Challenges in the Translation and Commercialization of Regenerative Medicine

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Cell-based therapies and regenerative medicine are an emerging form of healthcare than offer significant potential to improve the practice of medicine and provide benefits to patients who currently have limited or no treatment options. Ideally, these innovative therapies can complement existing small molecule, biologic and device approaches – forming a so-called fourth pillar of medicine – and allowing clinicians to identify the best treatment approach for each patient. Despite this potential, cell therapies are substantially more complex than small molecule or biologic interventions. This complexity poses challenges for scientists and firms developing cell therapies and regulators seeking to oversee this growing area of medicine.

In this project, we retrospectively examine the development of seven cell therapies – including three autologous interventions (Epicel, Carticel and Provenge) and four allogeneic interventions (Apligraf, Dermagraft, Prochymal and Osteocel Plus) – with the aim of identifying common challenges and promising strategies to help scientists, firms and regulators successfully bring new cell therapies to market. We complement this analysis with a series of qualitative interviews with experts in various aspects of regenerative medicine, including people working in academia and industry as well as those working in relevant portions of the financial sector.

Our analysis, developed through review of existing literature collected from company documents, newspapers, journals, analyst reports and similar sources, and refined through analysis of the qualitative interviews, identified several common challenges that cell therapy and regenerative medicine firms must address in both the pre- and post-market stages. Key pre-market challenges included identifying and maintaining stable funding to see firms through lengthy developmental timelines and uncertain regulatory processes. These challenges are not unique to cell therapies, of course, but the novelty of cell-based interventions complicates these efforts compared to small molecule or biologic interventions. The atypical nature of cell therapies also led to post-market difficulties, including challenges navigating the reimbursement process and convincing providers to change their treatment approaches. In addition, managing the cost of producing, storing and distributing cell therapies at scale was a challenge that started pre-market and continued into the post-market phase. We conclude by identifying key pitfalls and best practices applicable to the development of future cell and stem cell therapies and considering how these lessons might apply to other emerging technologies.