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Middle East HEALTH

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November- December 2024

SUPPLEMENT

Biomedicine in the United States:

Advancing global health through scientific innovation

Leading US hospitals showcase their specialties

Supplement to Middle East Health

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Cedars Sinai

BEST HOSPITALS U.S.News HONOR ROLL 2024-2025

Excellence in Healthcare

Cedars-Sinai has been named to the Honor Roll for the ninth consecutive year in *U.S. News & World Report*'s "Best Hospitals 2024–25" rankings. As a global leader in healthcare, we offer a full range of services to patients from around the world. Whether we're providing clinical services in your region via video visits or at our renowned medical center, our mission remains unchanged: to give patients access to excellence in healthcare. Located in iconic Beverly Hills, Cedars-Sinai blends cutting-edge medicine in a vibrant setting making it a leading destination for international patients.

Seven Cedars-Sinai specialties were ranked in the top 10 in the U.S.

- Cardiology, Heart & Vascular Surgery
- Diabetes & Endocrinology
- Gastroenterology & GI Surgery
- Neurology & Neurosurgery
- Obstetrics & Gynecology
- Orthopedics
- Pulmonology & Lung Surgery

Rodeo Drive, Beverly Hills

Learn more: cedars-sinai.org/international
cedars-sinai.org/globalcollaborations

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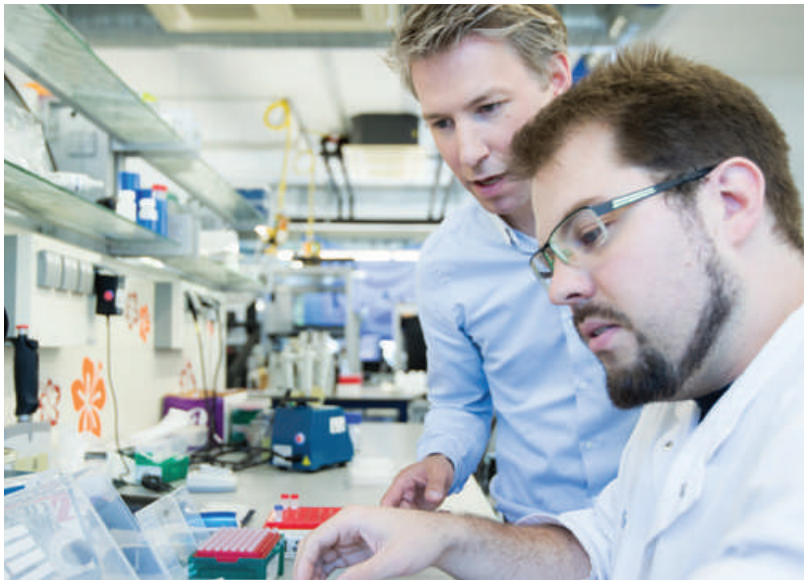

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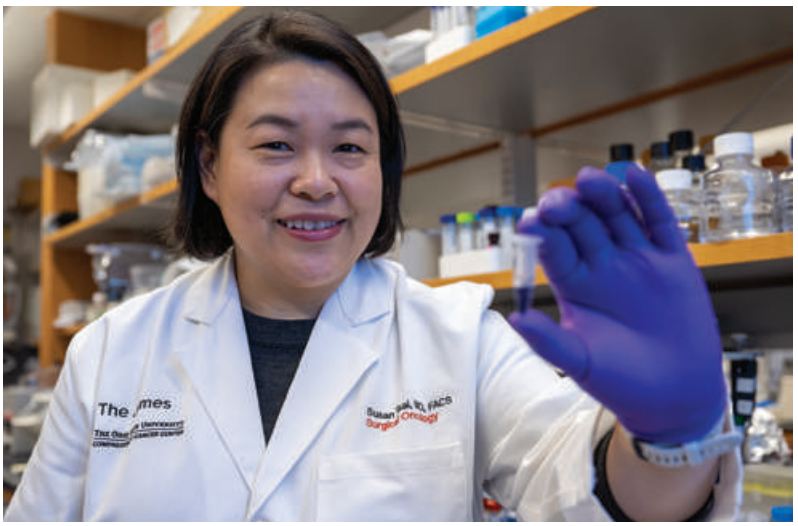
UNITED STATES HOSPITALS



