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Leaving the Lab

LA BioMed isn't just a research institute, it's become a thriving business incubator.

RINGO H.W. CHIU/ LABJ

BioMed Head: Executive Director David Meyer at the campus of the Los Angeles Biomedical Research Institute in Torrance. The institute shares a sprawling campus with Harbor-UCLA Medical Center.

MOST Angelenos haven't heard of the **Los Angeles Biomedical Research Institute**. But it was the point of origin of many noteworthy advances in health science.

It was launched by UCLA doctors in 1952 at the site of a decommissioned World War II Army camp in Torrance. Rats were a problem, but they were kept under control by stray cats that wandered the sprawling campus. Inside the former military barracks, researchers developed the paramedic model for emergency care, the modern cholesterol test and human egg transplants for infertile women.

Today, LA BioMed, a non-profit research organization, is still on the same site along with **Harbor-UCLA Medical Center**. In recent years, it's become an incubator of biomedical startups.

Spinoffs that have products on the way to market include **Kythera Biopharmaceuticals Inc.**, a publicly held Calabasas developer of an injectable treatment for chin fat, and **Emmaus Medical Inc.**, a Torrance pharmaceutical company working on treatment of sickle cell disease. The institute gets licensing royalties from products and technology developed there.

The descendants of the felines are still on patrol, fed by the scientists who work there. Some of the original barracks remain but have been converted to research facilities and offices. Others have been torn down and replaced by state-of-the-art laboratories.

Money to convert the buildings comes from government grants and corporate partnerships. For example, a Japanese company that's formed a partnership with Emmaus to develop treatments for damaged organs paid to transform one of the old wartime health clinics into a modern laboratory that maintains the feel of the original site.

Much of the institute's research remains focused on the needs of the disadvantaged population served by the Los Angeles County hospital. For example, doctors develop treatments for chronic ailments prevalent among poor people, such as obesity and resulting heart disease and diabetes.

But the physician-researchers at the institute are also thinking entrepreneurially about niche medical devices and drug development.

"There is a rare spirit here that you don't find at other institutions I've worked at," said Executive Director **David Meyer**, who has fostered tech transfer at LA BioMed since he came in 2009. "We share a campus with a level-one trauma center that serves a tremendously underserved community. Our people don't stay holed up in their laboratories. It results in some exciting medicine."

At least two LA BioMed startups are slated to present their technology to potential angel and venture investors this month at the annual **Southern California Biomedical Council** Investor Conference in downtown Los Angeles.

In addition to their business ventures, most LA BioMed doctors conduct academic research, supervise medical residents and even make the rounds at Harbor-UCLA.

Dr. Matthew Budoff is an LA BioMed cardiologist who leads a team with patents pending on a computer workstation that can take cost-effective measurements of both bone density and plaque in the heart with a standard CT scanner. He notes that the institute's reputation for cross-collaboration among medical specialties helped it win grants that were used for creation of a $9.7 million multidisciplinary research facility. It helps that it's a more modest research facility than some of the area's heavier hitters.

"LA BioMed has really allowed me to work in an unfettered environment," Budoff said. "It's not a big behemoth like UCLA. We're small and nimble."

On the following pages, The Business Journal profiles five of the companies that started at LA BioMed and are at various stages of spinning off and putting their products on the market.

– Deborah Crowe

Also profiled:

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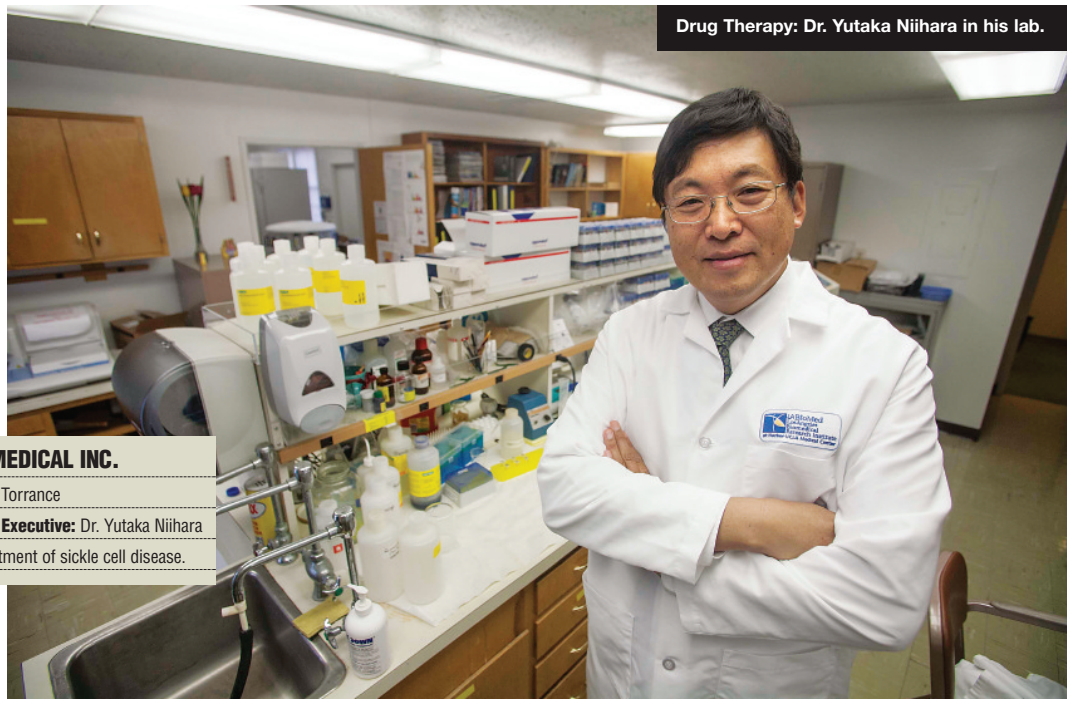
Treatment for Sickle Cell Pain



