Interview: Janet Lynch Lambert – CEO, Alliance for Regenerative Medicine (ARM), USA

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Janet Lynch Lambert, CEO of the Alliance for Regenerative Medicine (ARM), discusses the association's aims and membership, the importance of creating a supportive reimbursement environment for regenerative medicine, and the huge and expanding need for education in this critical area.

What was the initial inspiration behind setting up the Alliance for Regenerative Medicine (ARM)?

The Alliance came together based on the foresight of founders, Michael Werner and Morrie Ruffin, both of whom had pursued successful careers as senior officials at BIO. They saw that regenerative medicine was a very exciting, up-and-coming scientific niche within the biotechnology industry. They saw an excellent opportunity to provide the regenerative medicine community a watering hole of their own: a place to really discuss their issues, and to define what kind of policy agenda they will require for the future.

Since ARM's inception, we have been progressing in leaps and bounds. We now have more than 265 members at this point and can now deliver a very rich array of activities and member services. Having enjoyed a highly positive start-up phase, we now need to put the right elements in place to ensure a successful phase two and continue delivering value for our members, the sector and the

patients who rely on the successful commercialization of these therapies.

What are the priority issues on for the Alliance right now as you set about implementing this second phase?

Enabling a supportive reimbursement environment is a major focus for our members. The reimbursement landscape in the United States tends to be not only more complex than elsewhere, but also more dynamic. The science is so rich and we have so many products that are entering the market that it's crucial that the value of these unique therapies is appropriately assessed and reimbursed. It is important that we, as a group, are pursing an optimum agenda for making regenerative medicines accessible as broadly as possible. We are keen to work alongside regulators and all stakeholders to find win-win mechanisms that work well for payers, patients and therapeutic developers alike.

The passage last year of the 21st Century Cures Act was a major win for our sector. This legislation, includes a number of provisions that are specific to regenerative medicines, creating, for the first time in the US, specific regulatory approval pathways for regenerative and advance therapies, including enhanced interaction with the FDA and the use of real-world data.

Moreover, with more regenerative medicine products coming to market, we are going to be witnessing a rise in conversations around topics such as gene therapy, gene editing and cell-based medicine. Naturally, ARM is eager to play a role in shaping and guiding that debate. Our intention is to engage in this open dialogue by putting together a toolkit and series of communication campaigns to help people to properly understand the domain of regenerative medicine. Specifically we want to raise awareness about what makes this category of medicine unique and useful. There are many debates to be had about how we should be prescribing, regulating, consuming and paying for them, driving awareness and acceptance of these products, and speeding safe and efficacious products to patients in need.

At this particular stage, what should be the biggest driving force behind the future adoption of regenerative medicines? Which actors should be the lead protagonists in promoting this pathway?

Multiple stakeholders are important in each and every stage of the process. Patients are essential in understanding needs and thinking about tradeoffs and performing trials. We have patient advocates and several patient advocate organizations who are active members of our organization and we are always very interested in including their perspective into the deliberations. The immensely exciting thing about being at ARM is that our actions positively impact the lives of

people and their loved ones.

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Tell us more about the prevailing regulatory, reimbursement and pricing context. Historically the pharma industry has been poor at communicating why R&D costs have been so high. How can this pitfall be surmounted in the case of regenerative medicines?

Pricing is going to be a big issue and there is no getting away from that reality. For the regenerative medicine and advanced therapies community, it is not just a question of cost, but also the corresponding value of a product, in terms of the immense potential benefits these therapies offer patients, their families and society overall.

Take hemophilia, for example; the current course of treatment is often very expensive, and the gene or cell therapy approach to hemophilia may also be costly. Nevertheless, at the end of the day there is a trade-off to be made. The regenerative approach presents a more durable and possibly even permanent solution that brings tangible and material value to the patient. Also, it's important to keep in mind the cost trade off costs that would otherwise be spent on other kinds of treatment for the same patient.

In other cases, a cell or gene therapy might be introducing a treatment option into a space where there is presently none at all. It will be a question of providing solutions to patients who currently have no other course of action. A certain level of up-front investment is obviously going to be necessary, but, nevertheless, what is special about many of the regenerative therapies is their ability to be durable and, in some instances, even curative. This ability to deliver a potentially curative solution with a few doses, or even a single administration, is incredibly exciting to all stakeholders and I believe it has to be part of the future pricing conversation.

What do you see as the main concerns of the regulatory apparatus vis-à-vis regenerative medicine?

Right now, when we think about the regulatory arena in the US we think primarily about the FDA; their concerns almost always revolve around safety and efficacy. They aren't the folks worrying so much about pricing. Their foremost concern is whether products are safe to take, and whether they are as good or better than anything else on the market up until now.

The new head of the FDA, Dr. Scott Gottlieb, was recently appointed and we at ARM are encouraged by some of his initial comments about our sector. In a recent blog post, he revealed his team is working on the new regulatory framework on regenerative medicines. This framework, as

detailed in his post, endeavors to figure out how to effectively regulate the safety and efficacy of these treatments, while also making sure there are not unintended obstacles to the growth of the field and the ability of safe and efficacious treatments to reach patients.

To what extent does this sheer variety of players complicate matters?

In a European country, you will get a single answer from the authorities. It might not be the answer you desire, but at least it derives from a single source. In the United States, it's a much more fragmented system: we have Medicare, Medicaid, the inpatient apparatus, the outpatient apparatus, and each and every one of them have their specific reimbursement structures to navigate and to negotiate with. And that's just mentioning the big government programs.

