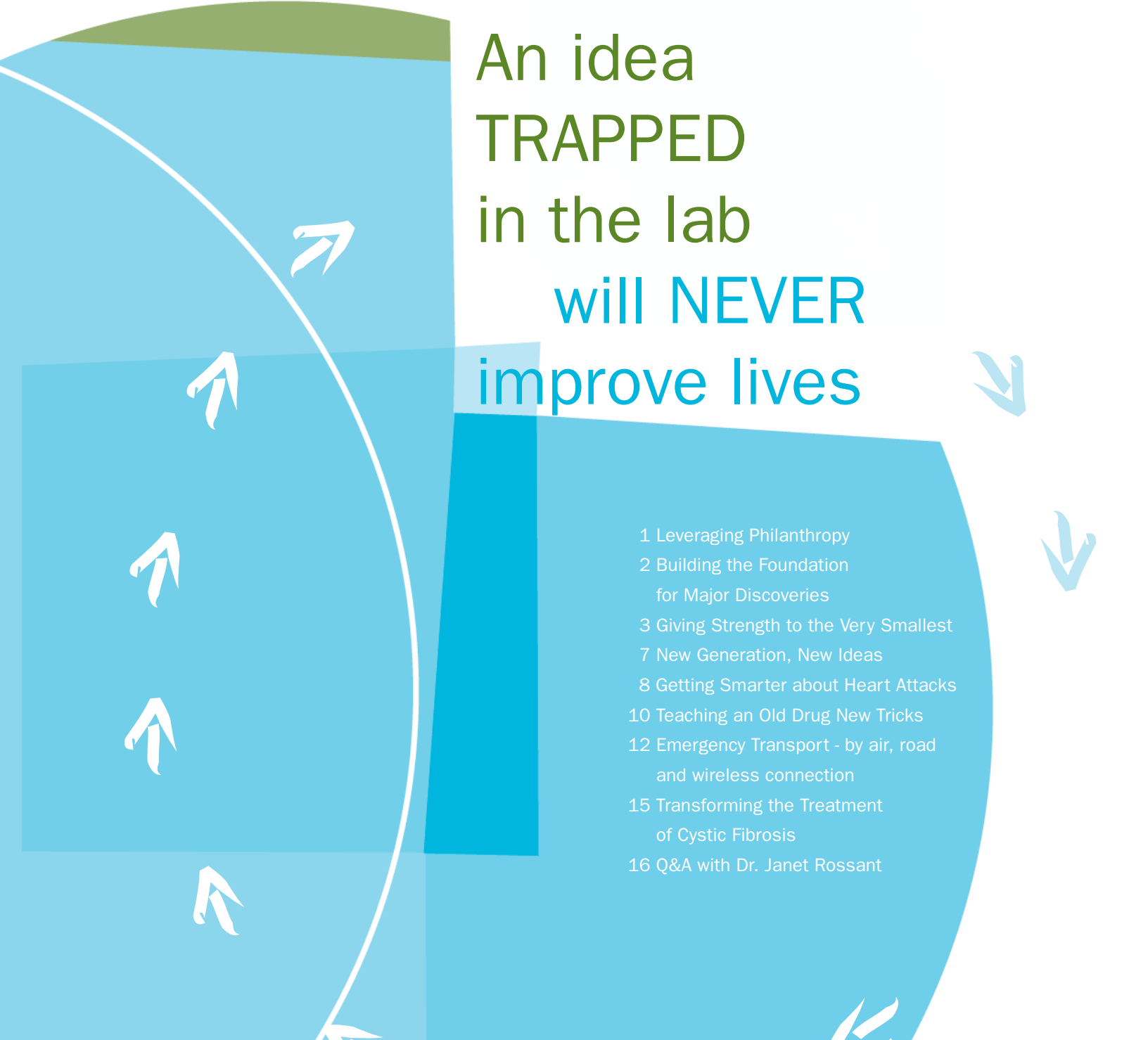


SickKids®

impact

Healthier Children. A Better World.™

Issue 2 Winter 2009



An idea
TRAPPED
in the lab
will NEVER
improve lives

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“Imagine if you could see into the future.”

More than 6,000 genetic disorders exist. It’s our job to revolutionize the way these disorders are diagnosed and treated. If we can identify the genes responsible, it will open the door to understanding your medical history and predict future health problems. With this knowledge we can improve outcomes and even prevent illnesses.

Out of your six billion genetic letters, the *giving* gene is perhaps the most important to us. With your support, five years from now you will have access to the DNA blueprint of your children’s health.

–Dr. Stephen Scherer
Senior Scientist, SickKids Research Institute



You can help discover your DNA blueprint. Exercise your *giving* gene by supporting the world-class research at SickKids.
Donate now at sickkidsfoundation.com or call 1.800.661.1083



Leveraging Philanthropy

Leveraging is not a term that many of our supporters hear about on a regular basis, but it is one of the most important principles at play in our fundraising efforts at SickKids.

SickKids Foundation is the largest hospital foundation in Canada and the largest non-governmental granting agency in children’s health. To put it simply, we exist because of the incredible generosity of donors within our community and across the country. However, what many people do not know is that through the donations we receive, SickKids is able to leverage even more funding for research and education through other means, such as government agencies and private sector grants. For example, in the past five years, for every dollar invested in a SickKids researcher, \$3.34 in external funding has been generated. This extraordinary return on investment would not be possible without the philanthropic spirit of thousands of individuals and organizations who continue to believe in SickKids.

In this issue of Impact magazine, we want to highlight the power that donating to SickKids Foundation can have on the process of turning research into reality. The Hospital for Sick Children has a long and proud history of making important scientific discoveries, and then translating those results into real products and technologies that can help not only Canadian children, but also countless others around the world. Perhaps one of our best known success stories is the development and commercialization of Pablum, a SickKids innovation that has proven to be instrumental in tackling children’s malnutrition globally.

