

Meeting report series

Report of the 3rd Teleconference of the WG on Biomarkers for Disease Progression and Therapy Response

April 11, 2014

Organization

Organized by: Scientific Secretariat

Hosted by: Teleconference

Participants

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Agenda

- 1) Feedback from the Therapies Scientific Committee
- 2) Analysis of the field of biomarkers: timeline and preparation of rationale
- 3) Report on the ENMC biomarker meeting in Naarden
- 4) Review of biomarker's criteria
- 5) Other topics

REPORT

Feedback from the Therapies Scientific Committee

The Therapies Scientific Committee (TSC) met in March in Paris and discussed the progress of the Working Groups. During this meeting, the TSC finalized its recommendations to be considered by the Executive Committee for the IRDiRC road map.

In these recommendations, the TSC agreed that an analysis of the biomarkers' field would be useful to identify gaps and opportunities. However, the Scientific Committee considered that such an analysis may require hiring one person for a few months (that could be hosted in the laboratory of one of the WG members) and is planning to request the IRDiRC Executive Committee for seed money.

In order to be able to present a consistent project, the TSC would like the WG to agree on a timeline (approximately 6 months) for this analysis as well as a rationale explaining its purpose, objectives and outcomes.

The present members of the WG on Biomarkers agreed to prepare a short proposal for the TSC.

Analysis of the field of biomarkers: timeline and preparation of rationale

In order to analyse the biomarkers' field, it would be necessary to know which biomarkers have already been identified. A survey of biomarker and natural history project leaders would need to be conducted in order to perform a gap analysis, identify potential clusters of biomarkers by disease and find information on biomarkers used in failed clinical trials. The objective will be to have a wide overview of the ongoing biomarkers' projects in the European Union (EU) and in USA. The main outcomes will be to have the possibility to get in contact with other professionals involved in biomarkers. The first phase of this analysis will consist in realizing a wide listing of biomarkers' projects, and in a second phase, a selection could be realized between those projects.

When the inventory will be finalized, a workshop could be organized by IRDiRC between the members of this WG and between the people involved in the biomarkers projects mentioned in the inventory.

Report on the ENMC biomarker meeting in Naarden

On January 24-26, 2014, a workshop organized by the European Neuromuscular Center (ENMC) on biomarkers in Duchenne Muscular Dystrophy (DMD) took place. This ENMC workshop aimed at sharing experience and results among the international community working on DMD and other neuromuscular disorders. 26 EU and USA experts participated. During this workshop, a comprehensive overview on biomarker research in DMD, highlighting both established and yet unpublished data was presented. Types of biomarkers, discovery tasks, strategies and clinical applications were extensively discussed. A session was dedicated to the already known DMD biomarkers (DNA, RNA, proteins) and their potential role in clinical trials.

The rationale and strategies of this workshop could be applied to all rare diseases, not only muscular dystrophies.

The report of this workshop will be published in Neuromuscular Disorders Journal and the IRDRIC biomarker WG could be mentioned in the report. The ENMC will be asked if it could be possible to write a short paragraph about the activity of this WG and this will be incorporated to the report.

The present members of this WG agreed with that proposition.

Review of biomarkers' criteria

It is important to be consistent with the biomarkers' definition and criteria.

A draft PowerPoint proposition of biomarkers' criteria was already circulated. The WG members were asked to give more feedback to this proposition to finalize these criteria and propose it to the TSC meeting in Berlin.

Main deliverables

- ▶ Prepare a short proposal to the TSC to hire a person that would analyze and make an inventory of the biomarkers' field (timeline, purpose, objectives, outcomes)
- ▶ Read the report on the ENMC biomarker meeting on Naarden
- ▶ Give a feedback on the biomarkers' criteria draft to finalize them and propose it to the TSC meeting in Berlin
- ▶ Schedule the next WG teleconference for the end of May