Cell Therapy Commercialization: Look for Headroom

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Of all the formidable challenges that a biotechnology company will stumble upon in its quest to commercialize, perhaps there is none more frustrating than a failure to get a product reimbursed by payers.

After years of navigating the gauntlet of risk that is biotechnology development, and sinking what is likely hundreds of millions of dollars into preclinical and clinical research activities, a company finally gets to enjoy the success of regulatory approval for marketing only to discover a year later that the product has failed to earn reimbursement.

Unfortunately, without reimbursement there is no adoption, and without adoption there are no sales. A technology is dead in the water.

The Centre for Commercialization of Regenerative Medicine (CCRM) is a unique not-for-profit group that is solely focused on developing and commercializing regenerative medicine technologies. CCRM, established in June 2011, is federally funded through the Networks of Centres of Excellence program (www.nce-rce. gc.ca) and has until 2015 to demonstrate its impact in Canada to receive a second, and final, operating grant. As CCRM prepares to launch companies, the necessity of reimbursement is being factored into each company creation strategy.

"Many biotech companies fail because they don't consider reimbursement early enough," explains Dr. Michael May, CEO, Centre for Commercialization of Regenerative Medicine. "Important questions to ask are 'who will pay?' and 'what value will this therapy offer to the health-care system?' At CCRM we're thinking about these questions, and others, at the beginning so that we can refine and focus technology development and abort a project if the assessment isn't favourable."

Given the high cost of goods associated with cell therapy products, this therapeutic class is particularly prone to reimbursement issues, and will continue to be until cell manufacturing is scaled to the point that costs can be driven down.

The advent of large-scale, closed system, 'turn-key' bioreactors will eventually allow the cell manufacturing industry to achieve the economies of scale and efficiencies necessary to temper costs. However, until then, we must be vigilant in selecting cost-effective cell therapy technologies for commercialization.

So, how can we strategize to sway the odds in favour of reimbursement success? Therapeutic headroom is a good beginning.

"Ready to walk the Reimbursement Maze?"

Therapeutic Headroom

Briefly, therapeutic headroom is the scope for improvement on a patient's quality of life resulting from a treatment, relative to other potential treatments for the same indication.

AMI (a heart attack) is an indication that is being pursued aggressively in the clinic by the cell therapy industry despite providing very little therapeutic headroom.

The average age of an AMI patient is 67. An individual of this age and of average health who has never experienced an AMI has a quality of life score of 0.80, while an individual of the same age who has experienced AMI has a quality of life score of 0.76 (actual numbers supported by the literature); a marginal difference of 0.04. If the individual who has AMI was treated with a cell therapy and returned to normal health, and then went on to live out his or her full life expectancy – on average 83 years – the overall health benefit that patient would receive as a result of treatment is an increase of 0.64 quality-adjusted life years (QALYs), or 234 days (0.04 x 16 years).

The current willingness-to-pay (WTP) by reimbursement providers is $50,000/QALY, which supports a cost of intervention of $32,000 for a cell therapy to treat AMI ($50,000 x 0.64 QALYs).

Investigational stem cell therapy interventions are currently running at approximately $100K - $200K per patient when all the costs are factored. This is well above the WTP for AMI. While this discrepancy is somewhat concerning from a reimbursement perspective, further efforts in manufacturing to lower the cost of goods should improve the landscape, at least for those technologies still in early development.

It is imperative that developers have a long-term strategy while commercializing cell therapies. Too often we get caught up in the science without considering the practicalities of getting a therapeutic to the patient's bedside, and demonstrating good value. This is not to say that interesting science and complex therapies should be overlooked, but if they come at significant cost they better have significant clinical results. Considering therapeutic headroom in selecting indications to target with cell therapies should more closely align treatment cost and willingness to pay, leading to fewer disappointments when it comes time to seek reimbursement.

Figures above are derived from Dr. Chris McCabe's research (University of Alberta). To read the full version of this article, please visit www.signalsblog.ca. To hear a podcast from Michael May on reimbursement, please visit http://www.ccrm.ca/node/304