Stem for Life
a CURA Foundation

Summer-Fall 2016

CURING DISEASES WITH CELL THERAPY

LifeLines

www.stemforlife.org

UNITE TO CURE
Welcome Message

The *Third International Conference on the Progress of Regenerative Medicine and Its Cultural Impact* was a huge success! It facilitated the exchange of knowledge and ideas between the different disciplines to advance research and clinical collaborations. During the conference, we explored how science, technology and communication will allow us to personalize medical solutions and remove inefficiencies in medical treatment reducing overall health care costs while improving clinical outcomes through better access to appropriate therapies. We also explored the need to accelerate regulatory approvals, lower cost of goods and address distribution barriers so patients who need medical solutions around the world can access these innovations more readily.

To our benefactors and sponsors, I cannot thank you enough for giving us the opportunity to complete this initiative. I am very proud to share with you the global media coverage we obtained, which included over 1,000 news stories about the event, 510,050,696 print and online impressions, and 48,983,562 broadcast impressions including coverage in the Vatican on *Good Morning America, NBC,* and *CBS*.

We are grateful to all of you for being part of our global movement and your contribution to the progress of cellular research. We also thank the Vatican for their unwavering support for this initiative and are grateful to partner with the forward thinking individuals at the Pontifical Council for Culture who understand the importance of innovation and the need to prepare for the impact it will have on society.

Dr. Robin L. Smith
President and Trustee, Stem for Life Foundation

TO ACCLERATE DEVELOPMENT OF CELL THERAPY TREATMENTS AND CURES: **@STEMFORLIFE #UNITETOCURE**

PHILANTHROPY AND COLLABORATION DRIVING CURES

Victoria Jackson and Bill Guthy, Guthy-Jackson Charitable Foundation Founders

Sean Parker, Philanthropist and Entrepreneur, Dr. Jeffrey Bluestone, President and CEO of the Parker Institute for Cancer Immunotherapy and Katie Couric, Yahoo Global News Anchor and Co-Founder, Stand Up to Cancer

Philanthropists are helping to accelerate medical research by requiring collaboration of researchers and physicians. Victoria Jackson and Bill Guthy were among the first ones to enforce collaboration among their grant recipients studying neuromyelitis optica (NMO). When their daughter Ali was diagnosed with NMO, a rare autoimmune disease that is often misdiagnosed, entrepreneurs Victoria and Bill established the Guthy-Jackson Charitable Foundation. This foundation has been successful in building trust in the NMO community by making funding contingent on data sharing and collaboration among the researchers in their ecosystem. To date the foundation has provided over $50 million dollars in funding for researchers in 25 countries studying NMO. This model of collaboration has helped to better understand the pathophysiology of NMO and has been a catalyst to conduct more research and clinical trials around the world focused on this disease.

Another type of collaboration was recently publicized when announcing the creation of The Parker Institute for Cancer Immunotherapy. The Institute unified six leading academic institutions including Memorial Sloan Kettering Cancer Center, Stanford University, UCLA, UCSF, the University of Texas MD Anderson Cancer Center and the University of Pennsylvania to accelerate cancer immunotherapy research forward by providing the funding, resources, operating frameworks and data and technology. “The Institute plans to invest big, fast and focused in high-risk, high-impact initiatives and initiate close linkages between basic and clinical research by breaking barriers and protecting intellectual property rights of scientists and their institutions,” according to Sean Parker, founder of The Parker Institute for Cancer Immunotherapy.

Collaboration and data sharing are key principles to driving progress and accelerating breakthroughs. These two models are great examples of how collaboration and trust can be achieved while recognizing individual scientific contributions of the researchers, removing barriers and driving medical progress forward.

SISTERLY LOVE

Sisters Sally-Ann Roberts, Co-Anchor, Eyewitness Morning News, WWL-TV in New Orleans and Robin Roberts, Anchor, Good Morning America

When her younger sister was in need, Sally-Ann answered the call. Sally-Ann Roberts, a longtime co-anchor at *Eyewitness Morning News* on *WWL-TV* in New Orleans, supported her sister, *Good Morning America* co-host Robin Roberts, who needed a bone marrow transplant. Officially declared to be in remission from breast cancer in 2007, Robin was exhilarated to begin her life again. But, by March 2012, she experienced progressive bone marrow failure caused by her cancer treatment and was diagnosed with myelodysplastic syndrome (MDS), also known as pre-leukemia. MDS is characterized by abnormal hematopoietic stem cells, anemia and neutropenia, all significant blood deficiencies. With this dire diagnosis, Robin’s doctors gave her less than two years to live; her only hope was a bone marrow transplant.

