DISCOVERY STARTS HERE

2021

Annual Report

CORIELL.ORG

CORIELL INSTITUTE
FOR MEDICAL RESEARCH

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FRONT COVER IMAGE:
3D illustration of a cell

LETTER FROM

Dr. Jean-Pierre Issa, Coriell's President & CEO

It was an exciting year for the Coriell Institute—certainly a year like none before. It was a year that tested our resilience and ingenuity, both here at Coriell and throughout the nation as we adjusted to the "new normal" of life during a pandemic.

Despite a year of unprecedented challenges, Coriell received major new funding for exciting research; we advanced and grew the offerings in our biobank; we developed new online tools to help researchers find the biosamples they need in new ways. And we redoubled our commitment to our community, bolstering access to scientific education and organizing our employees time and talents to support other local nonprofits.

All in a year with still much uncertainty due to the pandemic. When I composed this letter last summer, new case totals were dipping over the summer, but the rightfully dreaded winter was still to come. Vaccinations felt a lifetime away. It had been months since so many had in-person contact with loved ones.

Now I am encouraged by the successful push for vaccines. In record time, all around the world, scientists tapped state-of-the-art technologies to create entirely new vaccines that stop infections and prevent disease. This effort put into clear view for millions how important early and steady investment in scientific research is. Scientists were able to research, test, and produce many different vaccines because the groundwork had been laid over years, decades even, of scientific research. We learned more about the virus and how to treat the disease it causes. We rolled out a national vaccination campaign that made inoculation a possibility for hundreds of millions of Americans in short months.

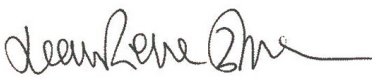
I remain inspired by the potential for science. This pursuit of knowledge is a part of us—*homo sapiens* means "the wise human" after all—and so is how that knowledge can be translated into new technologies and vaccines and cures.

Yes, I have worries about the future. New variants and new viruses will forever trigger panic in us after this pandemic. But I know that we will adapt, we will work together, and we will persist. We've done it before.

I am proud to work among the people that I do. I am proud that through it all, we've not only stayed together, but have stayed strong. But most of all, I am proud of the work we do together.

I hope you enjoy reading about that work in this report and share a passion for our mission.

DR. JEAN-PIERRE ISSA



BOARD OF TRUSTEES

Biobanking

The National Institute of General Medical Sciences (NIGMS) Human Genetic Cell Repository continued to grow in the last year with a focus on fostering relationships with groups that organize and advocate for people affected by rare, heritable diseases.

"Something inspiring about the rare disease community is how collaborative everyone is and how willing everyone is to build and grow networks."

— DR. MITCHELL

Rare Disease Day

Coriell's second Rare Disease Day at Coriell was a great success. This year's event, Patients: Heroes of Rare Disease Research, was held virtually and was the result of a new collaboration between Coriell and Penn Medicine's Orphan Disease Center. Scores of scientists and members of the rare disease community attended.

Matthew W. Mitchell, PhD, Co-Principal Investigator of the NIGMS collection, was one of several speakers who applauded the rare disease community for its role in research.

The event was hosted by the Orphan Disease Center's Director of JumpStart, **Deborah Requesens, PhD**, who previously served as the Principal Investigator of the NIGMS Repository at Coriell. Other featured speakers included **Anne T. Berg, PhD**, of the Northwestern Feinberg School of Medicine, **Eric Marsh, MD, PhD**, of Penn Medicine's Orphan Disease Center, **Scott Demarest, MD**, of Children's Hospital Colorado, and **Anna Pfalzer, PhD**, of COMBINEDbrain.

ISBER Meeting

At the International Society for Biological and Environmental Repositories (ISBER) Annual Meeting in early 2021, Coriell's **Dr. Kathryn Driesbaugh** and **Dr. Laura Scheinfeldt**, Director of Repository Operations and Director of Repository Science at Coriell respectively, hosted a roundtable discussion titled "Biobanking During COVID: Minimizing Risk and Maximizing Operations." A poster was presented by NIGMS Repository Project Manager **Sam Sander-Effron**, through which he provided an overview of research publications that cited the use of NIGMS Repository samples over a two-year period.

