

Meeting report series

Report of the 16th Therapies Scientific Committee Meeting

Teleconference

July 6, 18

Participants

Dr Diego Ardigo, Chiesi Farmaceutici S.p.A., Italy – Chair

Dr Virginie Hivert, EURORDIS-Rare Diseases Europe, France – Vice Chair

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Dr Sandrine Marreaud, EORTC, Belgium

Dr Anne Pariser, National Center for Advancing Translational Sciences (NCATS), NIH, USA

Prof Maurizio Scarpa, Horst Schmidt Klinik / University of Padova, Germany/ Italy

Dr Ken Sakushima, PMDA, Japan

Dr Marlène Jagut, Scientific Secretariat (Sci Sec), France

Dr Anneliene Jonker, Sci Sec, France

Dr Anne-Laure Pham-Hung d’Alexandry d’Orengiani, Sci Sec, France

Apologies

Dr Annemieke Aartsma-Rus, Leiden University Medical Center, The Netherlands

Dr Seng Cheng, Sanofi, USA

Dr Michela Gabaldo, San Raffaele Telethon Institute for gene therapy, Italy

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Agenda

1. Debrief of Vienna
 - Approval of Activity C proposal
2. Activity C
 - Review of examples of Building Block forms
 - Review of list of Building Blocks
 - Discussion on candidates for the Task Force

- Organization of the workshop
- 3. Data mining and repurposing draft
- 4. Any other business
 - Drug repurposing platform proposal by Jordi Quintana

REPORT

1. Debrief of Vienna

The Activity proposal C – Guidebook for Orphan Drug Development, was, after discussion at the TSC and the Consortium Assembly (CA) meeting, formally approved by the CA. All participants but two voted in favor of the proposal, and two members abstained from vote. No members voted against the proposal.

The discussion at the CA meeting provided rather positive input, and several CA members have requested to remain involved in the course of the activity. Therefore, the TSC Chair and Vice Chair decided to enlarge the participation of this activity, and to ask all CA members to provide feedback and suggestions to the Building Block list.

2. Activity C

2.1 Review of examples of Building Block forms

Based on the discussion in Vienna, an additional field was added to the Building Block form, namely the relevance to orphan drug development. This field is important

- ▶ To understand why and how the Building Block is relevant for supporting high quality drug development and how to expedite drug development
- ▶ For prioritization of filling out the Building Blocks

An example of a filled out Building Block form, the EMA Innovation Task Force, was attached as a preparatory document. In addition, the Chair and Vice Chair will prepare two additional examples. These will serve:

- ▶ As guide for filling out the all Building Block forms
- ▶ To start asking members to review forms, and to provide additional information

As a general workflow, the Sci Sec will start filling out forms, with information that is publically available (either on websites, articles or other sources of information). Members are asked:

- ▶ To review the information, and add further details when needed
- ▶ To provide the added value to the Building Block forms
 - The pros and cons of every Building Block
 - The relevance of every Building Block
 - Practical tips when using the Building Block

Ideally, all Building Block forms will be filled out by July 27, 2018. The Sci Sec will first prioritize the regulatory Building Blocks, as information can be easily obtained for these Building Blocks.

→ Fill out the Building Block forms by July 27, 2018

2.2 Review of list of Building Blocks

The list of Building Blocks was attached as preparatory documents. All members are asked

- ▶ To review the list
- ▶ Add additional Building Blocks as needed
- ▶ To add your name to the Building Blocks you intend to review and work on
 - Ideally each Building Block should be reviewed by at two or more members

The review of the Building Block list should be finalized by July 13, 2018.

→All TSC members to review the Building Block list by July 13, 2018

2.3 Discussion on candidates for the Task Force

A Task Force profile for Task Force members was circulated to all TSC members. Members are asked to review this profile, and to provide names of potential candidates that fit this profile. Several TSC members provided names already. These names are summarized and provided as preparatory document. For each potential candidate, the following information is needed:

- ▶ Name of the expert
- ▶ Job/ affiliation of expert
- ▶ Country
 - To ensure expertise from around the globe
- ▶ Key competence
 - To ensure a variety of expertise
- ▶ Contact details
 - To get in touch with the expert
- ▶ Name of proposer
 - In order to ask additional questions if needed

In parallel, CA members will be asked to also provide names and information on potential candidates, in order provide additional input and ideas.