Fortunately, Sally-Ann was a perfect donor match for Robin. In the summer of 2012, Sally-Ann underwent a minor procedure and had some of her stem cells withdrawn. Robin soon received the transplant containing Sally-Ann’s concentrated bone marrow cells. Four years later, Robin is now healthy, happy and forever grateful for the kindness of her older sister, Sally-Ann. Following Robin’s transplant, both sisters have become strong bone marrow donation advocates. In addition to MDS, more than 70 disorders can be treated with bone marrow transplantation, with nearly half of the 50,000 bone marrow transplants performed around the world each year requiring a donor. Today, there are more than 20 million voluntary bone marrow donors worldwide.

CELLULAR HORIZONS CONFERENCE HIGHLIGHTS

Dr. Sanjay Gupta, Chief Medical Correspondent, CNN Health, Wellness and Medical, Eduardo Bravo, CEO, TiGenix, and Dr. Mohit Khera, Associate Professor, Baylor College of Medicine

The *Third International Conference on the Progress of Regenerative Medicine and Its Cultural Impact* took place in the Vatican on April 28-30, 2016. The Conference brought together religious and political leaders, scientists, physicians, legislators and patients on three thematic days about the future of medicine.

Day 1: Hope for Our Children

Day 2: Cellular and Technological Breakthroughs in Cancer

Day 3: Cellular Frontiers: Research, Regulation and Funding

Dr. Max Gomez, Medical Correspondent, CBS News moderates discussion between Dr. Domenica Taruscio, Director, National Center for Rare Diseases, Italy (left) and Dr. Stephen C. Groft, Senior Advisor to Director, National Center for Advancing Translational Sciences and Former Director, Office of Rare Diseases Research, National Institutes of Health

ESTEEMED SPEAKERS AND GUESTS

"It is fundamentally important that we promote greater empathy in society and not remain indifferent to our neighbor's cry for help, including when he or she is suffering from a rare disease."

– Pope Francis

"What is clear is that there are immense possibilities fully within our reach that did not exist even five years ago."

– U.S. Vice President Joseph R. Biden

"There is a sea change happening within health care that will affect billions of people alive today resulting from a better understanding of genomics, cellular science and data. It could alleviate so much human and animal suffering; so let's keep up the pace and bring it on."

– The Edge

CONFERENCE HIGHLIGHTS

"The problem of tragic disparities in health care access and treatment is not limited to emerging nations. All of us around the globe need to come together to address this challenge of bringing adequate cancer treatment and care to our children. We need to deepen our commitment and our collaboration to move the 90% of children who die of cancer in developing nations into the category of the almost 90% who receive excellent care and survive their pediatric cancer and go on to live a full and long life."

– Rev. Fr. Kevin FitzGerald

Professor, Dr. David P. Lauler Chair in Catholic Health Care Ethics, Georgetown University

"We need a multimodal therapy to fix the entire gamut of rare diseases. This requires many clinical trials and financial support, which currently is coming from philanthropy, foundations and the disease advocacy groups in addition to funds from the traditional granting process."

– Dr. Joanne Kurtzberg

Director, Carolinas Cord Blood Bank, Chief Scientific Officer, Robertson Clinical and Translational Cell Therapy Program and Director, Pediatric Blood and Marrow Transplant Program

CONFERENCE HIGHLIGHTS continued next page

CONFERENCE HIGHLIGHTS

"Money is necessary, but not the only ingredient needed to accelerate treatments. The stakeholders (scientists, patients, advocates, physicians) need to synergize - gone are the days when we work all alone. With everyone engaged, we can move the normal 7-10 year timeline to go through basic science, pre-clinical efficacy, and then through clinical trials to a much shortened timeline of a few years."

– Dr. Jill Weimer

Director and Scientist, Children's Health Research Center, Sanford Research, Sanford Health
and Associate Professor in Department of Pediatrics, Sanford School of Medicine at the University of South Dakota

"I think if you truly want to treat the disease the ultimate goal is to prevent the disease. We have that unique opportunity now with genomics and all of the other epidemiological environmental information to dissect out why these individuals are actually losing their pancreatic beta cells. Once we've cracked that nut, then we can actually be prophylactic and identify and prevent the individuals that are going to have the disease from acquiring type 1 diabetes."