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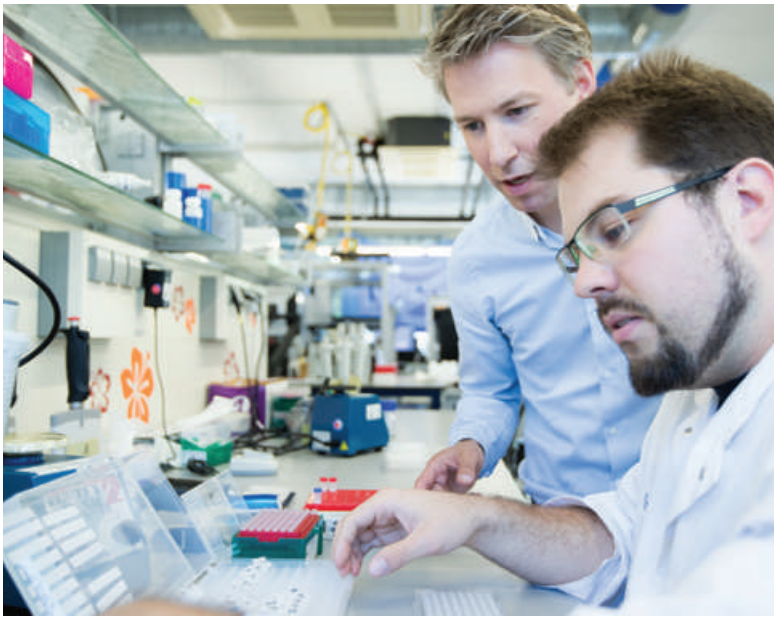
America's biomedical research domain: Advancing global health through scientific innovation



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From mapping the intricacies of the brain's waste-clearance system to developing breakthrough CAR-T cell therapies for childhood brain cancers, American biomedical research continues to push the boundaries of medical science. With the National Institutes of Health (NIH) supporting over 300,000 researchers across 2,500 institutions, US laboratories are making unprecedented advances in neurodegenerative diseases, precision medicine, and cancer treatment, among other medical specialties. These discoveries are transforming our understanding of human biology whilst developing innovative therapies for previously intractable conditions.

The United States maintains unparalleled dominance in biomedical research, driven by substantial federal funding and a sophisticated research infrastructure. At its core, the National Institutes of Health (NIH) operates as the world's largest public funder of biomedical research, with an annual budget exceeding US$45 billion [1], facilitating groundbreaking discoveries across the medical sciences.

The fiscal impact of American biomedical research extends beyond direct scientific outcomes. In fiscal year 2022, NIH funding generated $96.84 billion in economic activity, with a multiplication factor of 2.64 for every dollar invested. This funding supports a vast network of over 300,000 researchers across more than 2,500 institutions, creating a research ecosystem that spans the nation's academic and medical centres.

Neuroscience innovation

Recent breakthrough studies in neuroscience exemplify American research sophistication. Researchers at Oregon Health & Science University have definitively demonstrated the existence of the glymphatic system [2] in living human subjects, a significant advancement from previous mouse model studies. Using gadolinium-enhanced MRI with T2/FLAIR imaging, they visualised distinct cerebrospinal fluid channels along perivascular spaces, confirming the presence of this crucial brain waste-clearance system.

This discovery holds particular significance for understanding neurodegenerative disorders, as the glymphatic system's function appears closely linked to sleep-dependent clearance of pathological proteins, including beta-amyloid and tau. The confirmation of this system in humans provides new therapeutic targets for conditions such as Alzheimer's disease and other neurological disorders.

Immunotherapy developments

Stanford Medicine's groundbreaking work with CAR-T cell therapy [3] for diffuse intrinsic pontine glioma (DIPG) demonstrates America's capability to rapidly translate basic science into clinical applications. Their approach, targeting the GD2 surface marker on DIPG cells, achieved significant clinical responses in a phase I trial. Of eleven participants, nine showed clinical benefits, with four experiencing tumour volume reductions exceeding 50%, and one achieving complete response, meaning his tumour disappeared from brain scans. Although it is too soon to say whether he is cured, he is healthy four years after diagnosis, the study authors say.

The trial's success in treating DIPG, which typically has a median survival of approximately one year and a five-year survival rate below 1%, represents a significant advancement in paediatric neuro-oncology. The therapy's receipt of regenerative medicine advanced therapy designation from the FDA exemplifies the regulatory framework's ability to accelerate promising treatments to clinical use.

Precision medicine and sexual dimorphism

American researchers are advancing understanding of sexual dimorphism in therapeutic responses. Recent studies at the University of California, San Diego, revealed distinct mechanisms of meditation-induced pain relief between sexes [4]. While both males and females experienced pain reduction through meditation, only males showed dependence on the endogenous opioid system, as demonstrated through naloxone blockade studies. This finding suggests fundamentally different pain-processing mechanisms between sexes, with implications for analgesic development and pain management strategies.

