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Talking about Regeneration
‘There’s no reason Philadelphia shouldn’t be a hub’

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Every day in the labs of Philadelphia-area academic medical centers, research institutions, established life sciences companies and startups, scientists and clinical investigators are attempting to find new ways to repair faulty hearts, restore bones and even regenerate human organs.

They are all striving for the next breakthrough in one of the fastest-growing health-care fields.

All this activity leads to the question: Could Philadelphia become a hub for regenerative medicine?

“The one word answer to that question is yes,” said Dr. John Gearhart, director of the University of Pennsylvania’s Institute for Regenerative Medicine.

Getting to that point will require money. What’s needed, experts say, is government funding or private donations to support early-stage research, and investment capital from venture capital firms and pharmaceutical companies to help commercialize research discoveries.

The Philadelphia region already has a large inventory of scientific researchers and clinicians and a close proximity to many of the country’s major pharmaceutical companies.

“One of the issues is how do we get all of these people together?” Gearhart asked.

“There is a desire to do it, but how do we do it? It’s difficult to build bridges. When does industry invest? How far ahead do you come in and make a large investment when the return is years and years away? What would be ideal is for [state] government to appreciate the economic potential for investing in a hub, perhaps partnering with academic [entities] and venture capital firms to make it a reality.

Becoming a leading center for regenerative medicine would have a financial payoff for the region. Such a standing would attract venture capital, private donations and top researchers, while spurring new company formation and generating jobs.

“There’s no doubt there’s a huge amount of interest here in regenerative medicine,” said Gary K. Beauchamp, president of the Monell Chemical Senses Center. “There’s no reason Philadelphia shouldn’t be a hub. We are strong on biomedical research and have almost too many hospitals. This should be a hotbed.”

Strong Starting Point
The Philadelphia region is already active in the field of regenerative medicine. Consider:

► In West Philadelphia, researchers studying human baldness at the Penn’s Perelman School of Medicine successfully took human skin cells and transformed them into stem cells capable of generating hair follicles in mice.

► Out in Exton, Fibrocell Sciences – the only local publicly traded regenerative medicine company – markets a wrinkle treatment that involves extracting a person’s fibroblast cells, multiplying the sample into tens of millions of cells, then re-injecting those cells back into a patient’s skin to treat wrinkles.

► In South Jersey, the Coriell Institute for Medical Research in Camden manages a repository of pluripotent stem cells (adult cells that have been genet-
ically reprogrammed to an embryonic stem cell–like state) for use by researchers worldwide.

Also around the region, scientists at academic medical centers, researcher centers and startup companies are working on: grafting cells into the heart to repair faulty hearts (Penn); using computer-aided engineering to design and create human tissue to replace or repair organs (Drexel University) and creating a pill people can take instead of undergoing a root canal (Temple University).

**PENN’S POINT MAN**

Gearhart was recruited to the Penn Health System from Johns Hopkins University in 2008 to lead the Penn Institute for Regenerative Medicine, which was formed to promote basic discoveries in stem cell biology and regeneration — and to translate those discoveries into new therapies.

Gearhart is not a big fan of the term “regenerative medicine.”

“My problem with this term is that it is very broad and not intrinsically clear as to what it means,” he said. “Stem cell biology is one platform, but it is believed to be the most promising for treatments or therapies that are safe and effective. Stem cell biology covers not only the use of various sources of stem cells (not solely embryonic stem cells) to rebuild our bodies, but also the information attained through basic science research on stem cells that can be used to improve the capabilities of our bodies to rebuild our bodies following injury or disease.”

Gearhart also noted stem cells are just one component in a field that also encompasses devices, biologics and pharmaceuticals. “Anything you do to improve function when something is lost or damaged is regenerative medicine,” Gearhart said. “The scope of this is enormous.”

Ten companies created through Penn’s Upstart Program, which helps faculty form businesses based on their inventions and innovations, are involved in some aspect of regenerative medicine.

