BLOG

31 March 2025

The AstraZeneca acquisition of EsoBiotec – important validation for Genespire's lentiviral vector platform

We noted with interest the recently announced news that AstraZeneca will be acquiring the Belgium based gene therapy company EsoBiotec for a total consideration of $1 billion – split between an upfront payment of $425 million and up to $575 million in developmental and regulatory milestones. EsoBiotec's platform uses lentiviral vectors to deliver genetic instructions to T cells to recognize and destroy either tumor cells for cancer treatment or potentially autoreactive cells for immune-mediated diseases.

This is a significant deal in the gene therapy field and for Genespire for a variety of reasons:

1. AstraZeneca entering the "off-the-shelf" cell therapy space shows the rising interest in *in vivo* gene therapy and, in particular, lentiviral vectors
2. It provides important validation for Genespire's immune shielded lentiviral vector platform, a technology originated and initially developed at the San Raffaele Telethon Institute for Gene Therapy and out-licensed to Genespire for liver-directed gene therapy

Lentiviral vectors offer the advantage of long-term expression of the therapeutic gene and are particularly suitable for duplicating cells such as in organs during growth, where non-integrative approaches such as adeno-associated vectors (AAVs) can lead to suboptimal results. However, the use of lentiviral vectors for in vivo gene therapy is limited by the immune system

Genespire's proprietary platform of lentiviral vectors are shielded from the immune system ("immune-shielded lentiviral vectors", ISLV) and, like EsoBiotec's platform, are designed to be injected directly into the patient's peripheral blood. Genespire's ISLVs migrate to the liver and insert the therapeutic gene directly into the DNA of the patient's cells.

Genespire is developing GENE202, the company's lead candidate, for the treatment of Methylmalonic Acidemia (MMA), a devastating genetic disorder impairing the metabolism of certain amino acids and fats and raised €46.6 million (~$52 million) in September last year in a Series B round to advance it into the clinic.

Dr. Karen Aiach-Pignet, CEO, Genespire
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