

## Meeting report series

### Report of the 14th Therapies Scientific Committee Teleconference

Teleconference  
May 3rd, 2018

#### Participants

Dr Diego Ardigo, Parma, Italy (Chair)  
Dr Virginie Hivert, Paris, France (Vice Chair)  
Dr Karin Rademaker, Utrecht, the Netherlands  
Prof Maurizio Scarpa, Wiesbaden, Germany  
Dr Anne Pariser, Bethesda, USA  
Dr Seng H. Cheng, Framingham, USA  
Dr Michela Gabaldo, Milan, Italy  
Dr Robin Conwit, Bethesda, USA  
Dr Anneliene Jonker, Paris, France  
Dr Marlene Jagut, Paris, France  
Dr Anne-Laure Pham Hung d'Alexandry d'Orengiani

#### Apologies

Prof Annemieke Aartsma-Rus, Leiden, the Netherlands  
Dr Sandrine Marreaud, Brussels, Belgium  
Prof Josep Torrent i Farnell, Barcelona, Spain  
Mr Yann Le Cam, Paris, France  
Dr Ken Sakushima

#### Agenda

1. Preparation for Vienna
  - Review of presence to the meeting (F2F or over the phone, if possible)
  - Presentation of the agenda for the TSC meeting in Vienna
  - Discussion
2. Activity C
  - Review of approach, plan, and timelines with a specific focus on the period from TSC TC and TSC meeting in Vienna
  - Activity C goals for the meeting in Vienna
  - Presentation of the Development Board

- Review and brainstorming around the Building Block list
  - Review of and discussion on the Building Block form template
3. Any other business and next meetings/ TC
- Any other business or initiative to be proposed/ discussed
  - Plan for 2018/19 TSC TCs

## REPORT

### 1. Preparation for Vienna

The TSC discussed the proposed agenda, and asked members to provide their input. No changes were made to the agenda, everyone present agrees to the suggested schedule. A final version will be sent to all.

TOPIC	DESCRIPTION	DURATION
Update from IRDiRC Op Comm	<ul style="list-style-type: none"> <li>• 2027 strategy update</li> <li>• Ongoing/ started activities</li> </ul>	~ 10 min
Update on TSC membership	<ul style="list-style-type: none"> <li>• Current TSC membership and potential new members</li> </ul>	~ 10 min
Update on ongoing/ completed activities/ TFs	<ul style="list-style-type: none"> <li>• SPCT TF paper update</li> <li>• DMR TF follow-up: what's next?</li> </ul>	~ 20 min
Brainstorming on TSC follow-up to completed TFs	TSC free discussion on potential follow-ups to completed TFs and past initiatives	~ 30 min
Activity C	<ul style="list-style-type: none"> <li>• Review of approach, plan, and timelines</li> <li>• Review and approval of the first draft of tools (Development Board, Building Blocks, and Block Form)</li> <li>• Brainstorming and prioritization on Building Blocks list</li> <li>• Creation of the core steering group for the workshop</li> <li>• Allocation of responsibilities for the preparation of the first draft of the Building Blocks</li> <li>• Planning of the workshop</li> </ul>	~ 90 min
Activity D (RWE data collection)	Discussion on TCS contribution to the activity	~ 20 min

TOPIC	DESCRIPTION	DURATION
Activity E (reframing of research agenda)	Brainstorming and planning of the TSC survey	~ 20 min
Update on the IRDiRC counter	<ul style="list-style-type: none"> <li>Review of data generated with the new counter and discussion on finalization of counting rules</li> <li>Planning for the counter paper</li> </ul>	~ 30 min
AOB and next meetings/ TC	<ul style="list-style-type: none"> <li>Any other business or initiative to be proposed/ discussed</li> <li>Plan for 2018/19 TSC TCs</li> </ul>	~ 10 min

## 2. Activity C

### 2.1 Review of approach, plan, and timelines with a specific focus on the period from TSC TC and TSC meeting in Vienna

This project aims at creating a simple guidebook for academic and industrial drug developers describing the available tools and initiatives specific for rare disease development and how to best use them.

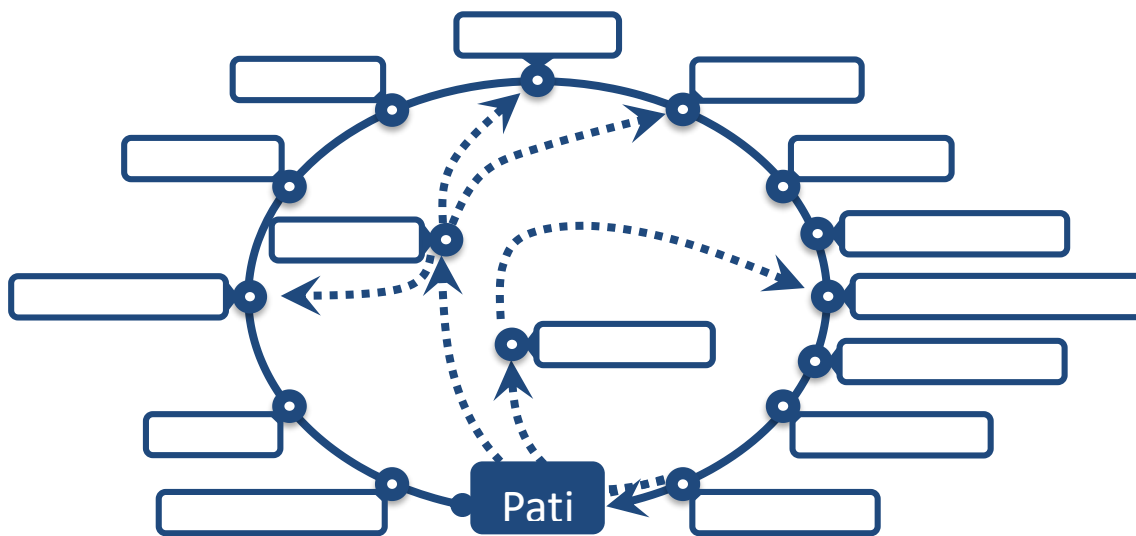
- ▶ The approach for Activity C is like a game, the framework being the game board, with all classical steps of drug-development.
- ▶ Building bricks of the game are all single initiatives and tools. Each building brick will have a description sheet that will enable developers, academic researchers, and others to decide which block to use, according to their need in the process of orphan drug-development.