**MICRO ENVIRONMENTS**

One established Center City company that’s focused on regenerative medicine, absent the use of stem cells, is Bioquark Inc.

According to CEO Ira Pastor, Bioquark’s focus is on developing biologics for the regeneration and repair of human organs and tissues.

The company’s emphasis is on repairing diseased, damaged or aged tissues.

“You have the two ‘R’s,’” Pastor said. “Regeneration is the sexy one that conjures up the major dreams. The other ‘R’ is repair, where the market is 10 times larger.”

Bioquark is attempting to advance technology developed by the University of South Florida researcher Dr. Sergei Paylian, the company’s founder and chief scientific officer, that creates what the company describes as micro-environments that induce efficient and controllable regeneration and repair by creating dynamics in mature tissues that are normally only seen during human fetal development, as well as during limb and organ regeneration in organisms like amphibians.

“The idea is to slingshot back in time, and then take it forward,” Pastor said. “We’re not lacking for interesting applications.”

The company is working on a variety of fronts including: treating cancer by reprogramming tumor cells; developing cosmetic application to treat aged skin; and repairing organs and tissues in patients suffering from traumatic brain injury and end-stage kidney disease.
Pastor said what would be ideal for galvanizing the region’s regenerative medicine sector can be found about 500 miles to the south. Last year, David Murdock, the 90-year-old chairman of Dole, committed more than $500 million to create the North Carolina Research Campus and the David H. Murdock Research Institute in Kannapolis, N.C. The research complex is being designed as a place where researchers from government, industry, nonprofits and universities can use advanced technology and agricultural resources to collaborate.

“If you could create an ecosystem like that here for regenerative medicine, and just a small percentage of companies that are created because of it stay here, you’d have a really strong industry,” he said.

**ISOLATED POCKETS**

“Right now, we have a lot of isolated pockets” where regenerative medicine research is taking place, said Peter I. Lelkes, director of Temple University’s Institute for Regenerative Medicine. “What it would take to capitalize on the region’s strengths is a focused attempt to bring these people together under a funded umbrella. If you want to see how it could work, go to Pittsburgh.”

Pittsburgh is home to the McGowan Institute for Regenerative Medicine, which focuses on developing technology to address tissue and organ insufficiency. The institute traces its roots back to a $1 million donation from William and Sue Gin McGowan. William McGowan is the former CEO at MCI Communications. He became involved in supporting regenerative medicine research after undergoing a successful heart transplant at the University of Pittsburgh Medical Center in 1987.

With Philadelphia and Pennsylvania governments strapped for cash, it’s doubtful a regenerative medicine institute in Philadelphia could attract significant government backing. Lelkes, like others, said the region’s best bet would be a private donation from a corporation or individuals.

“Philadelphia is home to Comcast, which is paying $45 billion for Time Warner Cable,” Lelkes said. “If the company would consider donating one one-thousandth of that amount for [a regional regenerative medicine program], you’d have $45 million to get something off the ground.”

**CAUCUS SUPPORT**

State Sen. Andy Dinniman, co-chair of Life Sciences and Biotechnology Caucus, said he would favor Pennsylvania investing in an effort to make the Philadelphia region a center for regenerative medicine — but he acknowledged any financial support would likely have to wait until the states make a stronger economic recovery.

“If we could become a world center in this new area of biopharmaceutical and medical research there would be an economic return, but we are also talking about changing for the better the lives of thousands and thousands of patients,” said Dinniman, a Chester County Democrat. “If there’s a shortage of funds to do this right now, we could focus on figuring out the infrastructure we would need and identifying the key industry players and our strengths and weakness. That way when the economy improves we’d be ready to do something.”

Dinniman thinks the state took a step back when it shifted tobacco settlement funds from medical research to the state’s general fund. He said he would work with the Biotechnology Caucus to create bipartisan support for a proposal that would position the region as a leader in regenerative medicine, and work to get the support of the governor for such a plan.

