**Where Do Cures Reside? Morrie Ruffin and Michael Werner of the Alliance for Regenerative Medicine Think the Answer Is in Regenerative Medicine**

The practice of medicine is being transformed. The journey has been arduous, but revolutionary stem cell, gene and immunocellular therapies are rapidly moving toward pivotal milestones—and investors in the space should strap in for a rewarding joy ride. In this interview with [***The Life Sciences Report***](http://www.thelifesciencesreport.com/)*,* Alliance for Regenerative Medicine cofounders Morrie Ruffin and Michael Werner consider how new federal guidance might enable new standards of care in cardiovascular, neurologic and oncologic medicine, and offer a preview of next week's Biotech Showcase in San Francisco.



***The Life Sciences Report***: On Dec. 22, the stem cell and regenerative medicine space saw a very significant development. The U.S. Food and Drug Administration (FDA) published a new [**draft guidance paper**](http://www.regulations.gov/#!documentDetail;D=FDA-2014-D-1696-0002) on what constitutes minimal manipulation of human tissues and cells. Although this document is not binding, it does tell us what the agency is thinking. In effect, tissues and cells that have been processed in some way will be treated as drugs, and will come under the regulatory umbrella of the FDA. What does this mean for the industry?

**Michael Werner**: As sometimes happens with government agencies, major regulatory documents are released right before the holidays. As far as the Alliance for Regenerative Medicine (ARM) is concerned, our member companies are currently looking at the paper and trying to digest it. This draft guidance is open for comment for 60 days after its publication in the Federal Register. We will go through it with a fine-tooth comb, and we will prepare formal comments, which we will submit to the FDA early this year.

Generally speaking, I think the most significant aspect of the guidance is that it provides greater clarity on the FDA's definition of minimal manipulation and, therefore, which products can qualify as minimally manipulated, and which will be regulated as biologics or drugs.

What ARM has said, from its inception many years ago, is that this industry needs a clear and predictable regulatory pathway. The regulations for minimal manipulation have been around for a while, but as this field has expanded, and as the technology has changed, questions have arisen about how the FDA is applying its regulations, and what “minimally manipulated” means in the context of new, tissue-engineered products.

At the very least, it's important that the FDA has, through this document, been transparent about its views.

**TLSR**: Michael, will you explain further why this transparency is important?

**MW:**Companies need to know what the FDA is thinking. We've had conversations with individual companies that think the FDA will regulate them as a 361 HCT/P, but are not totally sure, which makes it hard to plan clinical programs. The fact that the FDA has come forward with a document like this is great. As always, the devil is in the details, and we will certainly be going over the document carefully. But published guidelines are a good thing.

**TLSR**: Could this guidance, in effect, run retail or storefront stem cell therapeutic clinics out of business?

**MW:**Time will tell. In terms of its impact on what you describe as storefront, I think it really comes down to this: If you are running one of those kinds of operations, or if you perform so-called stem cell tourism, you are now on notice about what kinds of technologies, processes and products are subject to FDA regulation. If you're a storefront clinic, you now know that the FDA not only believes it has jurisdiction to regulate, but how it's going to regulate in the days ahead. It lays the foundation for FDA's enforcement actions going forward.

***"Over the next year or two, you will see a number of very significant data events around cell therapy."—Morrie Ruffin***

I think we can expect the agency to use its enforcement discretion in a more consistent way. How aggressive it will be in terms of going after storefront clinics remains to be seen.

**TLSR**: Do you feel that this, in the long run, is going to be positive for the regenerative medicine and stem cell industry?

**MW:**I think it will. If you're a product developer, what you want is clarity. You want to know where the goalposts are, and you don't want the goalposts to keep moving. If the FDA is transparent, then that's 80% of the ballgame for the product developer.

Now, in terms of specific policies, the specific ways FDA is going to apply the regulations—that remains to be seen. Companies can—and will—go back to the FDA and say "Here are our thoughts about some of the specifics." Of course, each individual company must go through an iterative process with the agency regardless.

**TLSR**: It feels like the track to progress, and to the market, for the regenerative medicine industry has been even more difficult than it was for monoclonal antibodies, for lack of a better comparator. What do you think has held the industry back?

**MW:** One thing has been the clarity of the regulatory pathway. You describe the industry as being held back, but I'd like to say, more positively, that more clarity and predictability in the pathway would enable more products to move forward.

Another key issue involves standards, which has been acknowledged by the FDA and industry as important in terms of enabling more products to come to market. We also need changes to the reimbursement and payment systems, both public and private, to reward innovation. These are different products and models, differentiated by how they treat diseases at the underlying molecular and cellular level, and they could lead not only to treatments, but also to cures.

