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editorial

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The challenges of product development and commercialization in a convergence technology world: focus on regenerative medicine

A new paradigm in health care

Regenerative medicine has long been thought of as the future of medicine – science fiction, even. Yet, within the past several years, what was once 'science fiction' is becoming reality, and has the potential to become a far-reaching reality in the coming years.

By focusing on restoring tissue damaged by injury, disease or the body's natural aging process, to normal structure and function, the field of regenerative medicine represents a new paradigm in health care that will fulfill unmet medical needs and hold the promise of transforming the way companies discover and produce new treatments, and the way providers deliver health care. By enabling delivery of state-of-the-art treatments for diseases ranging from diabetes to cancer, regenerative medicine is a true universal medical advancement.

Daily developments in the field of regenerative medicine bring the future of medicine closer within our reach. So far, researchers have grown beating hearts, created artificial blood, and bioengineered skin, ears, bladders and ligaments by re-growing tissue. In addition, various regulators, such as the Food and Drug Administration (FDA) and the European Medicines Agency (EMEA), have approved some regenerative medicine products – which often represent a convergence of pharmaceutical, device and biologic technologies – including dermal and joint substitutes and bone marrow for orthopedic uses. However, we have only begun to scratch the surface of possibility.

Because of the tremendous potential of regenerative medicine, commentators have sensationalized the field as a 'miracle medicine,' resulting in unrealistic expectations around the promise of regenerative medicine, as depicted in the Peak of Inflated Expectations in the Gartner Hype Cycle (Fig. 1) [1]. Yet, as researchers learn more about living cell technologies and continue to educate about progress in the field and the potential of products to help restore and rebuild living tissues, the industry is pushing up the Slope of Enlightenment toward the Plateau of Productivity.

To help reach the Plateau of Productivity, a great deal of work needs to be done to educate the systems that oversee these complex components to fully understand the new science/business approach to regenerative medicine. This has created a need for the regenerative medicine community – academic and corporate, including large, established companies and start-up companies – to speak with one voice about the immense clinical value of regenerative medicine technologies. In response to this, last year leaders in the regenerative medicine community created the Regenerative Medicine Foundation (RMF), a not-for-profit organi-

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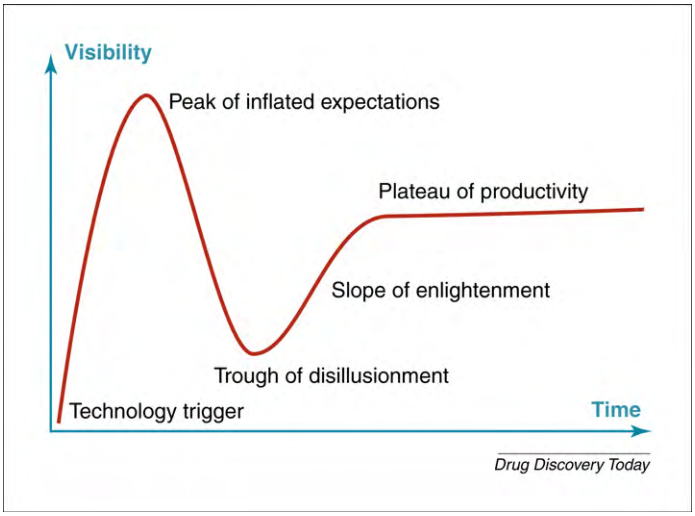

FIGURE 1
Gartner Hype Cycle.

zation, to advance new treatments and therapies based on tissue engineering and regenerative medicine. Through education, advocacy, and research, the RMF aims to facilitate and advance the current regenerative medicine science from laboratories to clinical settings.

The RMF was a founding member of the Alliance for Regenerative Medicine (ARM), also formed in 2009, to educate key policy makers about the potential of regenerative medicine and to advocate for favorable public policies that enable advances in the field. Today, the ARM has more than 40 member companies, foundations/associations, research institutions and investors, and continues to add new members.

A different approach

The RMF and the ARM are making great strides in educating and advocating for the field of regenerative medicine, and companies in the industry remain committed to innovating some of the most fascinating technologies. However, successfully commercializing these complex products will require developers to take a vastly different approach to product development and commercialization than pharmaceutical, device and biologic technologies currently require.