– Dr. David Pearce

President, Research, Sanford Health; Senior Scientist, Children's Health Research Center; and
Professor, Department of Pediatrics, Sanford School of Medicine of the University of South Dakota

"There's optimism. We're actually saving lives and curing people. It's not an issue of whether it can happen anymore, it's how quickly we can bring them to patients and how often will they work. It is just a matter of putting our heads together and collaborating and acknowledge that it's a great time to be in this field."

– Dr. James P. Allison

Professor and Chair, Immunology, The University of Texas MD Anderson Cancer Center

"There are three big Cs in the space of immuno-oncology. The first is our trend towards combinations. The second is that of collaboration because no single player, be that in academia or in industry, can do that alone. The third is then the cure word."

– Dr. Axel Hoos

Senior Vice President, Therapeutic Head Oncology Research and Development, and Head of Immuno-Oncology, GlaxoSmithKline

"We have the ability today to extract information, compress data into codes, which are actionable, intelligent and transparent. If you make the data clear, accurate and available for all to see, it will change the behavior of physicians, patients and payers leading to better outcomes and less dollars spent."

– Dr. Andrew L. Pecora

Chairman and Founder, COTA and Chairman and Executive Administrative Director,
John Theurer Cancer Center at Hackensack University Medical Center

"We have an extraordinary opportunity at this moment in history with great discoveries, major conceptual advances, and unprecedented technological capabilities. It's only when those discoveries are translated into clinical endpoints with supportive policy that the human condition can be impacted."

– Dr. Ronald DePinho

President, The University of Texas MD Anderson Cancer Center

"The Holy Father is calling us to remember the poor and to figure out how to distribute good quality education and health care to all children and people in need. I am concerned that our society doesn't have the moral resources to ensure that the difference between those who are privileged and those who are not will not get bigger. It doesn't frighten me, it actually saddens me."

– Rev. Fr. Nicanor Pier Giorgio Austriaco, O.P.

Professor of Biology and of Theology, Providence College

"Mesenchymal Cell Therapy is a potentially disruptive technology platform. What does that mean? It means we're different, we're novel, we're not the standard small molecules that drug companies and regulators are used to looking at. Sometimes pharmaceutical companies consider this as something that may change the way medicine is practiced and therefore may dramatically impact their business models."

– Dr. Donna L. Skerrett

Chief Medical Officer, Mesoblast Limited

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CONFERENCE HIGHLIGHTS

"New technologies in cellular therapy should translate into huge cost savings, because an 80% burn patient will stay in the hospital six or eight months at a $10,000+ per day cost. You can shorten their hospital stays by three or four months by using new technology, improving patient care and saving money."

– Dr. David Ahrenholz

Former President, American Burn Association

"What we've learned is that in nature, if someone has a heart attack, they express (SDF1) to recruit stem cells to the site of tissue injury, so that if you can prolong the higher levels of (SDF1) for two to three weeks, it is sufficient to recruit a sufficient number of stem cells to the site of tissue injury for adequate repair to grow a microvascular network of small blood vessels and activate and recruit tissue-specific stem cells (cardiac stem cells)."

– Dr. Marc Penn

President and Chief Medical and Scientific Officer, Black Beret Life Sciences

"I don't believe the science and the technology are any longer the daunting barrier they once were, but the gap between what is possible and what we're delivering to patients will continuously get wider and become a chasm if we don't address the regulatory and reimbursement policies."

– Dr. Andrew C. von Eschenbach

Former Commissioner, US FDA and 12th Director of the National Cancer Institute, NIH

"Our Food and Drug Administration has been structured to look at pills, pharmaceuticals, medicines and devices but what we need is an evolved process, a separate pathway to evaluate and address regenerative medicine, which are not pills, medical devices or traditional pharmacological treatments. It is a process and we don't have that structure and it is time to modernize that."