Molecular neurology and disease progression

The Seattle Alzheimer's Disease Brain Cell Atlas consortium has mapped cellular and molecular changes in Alzheimer's disease

progression with unprecedented resolution [5]. Their analysis of the middle temporal gyrus from 84 donors revealed two distinct disease phases, characterised by different patterns of cellular dysfunction and protein accumulation.

The early phase showed sparse amyloid-β plaques and tau tangles with slow growth, accompanied by microglial inflammatory gene activation and oligodendrocyte loss. The later phase demonstrated exponential increase in pathological protein accumulation and widespread neuronal loss. This detailed temporal mapping of disease progression provides new therapeutic windows and cellular targets for intervention.

Research infrastructure advantages

Several structural elements contribute to America's continued leadership:

1. **Funding mechanisms**: The NIH's peer-review system ensures rigorous project selection while maintaining scientific independence. This system supports both investigator-initiated research and targeted program announcements, allowing flexibility in addressing emerging health challenges.
2. **Clinical trial infrastructure**: An extensive network of academic medical centres and research hospitals enables rapid protocol development and patient recruitment. This infrastructure supported over 60,000 NIH grants in the last fiscal year alone.
3. **Technological innovation**: American institutions maintain cutting-edge technology platforms, including advanced imaging systems, high-throughput screening facilities, and sophisticated data analysis capabilities.
4. **Regulatory framework**: The FDA's adaptive licensing pathways, including breakthrough therapy and regenerative medicine advanced therapy designations, facilitate rapid translation of promising treatments.

Global impact and knowledge dissemination

The influence of American biomedical research extends globally through various mechanisms:

1. **Open science initiatives**: NIH-funded research typically requires open access publication, facilitating global knowledge dissemination.
2. **International collaboration**: American institutions frequently engage in multicentre trials and collaborative research networks, enhancing global research capacity.
3. **Scientific training**: U.S. research centres train international scientists in advanced methodologies and experimental techniques, creating a global network of expertise.
4. **Therapeutic development**: Novel treatments developed in American laboratories often become available internationally through licensing agreements and clinical trials.

Despite its leadership position, American biomedical research faces several strategic challenges:

- **Funding competition**: Emerging research powers, particularly in Asia, are increasing their investment in biomedical research infrastructure.
- **Workforce development**: Maintaining technical expertise requires sustained investment in advanced training programs.
- **Translation efficiency**: Optimising the pathway from discovery to clinical implementation remains crucial for maintaining research impact.

The United States maintains its position as the global leader in biomedical research through a combination of substantial funding, sophisticated infrastructure, and strategic regulatory frameworks. This leadership continues to produce significant advances across multiple medical disciplines, from basic science to clinical applications. As global health challenges become increasingly complex, America's research capabilities remain essential for developing evidence-based solutions that advance medical science worldwide.

The success of American biomedical research relies on continued commitment to scientific excellence, sustained funding, and adaptation to emerging research paradigms. This framework ensures that discoveries made in American laboratories continue to drive global medical advancement and improve patient outcomes across the world.

Strength of biomedical research lies in collaborative ecosystem

The strength of American biomedical research lies significantly in its collaborative infrastructure. Beyond individual institutional excellence, the US has developed sophisticated networks of research collaboration that span academic medical centres, government laboratories, biotechnology firms, and pharmaceutical companies. This interconnected approach enables rapid translation of basic science discoveries into clinical applications.

The NIH's research centres exemplify this collaborative ethos. For instance, the Seattle Alzheimer's Disease Brain Cell Atlas (SEA-AD) consortium brings together researchers from multiple institutions, combining expertise in neuroscience, genomics, and clinical medicine. Their recent mapping of cellular changes in Alzheimer's disease progression demonstrates how shared resources and cross-disciplinary collaboration accelerate scientific discovery.

Stanford Medicine's breakthrough in childhood brain cancer therapy further illustrates this collaborative approach. Their CAR-T cell therapy development involved partnerships with the Parker Institute for Cancer Immunotherapy, Johns Hopkins School of Medicine, and multiple funding foundations. Such multi-institutional efforts enable sharing of technical expertise, patient resources, and research costs, whilst accelerating the pace of innovation.

Cross-institutional data sharing represents another crucial aspect of US research collaboration. The NIH's BRAIN Initiative has established standardised protocols and reference datasets that researchers nationwide can access and build upon. This open-science approach prevents duplication of effort and ensures that discoveries in one laboratory can rapidly inform research across the country.