Companies that successfully commercialize regenerative medicine products are challenged every day to strike the right balance of resource allocation between product development and commercialization efforts, including regulatory, research and development, manufacturing, logistics, reimbursement, and sales and marketing strategies. By addressing these challenges early in the product development process and combining competencies from pharmaceutical, device and biologic technology sectors, companies are more likely to succeed and reach their commercial goals. Let us take a look at each of these efforts:

Regulatory

The regulatory pathway for regenerative medicine products remains largely undefined, because the standards for safety, efficacy and consistency have not been fully established. The challenge lies in the fact that many regenerative medicine therapies contain components from various product categories that face different regulatory review. Standards and guidelines are evolving, but until regulatory agencies like the FDA or the EMEA refine a process for reviewing convergence technology products, exact regulations on the manufacture of a convergence technology product will depend on the primary designation each agency applies to the product. Different regulatory agencies have different requirements, necessitating companies to establish processes that comply with the strictest regulations for which the company intends to seek approval.

Research and development

In developing regenerative medicine products, the living cells that comprise the product are central to the research and development effort. Scientists are responsible for growing cells that are optimal for therapeutic uses and free of contaminants, that allow for safe and reliable expansion, and that are controlled for differentiation.

However, there are many components beyond the living cells that companies need to consider during the research and development phase. For example, many regenerative medicine products require a certain structure for delivery to the recipient. Seeding cells into a biodegradable scaffold has evolved as a method for the reconstruction of various tissues and organs [2]. Putting cells on a scaffold requires not only biology, but also engineering mechanics. Delivery of the product to the intended site is another example. Companies must consider how they will deliver the product, whether through an injection, surgical procedure or another process. As a result, a company's research and development team must possess the expertise and capabilities enabling them to address these issues during development.

Manufacturing

To fulfill manufacturing requirements of these complex products, companies must comply with a vast array of regulations covering product manufacturing, packaging, facility validation, consistency and safety of materials, sterility of the product, and where appropriate, sterilization and viral inactivation procedures.

Manufacturing requirements can vary greatly between international regulatory agencies. Consequently, establishing manufacturing guidelines at the onset of product development that might meet one agency's requirements might not be sufficient for a different regulatory agency. An early understanding of the regulatory requirements of all potential markets where the product might be sold is essential. Changing any step of the manufacturing process would be very difficult without requiring an entirely new clinical development program, adding an increased level of importance to the early stages of the manufacturing processes.

Further, because of the ever-changing regulatory environment and increased stringency of regulations, it is crucial for manufacturers to continuously assess their processes and accreditations to ensure they remain in compliance with current regulations.

Logistics

How a company manages logistics of shipping live cells is another important consideration that companies should address during product development. Living cell products must be handled and transported under precisely controlled conditions, in a time-sensitive manner, and might require transportation in validated

shipping containers. Further, because these are typically living cell products that might require cryopreservation, companies might need to implement cold chain logistics and distribution capabilities, increasing the complexity of the process.

Reimbursement

The process for obtaining reimbursement by private insurers and the Centers for Medicare and Medicaid Services (CMS), the largest insurer in the U.S., can be challenging because of the emerging state of the industry. However, similar to pharmaceutical, biologic and device products, it is imperative that regenerative medicine products be reimbursed by the payor system if companies are to successfully deliver them to patients on a long-term basis.

Companies that understand the system and where their products fit within the economic and healthcare ecosystem before market launch are suited to succeed commercially. In doing so, a company might define how they are improving care with their product compared with the current standard of care and make a justification based on the cost-effectiveness of their solution. Although relatively established in Europe, this move toward cost-effectiveness as a key element in the approval and reimbursement of a product is evolving and becoming more crucial in the U.S. healthcare system.

Sales and marketing

Awareness and understanding of regenerative medicine is essential to the success of each emerging product in the field. Just as the RMF and the ARM are increasing awareness of the field of regenerative medicine by educating consumers on new treatment paradigms, companies can help further awareness of the field and in turn, acceptance of their products as new treatment options. The complexity of regenerative medicine products enables companies to gain a competitive advantage by deploying a specialized science-based commercial team that understands the clinical need for and benefits of a product.

Conclusion

Although regenerative medicine has yet to achieve its full potential, there are a few companies that have been successful in commercializing regenerative medicine products. The number will grow as the industry gains a better understanding of how to develop and commercialize products in this new convergence technology landscape. The crux of this challenge is not becoming wed to a single way of thinking, for example, pharmaceutical, device or biologic. Organizations that recognize this early on, and gain a thorough understanding of the various components of product development and commercialization, will achieve greater success in meeting the challenges of bringing new products to market.

References

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