



Co-operation profile details from Enterprise Europe Northern Ireland

12 ES 252K 3OPS - A repositioned drug for the treatment of Familial Transthyretin Amyloidosis (ATTR) ready for Phase II clinical studies Technology collaboration OFFER

Abstract

Catalan repositioning company has developed a reprofiled drug for the prevention and treatment of Familial Transthyretin Amyloidosis (ATTR), a rare genetic disease characterized by severe wasting, immobility and death within 5-15 years after first outbreak symptoms. The drug is currently ready to initiate clinical Phase II for this indication and is considered the most cost-effective and risk balanced treatment of ATTR. Partners for licensing and co-development agreements are sought.

Description

Familial Transthyretin Amyloidosis (ATTR) is a rare disease caused by mutations in the TTR gene. It is characterized by a misfolding of the TTR molecule that leads to its aggregation and deposition of amyloid proteins in nerves leading to a slowly progressive neuropathy, cardiomyopathy and nephropathy. ATTR can result in severe wasting, immobility and death within 5-15 years after symptoms first develop. Currently, the only effective therapy for ATTR is the liver transplantation, which removes the main production site of the amyloidogenic protein. Also, tafamidis - a benzoxazole derivative - has recently obtained a conditional approval for the treatment of ATTR only in the EU but not in the US. The company has reprofiled a commercialised drug intended to prevent and treat this orphan disease presenting a more cost-effective therapeutic alternative to the liver transplantation and also a better efficacy and well-known safety profile than tafamidis.

The new use of this drug has been identified through a proprietary virtual screening technology able to identify new drug activities for already marketed drugs. In two years, the company has evaluated more than 20 drugs, identified at least 14 candidates to develop and has 2 products under development/licensing for Phase II programs.

A reprofiling project presents several advantages in front of the traditional drug development: shorter development time and cost, toxicological and safety profile available and overall less-risky development strategy.

The new use of the drug is protected by an international patent. In addition, the patent covers its synergic activity with other compounds - including tafamidis - and also different routes of administration and several dose regimes.

The company business model is based on licensing and joint-venture agreements for the development of reprofiled drugs. The company is open to any kind of agreement, from a full-sell of the project to a joint-venture for co-development.

Innovative Aspects:

- The drug is positioned as a new treatment for ATTR presenting a more cost-effective therapeutic alternative to the liver transplantation with a better efficacy and well-known safety profile than tafamidis.
- It is a commercial drug available in several countries for more than 10 years approved by EMA and FDA.
- It has been tested in several in vitro and ex-vivo tests showing a promising profile and higher efficacy results than tafamidis.
- A complete preclinical package for this new indication is already available and is ready to initiate clinical proof-of-concept.
- Fast track to the market for this new indication.
- Designation as an orphan medicinal product by the EMA and FDA may be requested.
- Having the orphan status, the Sponsor will benefit, among others, of fee reductions, protocol assistance, regulatory scientific advice, market exclusivity, etc.

Target partner expertise sought:

- Type of partner sought: Pharmaceutical industry active for example in: Orphan, Neurological or metabolic diseases.
- Specific area of activity of the partner: Pharmaceutical and/or Biotechnological industry.
- Task to be performed: Clinical trials, regulatory approval.

Key information:

Country of origin: SPAIN

Listed under: Medicine and Health \ Biosciences and Health \ Drug Discovery and Development

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