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To learn more, contact Northwell Health's International team at (212) 434-6000 or email signature@northwell.edu

Northwell Health®

New leadership at Baptist Health Miami Cardiac & Vascular Institute plans major expansion

Since its founding in 1987, Baptist Health Miami Cardiac & Vascular Institute has become a global leader in cardiovascular medicine. Now, under the leadership of Dr. Tom C. Nguyen, the Institute is embarking on its next phase of growth, focusing on research, innovation, and patient-centered care.

The Institute's latest advancement includes a dedicated arrhythmia center, the first in South Florida and among the few in the U.S. Dr. Nguyen plans to introduce new programs, such as robotic heart surgery, and continue recruiting top cardiothoracic surgeons and specialists from around the world. "Our goal is to make Miami Cardiac & Vascular Institute the crown jewel of cardiovascular care, while always prioritizing the community's needs," he says.

Dr. Nguyen is a nationally recognized expert in minimally invasive heart surgery, with a focus on valvular heart disease. With more than 300 peer-reviewed articles, his research and expertise have been instrumental in advancing heart surgery techniques. Prior to joining Baptist Health, Dr. Nguyen led the cardiothoracic surgery program at the University of California, San Francisco (UCSF), where he co-directed the Heart and Vascular Center and oversaw groundbreaking clinical advancements.

Dr. Nguyen's journey to becoming an internationally recognized surgeon is rooted in resilience. A refugee from Vietnam, he fled the country with his family at the age of four. They arrived in Houston with no knowledge of English and lived in poverty. These early hardships instilled in him the value of second chances and a deep commitment to helping the most vulnerable patients. "Everyone deserves a second chance," he says, reflecting on his mission to serve patients who have been told there are no more treatment options.

His determination to succeed was supported by a strong community and mentors along the way. After graduating with honors in economics from Rice University, Dr. Nguyen earned his medical degree from Johns Hopkins University School of Medicine. He completed a general surgery residency at Stanford University, followed by a cardiothoracic surgery fellowship at Columbia Presbyterian and a transcatheter aortic valve replacement (TAVR) fellowship at Emory University.

A turning point in Dr. Nguyen's life occurred when he returned to Vietnam after medical school and witnessed a man his age working long hours on a shrimping boat. "I wasn't stronger or smarter, just luckier," he recalls. This experience reinforced his dedication to helping others, especially those facing significant challenges.

In his approach to surgery, Dr. Nguyen emphasizes the importance of precision and teamwork. "There are over 100 critical steps in heart surgery with small margins of error. You have to stay composed, and the entire team needs to work together seamlessly," he explains. His collaborative approach is central to the success of the Institute's heart surgery programs.

Dr. Tom C. Nguyen

Beyond his professional achievements, Dr. Nguyen is a dedicated family man. He and his wife, Dr. Gina Landinez, an interventional radiologist, have two young daughters. He is also a passionate advocate for diversity, equity, and inclusion, recognizing the importance of diverse perspectives in healthcare and supporting women's contributions to the field.

"My experiences have shaped me as a doctor and as a person," Dr. Nguyen says. "It's a privilege to care for patients who place their trust in you. Seeing them return to the activities they love, whether it's fishing or hiking, is incredibly rewarding."

For more information, visit *BaptistHealth.net/Heart*, call +1 786-596-2373 or email us at *International@BaptistHealth.net*

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HIGH PERFORMING HOSPITALS U.S.News 2024-2025 HEART ATTACK

HIGH PERFORMING HOSPITALS U.S.News 2024-2025 HEART FAILURE

HIGH PERFORMING HOSPITALS U.S.News 2024-2025 STROKE

For more information or to schedule an appointment, contact us at **International@BaptistHealth.net** or call **786-596-2373.**

Baptist Health

Miami Cardiac & Vascular Institute

Excellence in healthcare

Cedars-Sinai, a leading destination for patients worldwide

Cedars-Sinai has been recognized for nine years in a row on the "Best Hospitals" Honor Roll by *U.S. News & World Report*. Cedars-Sinai's sterling reputation and world-class services make it a leading destination for patients worldwide.

The *U.S. News & World Report* rankings place Cedars-Sinai among the top hospitals in the U.S. in multiple healthcare specialties, including Cardiology, Heart & Vascular Surgery; Gastroenterology & GI Surgery; Diabetes & Endocrinology; Neurology & Neurosurgery; Obstetrics & Gynecology; Orthopedics; and Pulmonology & Lung Surgery.

Specialized care for international patients

Cedars-Sinai's groundbreaking research and specialized care have garnered an international reputation for excellence. Also, located in Los Angeles, California, in the beautiful neighborhood of Beverly Hills, Cedars-Sinai provides patients with the rich surroundings of the lively city, known for its art, culture, music, sporting events, famous landmarks and warm weather.

