologies emerged providing consumers like us with the ability to store and access our files and applications over the Internet from anywhere, on multiple devices like our phone and tablets in addition to our computers at home. For the consortium, the Cloud provided global on-demand computer resources that could be accessed, shared, and analyzed remotely rather than from one location. The Cloud fueled innovation of a different kind, unburdening the consortium from the investment and headaches of computing networking, allowing them to focus on their discoveries.

The consortium created “*Cavatica*”³ (named for the spider in the popular children's story Charlotte's Web) utilizing Amazon Web Services (AWS). *Cavatica* contains all of the genome data from the specimens in the CHOP biorepository after they are genomically sequenced, cell lines are developed, and the results thoroughly documented and shared. [Cavatica](#) grew to include big data analytics and provide an open infrastructure not only for the consortium members, but researchers everywhere, facilitating further community engagement.⁴



CAVATICA

Glimmers of Hope

As hospitals joined the consortium, and more tissues and data became available, it became possible for researchers to leverage the genomic data to find breakthroughs in precision medicine. The data became invaluable to better match treatments for specific pediatric cancer patients. It became clear that some drugs may be effective to treat certain cancers, but if the patient had an additional gene mutation, that same treatment could actually stimulate the

² [Seven Bridges Genomics Case Study](#) (on Amazon AWS)

³ [CHOP, Seven Bridges form Cavatica for pediatric genomics data](#)

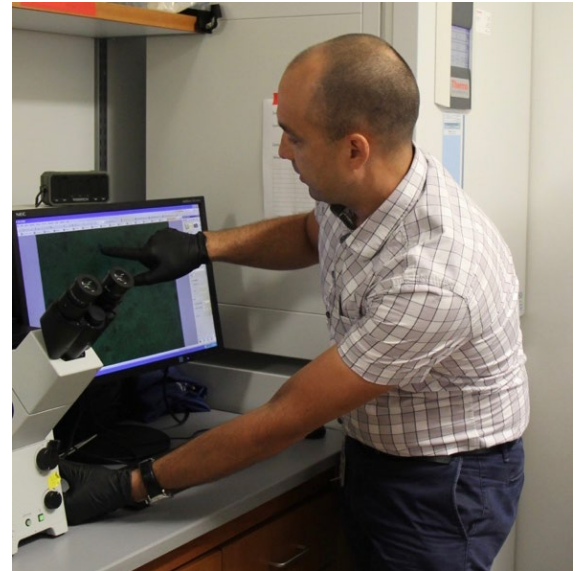
⁴ [CHOP's Resnick: Big data will transition from research to the standard of care in the clinic](#)

cancer. The researchers could identify not only what is the right drug for a particular patient, but also what may be the wrong drug. This dramatically changed the way the clinicians may treat patients in a precise, individualized manner.

Providing Time

This is a race against time, time the children and families don't have. Traditional methods took years to get from a specimen to useable genome and RNA sequencing and cell lines. Through the CBTTC, this project data is typically available within one-year following receipt of the specimens. The data is released real-time, with no embargo period.

Dr. Resnick speaks of a child in France where these results were translated into clinical care. By identifying the treatment that matched the child's gene mutation, the doctor was able to give her family more time; time that wouldn't be possible otherwise. Time is absolutely priceless, **every... single... day**. But cures are the mission, and long normal lives after treatment.



Genome & RNA Sequencing & Cell Lines
Mateusz Koptyra, PhD, CBTTC Lab Director

Platform for Cure

The CBTTC got past critical mass, it continued to partner and bring in additional data sets and samples from other institutions. Last year, the first dataset was released (the Brain Tumor Atlas, 9/10/18)⁵ which included 30 different types of pediatric brain tumors representing over 1000 subjects.

