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Lupus: peptide P140/Lupuzor™ effectiveness confirmed

A clinical trial with 149 patients suffering from the very disabling autoimmune disease systemic lupus erythematosus, has shown the effectiveness of a synthetic peptide developed by a team of researchers led by CNRS biologist Slyviane Muller at the Institut de Biologie Moleculaire (IBMC) in Strasbourg, France. The peptide, known as *P140/Lupuzor™*, is well tolerated by patients and leads to regression of the disease. Under the CNRS patent, ImmuPharma-France, which funded the trial, has an exclusive license to use the peptide. Now the final phase of clinical tests should soon confirm these results and contribute to the development of a drug without the side effects of existing treatments, which use cortico-steroids and immunosuppressants. These results are published online in the *Annals of the Rheumatic Diseases*.

Lupus is an autoimmune disease that affects 5 million people worldwide, mostly young women, and against which there is only non-specific, palliative treatment. In 2003, Sylviane Muller's team designed a peptide known as P140 (1) that proved capable of delaying the development of lupus in a mouse model (2). Since then, several regulatory clinical trials have been carried out by ImmuPharma-France, under CNRS license.

After very encouraging results with lupus patients in a single-center, open Phase IIa clinical trial, ImmuPharma-France began a Phase IIb trial. The 149 patients enrolled on the trial came from 21 different centers (to avoid bias) in 3 European countries (Bulgaria, Rumania, Spain) and in Argentina. The patients received 200µg of the P140/Lupuzor peptide (group 1) or a placebo (group 3) as a subcutaneous injection once every four weeks. The trial lasted three months and patients also received their normal long-term medication at very low doses to avoid influencing the results. These showed that the peptide P140/Lupuzor is effective and, after the twelfth week of treatment, had caused the disease to regress in 62% of patients in group 1, compared with 39% of patients in group 3 (placebo). The patients also tolerated P140/Lupuzor very well, with no side effects.

Now Lupuzor has received the necessary authorizations from the American Food and Drug Administration (FDA) to begin a Phase III clinical trial. After this phase III trial, if the results confirm the phase IIb results, Lupuzor can be marketed and will be key in the treatment of lupus patients.

(1) The peptide P140 corresponds to the sequence 131-151 of the nuclear ribonucleoprotein U1-70K in which residue 140 is a phosphoserine.

(2) Muller et al. *Arthritis Rheum*. 2008.

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For more information:
http://www2.cnrs.fr/presse/communique/823.htm.
http://www2.cnrs.fr/presse/communique/970.htm.

Clinical trials have a series of phases:

Phase I tests the safety of the new medicine using healthy volunteers.
Phase IIa is a clinical trial on patients who know they are receiving the active drug (a cohort of approximately 20 people).
Then phase IIb is the same comparative trial but with more patients and a placebo group. It is **multi-center** (i.e. patients are recruited in several hospital centers to avoid any possible bias), **randomized** (patients are chosen randomly and anonymously to receive either the active drug or the placebo, varying doses, etc.) and **double blind** (neither the patient nor the person administering the injection know whether it is the active drug or the placebo, so as not to influence them).
Phase III is the final, decisive, decision-making phase. The multi-center double-blind trial is carried out on an even greater number of patients. After this, if the positive results are confirmed, in compliance with the regulatory authorities in each country, a drug can be marketed.

Bibliography

Zimmer R., Scherbarth H.R., Rillo O.L., Gomez-Reino J. and Muller S. *Annals of Rheumatic Diseases* (doi: 10.1136/annrheumdis-2012-202460)

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