Drug Therapy: Dr. Yutaka Niihara in his lab.

PHOTOS BY RINGO H.W. CHIU/ LABJ

DR. Yutaka Niihara has devoted nearly a quarter-century to developing drug therapy that will relieve the pain of millions of sickle cell anemia patients around the globe. Clinical trials are scheduled to wrap up in December and his company plans to make the drug available by prescription next year. But it's too early to celebrate.

"I'm very pleased, but I cannot be too overjoyed because you never know until the very end," Niihara said.

He developed the drug therapy in the 1990s with **Charles R. Zerez** and **Kouichi R. Tanaka** at **LA BioMed**, which has granted a license to **Emmaus Medical Inc.** for the treatment.

Niihara traces his interest in the disease to a hematology-oncology fellowship he did at **Harbor-UCLA Medical Center** starting in 1989. Many of his patients were hospitalized with complications of sickle cell anemia, a genetic disease that occurs in about 1 of every 500 African-Americans.

EMMAUS MEDICAL INC.

Headquarters: Torrance

Founder-Chief Executive: Dr. Yutaka Niihara

Business: Treatment of sickle cell disease.

Most of the sickle cell research at the time focused on cures or prevention. But Niihara and his colleagues at LA BioMed took another route.

"We looked at it from a simplified point of view as to how we could treat the effects of this gene," he said.

The disease causes too much oxidation of red blood cells, transforming plump, disc-shaped cells into hard, crescent shapes. The deformed cells stick together and block blood flow, causing organ damage and pain.

After Zerez came up with a way to measure oxidation levels within the red blood cells, researchers focused on reducing that oxidation. They developed a treatment based on a type of glutamine that reduced pain and can be given orally with almost no side effects, making it safe even for children and pregnant women.

Niihara and his team requested government funding and got grants from the National Institutes of Health and the Food and Drug Administration's Office of Orphan Products Development, which promotes research into drugs to treat diseases that pharmaceutical companies do not consider potentially lucrative enough to develop. Emmaus has also raised $40 million, mostly from private investors. A recent $7.5 million private placement attracted some institutional investors as well.

If approved, the drug would be the first new therapy for sickle cell patients on the market in 20 years and it would serve a fully diagnosed but highly underserved patient population. According to the latest figures, more than $1 billion is spent treating the 100,000 sickle cell patients in the United States annually.

Niihara is not sure whether Emmaus will form a partnership with a bigger company, be acquired or even handle production and distribution itself.

"Our main goal is to choose the right option so this medication will get not only to patients in the United States and Europe but also to Africa, India, the Middle East and South America," said Niihara, who retired from UCLA in 2009 but still volunteers as an instructor and runs a laboratory at LA BioMed.

The reward for a lifetime of work?

"I do talk to a lot of sickle cell patients who were on the earlier trials," he said, "and they are quite happy."

– Karen E. Klein

Research Patron: Agi Hirshberg, at her West L.A. office, lost her husband to pancreatic cancer.

Faster Track for Drug Testing

AGI Hirshberg is rare among medical research company chief executives. She's not a doctor or laboratory researcher like most chief executives at biomedical firms. Hirshberg comes from the sporting wear industry. She went into medical research in 1998, after her husband died of pancreatic cancer at 54, eight months after he was diagnosed.

"I lost my husband because there were no effective drugs to treat it," she said.

After his death, she established the **Hirshberg Foundation for Pancreatic Cancer Research** and funded a research laboratory and a chair in pancreatic cancer research at **UCLA**.

She didn't know she would go beyond that. But in 2004, Hirshberg met **Paul Lee** and **Laszlo G. Boros**, doctors who were working at LA BioMed to quantify the results of testing for drugs designed to treat cancer and diabetes. So she and three partners – Lee, Boros and attorney **David Manheim** – co-founded **Sidmap** to advance and commercialize their work.

