

Meeting report series

Report of the 18th Therapies Scientific Committee Meeting

[25/03/2019]

[Conference Call]

Participants

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

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

- 1- Ongoing activities
 - Orphan Drug Development Guidebook (Galaxy Guide)
 - “New” Drug Counter
 - Articles
 - Data Mining and Repurposing
 - Drug Counter
- 2- Activity plan for 2019-2020
- 3- Committee membership

Report

1- Ongoing activities

- Galaxy Guide: A workshop was organized in December 2018 in Dublin, and an internal report was communicated to the TSC. The group is currently reviewing the building blocks (BBs) and developing a graphical representation of them. The first version of the guidebook will be ready by April 2019, and the dissemination of the results will start in July 2019.
- “New” Drug Counter: This work was supported by the former Scientific secretariat (Sci Sec) and therefore, the new Sci Sec and the TSC have to understand how the information for measuring drug count was shaped, and how the metrics were assessed. This work is currently in progress.
- Articles
 - Data Mining and Repurposing: The manuscript is currently in preparation.
 - Drug Count: This manuscript is in preparation. It describes the data provided by the former Sci Sec until December 2018.

2- Activity plan for 2019-2020

The IRDiRC Roadmap 2020 must be drafted by the beginning of June 2019 since it is a deliverable of the EJP RD Annual Work Plan. The delivery of the Roadmap in due time is very important since EJP RD relies on the scientific recommendations of IRDiRC and the TF outcomes to develop its strategic plan. The content of the Roadmap 2020 will be discussed and validated during the CA-SC meeting in Leiden (May 22-24).

The following activities have been identified as highly relevant for the next TSC activity plan. They will be further discussed during an internal call on April 15.

- Follow-up on the Galaxy Guide activities: The next steps will focus on the representation and the sustainability of the tool
 - Create a user-friendly representation of the BBs. Costello Medical Writing will be contacted to investigate how the GG can be translated into a clear writing or graphical representation.
 - Design an interactive website
 - Ensure the maintenance and updates of the BBs. It has also been suggested to develop an editorial board within EJP RD to validate the updates and the sustainability plan of the BBs
- Alternative Business Models for RD: This activity plans to map the specific orphan drug companies (including patient-driven companies), and understand the characteristics of their alternative model of development through the creation of a survey. This activity should highlight the strengths and the gaps of these alternative models, and provide recommendations to funders and companies.

- Position article on very low prevalence RD. This article could also be linked to the Alternative Business Models for RD activity, since patient-driven companies are also conducting research on very low prevalence RD.
- Development of a guide for drug repurposing. This activity could be closely associated to the GG development.

Other activities were mentioned during the TSC meeting but were not classified with high priority.

- Case studies/survey: learning from drug development cases that went wrong
- Technical track: Is there a need for a harmonized approach?
- Stakeholder-driven development: Guidelines and best practices for the different stakeholder interactions in RD

3- Committee membership

The TSC is facing membership turnover in 2019 since Karin Rademaker and Josep Torrent y Farnell reached the end of their mandate, Anne Pariser joined the FCC, and Yann Le Cam will end his mandate in October 2019.

- The TSC will therefore open 4 new positions in 2019.
 - Thomas Morel (KU Leuven, Belgium) and Sangeeta Jethwa (Roche, Basel, Switzerland) were identified as potential candidates by the Chair and Vice Chair of the TSC.
 - The membership application of Thomas Morel was approved by the CA during the last meeting on March 2019.
 - The membership application of Sangeeta Jethwa will be reviewed in Leiden.
 - Two more candidates will be identified during the year.

Next steps and actions

- Finalize the guidebook and disseminate the results by July 2019
- Identify the new methodology for drug counting
- Submit the different manuscripts in preparation
- Validate the TSC activities for 2020
- Follow-up on new members