"Every year, thousands of patients come to Cedars-Sinai from around the world," said Heitham Hassoun, MD, chief executive of Cedars-Sinai International, "We care for patients from more than 100 countries every year. Many need to be treated for complex conditions that the healthcare systems in their home countries are not yet able to manage."

This year, and for the second consecutive time, Global Healthcare Accreditation has recognized Cedars-Sinai for providing international patients with the highest-quality healthcare in the U.S. An exceptional level of personalized concierge care is available to international patients at Cedars-Sinai, including assistance with short- or long-term housing, airline ticketing, ground transportation and medical document review.

Cedars-Sinai's physicians create individual treatment plans for all international patients, and our multilingual staff provides support and advice throughout the full cycle of care.

Last year, Cedars-Sinai opened its International Lounge, a place where patients and their companions can relax in comfort. It includes a quiet room for meditation and prayer as well as conference rooms for meetings and consultations, among other amenities.

"Our goal is to support patients and their families and to make them feel comfortable throughout their care journey. Emotional support is critical to overall health, and we want them to know they have a team by their side at all times," said Christina Khatchadourian, Concierge Services lead.

Big changes

"There are big changes taking place in 21st century global healthcare," Dr. Hassoun said. "Impressive new healthcare facilities are being established in key regions, including the Middle East, Southeast Asia, Eastern Europe and Latin America. Cedars-Sinai continues to expand our collaboration with facilities around the globe."

With a regional office having opened in Mexico City in 2023 and another having opened in Singapore earlier this year, Cedars-Sinai now has multiple regional offices – and more are planned in Ecuador, Qatar and elsewhere.

"By meeting patients where they are, we can connect them with expert care providers at Cedars-Sinai and help coordinate their medical travel to Los Angeles or provide remote video consultations," said Benjamin Seo, Cedars-Sinai International's director of Global Business Development. "We're working to support local healthcare while providing the best possible experience for those who are able to travel to our medical center in Los Angeles."

It's all about the patient

Cedars-Sinai's Patient Experience team continues to strive for excellence at every level of healthcare services.

"Our global network continues to grow, and our expert doctors, nurses, researchers and managers are now reaching people all over the world," Dr. Hassoun said. "But I can guarantee that one thing won't change: The patient is at the center of everything we do. Everyone who comes to Cedars-Sinai can trust that they will receive the very highest quality of healthcare from people who are dedicated to their well-being and progress." MEH

Learn more:
International Patients | Cedars-Sinai
https://www.cedars-sinai.org/international-patients.html
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https://www.cedars-sinai.org/global-collaborations.html

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IU HEALTH Indiana University Health

Expert pediatric care in a healing environment

Located on the East Coast in historical Charleston, South Carolina, U.S.A., the Medical University of South Carolina (MUSC) Shawn Jenkins Children's Hospital (SJCH) has cared for patients and their families since the early 1900's. This long history of providing expert care in over 27 specialties ensures a thoughtful approach to all complex or rare pediatric conditions and diseases.

Opened in February 2020, The MUSC Shawn Jenkins Children's Hospital (SJCH) <*http://musckids.org*> consists of over 250 beds and includes a Level 1 trauma center, the state's only pediatric burn center and solid-organ and bone marrow transplant programs, the state's largest Level 4 neonatal intensive care unit, an advanced fetal medicine center and a top-4 ranked *U.S. News & World Report* children's cardiology & cardiac surgery program for two years in a row. Designed with input from over 200 providers and families passionate about building a healing environment, this new hospital provides unmatched attention and care to the patient and family experience.

MUSC Children's Health pediatric and congenital heart center located in SJCH performed over 15 heart transplants last year and is on pace to exceed that number this year. Our surgical outcomes are among the very best in the United States and our Society of Thoracic Surgeons (STS) data consistently outperforms benchmarks. The third floor of our children's hospital houses our heart center and includes a pediatric cardiology intensive care unit.

Our team treats the full spectrum of heart disorders ranging from babies still in the womb to adults. With a team of experts, including three cardiac surgeons, seven cardiac anesthesiologists, and more than 20 cardiologists, the MUSC Children's Health Pediatric and Congenital Heart Center is among the largest pediatric heart programs in the United States. We offer numerous sub-specialty programs encompassing all types of heart disease. Our depth and experience allow us to specialize in all areas of cardiology, including cardiac catheterization [1], cardiac critical care [2], echocardiography and advanced three-dimensional imaging, heart failure, arrhythmias and electrophysiology.