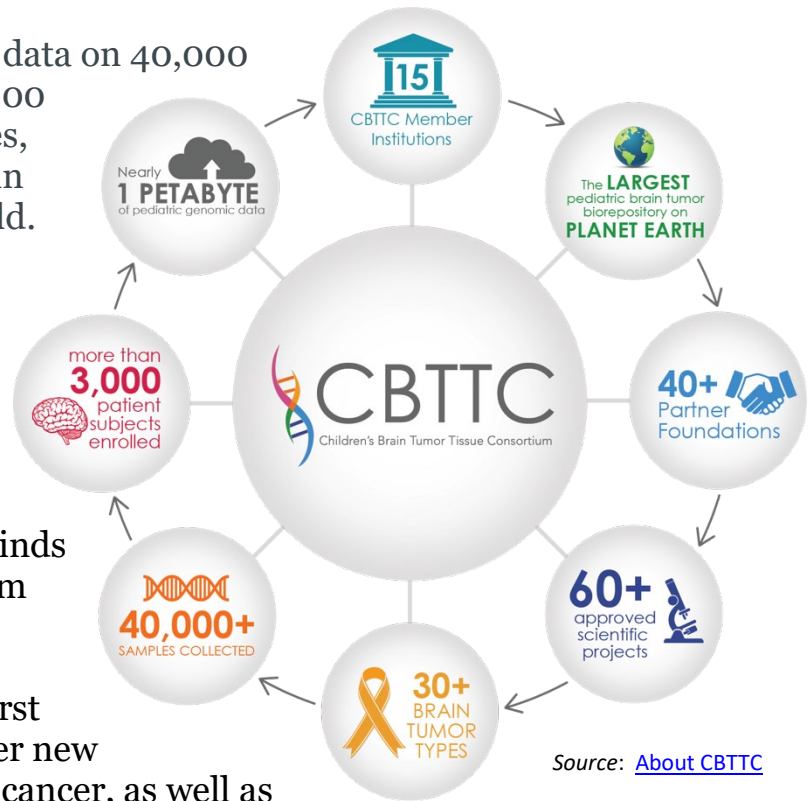
The open infrastructure platforms, shared discovery, and data sharing analytics attracted the attention of the National Institute of Health (NIH), and recent data science initiatives like the Cancer Moonshot. New funding came in the form of a 10-year Common Fund [Gabriella Miller Kids First Pediatric Research Program](#) and its large-scale Data Resource Center (DRC) for which CHOP is a prime recipient along with several key partners.

⁵ [Brain Tumor Atlas](#)

To date, the CBTTTC has now released data on 40,000 samples collected from more than 3,000 patients, for over 30 brain tumor types, from the world's largest pediatric brain tumor biorepository to the entire world.

Tomorrow's Breakthroughs Beyond Pediatric Brain Cancer

There are 12 different pediatric cancer types and over 100 subtypes. Because there are so many different kinds of childhood cancers, nearly all of them are considered 'rare,' but childhood cancer is a widespread, pervasive problem. The Gabriella Miller Kids First Program is to help researchers uncover new insights into the biology of childhood cancer, as well as structural birth defects. What the CBTTTC started with brain tumors, has now expanded not only to other pediatric cancers but other diseases focused on the discovery of shared genetic pathways between these disorders.



Research is finding that helping our children, will also help adults. Those mutations previously described, that helped the little girl in France? They can also be found in melanoma. Pre-clinical analysis and partners in new clinical trials are showing a very strong correlation in how to treat skin cancer from the brain tumor research.

It's a Long Road, We Need to Get There Faster

The statistics of pediatric cancer are overwhelming:

- 20% percent of pediatric cancers are still without a cure
- 43 children per day or 15,780 children per year are expected to be diagnosed with cancer (1 in 285 children)
- 7 children die a day due to cancer in the U.S.
- 2/3 of childhood cancer patients will have long-lasting chronic conditions from treatment
- Less than 4% of federal cancer research funding is devoted to kids

But these are just numbers. Numbers cannot convey the story of Sue and Hannah. They can't begin to describe Jim and Marian McCaffrey's little girl [Mia](#) with her beautiful crystal blue eyes, courageous and strong spirit, forever 6 after her 364 battle against rhabdomyosarcoma. Childhood cancer is the leading cause of death by disease among children in the U.S.; **the leading cause**.



Mia McCaffrey

What the CBTTC taught me is that there are answers, there are people with great ideas and visions, there are ways to accelerate on the path to solutions. But we have to go beyond conventional methods, we have to fuel innovative ideas and methods, and tap into unconventional resources. We aren't there yet, too many kids are dying, we can do better; we have to do better. Current cancer treatments are toxic with long term side effects; we have to do better. There is an overwhelming need for clinical trials to test what researchers have discovered, leveraging these precision-based approaches to ensure that we are not only curing, but also providing for a happy and long-lived normal life for a child.

We can do this by emulating what the CBTTC has done, work together. For those who also want to make a difference, donate to your philanthropic organizations who are fueling these discoveries and helping families who are fighting the battle. Better yet, donate your time and energy, just as Dr. Storm, Dr. Resnick, Sue, Andrea and Jim do **every... single... day**.

About CBTTC

The Children's Brain Tumor Tissue Consortium (CBTTC) is a collaborative, multi-institutional research program dedicated to the study and treatment of childhood brain tumors. The CBTTC supports the research of new prognostic biomarkers and therapies for children with pediatric brain tumors. As part of this research effort, the CBTTC has developed a network of informatics and data applications which allow researchers from across the world to work together to discover cures. "Innovation through collaboration" is made possible by the CBTTC's state-of-the-art biorepository as well as expertise of leaders in the field of biomedicine.