Gene Search

The biobanking team at Coriell launched a new tool for research last summer. This tool, Gene Search, allows researchers all over the world to explore high-quality, whole-genome sequencing data collected from NIGMS and the National Human Genome Research Institute (NHGRI) repository biospecimens. Gene Search makes it easier for researchers to identify the most appropriate samples for their genetic and genomic research. This tool was highlighted in an NHGRI Repository poster presentation at the Cold Spring Harbor Laboratories Biology of Genomes 2021 virtual conference.

NINDS

The National Institute of Neurological Disorders and Stroke's (NINDS) Human Genetics Resource Center expanded Dystonia, Cerebrovascular Disease, and Population Control collections by over 120 participants despite submission challenges due to the ongoing COVID-19 pandemic. In addition, the NINDS Repository team highlighted a public resource of genomic data collected from NINDS Repository samples in a poster presentation at the 2021 Annual American Society for Human Genetics virtual conference.

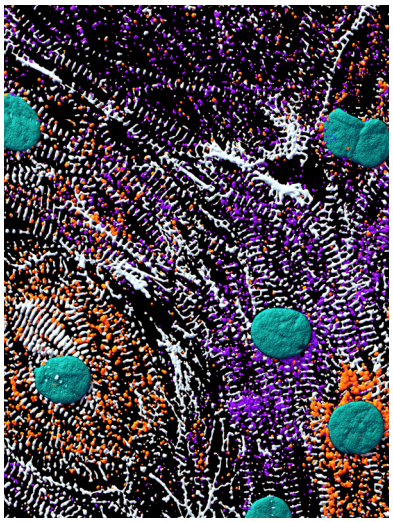
The Aging Cell Repository

The Aging Cell Repository, sponsored by the National Institute on Aging (NIA), held its Inaugural Scientific Advisory Committee Meeting in December. This group of leaders in the field are helping to guide the scientific direction of the repository moving forward. In an effort to continue to grow the repository, additional induced pluripotent stem cell (iPSC) lines were made available to the research community, bringing the total to 20 publicly available iPSC lines that represent various aging conditions.

Coriell also added a new collection of stem cells to its biobank, the result of a new collaboration between Coriell and the Rett Syndrome Research Trust (RSRT). This collection consists of 10 lines of human iPSCs created from blood donated by individuals with Rett syndrome, a rare, neurological disorder that presents itself in children—mostly girls—from 6 to 18 months old.

The Allen Cell Collection

The Allen Cell Collection, first launched at Coriell at the end of 2016, added several new lines to its highly specialized collection of stem cells, bringing the total number of lines to more than 50. This collection is a partnership with the Allen Institute for Cell Science and includes fluorescently-tagged hiPSC lines that target 40 key cellular structures/substructures.



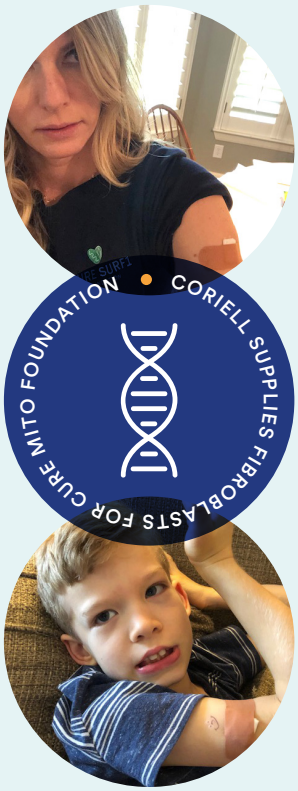
PICTURED:
Cardiomyocytes differentiated from the hiPS cell line AICS-0075 (clone 85), which labels the cardiac sarcomeric z-disk protein alpha-actinin-2 (pseudocolored in white).

