

ROSSIGNOL Nicolas (ENTR)

From: jkempeni@noxxon.net
Sent: vendredi 30 mai 2008 10:39
To: ROSSIGNOL Nicolas (ENTR)
Subject: Public consultation on the revision of Annex I to Directive 2001/83/EC (Advanced Therapies Regulation)
Attachments: Spiegelmer Technology Platform_mh.doc

Dear Mr. Rossignol,

We are pleased for the chance to make non-public comments on the upcoming amendment to the advanced therapy guideline and in particular to the definition of "gene therapy medicinal products" as described in paragraph 2.2.1.

As we understand, the main purpose of this guideline is to regulate RNA and DNA sequences which act by hybridisation with cellular DNA. We would like to take the opportunity to direct your attention to the fact that this is not the case for aptamers and Spiegelmers (as developed by NOXXON Pharma). Although these molecules are nucleic acids by chemical composition, they do not act by hybridisation to other nucleic acids. Their mode of action is rather a structure-dependent high-affinity binding of their target molecule (e.g. a protein). There is no relationship between the nucleotide sequence of a Spiegelmer or aptamer and their target molecule. Spiegelmers or aptamers do not encode a gene product and are not translated into proteins.

We therefore believe that aptamers and Spiegelmers should not fall under this guideline and propose to add a clarifying sentence to paragraph 2.2.1 like

- "Synthetic oligonucleotides not targeting genetic sequences and not encoding for a gene product are not considered to be gene therapy medicinal products"

I attach a description of the Spiegelmer technology for your information and convenience and am happy to answer any question you might have.

Best regards

Joachim Kempeni

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