MUSC Children's Health neurosurgeons routinely perform life-saving and complex procedures that influence global care. Our innovative approach to resolving undiagnosed conditions results in a highly respected program that offers safe and new techniques that families travel long distances to receive. The surgical team teaches their innovations to providers across the globe.

Our **pediatric orthopedic** team regularly cares for spina bifida, cerebral palsy, scoliosis and spinal deformity patients incorporating the latest robotic technology for enhanced surgical precision, shorter hospital stays, and fewer complications. Our three surgeons are all trained to safely perform the most complex skeletal procedures with excellent outcomes.

Housed within the SJCH, the Pearl Tourville Women's Pavilion (PTWP) [3] seamlessly integrates children's care and obstetrical services, thereby enhancing safety and improving outcomes in high-risk pregnancies. Working collaboratively with the advanced fetal care center for families expecting babies with complex congenital birth defects and medical problems, PTWP providers are one of only a handful of teams in the country able to offer couplet-care rooms, where newborns and mothers can recover together in an intensive-care setting. Our 80-bed NICU is one floor above the women's pavilion for swift transport to our neonatal specialists.

The lobby at Shawn Jenkins Children's Hospital

Additionally, we offer excellent outcomes in **pediatric cancer care**, including bone marrow transplant, **ear, nose and throat (ENT)**, **burn care, and burn reconstructive surgery**. Our **ECMO** program is a platinum ELSO-designated hospital, one of 16 pediatric hospitals globally awarded this achievement. Our telehealth and technology allow you to stay in touch with your patient and our care teams.

We are changing what's possible for patients and families across the country and the world. For more information, visit *musckids.org*.

• To refer a patient, please contact our International Services Team at: *https://muschealth.org/patients-visitors/international-patients*.

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3. *https://muschealth.org/medical-services/womens*

Advanced Pediatric Care **is Here**

BEST CHILDREN'S HOSPITALS
U.S.News & WORLD REPORT
RANKED IN 6 SPECIALTIES
2023-2024

The **MUSC Shawn Jenkins Children's Hospital,** located in Charleston, South Carolina, USA, is one of the top-ranked children's hospitals in the Southeast United States. The new 11-floor hospital provides expert care in over 27 pediatric specialties including burn care, cancer, heart surgery, orthopaedics and neurosurgery. Our pediatric heart center is ranked #4 in the United States and achieves among the best outcomes in the nation. To learn more about MUSC pediatric experts, visit **musckids.org**

To refer a patient, please contact our International Services Team at: **muschealth.org/international**

muscShawn Jenkins Children's Hospital
Medical University of South Carolina

Shirley Ryan AbilityLab: Top Global Destination for the Best Rehabilitation Care

Born at just 25 weeks gestation, Matar was diagnosed with cerebral palsy, epilepsy and hydrocephalus, a condition that leads to excess fluid on the brain. His parents were told he wouldn't survive.

Against all odds, Matar did survive. However, his family initially was met with more heartache when they were told by doctors that he would never be able to stand, walk or talk.

At the age of three, Matar traveled with his family from their home in the United Arab Emirates to the United States in hopes of finding the medical support he needed. That journey led them to Shirley Ryan AbilityLab in Chicago, Illinois, and offered a renewed sense of hope.

"Being away from home is very difficult," said Othman, Matar's father. "Yet, we were committed to sacrificing anything and doing whatever it took to find a cure for our son."

When Matar arrived at Shirley Ryan AbilityLab, he was only capable of dragging his body across the floor and walking on his knees. He also could not speak and almost never used his left hand. As he received intensive speech, occupational and physical therapy during his six-month stay at the hospital, progress soon began to take form.

Destination: Ability

Matar's story is not unique. Thousands of patients travel great distances to receive care from Shirley Ryan AbilityLab, the global leader in physical medicine and rehabilitation. The hospital serves adults and children with the most severe, complex conditions – from traumatic brain and spinal cord injury to stroke, amputation and cancer-related impairment.

Its reputation precedes it. In fact, Shirley Ryan AbilityLab has been designated the No. 1 rehabilitation hospital in America by *U.S. News & World Report* annually since 1991. Each year, patients come from around the world to Shirley Ryan AbilityLab's state-of-the-art research hospital in Chicago. There, clinicians and scientists embrace a translational approach that integrates the latest research findings into care. This unique model enables patients to benefit from access to the brightest minds, the latest research and the best opportunity for recovery.

Curated, comprehensive offerings for global patients

For those coming from afar, the Shirley Ryan AbilityLab Global Patient Services (GPS) team members work with patients and their families before, during and after their care process. Beyond coordinating international travel arrangements, GPS case managers also collaborate with numerous third parties, including working with embassies on everything from visas to patient sponsorship.