As you read through these pages, we think you will be surprised at the variety and ambition of the discoveries coming out of SickKids into the marketplace. Some of them include complex studies targeting a tiny but incredibly vulnerable segment of the population, such as groundbreaking treatment options for the neurological disorders Sandhoff and Tay-Sachs. Others are new concepts for re-shaping old processes, such as the Bedside Paediatric Early Warning System, which aims to save hundreds of lives a year by catching cardiac arrests before they happen.

As varied as the innovations are, SickKids applies the same philosophy to each of them: to ensure that our knowledge and discoveries have the greatest impact possible, reaching the largest number of patients as quickly as practical. We know that our supporters share this philosophy, and that is why we will continue to advance our vision of “Healthier Children. A Better World.™”

Patsy Anderson
Chair, SickKids Foundation Board of Directors

Building the Foundation for Major Discoveries

The SickKids Research Institute has attracted some of the most gifted scientific minds in the world to work on projects that will ultimately improve the health of children. Philanthropy is what enables us to retain these leading researchers, and provide an environment in which they flourish.

Essential to The Hospital For Sick Children's success in research is providing talented researchers with state-of-the-art laboratories and equipment. Their research flourishes in collaborative, open-concept environments where they work in close proximity to other scientists. This dynamic, inventive space is funded, in large part, by SickKids Foundation.

This year, the Foundation granted \$39.9-million to the Research Institute, 23 per cent of the total \$150-million operating budget. The grant helps fund scientists' salaries, work space, equipment, training and research support. None of these operating costs is funded by government and all are essential to enable scientists to carry out their research.

"World-class infrastructure and core support for research lays the foundation for research of the highest quality," said Gwen Burrows, director of strategic projects at the Research Institute.

Of the total operational grant, \$13-million went towards the costs of laboratory space in the Toronto Medical Discovery Tower, two blocks from the hospital. This facility is part of the Toronto Discovery District, a major research medical and biotech cluster, thus facilitating collaborations with industry and other research hospitals. The space includes five floors of SickKids research facilities that house

geneticists, molecular scientists and developmental researchers in an open-concept laboratory setting.

"When scientists and their team of researchers and trainees are working next to one another, they start to talk about their work," Burrows said. "They may begin to exchange techniques, or start to see some synergies between their projects, and new innovations in research will come about because of that."

Currently, there are more than 2,500 staff and trainees involved in more than 1,160 funded projects at the Research Institute. The groundbreaking research conducted at SickKids could not happen without the energy and intelligence of young, talented trainees. The Research Institute has a mandate to train and educate future scientists, including clinician-scientists, who are an important bridge between research and patient care.

SickKids Foundation supports the Institute's Research Training Centre. Last year, the Centre trained more than 1,000 young scientists from 43 different countries.

"SickKids has a strong teaching mandate, and this is equally true within the Research Institute," Burrows said. "Trainees contribute a great deal to the research that takes place at SickKids — and we need to make sure we are training the next generation of world-class researchers in child health."

Private gifts also help fund young scientist who are at the very beginning of their careers by providing some of the start-up laboratory and personnel costs. SickKids researchers typically leverage this early support by getting external funding from granting agencies. Over the past five years, for every dollar invested in a researcher, \$3.34 in external funding has been generated — an extraordinary return on investment, which would not be possible without philanthropic support.

With support from SickKids Foundation, the SickKids Research Institute will continue as a world leader in discoveries that contribute to the well-being of children and their families. ●



Tom Ciconte (left) with his son, Luke (right).

Giving strength to the very smallest

Prototype fortifies breast milk for premature infants

Liz Wilson-Ciconte and her husband Tom Ciconte waited 10 long years for their first child. But in the end, his birth came too soon. Luke arrived at 31½ weeks gestation, weighing only 1.4 kilograms. He spent his first seven weeks confined to the Neonatal Intensive Care Unit.

Luke's story is not uncommon. In 2006, more than 26,000 Canadian infants arrived before 37 weeks, some 2,500 of them weighing less than 1.5 kg. Thanks to modern technology, the vast majority of premature infants now survive. But with survival comes serious health risks, such as sub-optimal nutrition. Born without nutrient reserves and too few enzymes to metabolize those acquired after birth, premature infants face many challenges that tax their tiny nutrient stores, including frequent medical tests, infections and problems with sucking and swallowing.

While in hospital, premature infants are provided with extra nutrients. Once home, formula fed infants may



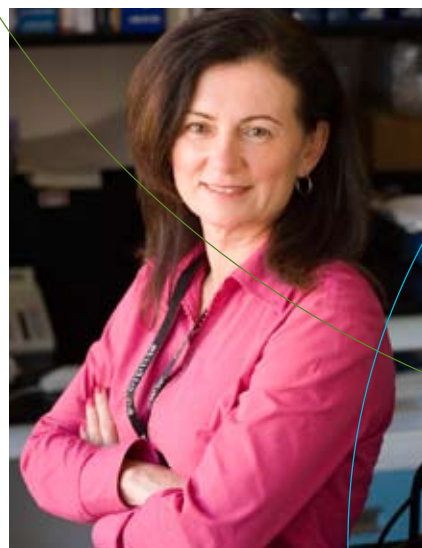
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PROBLEM: After discharge from hospital, premature infants are at risk for poor growth and development, repeat hospital admissions and long-term health problems.

SOLUTION: A multi-nutrient fortifier that parents can easily add to breast milk at home.

IMPACT: Better health, improved growth and a brighter future for the babies.

