2015 Executive Report

Solving Kids’ Cancer
Every Kid Deserves to Grow Up
Solving Kids’ Cancer is committed to significantly improving survivorship of the deadliest childhood cancers. 100% of all public donations are used to find, fund, and manage clinical trials and scientific programs to rapidly develop more effective and less toxic treatment options. Solving Kids’ Cancer is a 501(c)(3) public charity. To learn more about our work, please visit www.SolvingKidsCancer.org.
Changing the Future of Childhood Cancer Research

Solving Kids’ Cancer exists because children with aggressive forms of cancer need more effective treatment options now. Cancer remains the leading cause of death by disease for children in the United States. The reality for these children is that standard treatments such as chemotherapy, radiation, and surgery still don’t cure 50% of them.

Today, less than 5% of the exciting and potential ideas tested in the lab will ever reach children through clinical trials. Yet, when conventional treatment fails, children with cancer are left to rely solely on clinical trials for access to new and novel therapies. This is where Solving Kids’ Cancer is poised to fill the existing unmet needs — by turning preclinical innovations into new treatment options for children.

No parent should ever have to hear the shocking words that there’s nothing more to be done, no options left to save their child. Solving Kids’ Cancer is committed to significantly improving survival for children with the deadliest childhood cancers. Over the last eight years, Solving Kids’ Cancer has helped bring 19 clinical trials to children with 18 new treatment options made available. But there’s still more that must be done because Every Kid Deserves to Grow Up. With the rapid advances in research and the knowledge that scientists have gained to harness the power of the body’s own immune system, it is possible to cure childhood cancer.

But there is no magic bullet. What it will take is an integrated approach of Understanding the Research Landscape, and Strengthening the Science, which will lead to Curing and Caring for children with cancer. By expanding the menu of treatment options and innovations to benefit children who need it most, we can solve kids’ cancer sooner.

Scott Kennedy
Co-founder, Senior Director of Mission Programs
Solving Kids’ Cancer Appoints Bettina Bungay-Balwah As CEO

Bettina Bungay-Balwah, the Chief Executive of the Neuroblastoma Children’s Cancer Alliance UK (NCCA UK), has been appointed Executive Director/CEO of Solving Kids’ Cancer. She will oversee the merger of the NCCA UK as it becomes Solving Kids’ Cancer (Europe) and lead both organizations in the U.S. and in Europe in her new role.

Bettina will strengthen the profile, strategy and sustainability of the organization while pushing the agenda to further research for children with the most aggressive forms of cancer. She will be central in shaping the vision and goals for Solving Kids’ Cancer and preside over the organization’s core activities to fund research, provide access to treatment, and support education, awareness and parent services.

As the former Chief Executive of the NCCA UK, Bettina helped transform the charity from a $4 million charity to a $7 million charity in less than two years; she has played a key role in refocusing the strategic agenda on diversifying activity, strengthening governance and building the NCCA UK’s profile to attract partners, including Dell Inc., BBC, and Biosceptre.

Bettina grew up in Wimbledon, South London and studied biology at the University of Leeds, in Northern England. Before she joined the NCCA UK, she held a strategic management position at The Prince’s Foundation, one of the charities of His Royal Highness Prince Charles, where she led grassroots sustainable architectural and regeneration projects.

Bettina is a passionate advocate for children and families affected by cancer, actively reaching out to families, charities, and researchers to encourage international collaboration so that more children can survive the most aggressive forms of childhood cancer.
With a comprehensive understanding of the pediatric cancer research landscape, Solving Kids’ Cancer acts as an advocate to include the unique parent/patient perspective. Solving Kids’ Cancer seeks to identify areas of unmet need and strengthen the science by bringing a passion and a different perspective to the pursuit of developing better therapies for children with the deadliest childhood cancers. These novel and cutting-edge therapies include T-cell immunotherapies, oncolytic viruses, combination drugs, as well as enhanced delivery techniques. Since 2008, 13 partner charities have collaboratively funded 14 projects, which have led to new treatment options for children.

