

Meeting report series

Report of the 4th Teleconference of the WG on Biomarkers for Disease Progression and Therapy Response

27 October 2014

Organization

Organized by: Scientific Secretariat

Hosted by: Teleconference

Participants

Prof Alessandra Ferlini, Ferrara, Italy, chair

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Apologies

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Agenda

- ▶ Approval of the report of the previous teleconference
- ▶ Comments on EMA documents
- ▶ Discussion on inventory project
- ▶ Position paper on biomarkers in rare diseases
- ▶ AOB

REPORT

Approval of the report of the previous teleconference

Members of the WG approved the report of the 3rd teleconference (11 April 2014).

Comments on EMA documents

A member of the Therapies Scientific Committee (TSC) mentioned his concern about Guidelines on genomics biomarkers drafted by the EMA that only discuss SNPs as biomarkers, and wishes the WG's opinion on the guidelines. Two documents were circulated to the WG:

- ▶ Guidelines on key aspects for the use of pharmacogenomic methodologies in the pharmacovigilance evaluation of medicinal products.
- ▶ Concept paper on good genomics biomarker practices

Comments from the WG:

- ▶ Both documents are related to genomic biomarkers, including DNA and RNA biomarkers.
- ▶ Guideline on pharmacogenomics methodologies is probably not so relevant for this WG since it relates more to risk management and the presence of genetic polymorphisms influencing drug metabolism. The second is just a concept paper but the categories being suggested are reasonable and input would be most useful once the draft guidance is issued.
- ▶ There are guidelines from EMA but not from FDA. It is important to have some coordination between the statements from both agencies (for multicentre studies) as it would be more efficient for both patients and organizations developing drugs.
- ▶ It would best to have specificities for rare diseases in this type of documents.

⇒ Brief statement on these 2 documents will be prepared for the TSC.

Discussion on inventory project

A draft proposal of the project for establishing a research inventory on existing biomarkers in rare disease, to submit to the TSC, has been prepared by the Chair of the WG. However, this project appears to be more difficult than initially thought. The main problem is the need of people with different expertise for this type of work:

- ▶ 1st part of the project consists of getting the list of all research projects working on identifying biomarkers, not limited to rare diseases (Cordis, EMA, FDA, etc.).
- ▶ 2nd part involves developing a comprehensive view about these projects. It necessitates an expert in biomarkers, as well as a bioinformatics scientist to build an interactive map to represent in a schematic way the biomarkers disclosed in these projects.

- ⇒ It is difficult to find all the necessary expertise in one person. This project will also be time-consuming.

Another possibility is to obtain relevant information by sending a short survey (with Survey Monkey for example) to researchers working on projects mentioning “Biomarkers” with 2-3 questions (type of disease or tissue, etc.). This would allow a faster analysis of the field of Biomarkers and would help defining laboratories interested in developing collaboration.

Tasks:

- ▶ Interrogation of databases (NIH, Cordis, etc.) to obtain the list of projects with name and email address of PI: Scientific Secretariat? Key words to be defined by the WG.
- ▶ Preparation of survey
- ▶ Circulation of survey
- ▶ Analyze of the answers
- ▶ Preparation of report to TSC
- ▶ Realization of a schema to present the data? It would be helpful.

Distribution of tasks still has to be discussed. Would it necessitate hiring someone for a few months?

Position paper on biomarkers in rare diseases

Feedback from the TSC – Teleconference of 6 October 2014

The TSC would like the WG to consider the possibility and means to develop background paper for the funders on needs for specific groups of diseases as recommendation for funding priorities, starting with rare cancers. Another area could be considered in the next 6-12 months.

Discussion of the WG

Members of the WG present at the teleconference agreed on the principle of writing a position paper. This would required less work and give more visibility to IRDiRC and this WG than the inventory project. Several aspects (clinical trials, regulation aspects, etc.) of biomarkers could be approached. Each member of the WG could write a chapter in function of his/her expertise and specific issues. This paper could also include the type of work that can be done in the future.

In regard with the topic of the first statement paper, TSC recommended biomarkers for rare cancers. This would require inviting an expert to join the WG. Another possibility is to choose an area of expertise of one of the WG members.

- ⇒ Need to ask all members if agree on the principle of writing a position paper. If the answer is positive, a teleconference dedicated to this paper (topic, plan and distribution of task) will be organized.

AOB

Report of the workshop organized by the European Neuromuscular Center (ENMC) on biomarkers in Duchenne Muscular Dystrophy (DMD) that was held in January 2014 was accepted for publication by the journal “Neuromuscular Disorders”. A short statement about this WG was included in the manuscript.

Note post teleconference: the publication of the article is delayed to January.

Main deliverables

- ▶ Prepare a draft statement about the EMA document for report to TSC
- ▶ Sent a call for nominations to include more members in this WG
- ▶ Send publication on ENMC workshop on Biomarkers to Secretariat for circulation
- ▶ Contact the FDA for the replacement of the WG member that left