"Typically, the process from concept to drug is usually 10 to 20 years. I saw this as a fast track," she said. "And if I was going to make a difference in science, I wanted to do it bigger, better and faster."

SIDMAP

Headquarters: West Los Angeles

Founder-Chief Executive: Agi Hirshberg

Business: Drug development services.

Researchers at drug companies, independent labs and universities send samples and test results to Sidmap for analysis. Clients can use the results to verify their success or to invalidate faulty research and stop it earlier.

"We can save countless lives that might be lost when a drug is released and there are bad side effects," Hirshberg said, "or lawsuits over unanticipated side effects."

The company applied for government money but didn't get any. It was funded by Hirshberg and her partners and is now selling its testing services. The technology was developed at LA BioMed but a patent application was rejected so the company does not pay licensing fees to the institute.

While Hirshberg still laments the "snail's pace" of academic research, she is proud that her company delivers results to clients within six weeks.

Her experience in the apparel business, where she worked with brands such as Adidas, Under Armour and Speedo, doesn't give her much of an edge as a biomed chief executive.

"How does it translate? It doesn't," she said. "In a standard business, in 18 months you know if you either have a success or you don't. This is a long haul."

– Karen E. Klein

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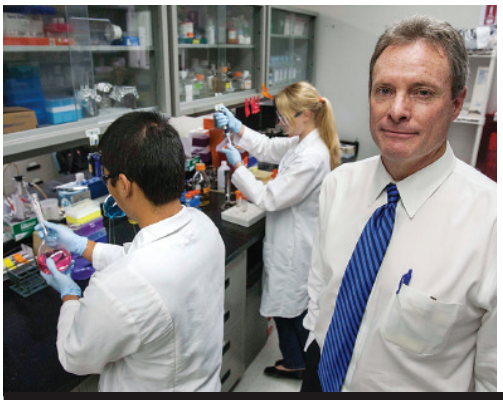
More Than a Shot in Arm



Yeaman: Developing vaccine with colleagues at his LA BioMed laboratory.

PHOTOS BY RINGO H.W. CHIU/LABJ

AT **NovaDigm Therapeutic Inc.**, scientists believe they are on to something. They're developing what they're calling a new generation of vaccines.

The company spun off from LA BioMed in 2005 to work on vaccine research and developed an unusual approach.

Traditionally, vaccines introduce elements of an illness, such as the flu, into the body. The immune system then learns to recognize the illness and create antibodies to fight it. But NovaDigm's vaccine employs a more complex model.

"One of the reasons our work is being seen as pioneering in next-generation vaccines is that it acts in a way that is unconventional," said co-founder **Dr. Michael Yeaman**. "Instead of generating antibodies exclusively, it seems to program our adaptive immune system so it can orchestrate the best defense."

The new vaccine might have at least two uses: It could prevent reoccurrence of yeast infections in women and also stop patients with weakened immune systems from picking up antibiotic-resistant infections during hospital stays.

NOVADIGM THERAPEUTIC INC.

Headquarters: Grand Forks, N.D.; founders remain at LA BioMed in Torrance

Co-Founder: Dr. Michael Yeaman

Business: Vaccines.

While Yeaman, **Dr. Jack Edwards** and colleagues continue their research at LA BioMed laboratories in Torrance, NovaDigm's corporate headquarters and clinical operations are at a University of North Dakota Laboratory biotech incubator in Grand Forks. Chief Executive **Tim Cooke**, a veteran of **Merck & Co. Inc.**'s vaccine division, telecommutes and travels from a one-man office near his Boston-area home.

"We are still a pretty virtual organization – our head of research and development lives in Philadelphia and our business development manager is in San Francisco," said Cooke, who relies on teleconferencing and cross-country flights to stay in touch with his people. "Technology and outsourcing enables us to be cost-effective, stay connected and have the best people working for us."

NovaDigm's experimental vaccine is in midstage clinical trials, with results expected by the end of next year. The company plans to apply for regulatory approval for the yeast infection vaccine first because those clinical studies will be less complicated and costly.

NovaDigm has raised $28 million from venture capitalists since 2008, with the most recent round closing last month. It also has more than $17 million in government grants. The company's venture backers include **Domain Associates** of San Diego and **RusnanoMedInvest** of Moscow.

If the vaccine goes to market, LA BioMed will earn licensing royalties. The institute also has a small equity stake that could pay off big if NovaDigm is later acquired by a larger drug company or goes public.

"They're really the research engine of our company and provide us with a lot of expertise in other ways as well," Cooke said of the scientists at LA BioMed. "They are very supportive and really easy to work with."

