

Gateway for Cancer Research has been devoted to funding Phase I and Phase II clinical trials for nearly 30 years. See how you advance medical science by making those trials possible!

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LETTER FROM THE PRESIDENT

by **Michael Burton**, President and CEO, Gateway for Cancer Research



July is Sarcoma Awareness Month. A large group of cancers of the bones and soft tissues, sarcomas are relatively uncommon, but certain types – such as osteosarcoma in children – can

be devastating. There hasn't been much improvement in patient outcomes for osteosarcoma in decades, which is one reason why Gateway is committed to advancing sarcoma research.

In this issue of *Cures TODAY* you'll read about a clinical trial of a promising new immunotherapy drug combination currently underway at Children's Hospital Los Angeles, sponsored by the Sarcoma Alliance for Research Through Collaboration, a non-profit research consortium and network of member medical centers in the U.S. The collaboration not only extends this potentially life-changing clinical trial to children at up to 13 other medical centers around the country, it also ensures the

patient's voice and perspective are the focus, which is the core of Gateway's mission.

Gateway exists to find and fund game-changing early-phase cancer research. In 2020 – a year like no other – we were unwavering in that commitment. At a time when many research foundations pulled back, Gateway charged ahead into a record year of grantmaking in our 30-year history. With the support of our founders, our funders and our partners in research, we awarded nearly \$10 million in grants for cancer research.

This kind of progress is only possible through the generosity of those who appreciate the value of investing in early-phase cancer research. The return on investment comes in the form of new treatments, improved therapies and practice-changing advances in cancer care that make a lasting difference in patients' lives.

As always, on behalf of all of us at Gateway, thank you for sharing our commitment to advancing cancer care and cures for the patients we serve. ■



99 CENTS OF EVERY DOLLAR RECEIVED DIRECTLY FUNDS CANCER CLINICAL TRIALS

EXTRAORDINARY MEASURES YIELDS EXTRAORDINARY RESULTS

For the nearly 5,000 Americans every day who receive a cancer diagnosis, the global COVID-19 pandemic delivered a double dose of complexity and heartache. Many vulnerable cancer patients opted to delay treatment, and clinical trials were suspended as cancer centers reallocated resources to pandemic response. Countless cancer patients around the world had limited access to clinical trials of promising investigational therapies.

To address this unprecedented threat to cancer care and clinical research, Gateway launched *Extraordinary Measures*, an innovative grant program



GATEWAY
EXTRAORDINARY MEASURES

to bring cancer clinical trials directly to patients' doorsteps. The program funds "site-less" early-phase clinical oncology studies that leverage technologies like telemedicine, wearables and smartphone apps, and remote monitoring to improve patient access to cancer clinical trials.

The generous response from Gateway's supporters redefines the word extraordinary. To date, *Extraordinary Measures* has raised nearly \$3 million for innovative, patient-centered, site-less cancer clinical trials, and has two funded studies currently underway.

Sameek Roychowdhury, MD, PhD, of The James

Comprehensive Cancer Center at The Ohio State University, is leading a clinical trial that delivers molecular profiling to the doorsteps of patients with

"In the midst of every crisis, lies great opportunity."

Albert Einstein

certain rare solid tumor cancers, utilizing telehealth consultation with local oncology practices.

At Princess Margaret Cancer Centre in Toronto, Canada, Gary Rodin, MD, FRCPC is studying the efficacy of iCALM, an online mental health program to help patients and their caregivers better manage the psychosocial implications of living with advanced cancer. iCALM can improve quality of life for these patients and expands access for those who cannot attend the in-person CALM program.

Cancer didn't stop for the pandemic. Thanks to *Extraordinary Measures*, neither did Gateway. We have additional "site-less" clinical trial applications currently under review for funding and have extended our search for the most promising patient-centered research until the end of 2021.

We are deeply grateful for the generosity of many donors who seized the opportunity to make a meaningful difference in the lives of those facing cancer at a time of unprecedented need. ■

Nearly
\$3 Million
Raised for Patient-Centered,
Site-Less Clinical Trials!

There's still time to support Extraordinary Measures!
To make a difference today, visit gatewaycr.org/EM.

VINO CON STELLE – AN EVENING UNDER THE STARS

The stars aligned in spectacular fashion on April 23, 2021 at the fourth annual *Vino con Stelle*, Gateway's signature Arizona fundraising event which raised a record-breaking \$772,000 for transformational early-phase clinical cancer research.

Vino con Stelle is a world-class culinary, wine and star-studded philanthropic experience that celebrates the transformational advances in cancer research and patient care that are made possible when leading cancer researchers, physicians and philanthropists come together with passion and purpose.



Global philanthropists Dr. Stacie J. Stephenson, Vice Chair of Gateway, and Richard J Stephenson, Gateway Founder and Chairman, served as hosts and honorary co-chairs for the event. Their generous underwriting ensures that 99 cents of every record-breaking dollar raised goes directly toward funding breakthrough cancer clinical trials at leading institutions worldwide.

This year's event at the Royal Palms Resort and Spa in Phoenix was emceed by multiple Emmy Award-winning journalist Tara Hitchcock, and featured live performances by recording artists Pia Toscano, Loren Allred, Justin Guarini and William Joseph. Pulitzer Prize-winning author, oncologist and Gateway-funded investigator Siddhartha Mukherjee, MD, DPhil, delivered a keynote address.