Dr. Deborah O'Connor created a multi-nutrient milk fortifier for home use, which promotes growth, weight gain and visual development.



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be provided with a specialty formula high in nutrients however, there is no easy way to provide extra nutrients to breastfed infants. Without the added nutrients, the smallest of these babies may suffer poor growth, repeat hospital admissions or impaired motor, language and cognitive development.

To avoid these problems, the nutritional gap between hospital and home must be bridged. Deborah O'Connor, a dietitian and nutritional scientist at The Hospital for Sick Children, says the answer is a multi-nutrient fortifier for home use. Her recent pilot study found that a hospital breast milk fortifier taken at home for 12 weeks after discharge promoted growth, weight gain and visual development, the latter serving as a proxy for brain development.

As a participant in the pilot study, the Wilson-Ciconte family was randomly chosen to receive the multi-nutrient fortifier. "We noticed a huge improvement in Luke's growth. He looked phenomenally healthy," his mother said. "It really gave us hope."

Encouraged by positive results, Dr. O'Connor and her team created a multi-nutrient fortifier specifically for home use. Development of a prototype was made possible with a \$50,000 grant from the Ontario Ministry of Research and Innovation's Ontario Research Commercialization Program. The funds are administered by *BioDiscovery* Toronto, a network that links medical and academic organizations to commercialize research. "This funding was vital," says Arlene Yee of SickKids Corporate Ventures. "Without it, there would be no prototype and no product development."

The prototype is a concentrated liquid full of vitamins, minerals, protein and fat, and is designed to be mixed with human milk to capitalize on the well-known benefits of breastfeeding. "For these very little babies, we needed to figure out how to support their needs for growth and promote breastfeeding. Our multi-nutrient fortifier does both in a simple, convenient way," O'Connor says.

The next step is a clinical trial to see how this prototype performs in the real world. If all goes well, the multi-nutrient fortifier could be commercially available in three to four years. The market for this niche product will be relatively small. "But the impact of helping premature babies will be enormous," says Dr. Yee. "This product is not available anywhere else, and is truly needed."

Wilson-Ciconte agrees. "It's critical that other families have the opportunity to give their child a chance to thrive." Her son, Luke, now three years old, is happy, healthy and on the run. ●



Liz Wilson-Ciconte (left) plays with her son Luke (middle) and her husband Tom Ciconte (right).

New Generation

Photos taken at the SickKids Summer Research (SSuRe) Program Symposium on July 24, 2008.



New Ideas

PROBLEM: The need for a fresh perspective to science.

SOLUTION: Students contribute novel ideas, enthusiasm and a willingness to learn.

IMPACT: Training the next generation of scientists.



When Gabrielle Boulianne began searching for the genetic causes of obesity, she employed more than just innovative research methods. She tapped fresh minds.

The SickKids senior scientist, who is an expert in developmental and stem cell biology, worked closely with three summer students to tackle one of the

most prevalent nutritional disorders among children worldwide. Together, the researchers produced a unique model for understanding obesity.

The students used fruit flies to determine which genes are responsible for regulating appetite and metabolism. "We developed three methods of screening for obese flies," said Aaron

Cheung, who joined Dr. Boulianne's team after completing his third year of studies at the University of Toronto. "We used these methods to screen through a library of mutant flies and identify which were obese, and hence identify which genes have a role in obesity."

Tomorrow's scientists are contributing in SickKids labs today

Cheung is one among hundreds of young scientists who have played an important role in advancing knowledge and research at SickKids. Over the years, students have made contributions in breakthrough studies, including identification of genes linked to autism and studying the origins of medulloblastoma, a childhood brain tumour.

Nicola Jones, SickKids scientist and chair of the SickKids Summer Research (SSuRe) Program, thinks students bring a special perspective to the table. "Students often think of new and creative ideas others haven't thought of before," she said. "They come up with different ways of looking at a problem, which can sometimes lead to major discoveries."

Established in 1978, SsuRe gives university students an opportunity to do research under the supervision of some of SickKids most renowned scientists. Through the 12-week program, the students have a chance to contribute to both existing and new research projects. However, a lack of funding limits the number of students that can be accepted each year.

John Brumell, a scientist in the Cell Biology program and last year's SSuRe chair, sees the program as a vital component in continuing the legacy of research at SickKids and would like more students involved. "The students have a fresh eye and an open mind," he said. "They are our future scientists and clinicians who have the potential to make a significant impact on research." ●

Getting smarter about heart attacks

A new process for tracking a child's condition may reduce cardiac arrests in hospitals

Each year, there are about 5,000 “code-blue” calls for immediate medical assistance for children in Canadian and U.S. hospitals. At least one out of every five of these children will die without leaving hospital.

Christopher Parshuram, Kristen Middaugh and the team at the Centre for Safety Research have devised a system of care to identify at-risk children and prevent code-blue events. If effective, the Bedside Paediatric Early Warning System (PEWS) could save the lives of 700 children in Canadian and U.S. hospitals each year.

SickKids Impact talked with Dr. Parshuram and Ms. Middaugh to discuss the system.

IMPACT: What exactly is the Bedside PEWS?

PARSHURAM: It's a comprehensive, integrated system to complement existing expertise. There are four components. First: a seven-item score measures the degree of a child's sickness. Second: an integrated documentation record permits real-time calculations of the score. Third: scores are matched to specific care responses. Fourth: an education program to support initial implementation and ongoing use.

IMPACT: How will this improve patient care beyond the system currently in place?

PARSHURAM: Our data show that current systems of care do not prevent late recognition of sick children, even though most hospitals have the expertise to help children at risk of cardiac arrest. Bedside PEWS removes barriers that prevent the timely referral of children who are getting sicker and require immediate help.