– Senator William Frist

Former US Senator from Tennessee and Former US Senate Majority Leader

"I think we can learn a lot from what's happened in organ transplantation in the past 30 years. The common goals for the emerging field of cellular therapy are much the same, with the need to identify the most appropriate donor cells for each potential recipient, and to ensure the quality of programs providing the treatment. The development of a public-private regulatory body and a common shared data registry that includes detailed information before and after treatment would greatly enhance progress in bringing cellular therapy to patients in need as it has for organ transplantation."

– Dr. Fred Sanfilippo

Director, Emory – Georgia Tech Healthcare Innovation Program, and Medical Director – Trustee, The Marcus Foundation

"Aging is a stem cell problem. It results from a shift in the balance of undifferentiated to differentiated cells. It disrupts the natural renovation and remodeling process that occurs in tissues, and it corrupts the normal response to injury. Thus, stem cells may, in fact, serve as the fountain of youth and allow us to retain high quality, high performing tissues and organs as we age. Stem cells are resident in all of our tissues, they drive the natural process of renovation and repopulation and restructuring of our organs and tissues in response to disease and injury."

– Dr. Robert Hariri

Founder and Chairman, Celgene Cellular Therapeutics and Co-Founder and Vice Chairman, Human Longevity, Inc.

"Improved global health is eminently achievable, because more than 80% of deaths from chronic diseases could be prevented. The science is now showing us that the specific choices that people make in their daily lives can play an instrumental role in reducing the burden of disease. Food is an untapped and equitable solution for health. That's because the cellular science is revealing new dimensions about the food we eat and liquids that we drink."

– Dr. William Li

President and Medical Director, Angiogenesis Foundation

"Stem cells really hold a promise to significantly improve patients' lives by curing diseases. To me, it is the medicine of the future providing solutions to unanswered problems, and philanthropy provides the vehicle to rapidly accelerate the pace of that discovery coming."

– T. Denny Sanford

Chairman and Chief Executive Officer, United National Corporation and Health Care Philanthropist

CELLULAR SOLUTIONS
A Hopeful Journey

Elizabeth Cougentakis

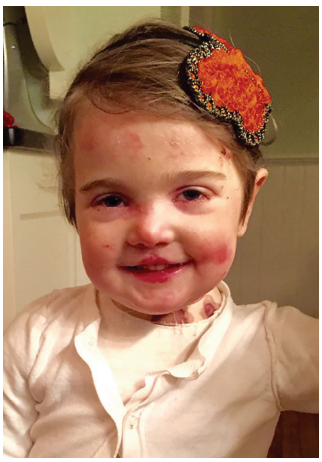
Elizabeth Cougentakis was healthy, athletic and an outstanding student until 2004, when at age thirteen, she was diagnosed with myasthenia gravis (MG). First, her vision and her ability to walk and feed herself were compromised. Then it became difficult to breath and quickly Elizabeth was completely disabled. Six months later, she was intubated to breathe and fed through a feeding tube.

MG, a neuromuscular autoimmune disorder, leads to muscle weakness and fatigue and can sometimes be managed with medications. Unfortunately, for Elizabeth, nothing worked and she continued to deteriorate. She was evaluated by several neurologists, had her thymus removed and underwent 17 sessions of IVIg and numerous plasmapheresis treatments. Desperate to save her life, her parents took her to Venezuela for an evaluation from another neurologist. Again, the treatment was ineffective and the doctors told her that she would be bedridden for the rest of her life.

In 2006, after nearly two years of hospital admissions and being dependent on ventilators and feeding tubes, Elizabeth joined Dr. Richard Burt's stem cell study at the Feinberg School of Medicine at Northwestern University in Chicago. Dr. Burt uses autologous hematopoietic stem cell transplantation to treat numerous autoimmune diseases, including MG. He harvests the patient's own stem cells, administers a short course of chemotherapy and then reinfuses the stem cells to regenerate the immune system.

Elizabeth felt the results of the procedure almost immediately when she gained control of her eyes. She could chew her food and progressively all of her symptoms disappeared. Within a year after the procedure, Elizabeth recovered and has been healthy requiring no medication for the last eight years.

STEM CELL THERAPIES FOR RARE DISEASES
In Development



Elisa McCann

When two-year-old Elisa McCann was born in Palermo, Italy, she was missing a patch of skin on the back of one hand. The following day, blisters erupted all over her body. Doctors in Rome later diagnosed her with recessive dystrophic epidermolysis bullosa, or RDEB, a rare genetic condition characterized by painful blistering skin injuries from the slightest scrape or bump. The skin of EB patients is so fragile that it is sometimes compared to butterfly wings. Children with severe cases live in constant pain and with chronic wounds that often become infected.