Guests enjoyed culinary wine pairings curated by renowned restaurateur Mark Tarbell, whose eponymous Tarbell's Restaurant in Phoenix has earned more than 50 "Best Of" awards, including Food & Wine Best Restaurant.



Gateway celebrated Arizona Cardinals wide receiver Larry Fitzgerald with its prestigious Leadership in Cancer Advocacy Award, which honors individuals or organizations who have made outstanding advocacy efforts on behalf of cancer patients and who are active in advancing treatments and cures for cancer. The Larry Fitzgerald Foundation issued \$1 million in grant funding for cancer research in 2020.

Rounding out the evening was a spirited auction, presided over by Yve Rojas, of one-of-a-kind experiences including Tarbell's Elite Collector's Experience, Getaway with the Mondavis, Exceptional Jewels by Robert Procop, and a "Vibrant Girls Getaway" wellness-themed escape to St. Thomas, with best-selling author and Gateway Vice Chair, Dr. Stacie Stephenson, the VibrantDoc™, which sold for \$75,000.

Gateway extends its deepest gratitude to the *Vino con Stelle* host committee members and all who came together for an evening under the stars.

Thank you for joining us in our life-saving mission to fund the transformative cancer research that will forever change the face of cancer care. ■

SAVE THE DATE – NOVEMBER 13, 2021

30th Anniversary Cures Gala

Mark your calendars and get ready to have a ball! *Cures Gala* co-hosts Dr. Stacie J. and Richard J Stephenson are excited to welcome you to an unforgettable celebration of 30 years of advancing early-phase cancer research and delivering on our promise to help those facing cancer feel better and live longer. Plan to join us! Together we can shape a world in which a cancer diagnosis is no longer feared. ■



A ONE-TWO PUNCH FOR OSTEOSARCOMA

Osteosarcoma is the most common malignant bone cancer in children, teens and young adults, and it is a significant challenge to treat. The current standard of care involves an intensive, multiple-drug chemotherapy regimen that causes detrimental side effects which can limit quality of life. The prognosis for long-term survival is poor, especially for those whose cancer has already spread at the time of diagnosis, or resists or recurs after initial treatment.

Gateway-funded investigator and osteosarcoma expert Fariba Navid, MD, of Children's Hospital Los Angeles (CHLA), is testing a new approach that enlists a patient's own immune system to attack the cancer and boost the effectiveness of a drug that shuts down the molecular signals that enable the cancer cells to grow.

In a multi-center Phase II study, patients five years of age or older with advanced osteosarcoma will receive a combination of immunotherapy drug, nivolumab, with targeted therapy, regorafenib.

Nivolumab belongs to a class of drugs called immune checkpoint inhibitors. A protein found on immune system T-cells, called PD-1, acts as a checkpoint "brake" on the ability of the T-cells to recognize and destroy cancer cells. Nivolumab blocks PD-1 signaling, releasing the "brakes" and increasing T-cells' cancer-killing potential. While anti-PD-1 inhibitors have proven effective against other cancer types, they have shown little benefit as single-line therapy for osteosarcoma, to date.

Regorafenib is a targeted small molecule drug which blocks the MAPK and VEGF molecular signaling pathways that cancer cells use to grow, proliferate, and create the network of blood vessels that support a tumor. Recent clinical trials of regorafenib have shown an improvement in progression-free survival of patients with relapsed or resistant osteosarcoma.

Dr. Navid and her collaborators believe the combination of the two drugs will extend patient survival longer than for those patients who were treated with regorafenib alone. The combination should also be well-tolerated and minimize side effects that can disrupt daily life activities, researchers say. If successful, they will have created a promising new treatment option for a cancer type that has shown no improvement in patient outcomes in over 30 years.

Sponsored by SARC (Sarcoma Alliance for Research Through Collaboration) in partnership with CHLA, and with funding support from Gateway and other organizations, this clinical trial (SARCO38) is already enrolling patients and will be opened at up to 13 SARC collaborating sites in the United States. SARC is a non-profit research consortium and network of member medical centers in the U.S. and globally dedicated to advancing sarcoma research and advocacy. The CHLA-SARC partnership supports the patient and caregiver engagement that is at the heart of Gateway's mission and brings a promising new therapy for osteosarcoma to children across the country.

Patients interested in participating in this clinical trial are encouraged to share and discuss this article with their doctors. Additional information regarding this trial may also be found at clinicaltrials.gov (NCT04803877). ■



Fariba Navid, MD

/ WORD CUREIOSITY /

SARCOMA

Definition: The general term for a group of cancers that develop in the bones and soft tissues, such as muscles, tendons, nerves and blood and lymph vessels that connect, support and surround other body structures. They can occur anywhere in the body but are most commonly found in the arms, legs, chest and abdomen.

Application: There are more than 70 types of sarcoma. They are relatively uncommon, with fewer than 200,000 cases diagnosed each year in the U.S. Treatment for sarcoma varies depending on type and location in the body and may include surgery, radiation therapy, chemotherapy or immunotherapy.