### 2.2 Activity C goals for the meeting in Vienna

- ▶ To have an initial understanding of the game board
- ▶ To have a first consolidated list of building blocks
- ▶ To define the information to attach to each of the building blocks (Building Block Form)
- ▶ To assign building blocks forms to members (workshops), both inside and outside of TSC, in order to prepare those sheets (20 people **maximum**)
- ▶ To plan workshops and place building blocks on the game board

### 2.3 Presentation of the Development Board

The following development board represents all classical milestones in the drug-development process in a wheel, rather than a continuous line.



- ▶ All other representations/modifications to the present board are welcome: members are asked to come with ideas at the Vienna meeting. (Ex: this representation does not include a timeline).
- ▶ Additional milestones/tasks/ single initiatives to be attached to this basic board, specific for rare disease drug-development

A question has been raised regarding how detailed each milestone should be, especially given that the Guidebook should be designed first, and foremost, to help developers in the pharmaceutical industry. This is an initial skeleton to build upon and refine, and this matter could be reflected upon in the next steps to come.

→ All TSC members are asked to come to Vienna with ideas and suggestions for the Development board

## 2.4 Review and brainstorming around the Building Block list

The list of building blocks represents either tools or initiatives specific for rare disease drug development. This initial list is non-exhaustive and is now open for discussion and modification. TSC members are encouraged to come to Vienna with ideas on how to complete the list of Building blocks.

- ▶ Keep in mind that the goal in Vienna is to review this list and from there, to prioritize/modify the list for relevance or feasibility

The issue of overlapping already financed ongoing projects (e.g. European Joint Program-EJP), such as mapping, structure coordination, increase diagnostics, policy making, etc... has been raised.

International projects/practices/tools can be added as building blocks. The Guidebook can act both ways, as to also benefit the community to questions raised in the EJP, for example. The main spirit of the Guidebook is to inform the community of what is available in the field and to use it to its best potential. That information will be handed out to developers through the form of fact sheets or building block forms.

→ *TSC members are asked to come to Vienna with ideas and suggestions to complete the list of Building Blocks*

## **2.5 Review of and discussion on the Building Block form template**

The purpose of those sheets is to give highlights, where to find detailed information, as well as where and when to use it for each initiative/practice/incentive. The template is now up for review, so all opinions and suggestions are welcome.

- ▶ The goal in Vienna is to set the content of the fact sheets. The next step is to fill a fact sheet for each building block

As filling these fact sheets may be tricky at first, a few examples will be shown in Vienna. In order to improve the entire process, TSC members are invited to pick one fact sheet and fill it to his/her best ability. As such, along examples displayed at the Vienna meeting, it will help all TSC members to have a clearer idea on the way to fill in those forms and how they work.

- ▶ Setting those building blocks and respective fact sheets in Vienna is crucial in order to prioritize them and proceed with the workshops at the end in the year

→ *TSC members are asked to bring a pre-filled building block form of your choice at the Vienna meeting.*

## **3. Any other business and next meetings/ TC**

### **3.1 Activity D: Natural History and Registry Platform for use in RWE Data Collection**

Activity D is a cross-committee project, between CCC, FSC and TSC, on the real-world evidence data collection. CCC has the lead on that activity and TSC remains an active member.

- ▶ Need to nominate a TSC representative to keep a continuous record of on-going developments for activity B and to seek TSC's attention whenever it is needed.

→ *TSC members are encouraged to step forward to volunteer to lead this activity*

### **3.2 Activity E: internal survey within the rare disease community to help define priorities and next steps to undertake**

Activity E is the implementation of an internal survey within the rare disease community to help define priorities and next steps to undertake. That survey will be run by the end of 2018 and will circulate internally.

- ▶ Necessity of a structure, list of questions and possibly one representative to become the lead agent to kick-off this specific activity.

→ TSC members are encouraged to step forward to volunteer to lead this activity

### 3.3 Plan for 2018/19 TSC TCs

- ▶ One meeting every two months seems a good working flow (including F2F meetings)
- ▶ Need to plan all upcoming TCs until the end of 2018 and possibly 2019
- ▶ Time remains 1 PM CEST (Paris/Rome) to cover a maximum of different time zones
- ▶ Members should let us know if particular days are consistently not possible for teleconferences

→ TSC members should let the Sci Sec no if consistently unavailable at particular days

→ Sci Sec will send out calendar invites for the next series of TSC TCs

#### Main action points

- ▶ Development board ideas at the Vienna meeting
- ▶ Review the building block list by the Vienna meeting
- ▶ Bring in a pre-filled fact sheet at the Vienna meeting
- ▶ Volunteer for Activity D liaison
- ▶ Volunteer for Activity E kick-off
- ▶ Tell us if you have any weekly imperative for planning TCs
- ▶ Send out calendar invites for the next series of TSC TCs