

How ARM's Edward Lanphier Is Fighting for Cell Therapy Cures that Save Patients and Portfolios

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COMPANIES MENTIONED

- Alliance for Regenerative Medicine

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Stem cell, gene therapy and genetically modified cell therapy companies have begun to attract serious capital from investors who understand biotechnology and have the wherewithal to move products into the clinic and into the market. But battles still loom on the political and reimbursement front. Edward Lanphier, founder and CEO of Sangamo BioSciences Inc. and current chairman of the Alliance for Regenerative Medicine, sees near-term milestones on the horizon, as well as additional inflows of validating capital from prominent investors. In this interview with [The Life Sciences Report](#), Lanphier describes upcoming milestones and share-moving catalysts, some of which will be revealed at ARM's Regen Med Investor Day.

Source: [George S. Mack of The Life Sciences Report](#)

The Life Sciences Report: You are chairman of the Alliance for Regenerative Medicine (ARM), which will hold its annual [Regen Med Investor Day](#) in New York City on March 25. How does ARM differ from broader industry groups like the Biotechnology Industry Organization (BIO) and the Pharmaceutical Research and Manufacturers of America (PhRMA)?

Edward Lanphier: There are several distinctions. ARM is focused on advanced therapies—technologies that involve development of stem cell research, gene therapies, and genetically modified cell therapies. These are cutting-edge technologies that ultimately, if successful, will change the whole paradigm of medical practice.

The goal for the majority of the groups represented by ARM—and the thing that really distinguishes companies working in the space—is moving past the existing model of pharmaceutical intervention. We endeavor to go beyond treatments and disease management to *curative outcomes*. Unique challenges confront companies working on this new paradigm.

"We endeavor to go beyond treatments and disease management to curative outcomes."

Beyond companies working in this area, ARM has a broader constituency of membership that includes patient advocacy organizations in specific disease areas, as well as research institutes focused on advanced therapies and regenerative medicine. This broad-based constituency drives how and where this technology can be applied, and exposes the challenges and opportunities encountered by such groundbreaking research.

TLRSR: Edward, the people involved with ARM have paved new ground for advocacy in a totally new sector of healthcare. They deserve a lot of credit, don't they?

EL: They do. Thanks to the leadership of people like Gil Van Bokkelen (chairman

and CEO of [Athersys Inc. \[ATHX:NASDAQ\]](#)), Geoff MacKay (president and CEO of Organogenesis [private]), Morrie Ruffin (cofounder and managing director of ARM and managing partner of Adjuvant Partners) and Michael Werner (cofounder and executive director of ARM and partner at Holland & Knight LLP), ARM now is the preeminent global advocacy organization for advanced therapies in the areas of stem cell, gene and cell therapies.

TLSR: A recent ARM presentation notes that 2014 was quite significant for financings for ARM companies. What got my attention was the significant investment from an important group of sponsors—the big pharma/big biotech companies. Can you comment on that?

EL: Yes. Over the last two to three years, investment in gene therapy, genetically modified cell therapies and regenerative medicine and stem cells has really accelerated. ARM has compiled data around this significant new investment, not just from the venture capital community, but from the public equity markets and also from large pharmaceutical and biotechnology companies. In the last two years, investment has risen on an exponential basis, and significant partnerships have been established between large pharmas and biopharmas and many of ARM's advanced therapy companies.

"Partnership payments from big pharmaceutical and biotech companies represent validation of the increasing maturity of these technologies."

TLSR: I'm looking at the numbers. Between 2013 and 2014, there are some very notable upswings in investment. Venture capital doubled—\$2.1 billion (\$2.1B) in 2014 versus \$1B in 2013—while initial public offerings (IPOs) almost tripled, to nearly \$1.4B. Follow-on private investment in public equity (PIPE) funding rose from \$1.4B to \$2.53B. But what really got my attention were the upfront partnership payments—not promises of milestone payments in the distant future, but rather actual cash payments—which rose from \$42M in 2013 to \$323M in 2014.

EL: That's what I'm talking about when I say growth has been exponential. The real industry growth in 2014 versus 2013 was unprecedented. Partnership payments from big pharmaceutical and biotech companies represent validation of the increasing maturity of these technologies. Future milestones from deals announced in 2014 could potentially reach \$9B, versus \$2.4B in potential milestones from deals announced in 2013. There is a lot of momentum in the space, and it has driven unique and transformative advances in advanced therapies.

TLSR: Discouraged investors have wondered when we would see critical mass in these industries, and we've been waiting for more than two decades. Are we now in the period of critical mass we have been awaiting?

EL: Yes, we are. Ultimately, I think we'll look back to this period and recognize cell therapy innovators who made a fundamental difference in the practice of medicine in the same way that Herb Boyer (founder of Genentech, now a unit of [Roche Holding AG \[RHHBY:OTCQX\]](#)), Bill Rutter (founder of Chiron Corp., now a unit of [Novartis AG \[NVS:NYSE\]](#)) and many of the other great scientists of the recombinant DNA generation were able to do. These scientists didn't just change medicine; they changed the lives of *patients*. I believe that people working in gene and cell therapy right now will have the same fundamental impact on both the practice of medicine and also on the quality of patients' lives.

TLSR: Let's talk about government relations for a moment. ARM is still very new, having been founded in 2009. One of the primary targets of the organization must be lawmakers, who you need on your side because you need policy on your side.

In addition, ARM members need funding from the National Institutes of Health (NIH). How do you talk to lawmakers? How do you explain these difficult concepts to them?