ARM's Reimbursement Committee is currently involved in the creation of a three-part white paper series that is investigating and illuminating the different models by which regenerative medicines category products can potentially secure value-based reimbursement in view of their different nature and pricing contours. It might be a question of embracing performance-related models where the payer only pays under certain conditions and pre-specified endpoints, when the patient continues to succeed on the therapy. Split payment over time could represent another pathway forward, among others detailed in our white papers.

We obviously don't expect to ever arrive at a one-size-fits all arrangement that is suitable for all types of gene and cell therapies, and other regenerative medicines. Nevertheless, there is merit in proactively defining some of the mechanisms in which an effective reimbursement approach can be secured for this category of products.

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In short, we strive to articulate the barriers to a more flexible reimbursement system that could consider value, durability and the curative nature of these products in a way that the standard reimbursement model presently does not. Our aim is to define those possibilities, and posit new models that rethink the reimbursement framework, in addition to offering specific policy recommendations to overcome some of the possible barriers to widespread adoption of these products. There is still much work to do, but we are confident we are already on the right track.

Is the United States ready and able to sustain a role as a leader in regenerative medicine in the long run?

The US has demonstrated considerable leadership in this domain through the enormous investment in basic research through the NIH. US leadership also stems from the government policies, clusters, universities, venture capital and entrepreneurs that have really driven innovation. I am confident that this will continue to be the case in the long run. Whether the US can prove to be an innovator with respect to the reimbursement side very much remains to be seen, but we remain hopeful.

What is the best way to mature and accelerate the development of the regenerative medicine sector? It be will more desirable to see more venture capitalist investment with what that implies or big pharma getting more heavily involved in structuring the segment and making faster connections with the patients?

We need a virtuous combination of those elements. Generally, we have a very vibrant environment that is well supported by venture capital. Then, once companies grow to a certain stage, there is also the possibility of big pharma placing investments, and buying or absorbing that company. This, in turn makes the space even more attractive to venture capitalists so it becomes a virtuous circle.

The big pharmaceutical companies obviously have global marketing and regulatory know-how. It is a sign of the maturity of the technology and the space when big pharma starts to get involved. It is difficult to determine whether one specific element is more important than the other but having all those components is thoroughly advantageous. Capital and know-how are the keys to a healthy enabling environment.

Such organizations, including ARM members Novartis, GSK, J&J and Pfizer are an immense help to furthering the development of our space because they maintain various cell and gene therapy divisions and know what they are doing on the business strategy and commercialization side, as well as having the requisite scientific expertise. Having this sort of support and backing goes a long way to motivating scientists developing this new category of therapies and is exactly what we believe is needed for the whole market to flourish.

Your annual Cell & Gene Meeting on the Mesa is coming up in October. Is this event just about advocacy or are you striving for something beyond?

It definitely goes beyond advocacy. Our Meeting on the Mesa is a three-day conference that combines a two-day sector-specific Partnering Forum with a final day Scientific Symposium. Over the course of the event, more than 850 attendees will hear 60+ companies presenting really exciting results from their clinical investigations, along with 10 interactive, expert-led panels, more

than 1,100 partnering meetings, 30+ poster presentations and more.

With the FDA's recent announcement regarding a fresh pathway for regenerative medicine, we are expecting to have a lot of discussions about the evolving, policy environment.

Some people have voiced ethical concerns about regenerative medicine. How much of an obstacle is this?

Science has allowed us to move beyond certain ethical questions, for example, with the advent and widespread of use of induced pluripotent stem cells.

Recently, Oregon Health and Science University conducted the first known US-based experiment utilizing gene editing technology to modify a human embryo, to demonstrate various scientific concepts in relation to what could be done to assist people afflicted with genetic-based diseases. While the results of this experiment demonstrate is a step forward for gene editing science, there is little doubt that moving in a direction that allows us to make fundamental choices about the genetic makeup of the person not involved in the decision does present ethical considerations, which will require further debate and investigation.

Given the important and still unresolved safety, ethical and legal issues, our organization does not support clinical trials of human germline modifications at this time.

This sector is very local in many respects. Why, then, are you seeking to tackle it from an international perspective?

We are trying to recognize the reality that our members are living in. Healthcare is a global business, therefore it makes sense for us to be a global organization. Our members are not trying to make a product solely for a US market. They are seeking to create a therapy to be delivered in the US, Europe, Asia, etc., to serve patients all around the world. That's really why ARM aspires to be even more global than we already are.

Moreover, we want to learn from the companies in Europe and their experiences with the European regulatory structures to determine how can we apply that to the US and vice versa. And, of course, the investor community is global, so we want to make sure that they are aware and knowledgeable about the space from an international perspective.

Is there anything else you would like to share with our global readership?

> "Right now, there is this huge and expanding need for education in this space."

It is ARM’s goal to be the ‘go to’ information resource in the sector, not only for our members and industry, but also for all of our stakeholders.

Right now, there is this huge and expanding need for education in this space. We need to drive awareness of the sector because we are not dealing with hypotheticals anymore. This is not about something that is going to appear 10 years down the road. In a few months time, we’ll likely have several entirely new categories of medicine hitting the market and regulators and reimbursement systems alike have to be ready for them.

We would like the international community to know this sector is at an important inflection point, both scientifically and commercially. During these times of radical scientific momentum and evolution, it is ARM’s mission to serve as the “tip of the spear” to address the policymaking, regulatory and market access needs of that space, ensuring these important products reach patients in need.

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