EB is rare, affecting twenty in every one million babies in the United States and comes in several forms that can range from mild to severe and can cause painful disfigurement and early death, typically before the age of 30. EB is universally regarded as one of the most painful and difficult conditions and children like Elisa suffer daily, with no cure available. Understanding this, Elisa's parents sought the best care for their daughter and reached out to Dr. Jakub Tolar at the University of Minnesota Masonic Children's Hospital. Dr. Tolar uses hematopoietic stem cell transplantation to treat rare genetic disorders, including RDEB. In mid-2014, the McCanns moved to St. Paul, Minnesota, where Elisa received a bone marrow transplant from her sister, Stella. Since then, she has shown improvement in the course of her disease, and Dr. Tolar is very optimistic about her future with continued treatments. For the first time, Elisa is able to walk and to hold her parents' hands. While her skin still blisters, it now heals on its own.

Dr. Tolar's experimental stem cell procedure may one day change the lives of children born with EB and help improve their condition with a life-altering therapy that will repair and strengthen their skin, extending both the quality and duration of their lives in the process. Elisa McCann, like many children with EB, is remarkably inspiring and exhibits unimaginable strength and courage in facing adversity.

LIVING WITH TYPE 1 DIABETES
Gary Hall, Jr.'s Story


Gary Hall, Jr.

Gary Hall, Jr., a ten-time Olympic medalist, was diagnosed with type 1 diabetes when he was 24 years old. He first noticed increasing thirst and trembling in his hands during workouts that were only corrected after drinking lots of Gatorade.

Hall was training for the 2000 Sydney Olympics when he heard from his doctors that his swimming career was over. Instead of quitting, he set out to take his practices day by day. He persevered and won 4 medals in Sydney including a gold in the 50 meter freestyle and went to Athens in 2004, where he defended his title.

But it hasn't been easy. After the 4 x 100 medley relay where he achieved the rank of the best swimmer in the world, he was found nearly unconscious, underneath the bleachers with a blood sugar of 23. Diabetes needs to be carefully and diligently managed and while there are moments of triumph or control, even, it is a deadly and devastating disease that may cause someone to lose his or her life in the blink of eye.

Since his retirement from swimming, Gary has become a diabetes patient advocate inspiring young T1D patients that diabetes should not be a barrier to achieving goals and dreams even if they include winning an Olympic gold medal. He is focusing on bringing awareness to the disease and funding for research, so that effective treatments and even cures can be realized through medical innovation and cell therapy.

FUNDING CELLULAR SOLUTIONS
Lessons from the Type 1 Diabetes Community


Photo courtesy of iStock

While the scientific and medical fields are often plagued by competition and lack of rapid information sharing, a collaborative network in the field of type 1 diabetes (T1D) research has been established to accelerate efforts toward preventing, treating and ultimately curing the disease. This network involves JDRF, The Helmsley Charitable Trust, and the T1D Exchange that coordinate their efforts with one another, the NIH and other funders, researchers and clinicians in order to advance the progress and minimize the time to impact.

JDRF is the world's largest and oldest private funder of type 1 diabetes research, investing over $2 billion dollars in research since its inception in 1970. The Helmsley Charitable Trust's type 1 diabetes program launched in 2008, which focuses on prevention, treatment and improvement of outcomes for T1D patients, has awarded over 350 grants totaling over $300 million dollars. Together they fund the T1D Exchange, established in 2009 and focused on patient engagement activities with a mission to aggregate patient data to accelerate clinical trial recruitment, physician participation and to create a comprehensive platform of researchers, clinicians and patients.

T1D Exchange is an integrated, patient centric research model that is the first to connect all parties in the development process - patients, researchers, clinicians and industry. It facilitates conducting better research faster by acquiring patient-driven data and providing access to resources to researchers to conduct clinical studies and improve clinical care. The Exchange aggregated data from over 28,000 patients and works with 75 clinics around the US that treat over 100,000 people. It has also created a biorepository with 2,000 samples and established the 'Glu' online patient and caregiver community with tens of thousands of participants touched by T1D. The Exchange is already a proven model of research acceleration and is now being shared through Unitio, a nonprofit established to manage the T1D Exchange and share its expertise with other disease groups.