EL: If you can get their time, and if you can look them in the eye, the outcomes and the goals of the technologies that ARM member companies are developing are instantly—let me repeat, *instantly*—compelling to lawmakers and policymakers. They rapidly grasp not only the fundamental differences between these technologies and previous approaches, but also recognize the differences in potential outcomes. If explained properly, lawmakers appreciate that these approaches could be "one-and-done"—long-lasting, curative therapies that will result in enormous savings in healthcare dollars over existing therapies.

TLSR: Give me an example of what you mean. What examples do you talk about with lawmakers and policymakers?

EL: There are numerous examples. Take the genetic disease hemophilia and factor VIII, a clotting protein. Hundreds of thousands of dollars are spent every year on an individual hemophilia patient, and that patient will need therapy all his life. If you could administer a one-time therapy to a very young child that causes him to express the normal human factor VIII protein at curative levels, the ultimate cost savings to the government and to payers could be enormous. If you then spread those savings across all of the disease areas where gene therapy and genetically modified cell therapies can be utilized, the cost savings are even more immense.

"Lawmakers appreciate that these approaches could be 'one-and-done'—long-lasting, curative therapies that will result in enormous savings in healthcare dollars."

In addition to the obvious benefit to patients from a quality-of-life perspective, there are incredibly important indirect benefits and cost savings. Sure, you speak to politicians about improving the quality of life of their constituents, but ultimately, these technologies have the potential to save the healthcare system significant amounts of money. You really don't have a hard time getting lawmakers' attention.

TLSR: What is the most pressing need of the regenerative medicine, gene therapy and genetically modified cell therapy industries that government resources could meet?

EL: I think there are three things.

First, we would like to see policies that stimulate durable investment. These would be regulations or laws that motivate and stimulate investment in these high-risk, high-reward areas.

Then there are the regulatory issues. We are developing a new paradigm of therapeutic technologies that evolve, so open dialogue with regulators and dialogue with groups that oversee the process for approvals is critical. We need to make sure those lines of communication remain open and productive.

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The third important area is reimbursement. As we think about the value of these therapies to patients and the overall healthcare system, especially with regard to the potential cost savings associated with curative therapies and the challenges

encountered by developers, it's important to secure fair value for innovators. The companies and investors that are taking the risks and developing these groundbreaking, cutting-edge therapies have to be fairly rewarded.

These are all areas that ARM is quite interested in, that we have done a lot of work on, and that we will continue to work on.

TLSR: What should investors in regenerative medicine, gene therapy and genetically modified cell industries be listening for?

EL: I think 2015 and 2016 are going to be incredibly important years in terms of clinical translation, clinical data and product approval. I think there are going to be a lot of catalysts: a lot of important, tangible milestones associated with Phase 2 data, Phase 3 data, biologics license application (BLA) submissions and, potentially, product approvals.

TLSR: Where do you think we will see the first product approvals?

EL: I think it's yet to be determined. I don't know that my crystal ball is quite that clear, but with that said. . .

There are a several *in vivo* gene therapy programs involving adeno-associated virus (AAV) approaches. One of those programs, for lipoprotein lipase deficiency (LPLD; also called familial hyperchylomicronemia), a very rare genetic disease, has already been approved in Europe and is undergoing some additional testing in the U.S. for potential approval here. The agent is called Glybera (alipogene tiparvec), and it was developed by the Dutch company [uniQure N.V. \(QURE:NASDAQ\)](#). Also employing AAV, there is an ongoing Phase 3 clinical trial in the ophthalmic space that I think is very important.

On the stem cell side of things, Athersys is moving forward with important clinical data. [bluebird bio Inc. \(BLUE:NASDAQ\)](#) has exciting data in hematopoietic stem cells in beta thalassemia. In the chimeric antigen receptor (CAR) T-cell space, companies like Novartis and [Juno Therapeutics \(JUNO:NASDAQ\)](#) may well be in a position to file for approvals in the 2016–2017 time frame. There's diverse and broad-based momentum, and what I've just mentioned is really just the tip of the iceberg.

TLSR: One of the great things about ARM's Regen Med Investor Day is that the company presentations are webcast, and then they are edited and archived for later viewing. Can you tell me about that so that readers can watch?

EL: Absolutely. The [live stream will begin at 8:00 a.m. EDT \(5:00 a.m. PDT\) on March 25](#). We will have the videos of the conference up on [YouTube](#) about a week later, with a great deal of additional content. I hope your readers will tune in live or watch later.

TLSR: I'll see you in New York.

EL: I look forward to that. Thank you.

[Edward Lanphier](#), chairman of the Alliance for Regenerative Medicine, is also the founder of Sangamo BioSciences Inc. He has served as president, CEO and as a member of the board of directors since Sangamo's inception in 1995. Mr. Lanphier has more than 30 years of experience in the pharmaceutical and biotechnology industry. From June 1992 to May 1997, he held various positions at Somatix Therapy Corp., a gene therapy company, including executive vice president,

commercial development and chief financial officer. Prior to Somatix, he was president and CEO of BioGrowth Inc., a biotechnology company that merged with Celtrix Laboratories to form Celtrix Pharmaceuticals Inc. in 1991. From 1986 to 1987, he served as vice president of corporate development at Biotherapeutics Inc. From 1984 to 1986 Mr. Lanphier served as vice president of corporate development at Synergen Inc. Prior to Synergen, he was employed by Eli Lilly and Company, in the strategic business planning biotechnology group. He currently serves on the board of directors of the Biotechnology Institute, the board of directors and as a member of the Executive Committee of the Alliance for Regenerative Medicine, the board of trustees for The Buck Institute for Research on Aging, and the Dean's Advisory Board for the University of Michigan School of Public Health. Mr. Lanphier holds a bachelor's degree in biochemistry from Knox College.

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