The CBTTC consists of 16 primary member institutions including:

1) Children's Hospital of Philadelphia, 2) Seattle Children's, 3) UPMC Children's Hospital of Pittsburgh and 4) Ann & Robert H. Lurie Children's Hospital of Chicago, 5) UCSF Benioff Children's Hospital, 6) Stanford University/Lucile Packard Children's Hospital, 7) Meyer Children's Hospital Florence Italy, 8) Weill Cornell Medicine Pediatric Brain and Spine Center, 9) Children's National Health System, 10) Joseph M. Sanzari Children's Hospital at Hackensack University Medical Center, 11) University of California Santa Cruz – Treehouse Childhood Cancer Initiative, 12) The Beijing Tiantan Hospital Neurosurgery Center (Beijing, China), 13) Genebank (Beijing Genomics Institute – Shenzhen, China), 14) Dayton Children's Hospital, 15) The Hudson Institute of Medical Research (Melbourne, Australia) and 16) Wake Forest Baptist Health – Brenner Children's Hospital.

The operations center of the CBTTC is located at Children's Hospital of Philadelphia and integrates genomic and molecular research, biorepository management and support for the informatics platforms of the CBTTC. This infrastructure serves an important role in the CBTTC's ability to provide free and open access to brain tumor data to researchers throughout the world."

From Hannah Duffy Foundation

The Hannah Duffy Foundation has been created in memory of Tinton Falls teenager Hannah Duffy, who lost her valiant battle with brain cancer on September 26th, 2013, at the age of 14. Our goal is to provide a lasting legacy through annual funding for cutting edge pediatric brain cancer research. I had the recent privilege to visit CHOP and present the CBTTC with a check for \$15,000 in memory of our beautiful Hannah. To everyone who has donated to our foundation, attended or volunteered at one of our events, this annual trip is made possible by your love and generosity. We continue to fulfill Hannah's two most important wishes...to never be forgotten and to continue to fight for a cure.



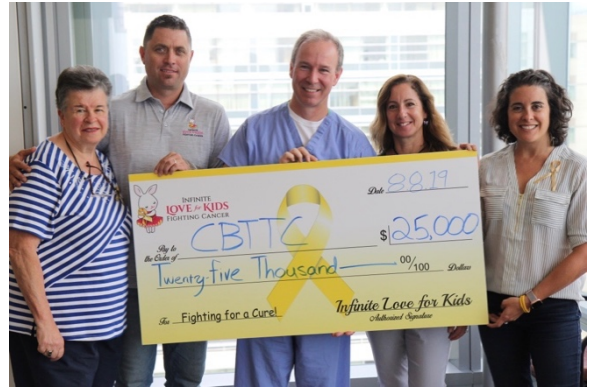
Long Time CBTTC & CHOP Support
Dr. Storm and Sue Funck, President/Founder of
The Hannah Duffy Foundation, Infinite Love
Board Member

From Infinite Love for Kids Fighting Cancer

Thanks to all of our incredible donors, we were able to present this check in the amount of \$25,000 to CHOP's CBTTC! What I love the most about the CBTTC is the simple fact that all egos are set aside to make room for anyone and everyone who wants to join in on the fight against pediatric brain cancers (ultimately contributing towards research discoveries for ALL other types of cancers).

When Dr. Resnick & Dr. Storm were in the planning stages of the CBTTC it was because they understood that "we" are stronger in numbers when it comes to research. The more hospitals (worldwide) who contribute their tissue samples and data discoveries, the faster and more efficiently breakthroughs can be found! Why have thousands of hospitals spent countless hours and millions of dollars all researching the same thing when they can share information and work TOGETHER towards a cure at a much faster rate?!

So THANK YOU to everyone who continues to donate to Infinite Love, to our many amazing sponsors and to our incredible Infinite Love team of volunteers who work so hard year round while asking for nothing in return!



Believing in the Mission

Linda Verdone Infinite Love Secretary, Jim McCaffrey, Dr. Storm, Sue Funck & Andrea Verdone Gorsenger

About this Series

The past decade has been an amazing time in technological innovation, but healthcare has been lagging far behind, until recently. This series highlights those technologies and companies in our region revolutionizing healthcare, making a difference in the lives of patients, and their doctors.

About the Author

Emily Nikoo is an innovative leader in technology, focused on patient experience. Her journey began in the space program and then cable television, media & entertainment (most recently at Comcast Business). After years of being a caregiver, Emily experienced firsthand how technology was lacking in the healthcare journey. To help drive innovative patient technology, and support nonprofit organizations in the community, she formed "[The Nikoo Group](#)", and joined the board of Infinite Love for Kids Fighting Cancer. Emily earned her Bachelor of Science in Electrical Engineering from Purdue University and Masters of Electrical Engineering at the University of Houston.



Infinite Love Team

Linda Verdone, Emily Nikoo, Jim McCaffrey, Andrea Verdone Gorsenger, & Sue Funck