Additionally, the team manages logistics for transporting patients with physical impairments and other medical needs. GPS case managers link patients and families with service partners who provide a range of resources – interpreting and translation, exchange of medical records, setting up bank accounts and handling other financial matters, obtaining a mobile telephone and cellular service, securing housing, and more.

"Like family"

By the time of his discharge, Matar was walking, talking and using his left hand as he continued to make strides in his recovery.

"Shirley Ryan AbilityLab is so different from any other rehabilitation facility we have ever seen – not only in terms of having the latest technology and the biggest building but, most importantly, because it has the greatest people," said Othman. "The therapists there are like family. From day one, we felt that they genuinely care about their patients. They are truly amazing, and they make miracles happen every day."

Connect with Shirley Ryan AbilityLab

Interested in learning more about Shirley Ryan AbilityLab's best-in-class care? Visit *http://sralab.org/international*

Many Journeys. One Destination.

When recovery from a complex condition is at stake, patients from all over the world choose Shirley Ryan AbilityLab.

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BEST HOSPITALS U.S.News & WORLD REPORT REHABILITATION 2024-2025

#1 Rehabilitation Hospital for 34 Years

Chicago, Illinois, United States

CAR-T cell therapy shows promise against fatal childhood brain cancer

Clinical trial demonstrates first major success using engineered immune cells to treat diffuse intrinsic pontine glioma, with one patient showing complete tumour clearance four years post-diagnosis.

Dr Michelle Monje, Professor of Neurology at Stanford Medicine

Stanford Medicine researchers have reported significant therapeutic benefits using CAR-T cell therapy in children with lethal brain and spinal cord tumours, marking one of the first successful applications of this immunotherapy approach against solid tumours. The findings, published in *Nature* on 13 November 2024, show particularly promising results in treating diffuse intrinsic pontine glioma (DIPG), a uniformly fatal childhood brain cancer that typically claims lives within a year of diagnosis.

Treatment approach

The trial enrolled 13 patients, with 11 receiving the cellular therapy. The treatment involved engineering patients' T cells to target GD2, a surface marker abundantly expressed on DIPG tumour cells. Of the treated patients, nine demonstrated clinical benefits, with four showing tumour volume reductions exceeding 50%. Most notably, one patient achieved complete tumour response, meaning his tumour disappeared from brain scans. Although it is too soon to say whether he is cured, he is healthy four years after diagnosis, the authors report. The researchers' preclinical studies had identified GD2 as a promising target in 2018, leading to this first human trial of CAR-T cells for DIPG.

"This is a universally lethal disease for which we've found a therapy that can cause meaningful tumour regressions and clinical improvements," said Dr Michelle Monje, the trial's lead author and Professor of Neurology at Stanford Medicine. The median survival time post-diagnosis was 20.6 months, with two patients surviving beyond 30 months. This represents a significant improvement over the typical median survival time of about one year.

Safety considerations

The research team identified optimal dosing strategies through the trial. Initial intravenous administration of CAR-T cells triggered cytokine release syndrome and temporary neurological effects due to tumour inflammation. Subsequent direct delivery into the cerebrospinal fluid proved better tolerated, with diminishing side effects in follow-up doses. The team determined that a lower initial dose led to less severe cytokine storm effects while maintaining therapeutic benefit.

Functional improvements

Beyond tumour reduction, the therapy demonstrated capacity to reverse neurological deficits. Several patients regained lost abilities, including walking and continence. Two patients showed functional improvement despite unchanged tumour volumes, suggesting broader therapeutic benefits beyond tumour shrinkage. "We could see clear evidence of reversibility," noted Dr Crystal Mackall, the study's senior author and Professor of Pediatrics and Medicine.

Participants underwent preparatory chemotherapy to prevent their immune systems from rejecting the engineered cells. Following initial intravenous administration, those who showed benefit received additional doses directly into the cerebrospinal fluid every one to three months. This approach allowed for sustained treatment with reduced side effects compared to intravenous delivery.

Future directions

The research team, led by Dr Monje and Dr Mackall, is now investigating methods to enhance the therapy's efficacy. This includes studying ways to suppress aspects of immune response that might favour tumour growth and exploring targeted therapies based on tumour biology. "Sometimes this tumour grows so fast that it feels like a race between the CAR-T cells fighting and the cancer cells replicating," explained Dr Monje, highlighting the need for complementary approaches to improve outcomes.

