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CC NEWS

Clinical Center News

May/June 2024

Center for Cellular Engineering Unveils New Manufacturing Facility

The Modular Terrace Facility will be used to produce cutting-edge cell and gene therapies for clinical research trial patients



The Center for Cellular Engineering (CCE) recently unveiled its new, roughly \$20 million Modular Terrace Facility.

First conceived in 2017, the custom-designed, 8,000-square-foot structure features four manufacturing clean rooms spanning 3,000 square feet and another 1,000 square feet of support space.

“There’s no research space in this building. It’s all dedicated to manufacturing,” CCE Deputy Director Dr. Rob Somerville says.

Unlike most commercial facilities, which focus production on one or two cell or gene therapy products, the CCE oversees a broad portfolio.

Somerville says the new Modular

Terrace Facility will directly support the CCE’s mission to produce therapies for patients enrolled in trials.

These range from stem cells for bone marrow transplants and CAR T-cell immunotherapies for cancer patients to the CCE’s most advanced product, a graft of retinal pigment epithelial cells for patients with macular degeneration.

To produce epithelial cells, CCE staff reprogram blood cells drawn from a patient sample to create iPSC, or induced pluripotent stem cells, a form of lab-generated embryonic stem cell.

“We then select basic cultures of these cells, pick the best one, and then ... go through a differentiation process to turn them into retinal pigment

epithelial cells,” Somerville says.

The cells are later transferred to a scaffold used by an ocular surgeon to implant into a patient who has suffered vision loss caused by macular degeneration.

“This is first in human. So far only a single patient has been treated with this therapy,” Somerville says, noting that the manufacturing process is protracted. “It takes multiple months, and we’re still just a proof of concept stage.”

Noting that requested cell and gene therapies for Clinical Center patients are constantly in flux, the scientist says the CCE’s new Modular Terrace Facility will increase production capacity.

“We can manufacture different products in different rooms, and they won’t impinge on whatever is going on in adjacent rooms, which gives us a lot of flexibility,” he says.

A key feature of the state-of-the-art facility is that it can be easily cleaned—a crucial requirement to ensure a sterile manufacturing environment to produce patient products free of hospital-acquired infections.

“This facility is a new tool that NIH can use for [many] years looking forward,” Somerville says. “Rather than designing a facility for where we are now, we’ve created flexibility ... It is forward thinking.”

—Sean Markey



Howard Harmony: Healing Through Music

Howard University's Music Department offered a special performance to celebrate Black History Month at the Clinical Center

To celebrate Black History Month, the Diversity, Equity, Inclusion and Accessibility Advisory Committee (DEIAAC) and the Office of Communications & Media Relations' Music in the Atrium series joined forces to present a special performance featuring the vibrant music of Howard University's (HU) Music Department.

The Feb. 27 event marked the first time a historically black college or university (HBCU) performed at the CC and featured student ensembles SAASy, the HU Jazz Singers and critically acclaimed Afro Blue, as well as classical voice soloists. The groups were backed by Kit Young and Samuel Prather on piano, Herman Burney Jr. on bass and Kelton Norris on drums.

Entitled "A Journey Through Song of the Underground Railroad," the moving, hour-long performance included classics such as "My God is So High," "Wade in the Water" and "Deep River" alongside two original compositions from Afro Blue, "The Trauma Express" and "Henry Box Brown," among other highlights.

The concert, hosted by HU alumnus and Clinical Center Acting Executive Officer, Ila Flannigan, drew a large audience, including Capt. Toni Jones, Clinical Center Patient Representative. She described the performances as "soul stirring, uplifting, and an unforgettable celebration of struggle, triumph and commitment to freedom for all people."

"Hearing and seeing these young

people tell these stories in traditional and contemporary reimaged ways laced with respect was particularly special," she said, "because it emphasizes the importance of passing down our history in a way our children can understand, honor and keep it alive to ensure it isn't lost or passed off as irrelevant."