A common vision to find and fund the most innovative clinical research led the Pierce Phillips Charity to partner with Solving Kids’ Cancer in 2010. After their two-year-old son Pierce passed away in 2009, Scott and Brandi Phillips hoped they could create a lasting legacy in his honor and prevent another family from hearing the words no parents wants to hear: “There’s nothing else we can do.”

“We wanted to do the most good in the quickest way possible, and clinical trials are the best way to make that happen. The model Solving Kids’ Cancer is using looks at survivorship as a top priority, and so do we. Get the ideas out there, do the research, fund the trials and see what happens,” said Scott Phillips, Co-Founder of the Pierce Phillips Charity.

“We felt as though we wanted more from the research community, more from the doctors, and eventually more from the treatments, not just treatment to prolong life, but real, new, and novel approaches to really making survivorship a priority,” added Brandi Phillips, Co-Founder of the Pierce Phillips Charity.

“Solving Kids’ Cancer is crossing borders and helping to fund some of the most important and innovative work in the world. They are results-oriented and motivated by all the right things”
— Syd Birrell, The James Fund

Together, in just three years, the collaboration between Solving Kids’ Cancer and the Pierce Phillips Charity has created six new treatment options for children with neuroblastoma through clinical trials enrolling children at 12 institutions.

Solving Kids’ Cancer remains independent from any single hospital, cancer center, or researcher. This allows us to collaborate with key stakeholders in...
Highly reputable scientific advisory board and a panel of informed parents, including charity partners, who review research proposals with utmost scrutiny.

Focused funding specific to clinical trials — innovative treatments that can make the greatest immediate impact — rather than funding pre-clinical research.

Dedicated to prioritizing high impact studies with strong rationale for efficacy — not incremental advances.

Strict criteria for strong trial design with dedicated investigators who put the best interests of the child first.

Incentive-based structure that includes negotiating from concept to completion and awarding milestone payments tied to accomplishment of goals rather than lump-sum grants.

Budgets that mandate the inclusion of essential line items only and that require progress reports.

academia, industry, and government in order to fully understand the complexities of the global research landscape, influence research priorities, and objectively fund only the most promising projects. Charities trust Solving Kids’ Cancer to uncover the most promising opportunities that hold only the highest promise of real solutions for children who urgently need more effective options today.

Solving Kids’ Cancer is jumpstarting the creation of more effective solutions for children by commissioning scientists to submit their best clinical ideas and help solve kids’ cancer sooner. The ideas are graded and weighed by an expert panel of leading scientists, with input from parents who add the all-important patient/family perspective.

To date, Solving Kids’ Cancer has four projects with partners to create clinical trials that harness the power of new immunotherapy approaches in both neuroblastoma and brain tumors, as well as introducing new cutting-edge approaches to unresectable bulky disease in neuroblastoma.

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Charity Partners

- Make Some Noise
- TLC
- Ronan Thompson Foundation
- Fishin For The Cure
- Evan Foundation
- Two Sides Foundation
Being at the cutting-edge of science and discovery requires the knowledge of what’s already been explored, what’s currently being investigated and where medicine is going in the future.

By absorbing relevant publications and data, attending research conferences, consulting with game-changers in the field as well as talking to families about what’s working and what’s not working, Solving Kids’ Cancer knows and understands the research landscape. We exist to make the voices of children louder and clearer.

Patients with an in-depth knowledge of their own disease are increasingly steering the direction of cancer research. Known as research advocates, these activists have pushed cancer researchers to prioritize the needs of people with cancer right now and those yet to be diagnosed. These research advocates have played an instrumental role in putting the patient experience at the forefront of medicine, most notably in breast cancer and adult leukemias.

The majority of children with cancer, however, are unable to act as their own research advocates, leaving them without representation in the research landscape. It’s up to the parents, caregivers and organizations like Solving Kids’ Cancer to act as advocates on their behalf. Research advocacy in childhood cancer means lending a voice for children to question the direction of research and ensure that the science is truly putting children first.