“Philadelphia is hosting next year’s Biotechnology Industry Organization’s [convention] where all the key people in the industry from

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**UPSTARTS**

**PENN’S LAUNCHING PAD**

_The University of Pennsylvania Upstart Program, which helps faculty members form businesses based on their inventions and technological innovations, has launched 10 companies involved in some aspect of regenerative medicine. They are:

- **Axonia Medical**, founded by Dr. Douglas Smith, is developing a novel technology aimed at repairing severed or damaged peripheral nerves.

- **DermoBiotech**, founded by Dr. George Xu, is developing a human hair follicle stem cell platform for dermatology-focused regenerative medicine applications.

- **Epigen Cardio**, founded by Drs. Jonathan Epstein and Peter Gruber, is working on epigenetic modifiers to restore cardiac function following myocardial infarction and to treat cardiovascular diseases.

- **Metamorphosis**, founded by Dr. Ben Stanger, is focused on cell-based and biologic therapies for generating beta islet cells and treating diabetes treatment.

- **Novapeutics**, founded by Dr. Xianxin Hua, is developing pathway modulators to restore beta cell mass and treat diabetes.

- **Innolign Biomedical**, founded by Christopher Chen, is developing engineered cellular microenvironments that could be used in the treatment of human disease.

- **Skelegen**, founded by Dr. Kurt Hankenson and Michael Dishowitz, is developing novel tissue engineering strategies to promote bone regeneration for clinical indications including fractures, segmental defects, spinal fusion, and dental and craniofacial reconstruction.

- **Prohibix**, founded by Jason Burdick and Brendan Purcell, is working on injectable hydrogel technology to responsively dose protease inhibition within the extracellular matrix of the myocardium.

- **TPeR Technologies**, founded by James Eberwine, is developing a transcriptome-induced phenotype transfer platform that can be used to convert one differentiated cell type into another for drug screening and therapeutic purposes.

- **Vibe Therapeutics**, founded by Robert Pignolo, has developed an innovative platform to identify mechano-responsive molecules which can serve as new-generation musculoskeletal therapeutics.
“Comcast is paying $45 billion for Time Warner Cable. If the company would consider donating one one-thousandth of that amount, you’d have $45 million to get something off the ground.”

PETER I. LELKES, Temple Institute for Regenerative Medicine, talking about the possibility for a regional regenerative medicine program.

around the world are gathering here,” he said. “What a tremendous opportunity to announce such an initiative.”

Dr. Edmund Pribitkin, a specialist in otolaryngology/head and neck surgery at Thomas Jefferson University Hospital, said there is already one pocket of regenerative medicine where Philadelphia is already a leader.

“In sensory research, Philadelphia is already ahead of everyone else,” said Pribitkin, noting that Jefferson, Penn, Drexel and Monell researchers are collaborating. “Our academic institutions are working together to tackle problems individual people alone can’t solve. When you create a mass of researchers looking at the same problem, that’s when great breakthroughs happen.”

Beauchamp at the Monell Chemical Senses Center said another funding source for biomedical research has also fallen on hard times, with the National Institutes of Health budget reduced by 5 percent last year under sequestration.

“We don’t see a light at the end of the tunnel,” Beauchamp said. “Talk about shooting yourself in the foot. The United States has always been known for scientific innovation and research. It’s a big reason people want to come here.”

Monell is working with Jefferson on a research project using regenerative medicine to treat people who have lost the sense of smell. Beauchamp’s hope is they can use the seed funding from a private donor to generate early clinical data it can use to leverage an NIH grant.

“The real strength of Philadelphia is researchers are willing to work together,” he said, noting that’s not true in all major cities. “We don’t need to build something from scratch. Medical research is one of the strengths of this region. We need to build a system that rewards innovation.” Pribitkin said to attract funding the medical industry has to do a better job educating people about regenerative medicine.

“Some people think falsely that regenerative medicine is all about stem cells that are stolen from embryos and being used by scientists to cook up horrendous abominations,” he said.

“Stems cells and regenerative medicine, it’s all about healing. It’s all about allowing the body to heal itself.”