Following the concert, HU Assistant Music Director Tyree Austin reflected on the event. "To see the students sing songs of and inspired by our ancestors is always such a powerful experience," he said. "It directly connects and reminds us of where we have come as a people and what more we still have to do to bring change in this world."

—Janice Duran

Clinical Center Honors Its Own Nobel Laureate



Left to right: Dr. Anthony Fauci, Diane Dowling, Dr. Harvey Alter, Dr. James Gilman, Dr. Monica Bertagnolli and Dr. Lawrence Tabak

The Clinical Center recently held a ribbon cutting ceremony for the NIH History Center and Stetten Museum exhibit on Dr. Harvey Alter. The exhibit highlights Alter's discovery of the hepatitis C virus and his work to make our blood supply safe which earned him the 2020 Nobel Prize for Physiology in Medicine.

For more on the exhibit, read the full CC New article here: <https://go.nih.gov/Alter>.

The Quest to Find Better Interventions for Children with Cerebral Palsy

For 30 years, Dr. Diane Damiano has searched for better ways to help children with developmental brain injury

Inside the NIH Clinical Center, a “pint”-size lab is making an outsized impact on children living with cerebral palsy.

Known as the Pediatric Imaging and Neuroplasticity Team, or PINT, the lab is part of the Rehabilitation Medicine Department’s Neurorehabilitation and Biomechanics Research Section and is led by Dr. Diane Damiano.

A physical therapist and researcher, Damiano has spent the past 30 years studying the underlying causes of movement abnormalities in children with cerebral palsy and other developmental brain injuries.

Her research has transformed the field, often defying conventional wisdom to produce novel insights and successful treatments to improve the quality of life of children with cerebral palsy, as well as their families.

Cerebral palsy affects half a million children in the U.S. and is the most common pediatric motor disability. Caused by damage to the brain, often from complications of very early preterm birth, it compromises muscle control and coordination, creating movement difficulties.

Early in her career, Damiano was inspired to help this population and worked as an early intervention specialist. “We weren’t really making a huge difference in reducing the level of motor disability. We really didn’t understand why,” she recalls. “I think it’s partly because we weren’t starting quite early enough.”

In the years since, the scientist has strived to reveal what works, what doesn’t and why.

One of her key insights is the need to actively engage the brain of cerebral palsy patients during therapeutic interventions, whether during physical therapy or when using robotic assistive devices.

“It’s a very different concept. This

whole idea of engaging the brain really changes how we design things,” she says. “We know that the child has to be actively moving on their own. They’re the ones that are going to make the change. They have to be thinking about it [and] they have to feel the sensory consequences. You just can’t do something passive and have them get better.”

One example is her idea for a computer-controlled pediatric exoskeleton leg brace to help children with cerebral palsy overcome a

“
*They get to play.
They fall, and they
have to learn to get
back up.*

— Dr. Diane Damiano

physically exhausting walking limitation known as crouch gait. (Now in its fourth iteration, the project is spearheaded by her former postdoc and current department colleague, Dr. Tom Bulea, in his spinoff NeuroRobotics Group (NRG) Lab.)

Damiano’s ongoing research focuses, in part, on how to strengthen the neural pathways of movement before they are lost during early brain development to children with cerebral palsy, as well as how muscle use, or movement, can help repair the brains of such children.

Among her many current projects is the “Baby G.” Installed in the PINT Lab, the custom-built device explains why her 10-person lab is staffed not only by physical therapists and pediatricians, but also research engineers, data scientists and tech-savvy postdocs.

Occupying the space of a small room, the device resembles a climbing harness attached to a cable. Suspended from above, the cable is attached to a nimble, intelligent motor that is free to move along wires of an X-Y axis strung from an overhead frame attached to the ceiling.

Free to follow movement in three dimensions, the Baby G provides just enough weight support to enable toddlers with cerebral palsy to more easily crawl, stand upright, walk, even gently fall down as they play.