***"More clarity and predictability in the cell therapy pathway would enable more products to move forward."—Michael Werner***

We need support from the U.S. government in terms of policies, in the same way that governments in Japan, the United Kingdom and elsewhere around the world have been enacting policies specifically designed to support the sector. The U.S. government needs to do the same.

The field is now poised to make a significant impact on healthcare. It's hard to say what's held the field back, but reimbursement is certainly a challenge that we need to get our hands around, to make sure the sector reaches its potential.

**TLSR**: Morrie, did you want to comment? The cell therapy/regenerative medicine sector has been in development now for about two decades-plus, yet I see only a couple of stem cell companies with a market cap above $1 billion ($1B), and all the rest are in the penny-stock to $500 million category. Why have cell technology companies lagged biotech?

**Morrie Ruffin**: We actually don't see it that way. That's one of the things we will show in the data that will be coming out in our Regenerative Medicine and Advanced Therapies State of the Industry Briefing on Jan. 12 at the [**Biotech Showcase**](http://www.ebdgroup.com/bts/index.php?utm_source=Biotech_Showcase&utm_medium=email&utm_content=link&utm_campaign=BTS15_streetwise121714) in San Francisco.

Clearly, as in any sector, there are going to be successes and failures, but we do see a very significant and recognizable upward trend in the amount of money being raised in the sector. The cancer immunotherapy space is drawing a lot of interest right now.

But if you look across the board at cell therapy, gene therapy and immunocellular therapy companies, we count nine to 10 public companies with $1B-plus market caps in the sector right now. And there are obviously a number of private companies raising significant amounts from private investors as well.

**TLSR**: Morrie, are we on the verge of seeing pivotal data come out of companies in the sector?

**MR:**We are about to see a number of very significant events in the sector. Clearly, both those working in the industry and investors are looking for positive clinical events—milestones—that signal we are making progress in the clinic, and that we understand how we might commercialize these therapies. Over the next year or two, you will see a number of very significant data events around cell therapy. We are already seeing this in the oncology space, which is why there is so much interest in what's happening with cell-based or immunotherapies. This has led to a significant interest in cellular therapies across the board. Investors are going to hear a lot in the near future, as people begin to understand the clinical milestones that are going to drive this sector over the next 12–18 months.

**TLSR**: What do you think is the greatest obstacle to this industry moving forward? Is that issue being addressed currently?

**MR**: One of the challenges in this space—and I think this is to be expected in any evolving sector—is the issue of commercialization, which concerns manufacturing, scale-up and all the things required to make these new products available and successful. I believe that we have made tremendous progress just in the last few years in understanding what this will take, whether it's an allogeneic therapy or an autologous therapy. I think this is one of the reasons we see manufacturing and tool companies making significant investments in the sector. Companies like GE Healthcare (a unit of [**General Electric Co. [GE:NYSE]**](http://www.theenergyreport.com/pub/co/2190)),[**Thermo Fisher Scientific Inc. (TMO:NYSE)**](http://www.thelifesciencesreport.com/pub/co/4662), the [**Lonza Group AG (LONN:SIX; LO3:FSE; LZAGF:OTCPK)**](http://www.thelifesciencesreport.com/pub/co/4920) and others are heavily engaged in this space. They see the huge opportunity here.

***"Infrastructure is going to be an important part of how these therapies are brought to the market, and to the patient."—Morrie Ruffin***

The cell therapy companies also understand that infrastructure is going to be an important part of how these therapies are brought to the market, and to the patient. One of the things we have been anticipating is having the processes of manufacturing, handling, and scale-up catch up with the lab bench science, and then duplicating that science on a large, profitable scale. I think we are on the cusp of that.

**TLSR**: Michael, you raised a point about government being involved in regenerative medicine, and you mentioned Japan, which has a new regulatory pathway in place whereby companies, in effect, have to show safety and the equivalent of Phase 2 proof of concept to get conditional approval for a cell therapy. Do you see the U.S. doing anything like that?

**MW**: I'll put it this way: I think it's possible the U.S. will take steps analogous to what other countries are doing to support the field. Is the FDA going to give conditional approval if a company shows certain kinds of safety data? I'm not sure about that, but I do think it is possible to take steps to smooth the pathway forward. For instance, we've talked with the FDA about standards in product development and manufacture. This is an idea that the FDA itself has said is critical to its product review process. Other ideas could include expedited approval programs. Such a program was recently created in the U.S. to support development of antibiotics.

The question is whether the kinds of programs that already exist can be applied or adapted to regenerative medicine, cell therapy and gene therapy products. I think you're more likely to see changes in existing U.S. policy, rather than copying Japan's policy or copying what other countries are doing.

