EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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EMA/CAT/463795/2011

Report from CAT-Interested Parties Focus Groups (CAT-IPs FG) on system to navigate guidelines for ATMPs

12th May 2011- 9:00 -13:00 (UK time)

Chair: Christian Schneider

Item	Draft agenda/Summary of discussions
1.	**Introduction of participants** (see list of participants at the end of the document)
2.	**Scope of CAT-IPs FGs and objective of the meeting** A summary document (EMA/CAT/769749/2010) explaining scope, role, composition, duration of the CAT-IPs FG was distributed to all participant.
3.	**Brainstorming: how to improve the system by which developers can navigate the maze of ATMPs guidance documents.** The participants examined as an example a list of guidance documents applicable to gene therapy medicinal products. It was acknowledged in general that guidance documents applicable to ATMPs are published in different web pages on the EMA website. This constitutes a complex system where information is not easily accessible by developers. As a general aspect it was briefly discussed the content of specific ATMP guidance documents. Interested Parties representatives (IPs) highlighted that guidance documents are highly appreciated when give clear directions to developers hence enhancing predictability of regulatory outcome. Guidance documents applicable to specific category of products should provide additional information rather that duplicating what is already contained in general guidelines. IPs stressed that some guidance documents are written from the perspective of the marketing authorisation, thus leaving out developmental aspects. ATMP developers need

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	to also know more about what regulation is applicable and regulators expectations of data for compliance at the different stages of the product development. Concerning the specificity of the guidance documents content, CAT/EMA participants clarified that guidelines can only address common issues for a group of products while advice on development for individual products can only be given in the context of scientific advice procedures. Moreover, the SA procedure offers the opportunity to discuss specific issues on which guidelines are not applicable. These considerations explains also the choice of CAT and its working parties to issue 'Reflection Papers' rather than 'Guidelines'. A reflection paper is normally developed to communicate the current status of discussions or to invite comment on a selected area of medicinal product development or a specific topic. It can provide a framework for discussion or clarification particularly in areas where scientific knowledge is fast evolving or experience is limited. Unlike guidelines, reflection papers do not provide scientific, technical or regulatory guidance, but may contribute to future development of such guidelines, or related documents. IPs highlighted that from the point of view of developers, all guidance documents are perceived as having the same value in terms of their applicability.
4.	**Wrap-up:** The following summary emerged from the discussions as a collection of significant points highlighted by interested parties to be reported to CAT. A number of correspondent actions were also proposed that could be further explored. <u>Summary of discussions</u> • At the start of the meeting, it was highlighted that CAT-IPs FGs aim at improving CAT's interaction with interested parties and propose shared solutions on some of the issues previously identified in general hearings with Interested Parties. Therefore the outcome of the meeting will be reported to CAT and the stakeholders for further considerations. • The group discussed a proposal to consolidate all guidance documents applicable to gene therapy products in one document. Most IPs were of the view that such consolidated document may still be difficult to navigate and difficult to maintain. An interactive flowchart hyper-linking all applicable documents would be a preferred option. • The proposed flowchart would take into account the different lifecycle phases (development/pre-MAA, MAA, post-authorisation) and features (quality, pre-clinical, clinical) and it would gradually guide the applicant from general to product-specific guidances. • It would be valuable to increase transparency on product or class specific information/advices. As an example it was stressed the usefulness of the assessment and public presentations during Public Discussions published by the

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	USA NIH Recombinant DNA Advisory Committee (RAC). • IPs agreed that Question and Answers document are generally very useful and easier to be updated. • IPs stressed that the ATMP webpage is not easily accessible as the banner is not available on the homepage. The 'quick-link' can be found only if the user is aware of its existence. The current section on ATMP guidelines is also not linked to the other scientific guidelines. In this respect it would be useful to have a short explanatory document/page to explain how to navigate the EMA's website. • IPs reported that it would be helpful for developers to find in scientific advice letters the references to the applicable guidelines.
5.	**Proposed actions:** • Focus group in cooperation with CAT and its working parties to work on an interactive flowchart to help applicants to navigate the guidance documents applicable to ATMPs. The proposed structure of the flowchart would be tested by stakeholders before the start of the drafting phase. • CAT and its working parties to revisit available guidance documents and to explore, (within the gene therapy, cell-based products categories) possibility to merge existing guidelines when the content is overlapping. It would be helpful to explore harmonization of the number and titles of guidelines for cell-based medicinal products and gene therapy medicinal products. • CPWP to perform horizon scanning on available guidance and produce a list of guidance documents applicable to cell-based medicinal products and a gap analysis of what is missing. • In the context of the current CAT work programme, to engage in the dialogue with National Competent Authorities to understand how guidance documents are interpreted at national level. In preparing this discussion forum, Academia representatives and National Funding Research Organisations could be engaged. • Explore how navigation of EMA web pages on ATMPs could be improved (e.g. including a direct link from the home page, mind maps and hyperlinks to guidelines that are found in non-ATMP related web pages). • To publish on ATMP web pages links to useful publications (e.g. scientific papers published by CAT, other documents that can inform on EU validated/approved biomarkers). • IPs to provide input on specific areas where further guidance is needed. • To report to the relevant WPs the proposal to include more references to

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	applicable guidelines in scientific advice letters.
6.	**Conclusions:** The summary of the discussions held and the proposed actions will be reported to CAT in June 2011 for endorsement. It was agreed that such focus group meetings, with more specific content, are a helpful addition to the usual meetings of the CAT with interested parties. The next meeting will be held when significant progress has been made on the majority of the proposed actions (not earlier than July 2011). The Chair thanked all participants for the fruitful discussions and closed the meeting.

LIST OF PARTICIPANTS	
Christian Schneider	CAT Chair
Paula Salmikangas	CAT member
Monica Neagu	CAT member
Pablo de Felipe	CAT WP expert
Sol Ruiz	CAT member
Lucia D'Apote	CAT Secretariat
Caroline Voltz	GTWP Secretariat
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