IMPACT: When a child is admitted to the hospital, how is the Bedside PEWS applied?

MIDDAUGH: Bedside PEWS is for children on hospital wards, not for those in intensive care units who are already closely monitored. When a patient is first admitted, a nurse measures a series of vital signs and records them in the Bedside PEWS documentation. These numbers are then added up to a single score, which classifies the type of care a child requires.

IMPACT: What kinds of recommended care are embedded in the system?

MIDDAUGH: When a relatively healthy child registers a low score, the system calls for routine monitoring by physicians and nurses. A child who registers a higher score calls for more frequent attention from the entire health-care team — perhaps as often as every 15 minutes. The score also has an impact on the number of patients the primary health-care nurse can handle at a time.

IMPACT: What are the advantages of having the Bedside PEWS in place?

MIDDAUGH: Bedside PEWS is an objective measurement of severity of illness that can be used at a single point in time to describe changes in clinical conditions over a longer period. It improves communication between health-care professionals, it can reinforce the clinical expertise of frontline staff and it may improve allocation of health-care resources.

IMPACT: Has there been any formal evaluation to date?

PARSHURAM: We are completing our evaluation of the clinical implementation of the Bedside PEWS and preliminary results are favourable. Our earlier work confirms that the Bedside PEWS score can easily distinguish sick from well patients. The score increases as sick patients get sicker and is correlated with the opinions of frontline nurses. We have held education sessions involving more than 100 health-care workers and received positive evaluations. The results of our 2,000-patient multi-centre validation study will be available early next year.

IMPACT: Is Bedside PEWS meant to replace current systems?

PARSHURAM: We are doing a good job now, but Bedside PEWS will allow us to enhance patient monitoring and ensure patients receive the best care possible in a timely manner. While we're fortunate to have the expertise to treat very sick children in our hospitals, Bedside PEWS is a powerful tool to improve how we bring that expertise to the children who need it most. ●

SickKids researchers have developed a system that could save the lives of hundreds of hospitalized children in North America every year.

PROBLEM: A Canadian and U.S. survey of 181 paediatric hospitals, published in 2006, indicated that in spite of current safety systems designed to identify children at risk of cardiac arrest, about 5,000 “code-blues” still occur every year.

SOLUTION: An objective system that identifies children at risk for cardiac arrests and specifies specific care responses.

IMPACT: The Bedside Paediatric Early Warning System could save the lives of 700 children in Canadian and U.S. hospitals each year.

COMMERCIALIZATION: There is an opportunity for software companies in the health-care industry to create an electronic data management and scoring system based on the Bedside PEWS technology.

Research was made possible by SickKids Foundation and funding from the Heart and Stroke Foundation of Ontario.

Dr. Christopher Parshuram (left) helped create the Bedside Paediatric Early Warning System (PEWS).

Hex A

Teaching an Old Drug New Tricks

Researchers think an old malaria drug could be a new treatment for a rare neurological disorder



Drs. Don Mahuran (left) and Michael Tropak (right)

PROBLEM: There is no medical solution for the 200 North Americans living with late onset Sandhoff and Tay-Sachs diseases.

SOLUTION: Identification of Pyrimethamine, a malaria drug, as a potential treatment.

IMPACT: Hope for the children and adults living with late onset Sandhoff and Tay-Sachs diseases.

Pyrimethamine

Debbie De Pinto encourages her two sons to experience life to the limit. That's because Sean, 24, and Chris, 22, have juvenile onset Sandhoff disease, a rare genetic neurological disorder that will significantly shorten their lives and steadily rob them of their ability to speak, walk and do other things healthy people take for granted.

There is no medical solution today to shield Sean, Chris and the 200 other North Americans living with late onset Sandhoff, and its sister disorder Tay-Sachs, from a bleak future. Without a scientific breakthrough, the afflicted face a future of increasing tremors, weakening muscles and possibly even psychological disorders.

The Hospital for Sick Children senior scientist Don Mahuran and research associate Michael Tropak have never met Sean and Chris, but they have worked with cells cultured from the brothers' own skin.

The researchers believe they may have uncovered a viable treatment for the effects of late onset Sandhoff and Tay-Sachs. Their findings could give the De Pinto family, of Richmond Hill, Ontario, new hope.

Sandhoff and Tay-Sachs diseases are rooted in a genetic mutation that leaves two particular proteins unable to form a functional enzyme, called Hex A (Hexosaminidase-A). Without Hex A, a specific fatty material cannot be broken down and accumulates in the brain

For people like Sean and Chris, who produce extremely low levels of Hex A, the fatty materials build up and eventually cause brain cells to die. The process triggers their symptoms: slurred speech, muscle weakness and difficulty walking. Each of the brothers also faces a 40 per cent chance of developing debilitating psychological disorders.

Through their research, funded in part by a donation to SickKids Foundation, Dr. Mahuran and Dr. Tropak have shown that an existing malaria drug, called Pyrimethamine, functions as a molecular 'chaperone' to help mutant Hex A form a functional enzyme.

"It was surreal at first," Tropak recalls. "We had actually found a real chaperone for Hex A – a compound that was already tried on patients with malaria and shown to be safe."

Mahuran is equally excited at the prospect of teaching an old drug new tricks. He is very optimistic that Pyrimethamine will be as effective with actual patients as it has been with their cells.

The Hospital for Sick Children, through the Corporate Ventures office, has filed patent applications in Canada and the United States on the Pyrimethamine compound for use as a pharmacological chaperone. Corporate Ventures has also licensed it to a molecular therapeutics development company, ExSAR Corporation. The New Jersey-based firm has completed the initial groundwork and is seeking funding for Phase I clinical trials. With further funding to support Phase I and II trials, as well as additional research and development, Pyrimethamine can be fully assessed as a potential treatment for these diseases.