Coriell Teams Up with Cure Mito Foundation for Urgently Needed Fibroblasts

THE MISSION was clear. Kasey Woleben needed to collect dozens of skin punches from members of her group, Cure Mito Foundation (formerly known as Cure SURF1), and she needed to get fibroblast cells from those samples safely to the University of Texas Southwestern Medical Center. Researchers there had been working on a potential gene therapy treatment and it was vital to have the cells to prepare an application to the FDA.

Unsure of where to start, Woleben turned to Coriell in September of 2020. Within just a few short months of bringing Coriell into the mix, the researchers had the cells in hand.

PICTURED:
Kasey & Will Woleben



"Without Coriell, we wouldn't be filing our IND (Investigative New Drug) application this year. We are very blessed and cannot thank Coriell enough," Woleben said.

Woleben formed her organization in 2018 after connecting with other families affected by the mitochondrial disease Leigh syndrome. Symptoms of Leigh syndrome include a loss of motor skills, developmental delays, and trouble gaining weight. Woleben's nine-year-old son, Will, had been diagnosed with the disease years earlier—Will's version is caused by a mutation on his SURF1 gene—and her family wanted to do all that was possible to push for a cure.

That led Woleben to UT Southwestern. A team there was launching a new gene therapy program and—after some lobbying from Woleben—they felt this gene was a good candidate for genetic therapy. Her group raised more than $1 million for research, making it possible for the preclinical work to begin.

But the request last year for fibroblasts was a hurdle. Having them would strengthen the case for the IND application, but the families Woleben works with are from all around the world. How was the group going to collect dozens of skin punches from all over and get them safely to Dallas, Texas?

"We realized Coriell can help us collect these samples and save us a lot of money doing so," she said. "Coriell is internationally connected, which really matters when patients are around the world."

Coriell houses the NIGMS Human Genetic Cell Repository, a diverse collection of research resources that includes samples representing more than 1,000 rare, heritable diseases. To build this collection, the NIGMS team at Coriell works with patients, families, and rare disease organizations to collect blood and skin samples for research.

The team at Coriell knew it could help. Shortly after being contacted by Woleben, Coriell's Sherryann Wert, a Senior Project Manager on the team, hosted a webinar with Woleben, the researchers, and the families, so everyone could learn why these cells were important and how the donation process would work.

Coriell then sent sample collection kits to nearly 40 people spread across nine countries. Those individuals had skin punches taken and followed Coriell's instructions to return them. Woleben and her family were the first donors, and they documented the process with photo and video to help others. Coriell was available for questions at any time.

Nearly every skin punch was viable when received by Coriell. "On top of monitoring shipments and culturing the samples into usable fibroblasts, a great deal of clinical data was collected as well. That information was translated, organized, and all relevant information was included and reviewed," said Wert. "This was a real team effort at Coriell."

Researchers at UT Southwestern started receiving the fibroblasts in February. Now, Woleben and her organization are preparing their application for a gene therapy they hope will help her son Will—the fourth grader and die-hard fan of the Pittsburgh Penguins—and the dozens of others with the same mutation.

> "Coriell understood our timeline needs... Without the fibroblasts, I don't know where I'd be. I can tell you that."
>
> — KASEY WOLEBEN

Research

This year was one of growth and expansion for the biomedical research conducted at Coriell.

The Jian Huang Lab

Jian Huang, MD, PhD, an Associate Professor and Senior Scientist for Stem Cell Biology and Genetic Engineering at Coriell, joined the Institute last summer and established The Jian Huang Lab to continue his study. Dr. Huang's team is investigating how the self-replicating processes of hematopoietic stem cells (stem cells that can develop into any type of blood cell) are regulated and how the stem cells differentiate into the many different blood cell types.

In early 2021, Dr. Huang was awarded a $2 million, five-year grant to research ways to reduce drug resistance in patients suffering from acute myeloid leukemia (AML), a form of leukemia which arises from the blood system.

A New Grant

Jozef Madzo, PhD, Coriell's Director of Bioinformatics, earned a new grant from the Gordon Charter Foundation. This one-year grant supports research into the use of machine learning to discover a novel cancer signature that can be used to determine whether someone is a good candidate for immunotherapy treatments or not.

