

## Consultation on IMI Advanced Therapies Concept Paper

The key challenges for a collaborative development between public and private for ATMP are well defined and described through this concept paper.

To be precise about Chondrocelect, one should know that the French High Health Authority gave in 2013 unfavorable opinion for reimbursement of Chondrocelect, judging insufficient the improvement in actual benefit.

To complete our French vision, we do not think that there any resistance issue to industry commercialization from academic but a deep lack of know how to proceed.

It appears, while reading one point after another, that the global issue is the disconnection between public and private sectors that reveals a lack of common objective and interest.

What would be the main challenge would be to identify in one hand crucial short term issues to consider for both parts; and in the other hand the strength that each is able to provide where the other failed with comparable effort. The current collaboration is planned according to pharmaceutical mode of operation.

The field is guided at first steps from a medical point of view and research uncertainties towards final steps where economical need for industry takes advantage. The goal is to introduce economical skills, and the expertise of industrial process at very early stage to guarantee the robustness of scientific grounds. These skills and expertise should be assumed by key persons from industry within public hospital. Those differences between organizations, fundings, core business or mission, obligation to get results, career profile and management, can't be neglected.

We must think to an acceptable scale of time and costs for both project management and expenses in either side considering that 4 years and 511 clinical trials were needed to put on the market 3 ATMP. In this way, the concept paper should be enriched regarding economical models. The lack of an economical model is one of the main obstacles for the development of the ATMPs. Most of the ATMPs are border to scientific knowledge and basic science. Compared to conventional medicinal products, the development of ATMPs remains challenging . Each is different from another, at any step, from surrogate markers to biological markers able to demonstrate the efficacy and the quality. The great amount of scientific data that need to be provided before the beginning of a clinical trial or before a marketing authorization have a huge impact on the business plan, while many of the ATMP are currently developed to respond to patients awaiting for new treatments.

ATMPs are often the latest therapeutic alternative proposed to patients in dead-end with conventional care or grafts. The population is most of the time very limited (orphan disease, patient with relapse...) and the industrials encounter difficulties to glimpse what can emerge since the effective cost of the product become disproportionate compared to conventional medicinal product and compared to the number of potential patients treated.

It is also to be noticed that human raw material will always be under ethical condition, will always be variable in quality, will probably never be inexhaustible,

Over all, there is a regulatory challenge consisting in the need for competent authorities to share with researchers and industrials not only the specificities of ATMP products but also the considered treatment, potentially life-saving. A step by step assessment, taking into account these specificities through mutual exchanges and learning would help to enhance the accuracy of authorizations. As GMP are mainly made for chemical medicine, the level of requirements is currently adapted. Share inspection between AC would allow to harmonize the level of manufacturing practices.

Through the concept paper, priority should be given 1) to the registry, mapping and inventory; 2) to economical analysis and solution 3 to highly sensitive analytical tools.

The idea of international consortium or platform where academics would be given a full place should be investigate.

Communication to general public seems to be untimely

Focus on adjusting Academic and industrial issues is essential at that stage