While this may not sound revolutionary, the transformation is profound. Damiano shows before and after video from a pilot study of a little girl with cerebral palsy in a pilot study, who cannot walk or stand unassisted. To move, she scoots across the floor while sitting on her knees by flapping both legs and arms. To move, Damiano says, “her brain is learning [incorrectly] that my legs move together, my arms move with my legs.”

But supported by the Baby G, that same girl can walk, stand upright, push a toy shopping cart, gather toy blocks and play like a normal toddler.

“She doesn’t even look like she has cerebral palsy,” Damiano reflects. “It’s just unweighting her, which means it’s making it easier [to walk]. She doesn’t have to try so hard and bring in all the wrong muscles to try to make something happen.”

“We don’t really give kids [with cerebral palsy] experiences like this in therapy. We try to be very regimented in what they do and try to get small gains. But this ... is like coming to a fun gym class. They get to play. They fall, and they have to learn to get back up.”

Read the full article online at cc.nih.gov/ccnews

—Sean Markey



Rare Disease Day participants outside of Natcher auditorium

Leap(ing) Into Rare Disease Day

The worldwide observation of Rare Disease Day fell on Leap Day this year.

February 29 also saw the return of the annual NIH Rare Disease Day 2024 conference, which is sponsored by the National Center for Advancing Translational Sciences and the Clinical Center. This event seeks to raise awareness about rare diseases, the people they affect and NIH collaborations that address scientific challenges and advance research for new treatments.

This year, over 2,200 people participated in NIH Rare Disease Day live and virtual meetings, which included panel discussions on artificial intelligence (AI) and its role in rare disease research and advancements in gene therapy.

NIH Director Dr. Monica Bertagnoli was among the speakers sharing rare disease stories and the experience of clinical doctors.

The event offered networking opportunities and more than 100 exhibits and scientific poster presentations at the NIH Natcher Conference Center. Exhibitors included medical and pharmaceutical companies, patient advocacy

nonprofits and NIH programs.

In an address, Clinical Center CEO Dr. James Gilman welcomed conference attendees and touted the research hospital's long-standing involvement with rare diseases and medical milestones, including the development of chemotherapy to treat cancer and blood tests to detect HIV and hepatitis.

Gilman encouraged participants to visit the Clinical Center Office of Patient Recruitments booth at the event to share their personal stories in hopes that participants could be paired with clinical studies to advance research.

"This is the one day a year where patients with a rare disease have a voice, said participant Jennifer Noonan with Accessia Health. "We are here to lean more about what is coming, what our patients might need and how to fill in those gaps."

To learn more about NIH Rare Disease Day 2024, visit videocast.nih.gov/watch=52569.

—Yvonne Hylton

Microaggressions: Strategies for Change

Seeking to cultivate a culture of teamwork and collaboration, the Diversity, Equity, Inclusion and Accessibility Advisory Committee (DEIAAC) has sponsored a series of events to foster healthy workplace dynamics.

In January, Dr. Christine Piggee, a scientific diversity advisor in the Office of the Scientific Director of the National Institute of Mental Health, led an interactive workshop for CC staff entitled, "Microaggressions: Strategies for Change."

Microaggression is "... an everyday verbal or nonverbal slight, snub or insult—whether intentional or not—that communicates a negative message to a person based on their membership in a marginalized group," Piggee said, citing Derald W. Sue.

She noted that microaggressions are something experienced and defined from the perspective of the receiver and sometimes occur with no conscious malice intended by the perpetrator.

Piggee offered strategies for responding to microaggression, saying it is typically best to be direct and unambiguous, even though microaggression can be jarring to experience. Effective responses include, "Are you saying all ___ are ___?" and "When you said ___, that made me uncomfortable."

Piggee said it is important to consider whether it makes sense to respond publicly or privately, and whether there are situational consequences that make a response unnecessary.

—Dan Silber

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