# COAXING A DRAGON FROM ITS DEN WITH AUTOLOGOUS CELL AND GENE THERAPIES

By Akshay Kumar

A recent episode of Dragons' Den on the BBC featured an entrepreneur who pitched an impressive product with healthy margins and growing demand. Yet the Dragons declined to invest, citing 'low barriers to entry'. This triggered an idea: what if I pitched an innovative, autologous cell or gene therapy that cured patients with a disease where there are currently no therapeutic alternatives? Would any Dragon utter those magic words: 'I'm going to make you an offer?'

The Dragons may be tempted by the cutting-edge technologies involved and the transformational benefits for patients, but the high valuations being placed on companies developing these technologies may elicit some frowns. Clearly, the pitch would need to be highly compelling because autologous cell and gene therapies are not simple 'products', but complex procedures, involving conditioning, cell extraction, and genetic manipulation followed by transplant.

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Autologous cell or gene therapies genetically manipulate a patient's own cells ex-vivo, using viral vectors to carry and transplant the corrected gene or cells to cure the disorder. Some may be aware of recent acquisitions of CAR-Ts and autologous gene therapy biotechs by large pharmaceutical companies, the latter owning the genetic manipulation process while partnering with large centres to deliver other steps in the process. Novartis, for instance, has certified 50+ centres in the US to deliver Kymriah. These centres condition the patient and extract their cells, which are then manipulated by Novartis' central manufacturing facility before being returned to the certified centre for patient transplant.

While even the most cynical of Dragons could not fail to be impressed by the potential clinical benefits of these treatments, they, like regulators, payers, public policy-makers and academics, will be weighing benefits against factors such as cost-density, budget implications and job creation opportunities. This is giving rise to new, interesting partnerships, such as that between the Government of Canada and GE Lifesciences who are collaborating with academic and pharmaceutical partners to create a world class, centralised hub for the manufacture of CAR-Ts. Likewise, MHRA in the UK recently issued EU-recognised Manufacturing and Importation

Authorisation (MIA) and an MIA for investigational medicinal products to Cell and Gene Therapy Catapult's manufacturing centre in Stevenage. Firms and large treatment centres can work with Catapult to develop therapies that progress faster to clinical trials and commercial supply. Noteworthy also is Miltenyi Biotec's partnership with treatment centres across Germany and other markets to create an infrastructure that meets EMA's Good Manufacturing Practice requirements and enables manufacture for clinical trials and commercial supply.

While these developments will lead to faster innovation and make autologous therapies a reality for patients with many cancers and rare diseases, this also leads to the democratisation of technology. This enables a range of organisations to get involved in clinical trials and commercialise their promising technologies. Additionally, this may allow centres to develop 'home-brewed' cell and gene therapies which may become very attractive, especially if payers use mechanisms such as lump-sum tariffs or Diagnosis-Related Groups (DRGs) to fund these therapies while managing costs to the healthcare system.

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The pitch is over. The Dragons are shifting in their seats, their investor brains whirring fast, but will any invest in this innovative autologous cell and gene therapy business? A Dragon asks whether the current model is extendable – capable not just of generating revenue from the manipulation of patient cells, but of a more holistic approach involving the faster development of technology platforms, managing costs and risks around the entire treatment delivery process, long-term real world data collection, innovative contracting and patient services. This may, critically, be the route to establishing competitive barriers to entry… savvy investors, these dragons.

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