For Debbie De Pinto, any progress towards a treatment for the disease that infiltrates every aspect of her sons' lives is encouraging beyond her dreams. "Even remission, to be able to keep Sean and Chris where they are right now with the disease, would be wonderful. Then everything else, well, we can deal with that." ●

Research was made possible by SickKids Foundation and funding from the Canadian Institutes of Health Research, National Institutes of Health, a donation from the Uger estate and a salary grant from the Life for Luke Foundation.

hope



Each year, more than 700 newborns, like little Alexander featured in these photographs, travel to SickKids by road or air ambulance to receive lifesaving care.



Emergency Transport – by air, road and wireless network



SickKids created a Transport Remote Access Care (TRAC) system to help transmit clinical information to SickKids from remote locations to help ensure a safe and stable transport from one hospital to the next.

PROBLEM: The Acute Care Transport team, comprised of nurses and respiratory therapists, stabilize 700 newborns each year but rely on paper charts and telephone to get critical information to medical teams waiting at SickKids.

SOLUTION: A software system that quickly and accurately transmits clinical information to SickKids from remote locations.

IMPACT: Enhancing the transport team's ability to assess and treat patients while giving the staff at the hospital a way to gauge a patient's condition before they arrive at SickKids.

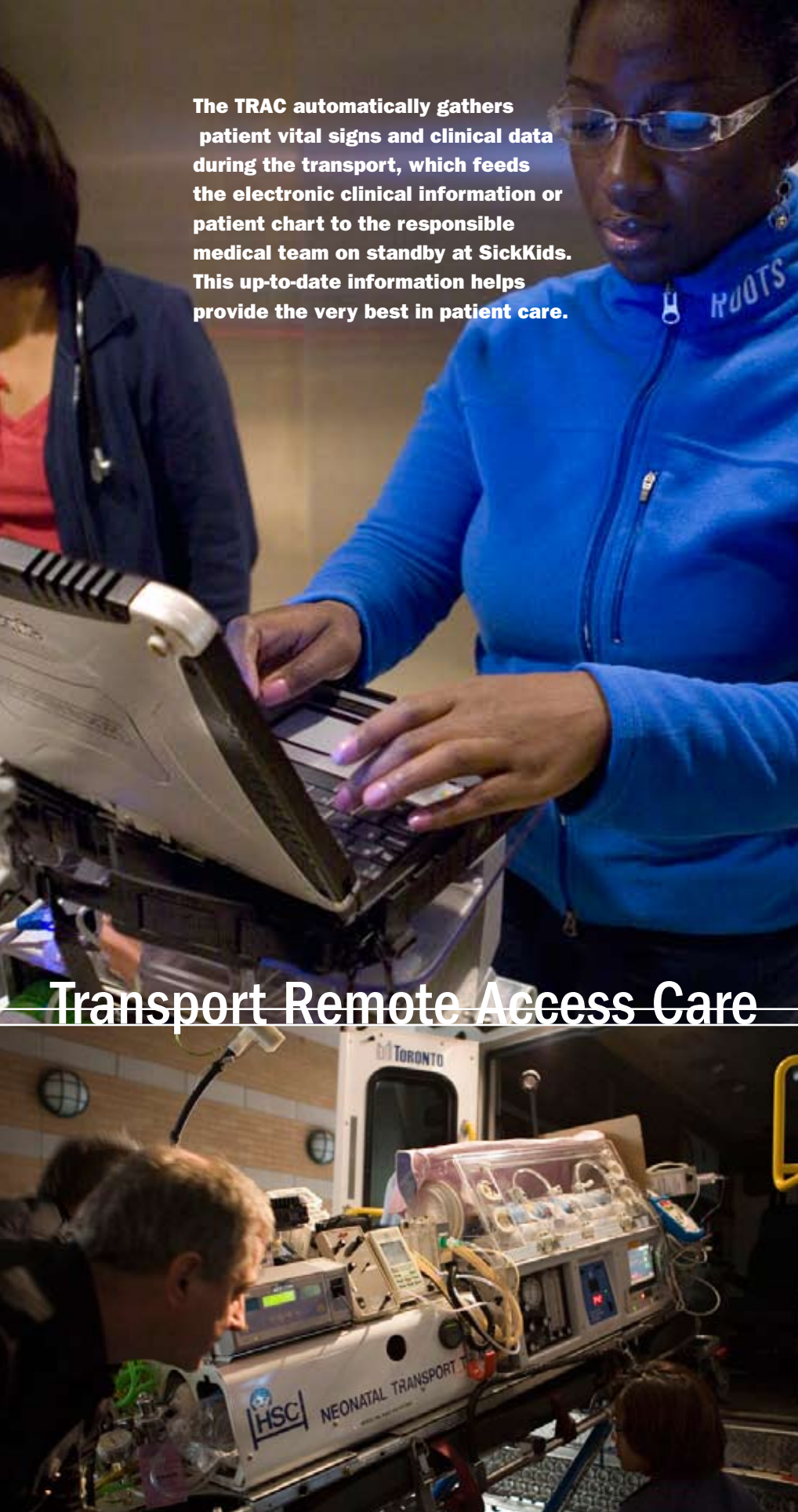
SickKids staff can now know what's happening when the ambulance is still miles away

Every year, the Acute Care Transport Services (ACTS) team stabilize more than 700 newborns en route by road or air ambulance to The Hospital for Sick Children.

Until recently, the specialists on standby at the hospital relied on paper charts and telephones to get critical information from the ACTS crews.

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The TRAC automatically gathers patient vital signs and clinical data during the transport, which feeds the electronic clinical information or patient chart to the responsible medical team on standby at SickKids. This up-to-date information helps provide the very best in patient care.



