



PRESS RELEASE

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Spinal Amyotrophy **A clinical trial is starting up on 20 patients**

Trophos, a biopharmaceutical company based in Marseille and specialising in neurological disorders, has begun a phase 1b clinical trial on 20 patients affected with spinal amyotrophy, a rare neuromuscular disease of genetic origin. The trial, conducted at Garches, Marseille and Lille, will assess the tolerance as well as the speed of absorption and elimination of a new molecule (*TRO19622*), already successfully tested on both healthy volunteers and amyotrophic lateral sclerosis patients. This innovative molecule has been granted 'orphan medicinal product' status by the European Commission.

Thanks to Téléthon donations, over the last 7 years Trophos has been receiving decisive financial support for this project from the AFM. And now, barely four years after the identification of a candidate molecule, a clinical trial in humans is under way. Once again this shows how a patient association can play a leading role in promoting drug development.

This phase 1b clinical trial aims to demonstrate the safety of molecule *TRO19622* and to study its pharmacokinetics (how this new molecule performs in the body – drug absorption and elimination speeds). The trial will involve 20 children and young adults age 6 to 25 years affected with types 1b, 2 and 3 spinal amyotrophy, and will take place in three French hospital centres: Garches, (Prof Estournet – Raymond Poincaré Hospital), Marseille (Prof Chabrol – La Timone Hospital) and Lille (Dr Cuisset – University Regional Hospital Group).

Molecule *TRO19622* was discovered and developed by Trophos using its screening platform. 40 000 molecules have been tested on neuronal cells in culture in order to identify compounds likely to treat effectively motor-neuron diseases. Once identified, the candidate molecule was subjected to preclinical tests which showed the neuro-protective and neuro-regenerative action of the molecule. In 2005 several phase 1 clinical trials were successfully carried out on healthy volunteers. In 2006 it was tested on persons affected with amyotrophic lateral sclerosis – another neuromuscular disease affecting the motor-neuron – which demonstrated its good tolerance and led to the characterisation of its pharmacokinetics.

In case of success and in function of the regulatory authorisations, this phase 1b trial will be followed in 2008 by a phase II/III European multicentred clinical trial aimed at testing the effectiveness of the drug in improving patient motor function.

Spinal amyotrophy (also known as Werdnig-Hoffmann disease) is a rare autosomal recessive hereditary neuromuscular disease. Its estimated prevalence is 1 birth in 6 to 10 thousand. It is due to the degeneration of certain nerve cells of the spinal cord, motor-neurons – the muscle motor nerves are damaged and die and the orders for voluntary movement no longer reach the muscles. As these muscles are inactive, they weaken, atrophy and contract. There are 3 types of spinal amyotrophy, defined by age at disease onset and patient motor capacities.

For more information about the disease: www.afm-france.org

Contacts Presse :

AFM : Estelle Assaf, 01 69 47 12 78, presse@afm.genethon.fr
Trophos : Andrew Lloyd, +44 1273 675 100, allo@ala.com