**TLSR**: What do you think will be the motivating factor for policymakers to accelerate cell therapy development?

**MW**: There is more recognition that this sector could, potentially, cure diseases that have gone untreated or have been deemed incurable. In diseases like stroke or heart disease, we are basically treating symptoms. We are certainly helping people, but we are not actually curing disease. We're not getting people all the way back to health.

***"We need changes to the reimbursement and payment systems, both public and private, to reward innovation."—Michael Werner***

I think there's a greater recognition on the part of policymakers, as well as others, that regenerative medicine—and we use that term very broadly—is where cures and treatments are going to reside. Therefore, you're starting to see more acknowledgment of that. The U.S. government needs to make sure there are policies in place that will allow this industry to thrive.

**TLSR**: Morrie, you and Michael are going to be at the Biotech Showcase in San Francisco. What will you and ARM members be doing for attendees?

**MR**: One of the things we're doing, at the kick-off for the showcase at 8 a.m. on Monday, Jan. 12, is presenting our Regenerative Medicine and Advanced Therapies State of the Industry Briefing. This will be our fifth briefing at the Showcase. In the two-hour briefing, we will provide information on the performance of the sector. We will look back at 2014 and 2013, highlighting the progress made in the sector. Then we will look forward to what we anticipate to be major milestones and major events over the coming two years.

**TLSR**: Will there be an emphasis on any particular topic, or will it be a very broad briefing?

**MR**: We will be talking about several different things, but we plan to spend a lot of time talking about the progress being made in the gene therapy sector and genetically modified cell therapies.

When we talk about advanced therapies in regenerative medicine, we are being inclusive of all the companies working in the *in vivo* and *ex vivo* gene therapy areas, whether that's cancer immunotherapy, or CAR (chimeric antigen receptor) T-cell receptors, or other strategies being employed to harness the immune system to attack cancer. This will be a big part of the briefing. We will also look forward to a number of the major data events that we anticipate in 2015, in a number of indications, including stroke, cardiovascular disease and a number of neurodegenerative disorders.

**TLSR**: Thank you both for your insights.

****[***Morrie Ruffin***](http://www.thelifesciencesreport.com/pub/htdocs/expert.html?id=6810)*has more than 20 years of experience in the biotech and healthcare industries. He is a founder and managing director of the Alliance for Regenerative Medicine, the global organization representing the interests of the regenerative medicine community. Ruffin is also the managing partner of Adjuvant Partners, a boutique regenerative medicine and advanced therapies business consulting firm. Prior to joining Adjuvant Partners, he was the chief executive officer of LifeTech Innovations LLC (LTI), a business development consulting firm. Prior to his position at LTI, he was executive vice president of capital formation and business development at the Biotechnology Industry Organization (BIO), the largest trade organization representing the biotech and drug development industries. Prior to joining BIO, Ruffin worked for U.S. Senator Arlen Specter for five years as his senior legislative assistant. He received his master's degree in international studies and economics from the Johns Hopkins School for Advanced International Studies and his bachelor's degree from the University of Virginia.*

****[***Michael Werner***](http://www.thelifesciencesreport.com/pub/htdocs/expert.html?id=11513)*is a partner in Holland & Knight's Washington, D.C. office. He has almost three decades of healthcare law, lobbying, regulatory, reimbursement and policy development experience in Washington. He is also is the cofounder and executive director of the Alliance for Regenerative Medicine, a Washington, D.C.-based organization whose mission is to advocate for federal funding, regulatory and reimbursement policies that will advance regenerative medicine research and product development. Before joining Holland & Knight, Werner was president of The Werner Group, a Washington, D.C.-based firm that provided lobbying, regulatory, and bioethics consulting services for biotechnology and pharmaceutical companies, physicians, health plans, investors, and patient advocacy groups. Prior to founding The Werner Group, he was chief of policy for the Biotechnology Industry Organization (BIO), representing over 1,000 biotechnology companies in the U.S. and other countries. Werner is also a founding member of the Board of Directors of the Coalition for the Advancement of Medical Research. He was senior healthcare advisor to U.S. Senate Majority Leader George Mitchell, a congressional investigator for the U.S. Senate Special Committee on Aging, and senior advisor to Maryland Governor William Donald Schaefer. Werner is a frequent media commentator and has appeared in*the Wall Street Journal, Science, Scientific American, the Washington Post, BIOWorld, Congressional Quarterly*and*the Baltimore Sun*, as well as on many TV and radio news programs. In 2013, he was named one of the Top 50 Global Stem Cell Influencers by Total BioPharma.*

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