

Meeting report series

Report of the 5th Companies Constituent Committee Meeting

Teleconference

July 11, 2018

Participants

Dr Mathew Pletcher, Roche, Switzerland – Chair
Dr Madhu Natarajan, Shire, USA – Vice Chair
Dr Tim Considine, Recursion Pharmaceuticals, USA
Dr James McArthur, Cydan II, USA

Dr Anneliene Jonker, Scientific Secretariat (Sci Sec), France
Dr Lilian Lau, Sci Sec, France
Dr Anne-Laure Pham Hung d’Alexandry d’Orengiani, Sci Sec, France

Apologies

Ms Karen Aiach, Lysogene, France
Dr Katherine Beaverson, Pfizer, USA
Dr Andrea Chiesi, Chiesi Farmaceutici, Italy
Dr Carlo Incerti, Genzyme, USA
Dr Ning Li, BGI, China
Dr Robert Mashal, NKT Therapeutics, USA
Dr Brett Monia, Ionis Pharmaceuticals, USA
Dr Tom Pulles, Ultragenyx, Switzerland
Dr Ellen Welch, PTC Therapeutics, USA
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Agenda

1. Update re regular CCC meetings
2. Debrief of the Vienna CCC meeting
3. Development of the proposal for Activity H - Background internal work on common knowledge base to drive rare diseases research
4. Change of leadership for Activity D – Natural history and registry (NH/R) platform for use in real world evidence (RWE) data collection

- Role of CCC in this activity
5. Identification of potential new members for CCC

REPORT

1. Update regarding regular CCC meetings

The Chair of the Companies Constituent Committee (CCC) welcomed the meeting participants and informed everyone of the CCC teleconferences (TCs) calendar. From now on, meetings will take place on a monthly basis, on the first Wednesday of each month.

- ▶ Long gaps between previous CCC meetings have proven to be inefficient for progress of the Committee
- ▶ There is a strong desire from the CCC to be active in the field and really help IRDiRC with its different goals
- ▶ Regulars calls will help members set goals, actively contribute, stay up to date with all current activities, and help everyone anticipate the next steps
- ▶ Invites have already been sent out to CCC members for the remainder of 2018; new dates for 2019 to be determined at a later stage

2. Debrief of the Vienna CCC meeting

Both the Chair and Vice Chair were unable to attend the CCC meeting in Vienna, and requested a debrief from members who were present.

Several points were discussed:

- ▶ The extension of the CCC with new members, to further increase working capacity of the Committee
- ▶ The leadership of Activity D (“Natural history and registry platform for use in real world evidence data collection”) has moved from the CCC to the Interdisciplinary Scientific Committee (ISC)
- ▶ There was a brainstorming session about activities the CCC could put in place that would be transformational and not incremental in nature
 - Reimagining therapeutic development to make it more successful and efficient, and provide a further basis for drug development to move away from the so-called “lower hanging fruit,” well-known and characterized diseases
 - How to create a basis to develop drugs for lesser known diseases
 - Accelerating the process of drug development to allow faster access to medicines
- ▶ A fully engaging activity of the CCC would allow members to be and feel more involved and consequently dedicate larger periods of time to the Committee
- ▶ **Important notice:** the timeline for generating new activities will change starting January 2019
 - New activity proposals must be submitted by June in order to be included in the annual work plan of the following year (e.g., June 2019 for inclusion in 2020 work plan)

3. Development of the proposal for Activity H - Background internal work on common knowledge base to drive rare diseases research

Most of companies' efforts are focused on a handful of rare diseases; often, a lack of knowledge surrounding rare diseases prevents companies from investing and launching development programs on lesser-known diseases.

- ▶ The CCC wishes to take the opportunity to fill the knowledge gaps and motivate companies to shift their interest from better-known rare diseases to others, where there are no or little research programs
 - ▶ The goal would not be to create a new drug discovery program, but to identify missing data types, including information that highlights potential commercial opportunity such as:
 - Prevalence data for all rare diseases, and specifically for those where there are no ongoing drug development programs
 - The number of patients that would benefit from a drug development programs on a specific rare disease or a phenotype
 - ▶ Proposed steps of action:
 - Firstly, generate a spreadsheet containing a list of data that would be of importance to gain deeper, wider understanding of broader selection of rare diseases
 - Data type of interest include:
 - Prevalence
 - Number of patients reported to have the disease
 - Existence of natural history data
 - Existence of well-maintained registries
 - Write up a proposal to create a Task Force
 - CCC will guide the Task Force on what to accomplish
 - Task Force will consider how best to assemble missing data/gaps
- ➔ *CCC members to generate a spreadsheet containing essential and/or available data type on rare diseases before the next CCC teleconference*

4. Change of leadership for Activity D – Natural history and registry (NH/R) platform for use in real world evidence (RWE) data collection

The CCC was initially slated to lead this activity which aims to improve NH/R data collection via the definition of standards to facilitate collection of high quality and interoperable data, and establishment of best practices to encourage the use of a universal, broad-based RWE platform.

- ▶ The leadership of this activity has shifted from the CCC to the ISC

- The CCC has a crucial role to play in this activity, but CCC members have to identify what role they want to play in it, and what outcome they expect of this activity
- There is a strong notion that all generated data should be exploitable by companies
 - The CCC could definitely have a consulting role to ensure that collected data is usable by companies
- CCC members could help define the necessary criteria, the quality measures, etc... needed by companies from these data collections
 - They could fill in the knowledge gaps and have a crucial role of being the bridge between companies and patient organizations for these types of data collections
- ▶ The CCC will await the launch of Activity D, but will communicate to the ISC the potential roles it could have and the outcomes it envisages to move this activity forward and ensure its success

5. Identification of potential new members for CCC

A list of potential members to invite on the CCC was generated during the Tokyo meeting and refined during the Vienna meeting.

- ▶ Potential organizations to contact were identified.
- ▶ Personal contact by CCC members, rather than a generic letter from the IRDiRC Sci Sec, is the preferred strategy to ensure an answer from some companies
- ➔ *Sci Sec to send a draft letter to CCC members, with a description of IRDiRC's activities, that can be personalized accordingly*
- ➔ *CCC members to contact these companies, and keep the Sci Sec informed on the status of these invitations*

Next steps and actions

- ▶ Generate a list of essential and/or available data type on rare diseases 1
- ▶ Send a draft letter of invitation to CCC members to personalize accordingly
- ▶ Invite companies to become IRDiRC members