The TSC will review and finalize the list of experts as soon as possible, in order to invite members so that they can save the date. Due to the summer, this finalization will be done by email.

Two potential experts were mentioned during the call, and should be added to the list:

- ▶ HTA expert with experience in Orphan Drugs
- ▶ Member of the ERN-EMA Task Force

In order to facilitate exchange between members, and to avoid working on different versions, a Google Drive folder will be created. Members will be given access to this folder, in which both the Building Block list, the Building Block forms and the list of experts is provided.

- All TSC members to review the Task Force profile for Task Force members by July 13, 2018
- All TSC members to provide names and information on potential candidates for the Task Force by July 13, 2018
- Ask CA for potential candidates for the Task Force
- Review and finalize list of experts, in order to invite experts to participate in the Task Force and the workshop
- Create Google Drive folder with list of Building Blocks, Building Block forms that are filled out, and list of experts

2.4 Organization of the workshop

- ▶ When: The Task Force workshop will be organized on December 12-13, 2018.
 - ▶ Where: nice location, preferably sunny in order to set up a good and stimulating working environment
 - ▶ Venue: needs to be given in kind, as Sci Sec contract does not allow to pay for a venue
 - Please inform the Sci Sec if you know a venue that fits these criteria
 - Current ideas are Mexico, Florida, and California
 - ▶ People: 20-30 people are expected to participate in the workshop
- Inform Sci Sec if you know a venue that fits the desired criteria for a workshop location, by July 27, 2018

3. Data Mining and repurposing draft

In November 2016, IRDiRC held a workshop for the Data Mining and Repurposing Task Force that resulted in a report, which includes a set of recommendations. In order to enlarge the audience of this report, a draft was prepared, that will be submitted to a scientific journal. The draft is currently prepared for *Orphanet Journal of Rare Diseases*. Interested members are asked:

- ▶ To review the draft, and to provide comments and suggestions where needed
 - ▶ To think about journals, either for a first submissions, or if the draft is rejected
- Interested TSC members are asked to review the data mining and repurposing draft by July 27, 2018
- TSC members are asked to provide suggestions for journals by July 27, 2018

3. Any other business

Following the before mentioned Data Mining and Repurposing Task Force, Jordi Quintana, a member of this Task Force, is currently setting up a consortium with various entities, in order to set up a platform for drug repurposing. He organized a teleconference, in which, among others, the Vice Chair of the TSC, and a member of the Sci Sec participated, discussing a very short preliminary proposal. Currently, the main bottleneck will be the lack of available funding, and lobbying is needed (potentially via the European Joint Programme for Rare Diseases), in order to obtain funding.

Members asked to provide a list of the next TSC teleconferences and meetings. The following meetings are scheduled:

- ▶ September 10, 2 PM – Teleconference
- ▶ November 5, 1 PM – Teleconference
- ▶ December 6-7 – CA Face-to-face meeting, Brussels, Belgium
 - Only the TSC Chair and Vice Chair will participate
- ▶ December 12-13 – Guidebook for drug developers workshop, TBD

Next steps and actions

- ▶ Fill out the Building Block forms by July 27, 2018
- ▶ Review the Building Block list by July 13, 2018
- ▶ Review the Task Force profile for Task Force members by July 13,
- ▶ Provide names and information on potential candidates for the Task Force by July 13, 2018
- ▶ Ask CA for potential candidates for the Task Force
- ▶ Review and finalize list of experts, in order to invite experts to participate in the Task Force and the workshop
- ▶ Create Google Drive folder with list of Building Blocks, Building Block forms that are filled out, and list of experts
- ▶ Inform Sci Sec if you know a venue that fits the desired criteria for a workshop location, by July 27, 2018
- ▶ Review the data mining and repurposing draft by July 27, 2018
- ▶ Provide suggestions for journals for data mining and repurposing draft by July 27, 2018