Breast Cancer Research

Shoghag Panjarian, PhD, Assistant Professor at Coriell, published findings in *Breast Cancer Research* about DNA methylation changes in normal breast tissue of patients with breast cancer. She found that a subset of patients with breast cancer has severely altered epigenomes characterized by accelerated aging phenotype. These findings pave the way for future studies to determine the potential use of the DNA methylation changes as biomarkers for early detection of breast cancer.

With Coriell's President and CEO, **Dr. Jean-Pierre Issa**, Dr. Panjarian also published a review on the role of epigenetic enzymes as novel therapeutic targets in aggressive breast cancer. This review was published in *Pharmaceuticals* as part of the special issue, *Potential Molecular Targets and Therapeutics in Triple Negative Breast Cancer*.

Camden Opioid Research Initiative

At the end of 2018, Coriell joined with two of its Camden neighbors, Cooper University Health System and Cooper Medical School at Rowan University, to launch the Camden Opioid Research Initiative (or CORI). Scientists involved in this three-pronged research project are investigating the underlying reasons as to why some people are more vulnerable to developing opioid use disorders than others.

The pandemic slowed patient recruitment for CORI, but the scientific work continued at full speed. CORI scientists authored an article detailing the design of the Genomics of Opioid Addiction Longitudinal Study arm of the research. This article was published in *BMC Genomics*.

CPMC Anniversary

The Coriell Personalized Medicine Collaborative (CPMC) celebrated the anniversary of a major milestone: the move from a focus on recruiting participants into the pioneering personalized medicine study to a focus entirely on research.

Laura Scheinfeldt, PhD, Associate Professor, Director of Repository Science at Coriell, and a longtime researcher on the CPMC, co-authored a paper that was published in a special edition of *Patient Education and Counseling* focused on the reporting side of personalized medicine.

Dr. Scheinfeldt and her team—consisting of researchers from Coriell, Geisinger's Genomic Medicine Institute, and the NCI's Behavioral Research Program—used data from the CPMC to test different ways that disease risk was communicated to participants and found that a report combining more than one approach reduced the chance a participant would misunderstand the numerical information presented to them.

In collaboration with scientists at Temple University and Geisinger's Genomic Medicine Institute, Dr. Scheinfeldt and **Dara Kusic, PhD**, published a new computational method to predict important genetic variants that impact drug response in the *Journal of Personalized Medicine*.

PICTURED:
Jian Huang, MD, PHD

National Cancer Institute Awards Coriell Scientist with Grant to Study AML Drug Resistance

THE NATIONAL CANCER INSTITUTE (NCI) has awarded a Coriell Institute for Medical Research scientist a $2 million five-year grant to study ways to reduce drug resistance in patients suffering from acute myeloid leukemia (AML), a form of leukemia which arises from bone marrow tissue.

The grant was given to Jian Huang, MD, PhD, an Associate Professor and Senior Scientist for Stem Cell Biology and Gene Engineering who will serve as principal investigator on the research project.

"Drug resistance in acute myeloid leukemia remains a major hurdle in treating this life-threatening cancer," said Dr. Huang "My research is focused on better understanding the genetic causes of that drug resistance and finding ways to determine which drugs are most effective for certain genetic makeups."

Previously, Dr. Huang and his team have conducted a CRISPR-based genetic screen in AML cells and identified several important genes whose loss-of-function leads to drug resistance to a new FLT3 inhibitor Quizartinib (AC220). This study was published in the journal *Cancer Research* in 2017. In the following studies, Dr. Huang's group will further investigate the molecular mechanisms by which those genes function in the drug resistance to AC220. As a very effective drug to treat FLT3-ITD+AML, AC220 is currently being tested in clinical trials.

In 2019, Coriell researchers published an article in *Clinical Epigenetics* detailing new biomarkers they found that indicated a patient's likelihood for success when their relapsed or refractory AML was treated with the novel epigenetic drug guadecitabine.

