

Meeting report series

Report of the 13th Therapies Scientific Committee Teleconference

Teleconference
March 7, 2018

Participants

Dr Diego Ardigo, Parma, Italy (Chair)
Dr Virginie Hivert, Paris, France (Vice Chair)
Prof Annemieke Aartsma-Rus, Leiden, The Netherlands
Dr Robin Conwit, Bethesda, USA
Mr Yann Le Cam, Paris, France

Ms Christine Cutillo, Bethesda, USA
Dr Anneliene Jonker, Paris, France

Apologies

Dr Seng H. Cheng, Framingham, USA
Dr Michela Gabaldo, Milan, Italy
Dr Karin Rademaker, Utrecht, the Netherlands
Dr Sandrine Marreaud, Brussels, Belgium
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Agenda

1. Welcome
2. Update on IRDiRC Membership and TSC Membership
3. Recap' on Strategic exercise performed by TSC before Tokyo CA and Updates on Roadmap and new Activities for the TSC – *Document 1, 2, 3*
4. Action plan for 2018 - activities, responsibilities and timeline
 - a. Defining new process for drug development and registration of innovation therapies for RD
 - b. Support the reframing of the current international research agenda for RD pushing

for focusing research efforts and funding

5. Introduction to the Guide to drug development– *Document 4*

REPORT

1. Welcome and introduction

The Chair welcomed all participants of the teleconference call and explained that the objective of the call was to provide an overview of ongoing activities and to continue on the brainstorming on the new activities for 2018.

2. Update on IRDiRC Membership and TSC Membership

The TSC is currently undergoing several changes:

- ▶ Adam Heathfield has left the TSC, as he served two consecutive terms
- ▶ Akifumi Matsuyama has left the TSC, as he retired
- ▶ Sandrine Marreaud has served one term, and has expressed her wish to serve a second term. This will be send to approval to the Consortium Assembly (CA).
- ▶ Ken Sakushima, has been approached to join as new member, on behalf of PMDA, the Japanese Medicine Agency. His nomination will be send to approval to the CA.

IRDiRC has seen several new members in the past year. Most notably, there has been an increase in the number of patient advocacy groups. IRDiRC currently has 9 patient advocacy groups on board, that are:

- ▶ US National Organization of Rare Disorders (NORD)
- ▶ Genetic Alliance
- ▶ EURORDIS-Rare Diseases Europe
- ▶ Chinese Organization for Rare Disorders (CORD)
- ▶ Advocacy Service for Rare and Intractable Diseases' multi-stakeholders in Japan (ASrid)
- ▶ Indian Organization for Rare Diseases (I-ORD)
- ▶ Canadian Organization for Rare Disorders (CORD)
- ▶ Rare Voices Australia (RVA)
- ▶ Rare Diseases South Africa (RDSA)

3. Recap' on Strategic exercise performed by the TSC before Tokyo CA and Updates on Roadmap and new Activities for the TSC

- ▶ Prior to the Tokyo CA meeting, a strategic exercise was held among TSC members, a GAP analysis to generate new actions for the TSC to take on
 - Resulted in a general framework for the generation of the TSC strategy
 - What needs to be done? – Strategic imperative
 - How to do it? – Strategic tools

- What are the stakeholders, who to communicate with? – Target stakeholders
 - Resulted in the identification of 4 strategic themes for action
- ▶ Themes and actions were presented at the Tokyo CA meeting, as were all actions from the other Scientific and Constituent Committees. Hereafter, actions were condensed by the Sci Sec, and presented for vote to the CA
 - Proposed TSC actions were approved, with over 90 % of votes for each activity
- ▶ Final list of actions of the TSC, and the outcome for each activity
 - Support the definition of a model for inclusion of patient's perspective into drug development and natural history research
 - Included in PACC led activities
 - No specific follow-up
 - Support the definition of a new master plan for the development and registration of innovative drugs specific for RDs
 - Activity TSC led
 - Voted for priority as ACTION C
 - Support the definition of a research framework and a business model for repurposing of existing drugs specific for rare diseases
 - No new action approved
 - Foreseen activities to be put forward as internal TSC follow-ups of the DMR TF
 - Support the definition of standards for use of data collected in health care practice for RWE generation, in particular for disease understanding and treatment monitoring
 - Activity shared with CCC and FCC
 - Voted for priority as ACTION D
 - Support the reframing of the current international research agenda for rare diseases pushing for focusing research efforts and funding
 - Activity TSC led
 - Voted for priority as ACTION E

4. Action plan for 2018 - activities, responsibilities and timeline

Including the new activities, the TSC has currently four activities for this year.