– Deborah Crowe

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Probing for Heart Trouble

UCLA pediatric cardiologist and **LA BioMed** researcher **Dr. Ruey-Kan Chang** leads a medical device startup, **QT Medical**, that has a full pipeline.

The company, incorporated in July, could one day commercialize Chang's collection of inventions. They range from a bedroom monitor to prevent Sudden Infant Death Syndrome to a pocket-size probe that tests newborns for a deadly congenital heart disorder.

Chang is QT's chief executive, but once he raises enough money from investors for clinical trials, he'll hire a more seasoned startup executive and open an off-campus office. That could start happening within the next few months.

QT MEDICAL

Headquarters: Torrance

Founder-Chief Executive: Dr. Ruey-Kan Chang

Business: Pediatric medical devices.

QT is one of two LA BioMed spin-offs set to present their technology this week at an annual investor conference sponsored by the **Southern California Biomedical Council** trade group.

"No sense to have all these ideas unless you can get them in the hands of doctors and parents," said Chang, who plans to present his pulse oximeter – the heart disease screening device for newborns – at Wednesday's conference in downtown L.A.'s Omni Hotel.

He is confident that there is a market for the device because the Health and Human Services Department in 2011 mandated that health facilities begin screening newborns for what is known as Long QT syndrome, a rare inherited heart condition that leads to a potentially deadly irregular heartbeat if not diagnosed and treated early. It can be detected with an electrocardiogram, but not reliably because they're not designed and calibrated for newborns. Chang's device would help hospitals meet the federal mandate, so he's filling a market void.

"About 100 babies in the United States die from this every year," Chang said. "What we have developed not only is made for newborns, but also can be made inexpensively enough to be covered by what the likely insurance reimbursement will be for the test."

Federal government grants have enabled Chang and his team to create prototypes for many of the devices that QT hopes to take to market. Chang himself does not have engineering training, but over the years has assembled a brain trust of device engineers who turn his ideas into reality. He said working at LA BioMed has provided him with the time and resources to work on these projects in between his work overseeing residents at **Harbor-UCLA Medical Center** on the same campus. The institute provided expertise in getting grants and collegial support.

"They give me so much flexibility and support in being inventive in my research," he said. "It's the reason I'm still working here."

– Deborah Crowe

Healing Arts: Dr. Ruey-Kan Chang at LA BioMed.

RINGO H.W. CHIU/ LABJ

Spinoff With Aesthetic Lift

AMGEN Inc. veteran **Keith Leonard** noticed that something was missing in the aesthetic medicine industry: scientific research to back up claims of beauty enhancement.

Also lacking: companies to meet the public's desire for new products.

"Over the last 12 years there has been a lot of patient demand for novel prescription products in the facial aesthetics market," Leonard said, "but not a lot of startup biopharma companies focused on that area."

At an industry meeting in 2005, Leonard met two UCLA dermatologists, **Adam Rotunda** and **Mike Kolodney**. Their research had demonstrated that deoxycholic acid caused fat cell destruction in vitro.

KYTHERA BIOPHARMACEUTICALS INC.

Headquarters: Calabasas

Founder-Chief Executive: Keith Leonard

Business: Injectable drug for the aesthetic medicine market.

So Leonard co-founded a venture capital-backed company, **Kythera Biopharmaceuticals Inc.**, that year that licensed the doctors' patent from LA BioMed. Through further research, Kythera developed a version of deoxycholic acid called ATX-101 that could be injected into the fat of the neck to reduce the double chins that often develop even in fit people as they age.

Leonard was especially excited about the prospect that ATX-101 could appeal to men in the facial aesthetics market, where patients are now 90 percent female.

"Market research shows that if men have wrinkles on their foreheads they're not particularly bothered by them," he said. "But if fat under their chin makes them look like their grandfather, or much heavier than they are, then getting rid of it is a pretty attractive prospect."

Kythera spent six years and about $200 million conducting global clinical trials, with positive Phase III results from the United States and Canada announced on Sept. 16. Leonard plans to file for approval from the U.S. Food and Drug Administration and expects sales to begin in 2015. The drug will be marketed under a name that is yet to be determined.

LA BioMed will get royalties on sales, Leonard said. He predicts that long-term U.S. and Canadian sales have the potential to reach $500 million annually.

"We think this will turn into a very large product that we can get into the hands of patients," he said. Bayer HealthCare holds the rights to develop ATX-101 outside the U.S. and Canada.

One year ago, Kythera went public. As of this month, the company's market cap was at $950 million. Shares have risen from $16 to about $45.

Leonard said LA BioMed was "incredibly important" to the company's origins and to its success. "It's harder than it looks to take these technologies and bring them out," he said. "But when it works it's really rewarding. LA BioMed is very good at getting their technology out into the world so it can be turned into real products."

– Karen E. Klein

Chin Up: Keith Leonard, chief executive of Kythera.