"My research is focused on better understanding the genetic causes of that drug resistance and finding ways to determine which drugs are most effective for certain genetic makeups."

JIAN HUANG, MD, PHD

A Legacy of Inn

A Future

ovation.

of

Possibility.

Community Engagement

Today's scientists stand on the shoulders of yesterday's giants. Important knowledge discovered years ago is the foundation for the work we do today, and that work will be the foundation for important work in the future.

A Virtual Science Fair

Recognizing that, we at Coriell feel a duty to educate the generation of scientists who will come next.

To help these young scientists, Coriell hosts a science fair each year, inspiring scores of students annually to pose scientific questions and design experiments to find the answers. This year was the fair's 40th anniversary, and due to the ongoing threat from the coronavirus, the fair was held virtually, just as it was in 2020.

About 100 students explored scientific interests in fields ranging from botany, computer science, biochemistry, and more.

They put forth valuable questions, conducted experiments to test their hypotheses, built projects and, finally, logged into video conferences to discuss their findings with a team of judges.

Student Earns First Place

The majority of this year's participants earned the right to advance onto the regional Delaware Valley Science Fair (DVSF). From that group, about 70 percent of the students either placed in their category or earned a special award at DVSF. One student, Maya Butani, even progressed onto the International Science and Engineering Fair (ISEF) where she earned a first place prize in her category of biochemistry. Inspired by patients in need of organs, Maya explored the utility of using plant materials in constructing tissue for transplant in humans.

Second Year of BRE

Unfortunately, because of the pandemic, Coriell was again forced to cancel its Summer Experience for Students, the hands-on summer program that Coriell hosts for high school and college students, and instead offered a second year of the Bioinformatics Research Experience (or BRE).

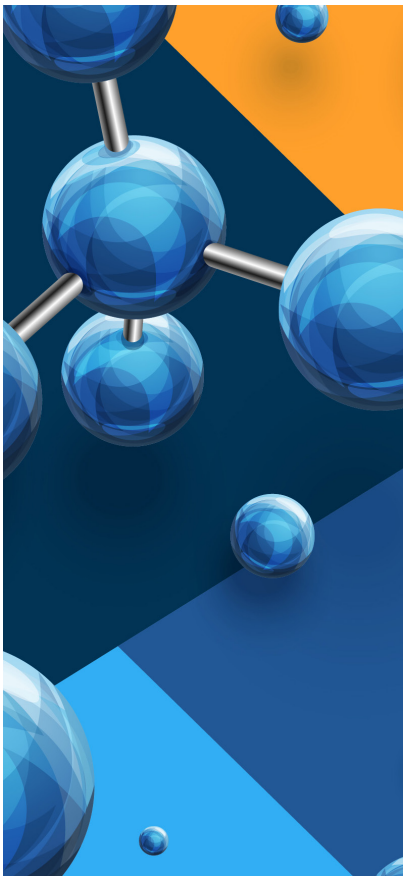
Like so many other pandemic-era programs, the BRE is held virtually. The purpose of the program is to teach qualified college students the basics of the new and rapidly growing field of bioinformatics. Students in the BRE learn basic general and biological data analysis and how to read and interpret scientific literature journal articles. The BRE runs through the month of July and students earn a $1,000 stipend on successful completion.

Commitment to Camden

But Coriell is not only committed to young scientists—Coriell is committed to the City of Camden, which it has called home since 1953. Following the unrest across the nation in the wake of the murder of George Floyd in Minnesota, Coriell redoubled its commitment to this community. Coriell created a new group, the Coriell Empowerment Initiative, to address needs in the immediate community, provide a space for employees to discuss topics such as race and inclusion, and encourage diversity at the Institute.

This group of dozens of volunteers collected hundreds of items, which were donated to Camden non-profits like Joseph's House, a shelter for unhoused persons, and Cathedral Kitchen, the city's most prominent emergency food provider; sponsored a local youth baseball team; held book club meetings to discuss relevant literature and topics; and organized educational seminars.

It is thanks to this group that Coriell now has a program to provide eight paid hours of time off to its employees to volunteer in community improvement programs.