Transport Remote Access Care

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Together with an IT partner, SickKids has devised a much better system for getting vital information to the people who need it. The Transport Remote Access Care (TRAC) system automatically gathers patient vital signs and clinical data during transportation and feeds it electronically to the responsible medical team at SickKids. The ACTS team is now even able to send live video feed of patients, enhancing their ability to assess and treat patients and giving hospital staff a critical look at patients before arrival.

SickKids neonatologist Hilary Whyte and Neonatal Intensive Care Unit systems manager Loreto Lecce teamed with Dapasoft Inc., a software development company, to devise TRAC in 2003. The hospital and the Toronto-based firm are now ready to make the system widely available by partnering with other health-care organizations. ●

COMMERCIALIZATION: By developing an effective way to monitor a newborn's vital signs and clinical data during transport, SickKids has enhanced patient safety. Through commercialization, Dapasoft and SickKids have the opportunity to partner with other health-care organizations across the world and make the TRAC system widely available.

Transforming the treatment of Cystic Fibrosis Scientists search for drugs to fix defects caused by mutant gene

After three decades working with cystic fibrosis (CF) patients, Peter Durie knows too well what an average life expectancy of 35 years looks like. The inherited disease creates thick, sticky mucus, which interferes with digestion and clogs breathing passages. Most people with CF die from damage caused by chronic lung infections.

"I've seen quite a few people pass away," says Dr. Durie, a gastroenterologist at The Hospital for Sick Children.

But he has also watched SickKids treatments aimed at controlling the symptoms and complications of CF push the lifespan of some patients into their 50s and beyond.

New work by a multi-disciplinary team at SickKids, in close collaboration with St. Michael's Hospital, is poised to help transform CF from a disease that cuts people's lives short, to one that can be managed throughout a normal lifetime.

Rather than treating the clinical consequences of this disease, such as using antibiotics to treat lung infections, current drug discovery efforts at SickKids and other North American centres are targeting the molecular breakdowns responsible for the disease. Early results for one drug being tested had such a positive affect that the disease could no longer be detected through standard techniques.

"Everybody was just beaming," says Christine Bear, co-director of the SickKids Cystic Fibrosis Centre.

CF is caused by a mutation in a gene discovered in 1989 by researchers at SickKids and the

University of Michigan. When that gene is not working properly, the protein it makes does not function correctly either, and thick and sticky mucus layers build up.

Research by Dr. Bear and others has shown that some forms of the mutant protein can be fixed and coaxed into doing their job.

Scientists have discovered drugs that have a good chance of "rescuing" certain types of mutant protein. Last spring, a small trial for one of these drugs reported surprising improvements in lung function as well as near normal results in a basic test measuring salt in a patient's sweat.

"We were just blown away," says Durie, who supervised the SickKids participants. "As proof of concept, it is really exciting."

The SickKids team is hopeful that other drugs targeting the most common protein defect in CF patients may also be successful in clinical trials.

"It's the best news we've had in a really long time," Bear says. "It means we're on the right track, focusing on finding drugs that will target the defects caused by the mutations in the CF gene."

Other research led by Bear and Durie is leading to discoveries that could one day add new diagnostic and therapeutic solutions.

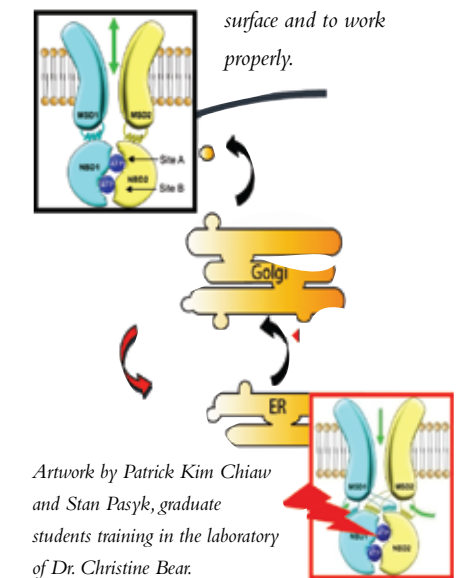
Both doctors are working closely with Corporate Ventures to commercialize these potentially valuable inventions. ●

PROBLEM: CF is most often fatal, cutting patients' lives short to an average of 35 years.

SOLUTION: Current drug discovery efforts at SickKids and other international centres are targeting the molecular breakdowns of CF.

IMPACT: Transforming CF from disease that cuts a life short, to a one that can be managed throughout a normal lifespan.

The average person's CFTR protein (in the black box) acts to regulate the movement of fluid (green arrow) and removal of air-borne bacteria from the surface of cells (such as those lining the airways). The major CF causing mutant protein (in the red box) gets trapped inside cells leaving them unable regulate the movement of fluid, which results in airway infection (brown arrows indicates this trap). There is hope that the defect caused by mutation (red lightning bolt) may be corrected with drug treatment to allow the protein to reach the cell



Artwork by Patrick Kim Chiaw and Stan Pasyk, graduate students training in the laboratory of Dr. Christine Bear.

Research was made possible by SickKids Foundation, funding from the Canadian Cystic Fibrosis Foundation, Canadian Institutes of Health Research and Cystic Fibrosis Foundation Therapeutics.

"It's the best news we've had in a really long time..."

This is a tough time for the economy. Does research spending really matter?



