Global expansion for Advanced Therapies companies: Top 5 Challenges and how to address them

By Kurt Gielen, Brightlands Chemelot Campus

During the 2015 Phacilitate meeting, an informal round table discussion was organized to look at the top challenges companies active in the field of Advanced Therapies are facing when it comes to global expansion. During the 1 hour discussion a number of challenges and issues were raised. In this article I'm highlighting the top 5 challenges. Where suggestions for solutions were given, I include these here as well.

Nr. 1: Every country is different.

Yes, this is stating the obvious. However, it is only when you have 12 people with diverse backgrounds from a variety of companies in the field of Advanced Therapies specifically addressing these issues that it becomes crystal clear how much of a problem this is for the advancement of the Regenerative Medicine Industry as a whole.

Whether it is differences in reimbursement, market approval, pricing, testing requirements, import and transportation of critical goods like living tissues or cells, to manufacturing; each and every one of these issues is evolving in itself as the industry matures. The fact that almost each country has different requirements makes it hard for the industry to move forward in a swift and coordinated manner.

Japan has to operationalize their accelerated program to benefit from the loosening of regulations.

(Jaewook Jeon, Tego Science)

What we all need at this point is conformity. And, more importantly, regulators need to be willing to work towards uniformity as well. Currently, the major players (EMA, FDA, Japan, and Canada) have regulations that are well known to companies active in those regions, and the participants of the discussions think it would be wise for the major regions to collaborate with other countries and regions that are not so far advanced yet.

E.g., South America as a whole region could benefit from what is already done in the U.S. in order to facilitate the local regulatory bodies when it comes to advanced therapies. EMA is already aligned with the Middle East and Australia looks to FDA, Canada, and EMA as a benchmark. It would be applauded if a similar approach comes to fruition in Asia Pacific, as currently there are a lot of countries working in an uncoordinated way. It was mentioned that for example the Philippines are very active in the field, unlike Malaysia where a large number of wealthy patients go to Germany to get

therapies that are regulated under hospital exemption. Uniformity needs to be applied at every level of government as well.

Obviously, everybody is looking at Japan to see how they are going to operationalize their loosened regulations. If this works out well, it was recommended that we, as an industry take this model to the other regions and work within our own network, big or small, to get this adopted as the preferred model.

Nr. 2: The impact of the C-word

How do you make it clear to regulators, health insurance companies, and the general public that in the past, most of what we call medicine was actually palliative care? And that what Regenerative Medicine is doing is actually curing diseases?

Scientific evidence is a given, that will get you at the table of investors. Today you need to have your health economics data ready and clear as well.

(Steven Breazzano, Piper Jaffray)

Of course, when it comes to Health Insurance companies, the first thing that they want to see is **hard data**. If they are going to reimburse these new, and often expensive therapies, they want to be absolutely sure that the results are solid and long lasting. What became obvious during our discussion in Washington is that, we as an industry, fail to meet these demands. As one of the participants commented: "*Scientific evidence is not enough to get reimbursed. In E.U., Canada, and Australia you need to show that your therapy is clinically and cost effective!"*

Too often, startups focus on getting financing for their clinical trials and the regulatory approval. However, you need to include enough financial support to get the reimbursement and market uptake financed as well. Because the end point is not market approval you need to have the right end in mind, which is market acceptance – and unfortunately not every therapy will be licensed or acquired by one of the big pharma's (see nr. 4).

As commented by the investor at the table, for investors clinical evidence is a given; without it you won't even be invited to the table. What will really make a difference is if you have the rest of your story worked out as well. Be creative when it comes to patient access schemes, identify patient organizations as potential partners to get you to a successful market uptake and plan all the way to market succes.

Nr. 3: Sharing lessons learned

We should not look back unless it is to derive useful lessons from past errors, and for the purpose of profiting by dearly bought experience.

(George Washington)

Because our industry is moving ahead at an amazing speed, it is key to stay focused on the road ahead as this is the time where big leaps forward can be made. At the same time, we have built up some (expensive) experience by now, so it is important to ever so often stop to derive useful lessons.

As in the quote from Mr. Washington above, the experience in the industry was bought dearly, let's make

sure we all learn something from it. A shout out was given to industry organizations such as ARM to continue their industry-wide work, focusing on lessons learned and identifying best practices. But organizations such as Phacilitate were also mentioned to stay closely aligned with the industry and to continue to adapt their conferences and shows to stay aligned with the evolving needs of the industry.

Only if we start to apply the lessons learned and start sharing best practices, we will be able to *transform this industry from an industry that is spending more than it earns to a viable industry.*

Nr. 4: Just not enough big pharma.

" Novartis slashes ante, inks another Gamida Cell buyout pact in $635M leukemia deal"

"Celgene commits $3.3B in whopper cancer stem cell deal with OncoMed"

"J&J pours another $20M into ViaCyte and sizes up its diabetes treatment"

With headlines like the ones above (all from 2014) it is easy to be misled that all hail will come from big pharma. If all 15 companies who are involved in pharma and doing more than $15B in annual revenue would just do 34 deals each, then all 517 companies involved in Cell and Gene Therapy would have a secure go-to-market strategy.

Unfortunately things don't work this way. While those big pharma companies still have tremendous M&A power, they too are more focused on their core business than ever. They are prioritizing innovation in disease areas that fit with their historic strengths. It is wise to move away from the idea that big pharma is interested in Regenerative Medicine as a whole as this is becoming too broad. GSK for example is primarily focused on ex-vivo T-cell work. Horribly complicated, they admit. However, this is what historically they've been working on.

A suggestion was made that the best way forward is to start assembling critical mass in specific disease or therapy areas. Wound healing was discussed as an example where a different look at the current market and its players might open up potential new companies to partner with. Rather than looking at cell therapy for wound care as a new paradigm shift, it was suggested to align this as an advanced therapy alongside more traditional treatments like wound closure products, anti-infectives and basic treatments, such as films and cleansing. By doing so you offer health care providers a step by step overview from basic to truly advanced treatments and they get a more aligned overview of available therapies. All of a sudden, you might find yourself looking at companies such as 3M and Molnlycke Health Care as companies to license your wound care cell therapies to.

Nr. 5: Paradigm shift after paradigm shift

One of the most striking things during this one hour discussion was how often the words "paradigm shift" were used. Whether it was a ***Business model paradigm shift*** that was discussed because innovation is coming from >500 new companies

rather than the big pharma, a ***Health Care paradigm shift*** from palliative treatments to true cures, or the ***Reimbursement paradigm shift*** that follows the above paradigm shift, it seems that the Regenerative Medicine industry is putting everything upside down. No simple solution was given to this general observation.

For me, chairing this roundtable discussion, the most important conclusion is this: We're all in this together; the only way to make this work is through open collaboration across the industry and beyond. And to extend our communications all the way to patient advocacy groups, health insurance companies, even to patients.

If you want to go fast, go alone. If you want to go far, go together.

(African Proverb)

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