The leaders of the three organizations agree that they are parts of one ecosystem with the same goal – a world without type 1 diabetes – but with different approaches to getting there. They were able to develop trust among their organizations through transparent practices, really engage with each other to be most impactful, create an environment where barriers and challenges can be addressed together, and to leverage each other's strengths and resources to advance the field forward.

ACCESS TO MODERN MEDICAL SOLUTIONS IN DEVELOPING NATIONS

There is a great disparity in access to proper medical care in countless communities around the globe – both in developing and developed countries. This is more pronounced in the low and middle-income countries, where nearly 2 billion people lack access to medicines and proper medical care. Ninety percent of childhood cancer deaths occur in developing countries. Approximately all 6.6 million (99 percent) of child deaths that occur in children under the age of 5 are caused by conditions that are easily treatable with access to modern medical care provided by medical personnel with knowledge of treatments and preventative measures.

There are various ways in which access to modern medical care can be brought to the emerging markets. This requires:

- infrastructure investments by the local governments or foreign partners to improve patient mobility and connectivity with points of care;
- training of medical personnel on standards of care and preventative measures;
- programs to bring modern equipment especially in diagnostics and affordable medicines to areas in need.

Pharmaceutical companies are beginning to do their part as we have a moral obligation to increase access to medical solutions in the developing world. GSK and Pfizer are 2 examples of drug companies improving access by adjusting pricing for low and middle-income countries. GSK, for instance, sells their patented medicines by keeping prices at no more than 25 percent of developed-world pricing.

Dr. Raphael Rousseau, Group Medical Director and Global Head, Pediatric Oncology, Genentech, Inc. believes that local physicians need to be trained on the newest developments in medical science and innovative drugs. This can only happen through collaboration between local and foreign entities. One way of doing so is “intellectual philanthropy,” described by Dr. Tanya Trippett of the Memorial Sloan Kettering Cancer Center in New York, as a way to leverage and offer the expertise and resources of western doctors to train doctors in low-income countries and use cooperation with international, private and public organizations to bring equipment and knowledge to places in need. Together with Ambassador Eugène-Richard Gasana, Agnes Gasana and The Eugène Gasana Jr. Foundation, Dr. Trippett is exercising intellectual philanthropy through the development of a regional pediatric cancer center in Kigali, Rwanda, which will provide medical care to children suffering from cancer in East Africa. To learn more please visit: http://www.eugenegasanajrfoundation.org.

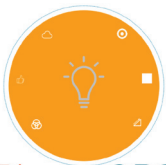
CLINICAL INSIGHTS

A New Paradigm in Drug Discovery and Development: The Impact of Big Data

The biggest barriers to success in developing pharmaceuticals are the length of time to get through the approval process, cost and incidence of failures in marketing approval. One in every ten drugs that make it to Phase 2 successfully reaches regulatory approval and captures significant market share. It is critical to have an early understanding of mechanisms of action and learn from the vast information in the literature and other trials to determine if, in fact, the drug that did not work for a single indication can be re-purposed (dose, delivery, patient population or indication) so that value can be recaptured from a failed development cycle. Each of the drugs that fail represents a significant investment that could potentially be recovered if a new, sounder scientific hypothesis leads to the right disease or patient segment.

Big Data can aggregate the many scientific discoveries in the life sciences (>5000 papers added to the published scientific literature every day), and the sheer amount of domain-specific data generated is a valuable repository of information to transform the current model of drug discovery and development. An example of this is the development of BXCL701. BXCL701 is an oral, small molecule immuno-modulator with established clinical safety, demonstrated modulation of relevant cytokine and immune cells in humans as well as single agent efficacy in immune-responsive tumors like melanoma. This compound was selected by the use of a comprehensive relationship map that links immune-evasion and immune-activation pathways and molecular targets with thousands of pharmacological agents and tumor indications, which was analyzed to rationally select a pipeline of clinically validated agents based on their ability to alter the tumor microenvironment and therefore be combined with approved immune checkpoint inhibitors to optimize their anti-tumor activity. The Big Data generated hypothesis by BioXcel’s proprietary cloud-based pharma analytics platform with input from the Chief Science Officer, Krishnan Nandabalan, Ph.D., created a new value across the entire drug lifecycle. It determined that such a compound would synergistically combine with immune-checkpoint inhibitors, which was confirmed in preclinical experiments and is in process of being tested in an upcoming clinical trial.