Absolutely. Scientific advancements are the engine for economic growth, and Canada's talent for innovation — particularly in the health sector — has produced world-changing advances:

- Research on the contributing causes of SIDS (sudden infant death syndrome) has led to behaviour changes resulting in a 40 per cent reduction in this syndrome.
- Today, three-quarters of Canadian children stricken with cancer—the second leading cause of death among children aged 1 to 14—are cured. That's a significant increase from five per cent just 40 years ago.
- Fifty years ago, a child diagnosed with cystic fibrosis might survive to age four. Today the median age for survival is 35-years old.
- The development of techniques for the surgical management of intractable epilepsy in children, based in part on information garnered with magnetoencephalography (MEG), are setting the standard in neuroimaging research and putting Canada ahead of the rest of the world.

We have good reasons to be proud of the extraordinary impact of child health research here and throughout the world, and can expect much more from the exciting research currently underway.

Research outcomes have a positive economic and social benefit when innovations are commercialized. SickKids Corporate Ventures works closely with our researchers to facilitate the process of moving research from the lab to the market and beyond, whether through patenting a new technology, seeking licensing opportunities or in creating a start-up company. Revenue gained through these business opportunities is reinvested to support future discoveries and innovations.

Q&A with Dr. Janet Rossant, Chief of Research

Are there particular areas of health research where Canada is considered a leader?

We are making important advances across the breadth of child health research — from the molecular to the population level.

Two particular areas are genetics and stem cell research. Progress in genetics has been remarkable — from the discovery of DNA's double helix structure in 1953 to the recent deciphering of the human genome. Scientists can now systematically discover the molecular basis of human disease, an understanding that holds the promise of new and effective therapies that will transform children's health. Our growing knowledge is already transforming medicine and has the potential to change other fields as well.

A second field where Canada leads is regenerative medicine. Stem cell research may successfully lead to treatments for many chronic diseases and injuries, saving lives:

- By substituting normal cells for damaged cells to treat diabetes, Parkinson's disease, spinal cord injury, heart failure and other disorders
- By providing safe and convenient models of disease for drug development
- By improving understanding of fundamental aspects of normal development and cell dysfunction

What do we need to do to take discoveries from the bench to the bedside?

We must be able to apply our findings as technologies or therapies that can be tested to ensure they are useful and not harmful. And then we must apply this new knowledge to improve health.

There are challenges. The first is speeding up the process from discovery to development of a treatment or device that can be tested. And we must ensure therapy is used in the community so it actually improves children's health.

How is today's economic climate impacting the health research environment?

Researchers must be able to take risks. Scarce resources mean that some scientists are abandoning some innovative and creative studies that may be less likely to be supported, leaving many good ideas behind. And fiscal constraints mean many scientists must downsize their labs while younger researchers must wait longer to obtain their first grants — a discouraging factor that keeps some talented young people from entering scientific research in the first place.

Why invest in research at SickKids?

Canada's children represent one-quarter of the population and — 100 per cent of our future. Healthy children are more ready and able to learn and, in the longer term are more likely to become healthy adults who will contribute as a productive citizenry and workforce. Yet our children face significant health challenges. Asthma is responsible for more than one-quarter of all school absences. According to a Parliamentary report released last year, one in four Canadian children is overweight or obese. This might mean that our children will live shorter lives than we'll live. An estimated 800,000 Canadian children — 14 per cent — experience mental health disorders that impair their ability to function, disorders that frequently persist into adulthood causing lifelong distress and disability. Almost 15 per cent of our children have a chronic condition. Injuries kill more children and young adults in Canada than any other cause and are the leading reason for hospitalization, even though most childhood injuries — even the fatal ones — can be prevented.

Advances depend on scientific discovery to translate research findings into clinical realities. SickKids researchers are devising extraordinary therapies, identifying ways to prevent illness before it occurs, and making discoveries that promise to aid in the transformation of children's health.

What role can philanthropy play?

Private funders have a unique capacity to invest in innovative and creative risk-taking strategies that may have high payoffs, such as support of young scientists.

Training is another area where the need for innovation presents a clear opportunity. The environment for health research is changing rapidly and in response, paradigms for training need to change as well. Our Research Training Centre has a history of establishing best practices; support is essential to the centre's continued success.

Encouraging youth to pursue science-based careers is critically important. Our Kids Science program, supported in part by Ontario's Ministry of Research and Innovation, helps keep kids in the pipeline even if they don't have role models working in science-related careers. We partner middle and high school

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students with researchers, trainees and physicians in the powerhouse that is the SickKids Research Institute, to investigate the problems scientists face in a typical work day. Guided by mentors, they get a firsthand look at a career in science and experience the exhilaration of discovering for the first time something that no one else knows.

Finally, as the potential “risk capital” in the system, charitable dollars need to find those niches where smaller investments can show the way for the more stable, larger investments to be made by government and industry. Our New Ideas Fund, supported by philanthropic donations, provides researchers with seed money to pursue non-conventional ideas that would not otherwise qualify for funding from conventional sources, yet have the potential to develop into a full external grant proposal, a commercialization opportunity and possibly a clinical practice change.

What are the challenges to getting philanthropic dollars?

A survey conducted two years ago by Research Canada indicated that the public favours investments in health research. A major challenge is to educate foundations and individuals on how they can provide support. We must overcome perceptions that health research is the sole province of government and industry — it isn't. One of the things we strive to do is translate knowledge, so Canadians understand how their support helps us improve children's health. ●

Dr. Janet Rossant

SickKids Chief of Research and a world-renowned expert in developmental biology, Dr. Janet Rossant is the definition of a trailblazer. Known for pioneering techniques for following cell fate and altering genes in embryos in the mouse, her current research focuses on stem cell development and cell differentiation in the developing embryo, important areas for the study of birth defects and regenerative medicine.

Dr. Rossant has been recognized for her contributions to science with many awards including the Killam Prize for Health Sciences and the CIHR Michael Smith Prize in Health Research, Canada's most prestigious health research award. She holds the Lombard Insurance Chair in Paediatric Research at SickKids and is a University Professor in the departments of Medical Genetics and Microbiology, and Obstetrics and Gynecology at the University of Toronto.