Every research project considered for funding by Solving Kids’ Cancer and every new treatment option that’s developed is carefully scrutinized by parents who act as research advocates and by a robust scientific advisory board. Their aim is to ensure that Solving Kids’ Cancer lives up to its name.

The research funding model of Solving Kids’ Cancer is not a one-way street from donor to institution. Solving Kids’ Cancer expects a return on investment, which is why we question investigators about clinical trial design, negotiate potentially effective improvements, and scrutinize research budgets — all in an effort to move the pace of research forward for children.

Staying connected to a global community of parents who are seeking reliable information and viable options for their children is also a critical aspect of research advocacy. Through a network of childhood cancer organizations around the world, Solving Kids’ Cancer is able to learn about updates on new clinical trials, converse with experts in the field about the future direction of research, and connect families with resources for their child.

Every week, Program Directors Scott Kennedy and Donna Ludwinski field requests from families all over the world who are looking for information. This two-way conversation enables Solving Kids’ Cancer to provide ideas for exploring clinical trial options, as well as being cognizant of the challenges and struggles that parents face in accessing the best therapies for their children.
Daily Life of a Research Advocate

Digging to uncover the who-what-when-where-why-how of childhood cancer research takes enormous effort:

- Learning the basic science, biology, pharmacology, and immunology to understand mechanisms of drugs and properties of cancer
- Expanding networks and forming relationships with thought-leaders and experts in the field
- Cross checking facts, opinions, and ideas by quizzing competing experts
- Absorbing dozens of peer-reviewed research publications
- Attending the most important scientific meetings where new data is presented
- Participating in scientific workshops/groups, regulatory bodies, and nonprofit advocacy expert panels
- Keeping a finger on the pulse of the global parent community to understand gaps, unmet needs, and share information

CASE STUDY: T-cell Clinical Trial at Baylor College of Medicine/Texas Children’s Hospital

Solving Kids’ Cancer supports the development of the most innovative and promising new therapies for children who desperately need potentially curative options when fighting recurrent or resistant disease. Typically, early phase clinical trials take a year to plan, and up to two years to enroll patients. So what happens when new advances are discovered during this lengthy process to ensure that children are receiving the most cutting-edge therapies? Children with cancer can’t afford to wait until the trial is finished and a new trial is opened to reap the benefits of new and novel therapies.

As a research advocate, Solving Kids’ Cancer presses investigators to amend their trials to incorporate new strategies and improvements so that children can benefit immediately. The GD2 CAR (chimeric antigen receptor) trial now ongoing at Texas Children’s Hospital for children with neuroblastoma is one such example of pushing for change to put kids first.

Solving Kids’ Cancer supported the development of a third generation T-cell CAR that was enhanced in design for safety and efficacy. The trial opened for enrollment in September 2013 and since then has had four amendments, adding improvements based on new evidence from adult trials elsewhere. These trials proved that using fresh T-cells, depleting the interfering immune cells, and adding more infusions of the T-cells was better for patient response.

Working with the principal investigator Andras Heczey, MD, at Texas Children’s Hospital, Solving Kids’ Cancer encouraged and supported a fourth and most exciting amendment to include a recently approved humanized antibody that takes the “brakes” off the immune cells to efficiently kill cancer cells. The anti-PD1 antibody pembrolizumab, which has had a significant impact on improving the disease of adults with very deadly cancers, will now be given in combination with T-cell CARs as part of the amended trial design for children with the deadliest childhood cancers.

Bringing this new immunotherapy combination therapy to children is an incredible achievement and huge advancement, not only in childhood cancer research, but in cancer research. The experimental therapy has great potential to help children with neuroblastoma, in addition to helping adults with different types of cancer.
SO HOW HAVE WE DONE IN THE PAST EIGHT YEARS?

This snapshot shows just how far Solving Kids’ Cancer has come in making significant achievements for children with neuroblastoma, sarcomas and brain tumors.