Harnessing big data in a way that can normalize, harmonize and make it receptive to the extraction of unique ‘signals’ can be transformative for the drug discovery, clinical development, and commercialization process lowering cost, shortening time to market and increasing probability of success. Interrogating large “data lakes” by posing complex questions can yield new pertinent information in the form of real-time actionable insights by highly skilled teams of domain experts.



2016 PONTIFICAL KEY INNOVATION AWARD

Sanford Health

From left to right: T. Denny Sanford, Kelby Krabbenhoft, Cardinal Gianfranco Ravasi, Monsignor Tomasz Trafny and Dr. Robin L. Smith

Delivery of medical breakthroughs relies upon transformative thinking and the ability to weigh cultural considerations with game-changing innovation that enhances patient outcomes and drives organizational efficiencies. Kelby K. Krabbenhoft and Sanford Health have taken this powerful way of thinking one step further by adding creativity and ingenuity, leading Sanford Health to deliver the highest quality of care, providing convenient access to expert medical services as well as cutting-edge technologies in world-class facilities. This level of innovation requires support from the community, as well as resources. T. Denny Sanford's philanthropy and generosity has enabled Sanford Health's mission and Kelby's vision to improve the human condition in the local community, across the region and around the world.

Sanford Health is improving the human condition through an exceptional commitment to research, education and community growth by establishing a global footprint with regional customization. Together, Kelby K. Krabbenhoft, T. Denny Sanford and the entire Sanford Health staff have made an impact, one that will undoubtedly extend into the future and have a lasting effect on society for which they were awarded the 2016 Pontifical Key Innovation Award on April 28, 2016.

2016 PONTIFICAL KEY PHILANTHROPY AWARD

Sean Parker

From left to right: Sean Parker, Cardinal Gianfranco Ravasi and Dr. Robin L. Smith

The 2016 Pontifical Key Philanthropy Award was awarded to entrepreneur and philanthropist Sean Parker on April 30, 2016, for his extraordinary generosity, vision and philanthropy. Sean Parker continues to show a resounding commitment to improving the human condition by tackling issues and projects that are not straightforward and require innovative thinking and resources. Mr. Parker's highest ambition is to ensure that all people have access to innovative, effective and affordable health care.

The creation of The Parker Institute for Cancer Immunotherapy with a $250 million gift is another example of Mr. Parker's out-of-the-box thinking and leadership. The Parker Institute builds on Mr. Parker's major philanthropic support, which will help to reinvigorate the world of charitable giving similar to what he accomplished with social media nearly a decade ago. He transformed online social activism and philanthropy for the masses via Facebook Causes, which has enlisted 180 million people to donate funds and take action for social causes. Five years ago, Mr. Parker helped to establish Stand Up 2 Cancer and the Cancer Research Institute's Immunotherapy Dream Team. In 2013, he was honored by CRI with the Oliver R. Grace Award for Distinguished Service in Advancing Cancer Research.

Mr. Parker has publicly affirmed his commitment to give away the majority of his fortune in his lifetime and in June 2015, he established the Parker Foundation with an initial gift of $600 million. As a father of two young children, he is an advocate of the moral imperative to "give back" and is personally deeply engaged in encouraging others to give, stating that he wants to create a model for other young philanthropists to follow.

CELLULAR HORIZONS AWARDEES

2016 PONTIFICAL KEY VISIONARY AWARD

Dr. Patrick Soon-Shiong

As a Chinese-American reared in apartheid South Africa, Dr. Soon-Shiong's efforts to enhance health care transcend borders and boundaries. Dr. Soon-Shiong is unique among all medical innovators of our day – his work blends visionary thinking with real action. He has developed an entire ecosystem of health care and technology companies (NantWorks), creating a transformative global health information and next generation pharmaceutical development network for the secure sharing of genetic and medical information. He has a burning passion to cure cancer and is developing cancer therapies based on unlocking the genetic code of cancers to allow the body's own immune system to destroy cancer cells.

Dr. Patrick Soon-Shiong received the 2016 Pontifical Key Visionary Award in the Vatican on April 29, 2016.