The results represent a significant advance in treating DIPG, which affects several hundred children and young adults annually in the United States and has a five-year survival rate below 1%. The normal distribution of treatment responses suggests the possibility of reproducible benefits in future patients. The U.S. Food and Drug Administration has granted the therapy regenerative medicine advanced therapy designation, facilitating an expedited approval process.

Ongoing research

The trial continues to enrol participants, with researchers refining protocols based on current findings. The team is analysing hundreds of biological samples collected during the trial to understand what distinguished the best responders. These insights are expected to inform protocol refinements and broader applications in brain tumour immunotherapy. "While this trial represents progress, we still have work to do to diminish the toxicity of treatment and enhance benefit for patients," Dr Mackall added, "But now we have a path forward."

Reference:

Monje, M., Mackall, C., et al. (2024). Cell therapy fights lethal childhood brain cancer in Stanford Medicine trial. *Nature*. https://doi.org/10.1038/s41586-024-08171-9

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Stanford MEDICINE

Committed to creating a cancer-free world

The Ohio State University Comprehensive Cancer Center – Arthur G. James Cancer Hospital and Richard J. Solove Research Institute (OSUCCC – James *https://cancer.osu.edu*) is dedicated to creating a cancer-free world by integrating scientific research with excellence in education and patient-centered care – a strategy that leads to better methods of prevention, detection and treatment.

Ohio State has been designated a National Cancer Institute (NCI) Comprehensive Cancer Center for nearly 50 years and is one of only a few centers funded by the NCI to conduct phase I, II and III clinical trials on novel anticancer drugs provided by the NCI. In its last three formal reviews for five-year re-designation, the university's cancer program, embodied in the OSUCCC – James, has received the NCI's highest descriptor of "exceptional."

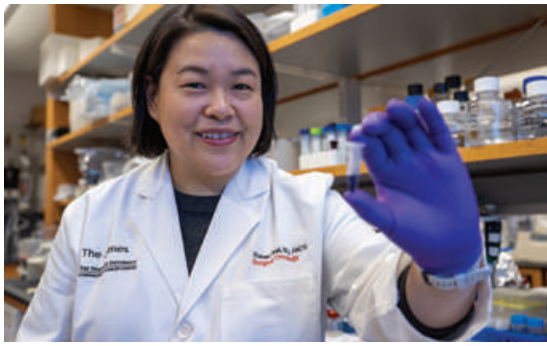
The OSUCCC – James has $140 million in active cancer-relevant research funding, and over $76 million of that total – more than half – comes from the NCI. In 2023 alone, Ohio State cancer researchers received 46 new research grants from the NCI.

Cancer research

The OSUCCC – James has over 320 full or introductory cancer researchers who collectively represent 12 of the 15 colleges at Ohio State. Each researcher is a member of one of five multidisciplinary research programs: Cancer Control; Leukemia and Hematologic Malignancies; Cancer Biology; Molecular Carcinogenesis and Chemoprevention; or Translational Therapeutics. In 2023, these researchers authored or co-authored 941 publications in peer-reviewed journals. In addition, OSUCCC – James researchers opened 140 clinical trials in 2023, bringing the total number of cancer clinical trials at Ohio State to approximately 650. These studies offer the latest cancer treatments, some of which are available nowhere else.

As the cancer program's 356-bed adult patient-care component, The James is the third-largest cancer hospital in the United States and one of the top cancer hospitals in the nation as ranked by *U.S. News & World Report*. With 21 floors and more than 1.1 million square feet, The James is a transformational facility that fosters collaboration and integration of cancer research and clinical care. Because early diagnosis is the key to successful cancer treatment, The James Cancer Diagnostic Center at the OSUCCC – James provides patients who may have cancer with direct, expedited access to diagnostic testing.

The OSUCCC – James since 2014 has enrolled nearly 70,000 patients in a Total Cancer Care® (TCC) protocol for voluntarily sharing de-identified clinical data that advances cancer research and personalizes cancer care. The TCC protocol has been adopted by all 19 member institutions across the United States that constitute the Oncology Research Information Exchange Network (ORIEN), a research collaboration co-founded and co-anchored by the OSUCCC – James and Moffit Cancer Center in Tampa, Florida. Through ORIEN, TCC-consented patients across the United States donate clinical data for research that helps scientists better understand cancer at the molecular level.



International reputation and global destination

Because of its international reputation for providing outstanding research-based cancer care, the OSUCCC – James is regarded as a global destination of choice, serving patients from all 50 U.S. states, three U.S territories and 90 other countries. The institution's Destination Medicine Global Health Care team <*https://cancer.osu.edu/destinationmedicine*> is dedicated to ensuring that distance and language are no obstacles to receiving the internationally recognized cancer care available at Ohio State.

The James

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