26 PROJECTS HAVE BEEN FUNDED

7 PRE-CLINICAL PROJECTS

14 agents were validated for further use in combination with other drugs

19 CLINICAL TRIALS

4 out of 7 pre-clinical projects led to further study

18 new treatment options were brought to the clinic

6 new pathways for treatment were discovered or targeted for the first time
Project Title: Phase I Nifurtimox for Relapsed or Refractory Neuroblastoma

Institution: University of Vermont/ Vermont Cancer Center

Status: Completed

Summary: After a published case study found that Nifurtimox (a drug used to treat a parasitic disease) led to dramatic results in tumor reduction for children with neuroblastoma, Solving Kids’ Cancer quickly brought researchers together to launch a clinical trial using Nifurtimox.

Impact: The trial accrued rapidly, and the results were published in the January 2011 issue of the Journal of Pediatric Hematology Oncology. A multicenter phase II study was launched.

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Project Title: Phase II Nifurtimox for Refractory Neuroblastoma or Medulloblastoma

Institution: Van Andel Institute

Status: Ongoing

Summary: After the successful completion of the phase I trial, Solving Kids’ Cancer launched a phase II trial to test Nifurtimox in more children with neuroblastoma, and also in children with medulloblastoma, a deadly type of brain tumor.

Impact: The clinical trial is now open and enrolling children in 13 different centers across the U.S.

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Project Title: Preclinical Neuroblastoma Drug Discovery and Development Program

Institution: The Hospital for Sick Children, Toronto

Status: Completed

Summary: Researchers isolated neuroblastoma cancer stem cancer cells from patients, and screened hundreds of drugs against them. The best drug candidates were tested in animal models with tumors and the most promising agents to come out of these studies were proposed for testing in children.

Impact: An agent was identified as having activity against neuroblastoma stem cells. A drug called rapamycin (sirolimus) was selected for a phase I clinical trial at many cancer centers in the North America.
Project Title: **Phase I Vaccinia Virus JX594 for Relapsed/Refractory Neuroblastoma and Other Pediatric Solid Tumors**

**Institution:** Cincinnati Children’s Hospital, Texas Children’s Hospital  
**Status:** Completed  
**Summary:** Solving Kids’ Cancer initiated and funded the first trial using JX594, a virus strain derived from the same vaccine used to eradicate smallpox, for children with solid tumors. JX594 had been previously tested in adults with cancer showing beneficial results.  
**Impact:** One of the children experienced a reduction in tumor size and showed increased immune system activity as a result of the virus. The study results were published in *Molecular Therapy* in March 2015.

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Project Title: **Reduced Intensity Haploidentical Transplantation With NK Cell Infusion for Pediatric Acute Leukemia and High Risk Solid Tumors**

**Institution:** University of Wisconsin Madison  
**Status:** Ongoing  
**Summary:** Prior research has shown that patients have significantly better survival and a very low relapse risk if the donor’s natural killer (NK) cells are activated in the patient’s system. In this trial, NK cells from the donor-parent are given to the child after the haplo stem cell transplant.  
**Impact:** This was one of the first studies to use haploidential stem cell transplants in the United States. This is an important development as the approach has shown some success in sustained remissions after relapse in children treated in Germany.

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Project Title: **Preclinical Oncolytic Virus Development Program for Neuroblastoma**

**Institution:** Children’s Hospital of Eastern Ontario Research Institute  
**Status:** Completed  
**Summary:** Solving Kids’ Cancer developed a novel program to harness cutting-edge technology to identify promising viruses to kill cancer cells. Maraba MG1 was tested in neuroblastoma cell lines grown from patients, including isolated cancer stem cells (tumor initiating cells), which were identified in a previous research project by Solving Kids’ Cancer.  
**Impact:** A virus known as the Maraba virus was identified as the most likely to be effective in treating neuroblastoma.