A member of the Canadian Institutes of Health Research Governing Council and founding member of The Stem Cell Network, Dr. Rossant played a leadership role in setting Canada's public policy regarding stem cell research, an area in which she is a respected voice. In April this year she was elected to the U.S. National Academy of Sciences, recognizing her distinguished achievements.

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Issue 2

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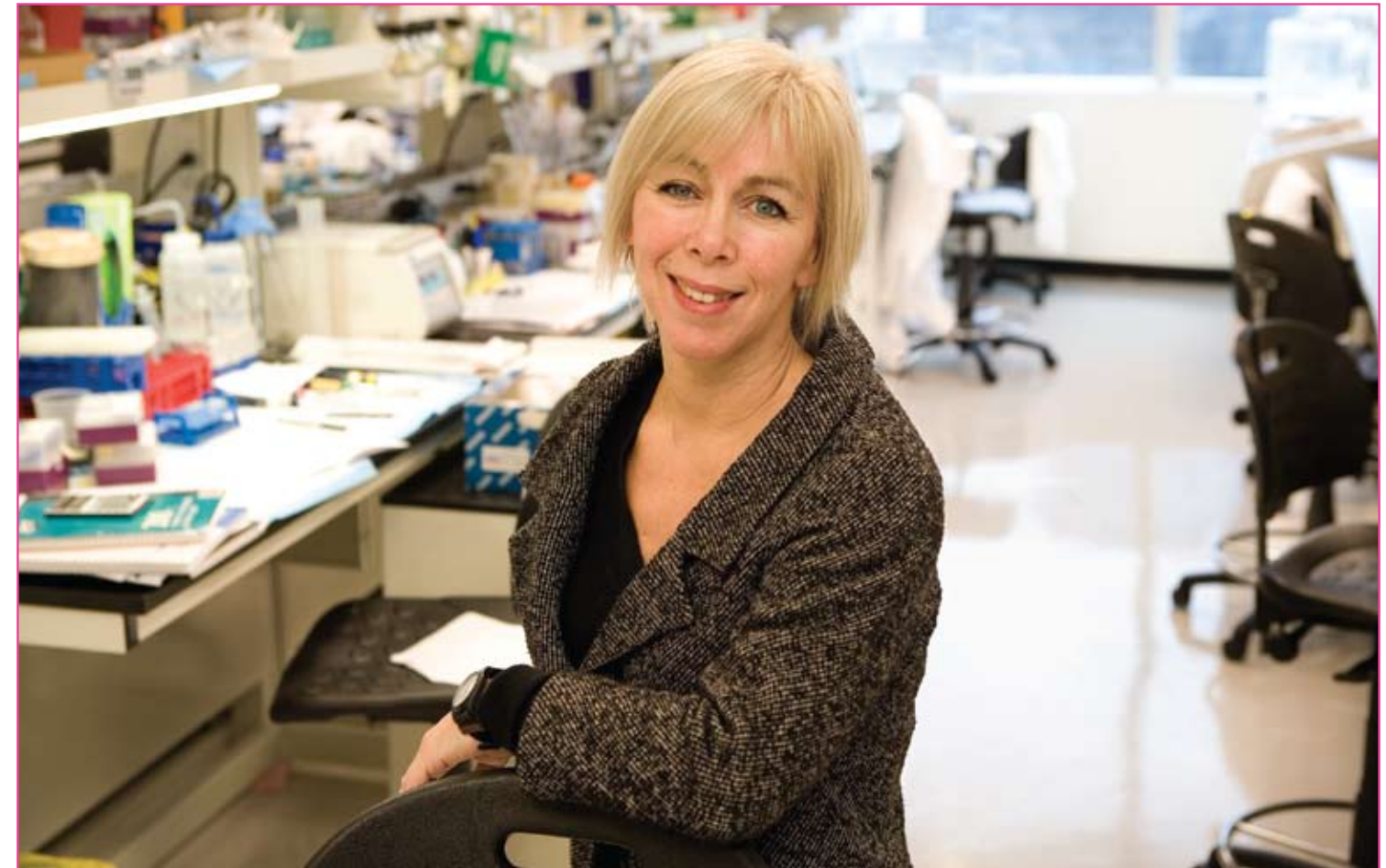
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“Picture a world where
damage could be undone.”

Nervous system diseases are one of the largest unmet medical needs worldwide. At SickKids, we had an unconventional idea to take stem cells from skin and use them to repair or regrow damaged nerves and tissues. The amazing thing is, it actually worked!

Philanthropy allows us to embrace out-of-the-box thinking and apply it in the lab. Your support gives us the opportunities that no one else is willing to take a chance on.

– Dr. Freda Miller
Senior Scientist, SickKids Research Institute

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FOUNDATION

You can help give our scientists the freedom to make a groundbreaking discovery by supporting the world-class research at SickKids.
Donate now at sickkidsfoundation.com or call 1.800.661.1083



SickKids[®]

It's Easy Being Green

when it impacts a healthy future for our children.

Early 2009, The Hospital for Sick Children (SickKids) will be in hot water; the solar-powered kind. To reduce energy costs and greenhouse gas emissions, SickKids will be the largest hospital to install a solar thermal energy site. 92 solar panels will be installed on the roof of the hospital and provide hot water heated by the sun for the next 10 years.

This is a small step in a larger strategy to work towards a 'greener' health-care institution. Over the past two years, 'green' initiatives at SickKids have resulted in more than \$1 million in energy savings.

As an organization dedicated to the health and well-being of our children, protecting the environment gets us one step closer to our vision: Healthier Children. A Better World.™