PICTURED:
Maya Butani

Coriell Institute Science Fair Winner Takes Top Prize in International Competition

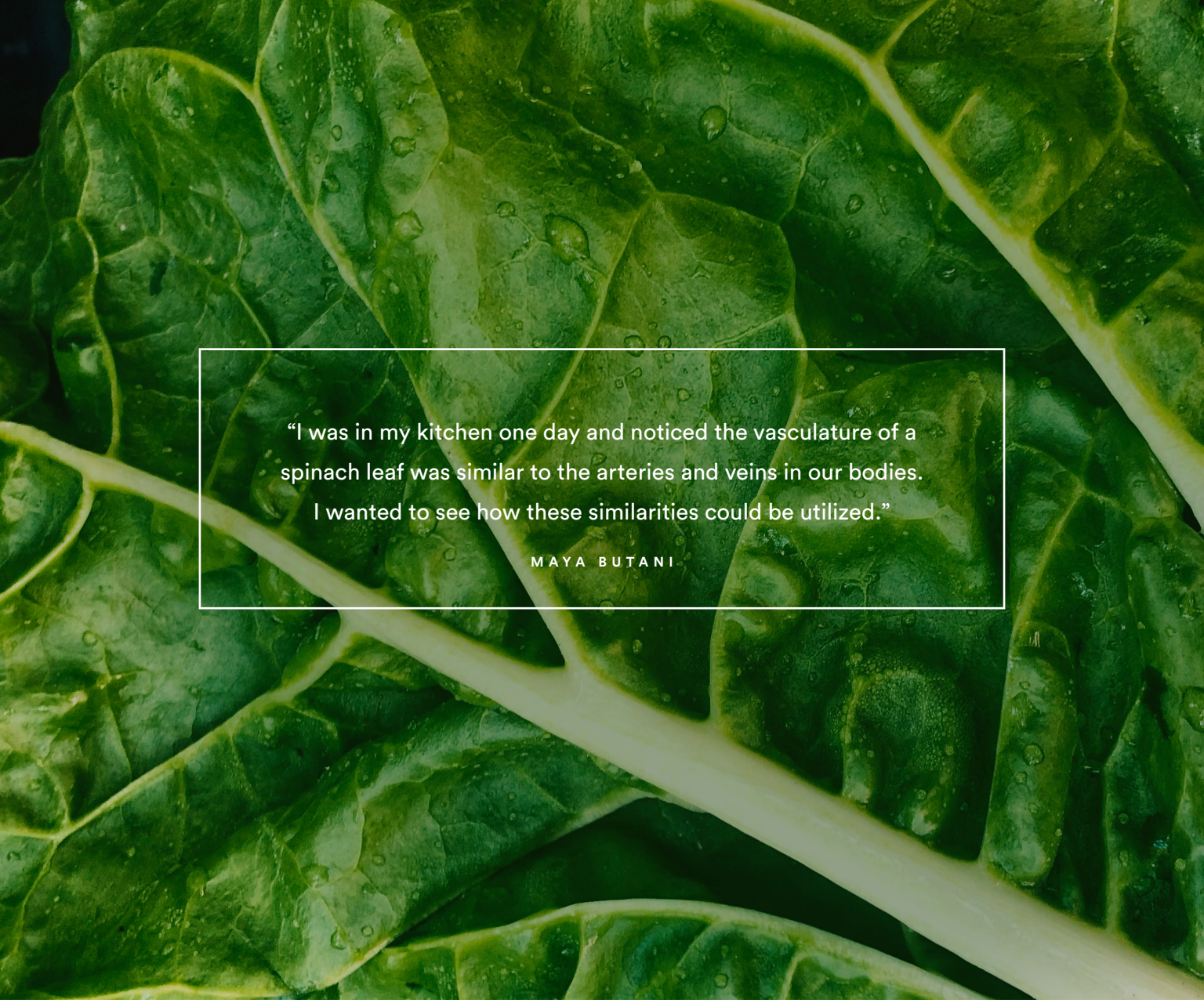
CONGRATULATIONS TO MAYA BUTANI, a winner in the 2021 Coriell Institute Science Fair, for her recent First Place Award in the 2021 Regeneron International Science and Engineering Fair (ISEF)!