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Project Title: **Phase I Vinblastine and Sirolimus in Pediatric Patients With Recurrent or Refractory Solid Tumors Including CNS Tumors**

**Institution:** The Hospital for Sick Children, Toronto  
**Status:** Completed  
**Summary:** A drug called sirolimus, or rapamycin, is currently approved for preventing rejection in organ transplant patients. Researchers discovered rapamycin was very effective in killing neuroblastoma cells, which led to a phase I clinical trial testing this drug with another cancer drug as a combination therapeutic option.  
**Impact:** The results were published in the January 2014 issue of *Pediatric Blood Cancer* and showed a partial response in one patient and stable disease in three children. The result of this study led other researchers to investigate the next generation of these inhibitors in other clinical trials.

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Project Title: **Dose Escalation Study of Intratumoral Herpes Simplex Virus1 Mutant HSV1716 Oncolytic Virus in Patients With Non-CNS Solid Tumors**

**Institution:** Cincinnati Children’s Hospital  
**Status:** Ongoing  
**Summary:** HSV1716 (Seprehvir) is a “first in class” engineered oncolytic virus derived from the herpes simplex virus, and has been beneficial for treating cancer patients in Europe in early trials. Solving Kids’ Cancer brought this cutting-edge research to the U.S. and developed the first clinical trial for children in 2010.  
**Impact:** This trial has been amended to include intravenous administration in addition to intratumoral route, which increases the potential for efficacy.
**Project Title: Preclinical Drug Development Program for Neuroblastoma Stem Cells**

**Institution:** The Hospital for Sick Children, Toronto  
**Status:** Completed  
**Summary:** Researchers designed a method to isolate populations of neuroblastoma cells with the properties of cancer stem cells. The goal of this project was to identify good targets and drug candidates to best kill these cells. Several new drugs are currently under evaluation for use in future clinical trials.  
**Impact:** The results of the study were published in the February 2011 issue of *Cancer Research* and confirmed that the PI3K1 target identified and supports the rationale for further research using these agents in children with neuroblastoma.

**Project Title: CHK1 Inhibition as Therapeutic Strategy for Children With Medulloblastoma and Neuroblastoma**

**Institution:** Children’s Hospital of Philadelphia  
**Status:** Completed  
**Summary:** Drugs called CHK1 inhibitors are able to sensitize cancer cells to chemotherapy and radiation. These are currently being used in clinical trials for other cancers and Solving Kids’ Cancer created this research project to test CHK1 inhibitors in medulloblastoma and neuroblastoma.  
**Impact:** Evidence from this preclinical work provided the rationale for a phase I clinical trial to open, and is now ongoing.

**Project Title: Phase I Image-Guided Convection-Enhanced Delivery of 124I-8H9 Monoclonal Antibody for Diffuse Intrinsic Pontine Glioma in Children**

**Institution:** Memorial Sloan-Kettering Cancer Center  
**Status:** Ongoing  
**Summary:** In this study, children with a deadly brain tumor that cannot be surgically removed are treated with an antibody connected to a radioactive isotope that is delivered directly to the tumor in the brain stem through a novel technique.  
**Impact:** This trial has shown excellent accrual and surprisingly no children have shown progression while on the study. At the higher dose levels a better response trend has been seen, and one child has been stable for more than two years. There have been no dose limiting toxicities or surgical complications.

**Project Title: Phase I Combining Decitabine and Vaccine Therapy for Patients With Relapsed Neuroblastoma and Sarcoma**

**Institution:** Kosair Hospital  
**Status:** Ongoing  
**Summary:** A child’s immune cells, called dendritic cells, are collected and isolated and “pulsed” with CT (cancer testes) antigens. This technique helps “prime” the immune system to kill cells with those CT targets when reinfused into the patient’s immune system in multiple doses.  
**Impact:** The first child treated had a complete response (published in the journal *Pediatrics* in January 2013) and there were three other positive responses from children with neuroblastoma. The trial has benefited by adaptive design, allowing amendments to improve the study, which now includes lymphodepletion and an immune adjuvant.
Project Title: Pilot Study of Imiquimod and Tumor Lysate Vaccine Immunotherapy for Diffuse Intrinsic Pontine Glioma (DIPG) in Children and Young Adults
Institution: University of Minnesota
Status: Ongoing
Summary: Solving Kids’ Cancer identified a novel trial using a cancer vaccine modeled after one that has been used in adults for a deadly brain tumor called glioblastoma. The vaccine has been combined with the drug imiquimod, which enhances the response of the immune system to attack and kill cancer cells.
Impact: The children treated on study thus far have shown no toxicity and no progression while on the study.

