2 February 2015
EMA/CAT/23438/2015
Procedure Management & Business Support Division

Report of the CAT Hearing with Interested Parties

Thursday, 11th December 2014 – 15:00hrs – 18:30hrs, room 3-E

Coffee break with CAT members: 15:00 – 15:30 (open area on 3rd floor)

Chair: Paula Salmikangas

Item	Agenda topic
1.	**Welcome of all participants by CAT chair and CAT secretariat**
	The CAT chair and the CAT secretariat welcomed all participants to the meeting.
2.	**Agreement of the agenda**
	The agenda was agreed.
3.	**Reflection paper on classification of ATMPs:** • CAT's current considerations on the following criteria for classification of ATMPs: 'substantial manipulation' and 'non-homologous use'; • Discussion and exchange of views; • Questions from the interested parties on ATMP classification (EuropaBio, EBE, BIA).
	Two presentations were given on the revision of the Reflection Paper on classification of ATMPs: B. Sekkali presented the CAT view on substantial manipulation; N. Ferry presented the concept of homologous/non-homologous use. B. Sekkali presentation N. Ferry presentation Following the presentations, the Interested Parties' (IP) participants highlighted following issues: - The definition on non-homologous use as proposed by CAT is difficult to understand. The inclusion of the parameter: location in the proposed definition of 'non-homologous use' was not fully understood. It would be better the use the term 'non-essential function(s)' as this is what is used in the legislation. - The absolute character of the reflection paper. CAT mentioned that if clear scientific data or justifications are presented by the applicant, this will be taken into consideration. However,

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	ATMP classification cannot change with every new scientific paper, it should provide some certainty that a product classified as ATMP will still be an ATMP in a couple of years' time. Some clear rules are therefore needed so that applicants know the regulatory status early in development: this has repercussions for the product development, but also for e.g. GMP requirements. - Non-binding character of the classification: could member states not agree, on a voluntary basis, to apply the CAT classification? - Harmonisation activities in EU and worldwide. CAT members mentioned a couple of initiatives: planned training of EU National Competent Authorities in 2015 and the activities of the International Pharmaceutical Regulators Forum (IPRF) for cell and gene therapies. It was noted that full international harmonisation will never be possible (different legal frameworks in the different countries or regions), however, similar scientific requirements for gene or cell based product should apply. - The IPs suggested to CAT to provide more guidance on previous classification (e.g. in a searchable database) and to include information in the reflection paper on what scientific data/justifications should be included in the application for ATMP classification. The CAT chair thanked the participants for their valuable observations. These will be taken into consideration together with the many comments received during the external consultation. CAT aims to finalise the revision of the reflection paper on ATMP classification in the first half of 2015.
4.	**The application of the concept of Risk-based approach (RBA) during the development and marketing authorisation of ATMPs.** • Introductory presentation from CAT; • Discussion and exchange of views; • Questions from the interested parties on the application of RBA (EuropaBio, EBE, BIA)
	E. Flory gave a presentation on what CAT understands under the Risk-Based Approach (RBA). It was mentioned that this is a very powerful regulatory tool, specific for ATMPs, which is currently underused by the ATMP developers and applicants. E. Flory presentation IP's made following observations and comments: - Most applications that are now submitted had their product development before RBA was in place. So that might be one of the reasons why it is not seen in marketing authorisation applications (MAAs). - How to use RBA as a development tool? It seems more to be a way to present the product development in the MAA (legal basis to justify absence of data) rather than a tool that can be used during the product development, e.g. in clinical trial application. This was further discussed: CAT mentioned that it is for their own benefit that ATMP developers can use this regulatory tool, i.e. to conduct some 'forward-thinking' on their product before conducting pivotal non-clinical testing / initiating clinical trials. ATMP developers can request scientific advice on the RBA-plan during development. This will avoid that unnecessary experiments are undertaken, resulting in additional costs and delays in development.
5.	**CAT workplan and CAT interested parties focus groups** • Presentation of the CAT Workplan 2015-2016 P. Celis presentation • Questions from interested parties (EuropaBio)
	Following the presentation by P. Celis on the CAT workplan 2015-2016, there was discussion on:

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	- CAT input on the revision of the ATMP Regulation: it was highlighted that the decision for revision has not yet been taken by the Commission, but if the legislation will be open, then the Commission will ask CAT's input on technical aspects. IPs mentioned the interaction with other legislation (for example the tissue and cell legislation) and the sometimes different national interpretation and implementation of these legislations. The IPs were asked by the Commission to provide a list of difficulties for ATMP developers stemming from national implementation and interpretations of other legislations. - Guideline development: this is not included in the CAT workplan, as guideline development is a routine activity of the Committee. The CAT chair indicated that CAT plans to initiate work on a Guideline on investigational Cell-based ATMPs. - In 2015, due to other priorities of the CAT, there is no intention to arrange meetings of the CAT-IP focus groups. Their work therefore remains on hold. CAT Work Programme
6.	**Discussions on other procedures and support to ATMP developers** (ATMP certification, adaptive licensing, one-stop-shop initiative) (CAT, BIA, IML)
	CAT provided feedback on the Adaptive licensing (AL) pilot, which is currently ongoing. There are some ATMPs included in this pilot. It was highlighted that AL will not replace normal licensing procedures: it rather provides for more intensive interactions between the developer, EMA (SAWP) and HTA's. IPs should keep an eye on the EMA website: reports will be posted on (interim) analysis of the AL-pilot. The roll-out of one-stop-shop initiative (more structured interaction during medicinal product development) is delayed until the first half of 2015.
7.	**Other issues and questions from the interested parties:** • Possible revision of ATMP regulation (Eucomed, EuropaBio) • Interactions with other stakeholders such as Competent authorities for tissues and cells, European Pharmacopoeia (EuropaBio) • Assessment of combined ATMPs (BIA) • Starting materials for ATMPs (BIA)
	The possible revision of the ATMP Regulation and the need for interactions with the Competent Authorities for tissues and cells were addressed under point 5. The topic on the assessment of combined ATMPs and starting materials for ATMPs were not specifically addressed.
8.	**Open forum discussion on any topic**
	Discussions on various issues took place under the other agenda points. The IPs thanked the CAT for the interesting and useful meeting, and requested for more frequent interactions and dialogue between CAT and the IP for 2015.
9.	**Close of meeting**
	The CAT chair closed the meeting at 18.30. She thanked all attendees for the open and useful contributions and exchanges of views, and wished everyone a Merry Christmas and a Happy New Year.

List of Interested Parties invited to the CAT hearing

Chair: Paula Salmikangas

Interested Parties	Participants (21)
1. Genetic Alliance UK	Nick Meade
2. Bio.be (Belgium)	Roland Gordon-Beresford
3. Eucomed	Dario Pirovano (via telephone)
4. Biomaterials Program CIBER-BBN	Aída Castellanos Páez
5. BioIndustry Association (BIA)	Christiane Abouzeid Christopher Sharpe Jacqueline Barry
6. Innovative Small Life Science companies (IML) (Sweden)	Vera Franzén
7. TOPRA	Sergio Fracchia
8. European Biopharmaceuticals Enterprises (EBE)	Piers Allin Florence Salmon Steve Hall
9. Science and Technology Studies Unit (SATSU)	Alex Faulkner
10. EuropaBio	Miriam Gargesi Decebal Bora
11. European Infrastructure for Translational Medicine (EATRIS)	Apostolos Gkazepis Jiří Deml
12. International Society for Cellular Therapy (ISCT)	Natividad Cuende Christopher Bravery
13. The Alliance for Advanced Therapies (AAT) and Alliance for Regenerative Medicines (ARM)	Annie Hubert
14. EURORDIS	Tsveta Schyns