Butani, a rising senior at Moorestown High School in Moorestown, New Jersey, took home the First Award in Biochemistry for her project titled "Stem Cell Behavior and Osteogenic Differentiation on Plant-Derived Scaffolds," in which she explored the utility of plant materials in manufacturing tissue for use in humans.

"There are more people who need organs than we have organs to give. We need to engineer a solution." Butani said. "I was in my kitchen one day and noticed the vasculature of a spinach leaf was similar to the arteries and veins in our bodies. I wanted to see how these similarities could be utilized."

Butani tackled her topic—discovering there was indeed potential, though more testing is needed—and presented her findings first at the Coriell Institute Science Fair in March. This was the 40th anniversary of the Coriell Institute Science Fair and, for the second year in a row, the event was held virtually due to the pandemic. Butani took home first place in her division.

From there, she moved onto the regional science fair, the Delaware Valley Science Fair, again taking a top placement that allowed her to move onto ISEF, the international competition, which also was held virtually.



> "I was in my kitchen one day and noticed the vasculature of a spinach leaf was similar to the arteries and veins in our bodies. I wanted to see how these similarities could be utilized."
>
> MAYA BUTANI

Butani was worried that the virtual nature of the big event would take away some of the aspects she was looking forward to, but she said they did an excellent job producing the fair. There were panels to attend and there was even a 3D modeling that allowed participants to move through a virtual space and observe projects as if they were there.

"My favorite part was meeting other finalists," she said. "I was upset that I might not be able to meet people, but I went out of my way, and I met a really nice group of people. It was great to meet other people around the world with a similar passion and similar drive."

Going into her senior year at Moorestown High School, Butani hasn't yet decided where she'll attend college, but she does know that her career will involve scientific research in some capacity. In the meantime, she'll continue sharing her passion with others.

"Science is something that is really important to me," she said. "I'm trying to start a new science club. I know that exposing students to experiential learning is the best way to introduce young students to science."

Philanthropy

As the world continues to battle the COVID-19 pandemic, one thing has become abundantly clear: Funding global biomedical research has never been more important.

We at the Coriell Institute believe preventing and curing disease is a global effort, and we've played a key role in it for nearly 70 years. Each day, we accelerate scientific discovery by creating and sharing world-class biomaterials and by conducting novel research in biobanking, personalized medicine, stem cell biology, and cancer epigenetics.

This pandemic has revealed that our global health is indeed fragile. Preparing for the next pandemic is essential and demands a dynamic, cooperative and sustained global biomedical research infrastructure. It's a challenge that must be met with the brightest minds of our generation, the collective might of the scientific community, and the support of our society at large.

Infectious disease, cancer, and genetic disorders all demand our attention.

Your support affirms that we need to act now to combat these global maladies and prevent the next pandemic. Your gifts allow Coriell to:

- Process and distribute research biomaterials to 85 countries worldwide, equipping more than 2,300 unique medical research programs last year alone;
- Embolden the rare disease community with purpose and hope. With more than 1,000 rare, heritable disease cell lines stored to date, our Coriell cell line establishment program launches the research journey against newly discovered maladies; and
- Train the next generation of medical miracle workers. Coriell's Science Fair has mentored thousands of STEM-centric students for over 40 years.

What we think of as medical miracles are the direct results of extraordinary people putting forth extraordinary effort to make the extraordinary happen. Big or small, a gift to Coriell will support and empower global biomedical research to find its next scientific breakthrough.

For more information on how to give, visit Coriell.org.

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We gratefully recognize donors who partnered in our success throughout Fiscal Year 2021 (July 1, 2020 - June 30, 2021).

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40

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INDIVIDUAL CELLS THAT
WE'VE CULTURED IN-HOUSE
AND SHIPPED

85

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100+

EMPLOYEES

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MEN IN SCIENTIFIC ROLES

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