Project Title: Improving GD2 T Cell Immunotherapy for Patients With Neuroblastoma
Institution: Baylor College of Medicine
Status: Completed
Summary: Scientists created artificial T cell receptors, called chimeric antigen receptors (CARs), to recognize the GD2 antigen on neuroblastoma cells. Solving Kids’ Cancer supported work to develop a new and improved type of GD2 CAR T cells that make the cells perform and persist better to kill cancer cells.
Impact: The development and validation work was completed quickly, met the stated goals and led to a new phase I clinical trial.

Project Title: Adoptive Cell Therapy for Adolescent/Pediatric Solid Tumors: Part I
Institution: National Cancer Institute Pediatric Oncology Branch
Status: Completed
Summary: Researchers are optimizing the activity of a CAR (chimeric antigen receptor) that targets GD2, an antigen on neuroblastoma cell by incorporating the chemokine receptor CXCR2 so that it will travel to the tumor site more efficiently. Children with rhabdomyosarcoma, osteosarcoma, and Ewing’s sarcoma have shown an immune response to this modified approach.
Impact: The preclinical work was completed quickly and resulted in the discovery of two new targets for cellular therapy in pediatric tumors.

Project Title: Phase I hu14.18-IL2 + KIR Ligand Mismatch Natural Killer Cells
Institution: University of Wisconsin Madison
Status: Ongoing
Summary: Scientists use a novel technique to collect, expand, and infuse a parent’s donor NK (Natural Killer) cells into a child. In this trial researchers use a humanized monoclonal antibody known as hu14.18-IL2, which specifically targets neuroblastoma tumor cells and the IL2 stimulates the NK cells in the tumor microenvironment.
Impact: The trial will provide access to a new treatment option with a potential to cure and have very low toxicity.

Project Title: Phase I Highly Active Cell Therapy for Neuroblastoma - RNA-transfected T Cell GD2 CAR
Institution: Children’s Hospital of Philadelphia, University of Utah
Status: Ongoing
Summary: Solving Kids’ Cancer worked to initiate a clinical trial using CAR T cell therapy targeting the GD2 tumor antigen, which is on the surface of the neuroblastoma cancer cells, that includes transient modification for enhanced safety. The new GD2 T cell CAR is expected to be a better way of treating relapsed and refractory neuroblastoma.
Impact: Solving Kids’ Cancer recognized an unmet need and called for the research community to submit ideas of intent to meet the need for improved immunotherapy. This trial is being conducted by a world-renowned team of scientists.
**Project Title: Pilot Study Peptide-based Vaccination for Recurrent Ependymomas**

**Institution:** Children’s Hospital of Pittsburgh  
**Status:** Ongoing  
**Summary:** Solving Kid’s Cancer supported this pilot study, which uses a combination treatment approach of vaccines and drugs to stimulate a child’s immune system. This cutting-edge research represents the first immunotherapy trial ever for kids with ependymomas, a deadly brain tumor.  
**Impact:** Three children have enrolled in the trial and one showed stable disease for more than 12 months, with no toxicity.

**Project Title: Pilot Study Intra-Arterial Chemotherapy for the Treatment of Progressive Diffuse Intrinsic Pontine Gliomas**

**Institution:** John Hopkins University Hospital  
**Status:** Ongoing  
**Summary:** The study delivers chemotherapy directly into the vertebrobasilar system, which represents a novel way to treat the disease by targeting the blood supply to these tumors. This unique method allows doctors to increase the dose of the drug to kill the cancer, while minimizing toxicity.  
**Impact:** Two children have enrolled in the study, which is showing that drug delivery is feasible and there is rationale for trying new agents with this delivery system to improve outcomes in this deadly tumor.