From left to right: Monsignor Tomasz Trafny, Dr. Patrick Soon-Shiong, Dr. Robin L. Smith and Cardinal Gianfranco Ravasi

NEW VATICAN APPOINTMENTS FOR DR. W. E. BOSARGE AND DR. ROBIN L. SMITH

Dr. W. E. "Ed" Bosarge Appointed Pontifical Council Admonitor and Senior Advisor for Regenerative Medicine and Adult Stem Cells

Dr. Ed Bosarge was appointed Pontifical Council Admonitor and Senior Advisor for Regenerative Medicine and Adult Stem Cells by the Pontifical Council for Culture on April 28, 2016. This is the third time Dr. Bosarge has been honored at the Vatican – he was awarded the Key Guardian Award in 2011 and Key Philanthropy Award in 2013. Dr. Bosarge has also served as Personal Advisor to the President of Pontifical Council for Culture Cardinal Gianfranco Ravasi in matters regarding regenerative medicine.

Cardinal Gianfranco Ravasi and Dr. W. E. Bosarge

Dr. Robin L. Smith Appointed a Dame Commander of the Pontifical Equestrian Order of Saint Sylvester Pope and Martyr

Pope Francis appointed Dr. Robin L. Smith Dame Commander of the Pontifical Equestrian Order of Saint Sylvester Pope and Martyr. The recognition is intended to honor Dr. Smith for her active involvement as a member of the Board of Trustees of the Vatican's Science and Faith Foundation and for her contribution to building bridges and fostering a dialogue between science, faith and culture. The award was presented to Dr. Smith by His Eminence Cardinal Pietro Parolin, Vatican Secretary of State on April 29, 2016.

Cardinal Pietro Parolin and Dr. Robin L. Smith

HUMANS 2.0

Advancements in bio-medical engineering and gene-editing will allow us to prolong life, design our children and intervene into the workings of our own biology on multiple fronts in the near future. This will bring a transformation for the generations to come and raises many anthropologic, ethical, societal and social questions and issues.

If you had a chance to prolong your life by 50 years, would you? This could strain our environment, already struggling with population growth. But if the years of life were not only plentiful but also high in quality with increased productivity would that make a difference?

If you can select for greater intelligence, physical fitness and ability in your future child, would you? What happens if tastes change? What seems great today may become passé in the next generation leaving future generations at a disadvantage. “One parent’s enhancement, is another kid’s curse,” according to Rev. Fr. Nicanor Austriaco. This rings true since our understanding of the human genome is still limited and changes of one gene may stay silent for many generations.

The CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) technology was first introduced in 2012 and is being used to edit DNA of mice and plants as well as to develop therapies for many human diseases. CRISPR holds the promise to be able to make man-made genetic changes so it is important to answer these questions today, as we must be cautious of the unintended consequences that may be passed

on to future generations.

In late 2015, U.S. National Academies of Sciences and Medicine together with the Chinese Academy of Sciences and U.K.’s Royal Society hosted The International Summit on Human Gene Editing discussing the ethical and societal implications of human gene editing. The Summit discouraged germline research until efficacy and safety concerns have been addressed so that optimal potential benefits can be realized in the future. It is believed that human genetic modifications will someday become useful but should these techniques be banned we would risk impeding the clinical translations of the technology in the future.

Nonetheless, the use of CRISPR technology is evolving and a clinical trial by Editas Medicine in Cambridge, Massachusetts, will commence in 2017 using CRISPR to treat a rare form of eye disease called Leber congenital amaurosis in humans to establish the safety and efficacy of this technique in treating diseases in adults. As to the answers to the challenging questions above, we will have to start answering these as a society in the upcoming decades.

THANK YOU TO OUR STEERING COMMITTEE

On behalf of the Stem for Life Foundation, The STOQ Foundation and Vatican’s Pontifical Council for Culture, we would like to extend a heartfelt thank you to the 2016 conference steering committee. We acknowledge your dedication and passion for this work and appreciate your invaluable contributions to the cell therapy community at large.

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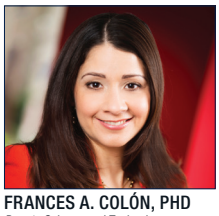

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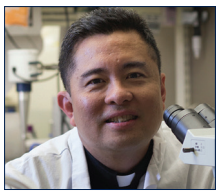

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