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Preclinical development of the antimicrobial peptide M33 and onset of regulatory procedures for clinical trials

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Background. This is a project aimed at the pharmaceutical development of a new antimicrobial peptide (M33) discovered at the University of Siena. M33 showed a strong activity in vitro and in vivo against a panel of bacteria generally involved in CF infections.

Hypothesis and objectives. Before arriving to human experimentation, a new drug must be developed preclinically and this includes the study of pharmacokinetic, bio-distribution and toxicity profiles in animals. This project was exactly designed for the evaluations of these issues. At the end of preclinical procedures of development, regulatory documents for the request of a Clinical Trial Authorization will be submitted to competent authorities.

Methods. Specific collaboration with CRO and public institutions were set up in order to conclude preclinical development currently in progress. Good Laboratory Practice (GLP) procedures have been followed for both M33 manufacture and animal experiments.

Results. Efficacy tests in vivo, pharmacokinetic analyses, biodistribution, along with anti-inflammatory and immunomodulatory activity has been evaluated with promising results for the set up of a new drug against Gram-negative bacteria, especially *P. aeruginosa* strains with multiresistant profile. The molecule resulted no genotoxic and apparently with a toxicity in vivo compatible for clinical application when administered systemically. Last toxicity evaluations in vivo and in vitro are in progress for the final set up of therapeutic index.

Expected results and their significance. Results obtained from this project allowed to move forward the preclinical characterizations necessary to the development of a new antibiotic drug. At the end of the project, when final results from toxicity and manufacturing will be available (few months), we will have the final information about the possible experimentation in humans of a novel antibiotic for severe infections in CF patients.

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