**Project Title: Preclinical Development of an Anti-ALK Antibody for Neuroblastoma**

**Institution:** Children’s Hospital of Philadelphia  
**Status:** Ongoing  
**Summary:** Researchers believe that a new antibody which targets the surface antigen ALK on neuroblastoma will alert the immune system to attack the cancer with few side effects in normal tissue. This research is to develop, test, and produce clinical grade anti-ALK antibody for future testing in children.  
**Impact:** This research has identified a drug antibody conjugate, which will target ALK expressing neuroblastoma with a cytotoxic payload. This is an original and innovative approach and will be available in a clinical trial in 2016.

**Project Title: Phase I/II MK1775 Wee1 Inhibition + Irinotecan for Medulloblastoma and Neuroblastoma**

**Institution:** Children’s Hospital of Philadelphia  
**Status:** Ongoing  
**Summary:** In this research project, a combination drug treatment that uses chemotherapy with inhibitors of DNA repair proteins (CHK1 or Wee1) causes cancer cells to become more sensitive to cytoxicity. This new treatment is being tested in children with medulloblastoma and resistant neuroblastoma.  
**Impact:** This trial is available in the Children’s Oncology Group phase I consortium centers and is accruing well. If results show activity, this may advance to frontline therapy for newly diagnosed children.

**Project Title: Phase I Activated T Cells Transduced With a 3rd Generation GD2 CAR and iCaspase9 Suicide Safety Switch for Neuroblastoma**

**Institution:** Baylor College of Medicine  
**Status:** Ongoing  
**Summary:** Solving Kids’ Cancer supported the preclinical work that led to this current clinical trial using a CAR T cell therapy approach with the goal of bringing the early successes in blood cancers to children with neuroblastoma and other solid tumors.  
**Impact:** The study is designed to be flexible to incorporate advancements immediately, and now includes a breakthrough amendment adding a PD1 antibody (checkpoint blockade), which is a first-in-human combination and is expected to dramatically increase the potential for efficacy.
Project Title: *Nepenthe (Match NB) - Phase I Trial Matching Targeted Agents Based on Next-Generation Sequencing*

**Institution:** Children’s Hospital of Philadelphia  
**Status:** Opening in 2015  
**Summary:** By understanding the genetic features of a tumor, researchers are able to use specific drugs that interfere with certain cell processes that cause unrestricted growth. Although researchers have discovered a few specific genetic abnormalities for neuroblastoma, a few potentially effective investigational drugs have been identified that may produce dramatic responses in children with chemoresistant disease.  
**Impact:** This is the first precision medicine trial for children that will robustly analyze the genomics and epigenomics of cancer and use combinations of investigational drugs to target specific mutations and alterations in the tumors.

Project Title: *Pilot Study of High Intensity Focused Ultrasound (HIFU) for Unresectable Neuroblastoma*

**Institution:** The Hospital for Sick Children, Toronto  
**Status:** Opening in 2015  
**Summary:** This clinical trial uses a new technology for childhood cancer called “High Intensity Focused Ultrasound guided by Magnetic Resonance.” This next generation experimental therapy destroys tumor tissue with targeted rapid temperature elevation, while leaving adjacent tissue and organs completely unaffected.  
**Impact:** This clinical trial will be the first time this noninvasive new technology will be tried in children with abdominal tumors that cannot be surgically removed.

Project Title: *Phase I Anti-PD1 Nivolumab in Children With Brain Tumors*

**Institution:** Memorial Sloan-Kettering Cancer Center; Johns Hopkins University Hospital  
**Status:** Opening in 2015  
**Summary:** Recent results from clinical trials in adults with various tumor types have shown exciting results. Nivolumab has not yet been studied in any children with brain tumors. This phase I trial will study the safety of nivolumab in children with recurrent brain tumors and then perform another phase I study of the combination of ipilimumab and nivolumab.  
**Impact:** This trial is the first time this exciting immunotherapy approach is being used in children with brain tumors.

Project Title: *Phase I Activated T Cells Armed With GD2 Bispecific Antibody in Children and Young Adults With Neuroblastoma and Osteosarcoma*

**Institution:** Karmanos Cancer Center; Memorial Sloan-Kettering Cancer Center  
**Status:** Ongoing  
**Summary:** This trial studies the side effects and best dose of activated T cells armed with GD2 bispecific antibody and how well they work in treating patients with neuroblastoma, osteosarcoma, and other GD2+ solid tumors.  
**Impact:** This study will provide new information on whether infusions of these activated T cells will not only kill the tumor but also “vaccinate” the patients against their own cancer resulting in significant improvement in survival for patients with relapsed GD2+ tumors.
Above and Beyond: A Caring Community

Solving Kids’ Cancer holds events throughout the year to raise awareness and funds for childhood cancer research. We can’t solve kids’ cancer alone. Through the support of generous individuals and companies who align with our mission and vision, Solving Kids’ Cancer is able to further the vital work of improving the lives of children battling cancer. These ambassadors for change have gone above and beyond to help put the spotlight on childhood cancer through events, both big and small. Whether it’s running a marathon, hosting a bake sale, organizing a shop-for-a-cause event, or supporting our annual Spring Celebration gala, each and every dollar that has been raised has made a big difference to Solving Kids’ Cancer.
Board of Directors

**John London**  
Co-founder, Chair  
John London co-founded Solving Kids’ Cancer in honor of his daughter Penelope, who once told him “I want no one to feel yucky Daddy.” He is Board Chair of Solving Kids’ Cancer and serves as a strategic advisor in all areas of therapeutic development. John is a Portfolio Manager at Hudson Bay Capital. He has an MBA from The Wharton School of Business, University of Pennsylvania and a Bachelor of Arts from Brown University.

**Scott Kennedy**  
Co-founder  
Scott Kennedy co-founded Solving Kids’ Cancer as a tribute to his son Hazen, who was diagnosed with neuroblastoma at age 3. As Senior Director of Mission Programs, Scott is integrally involved in the identification, funding, and management of the organization’s scientific programs. He has a BS in Chemistry and Psychology from Indiana University and an MBA from the Asian Institute of Management.

**Khalil Barrage**  
Khalil Barrage is a Managing Director based in New York. He joined Invus in 2003 and set up the Public Equity group, of which he is in charge globally. Prior to joining Invus, Khalil was a portfolio manager with The Olayan Group, New York in charge of its U.S. equity group. Khalil holds a BA from the American University of Beirut. He is a board member of the Children of Armenia Fund.

**Catherine London, JD**  
Catherine London co-founded Solving Kids’ Cancer in honor of her daughter Penelope with her husband John London. An author and writer, Catherine was previously a prosecutor at the New York County District Attorney’s Office. She graduated from New York Law School and has a BA from Brown University.

**Mark Savoye**  
Mark N. Savoye is a Vice President and Senior Business Leader, Emerging Payments at MasterCard. He joined MasterCard in 2008; prior to joining MasterCard, Mark served as a Director at American Express. Mark holds a Bachelor of Science degree from New York University Stern School of Business in Marketing and International Business.

**Ari Spar**  
Ari Spar is a Managing Director at Barclays Capital with more than 20 years of experience as a financial advisor. Ari was previously a Managing Director with Lehman Brothers. He graduated from the University of Michigan.

**Channing Stave, PhD**  
Secretary  
Channing Stave is Executive Vice President and COO of Disruptyx, a boutique market consulting firm with a unique heuristics-based focus in life sciences, financial services, and retail. Prior to co-founding Disruptyx, he held leadership positions at IBM, Pfizer, and Express Scripts. He has a PhD from New York University in Organizational Psychology and a BA from Columbia University. He is a founding board member of Solving Kids’ Cancer and currently serves as Secretary. He is also an executive advisor to Baruch’s Zicklin School of Business Consulting Organization.

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