- ▶ Follow-up of the work carried out by the Repurposing TF
 - Recommendations issued by the TF have not been made public, but will be done so simultaneously as the article is published
 - Article is currently being writing by Sci Sec, Diego and Virginie
 - Writing, review and submission is scheduled to take place in Q1-Q2 2018
 - Anyone from the TSC who would like to join is welcome

- ▶ IRDiRC Therapies Counter – phase 1
 - Sci Sec has conducted an analysis of drugs approved during previous IRDiRC goal (200 drugs for rare diseases)– comparing EMA and FDA times of approval, time differences between designation and MA, type of diseases, type of medication, etc
 - Article writing, review and submission - Q1-Q2 2018
 - Questions that could be included for analysis should be send to Sci Sec
- ▶ IRDiRC Therapies Counter – phase 2
 - New rules to be applied to gather additional information
 - Track all pharmaceutical products for rare diseases (with or without OD) approved by EMA, FDA, or PMDA starting from January 1, 2017, to count:
 - 1) New Rare Disease Pharmaceutical Products
 - 2) New Orphan Medicinal Products
 - 3) New rare diseases with a treatment
 - 4) New unique Orphan Medicinal Products
 - 5) Average number of new line extensions
 - 6) Average number of new Rare Disease Pharmaceutical Products approved for each disease
 - The new indicators are:
 - Indicators of science progression: 3 and 4
 - Indicators of R&D progression: 1 and 2
 - Indicators of market maturity: 5 and 6
- ▶ Support the reframing of the current international research agenda for RD pushing for focusing research efforts and funding
 - Same methodology than for the Strategic Exercise extended to the whole IRDiRC Consortium
 - Is scheduled to take place in Q3-Q4 2018
 - Design internal questionnaire, have a survey period, do a compilation of survey outcomes, set up a workshop in conjunction with CA meeting, write a report
 - TSC Members to give input on the questionnaire, as they have already taken part in this exercise previously.
- ▶ Defining new process for drug development and registration of innovation therapies for RD
 - Renamed the “Guide to drug development”, activity C in the IRDiRC Roadmap 2018
 - Will take place throughout the whole year and will therefore be the main TSC activity
 - Carried out together with additional experts when needed

5. Introduction to the Guide to drug development

The Guide to drug development:

- ▶ The current drug development model has been built for traditional pharma, based on clear guidelines and progressive de-risking
 - Model is not really suitable for orphan drug development, complex biotech products and does not suit the need of new players (such as non-profit organizations and biotechs)
 - Single initiatives are working to define new tools for drug development, but a new model has not yet been defined
- ▶ The scope of this project is therefore to create a guide for academic and industrial drug developers describing the available tools and initiatives specific for rare disease drug development and how to best use them
 - The Guide to drug development will be extremely helpful especially for education of junior investigators
 - Will make people aware the next steps in drug development they have to prepare for, thereby avoiding to find out the wheel if the process is already in motion
- ▶ Deliverables of this project are:
 - Fact sheet on new development tools
 - Roadmap with check-lists of “what to do” and “when to do it” for rare diseases
 - Handbook
- ▶ Project plan
 - Starting point is the development of the framework
 - Building blocks will be added to this
 - Standard cases will be added to this
 - IRDiRC Handbook is the final outcome
- ▶ The development framework
 - Framework is the skeleton for the whole exercise
 - Start with what is already there, but simplified and make it useful
 - Once framework is ready, the different elements can be placed, the building blocks, the tools, activities and initiatives
- ▶ Building blocks
 - Examples (in random order):
 - Small populations clinical trials design
 - Definition of PROMs
 - Early scientific advice procedures
 - HTA advices
 - Repurposing programs
 - Patient engagement in trial design
 - Involvement of clinical networks
 - Etc
 - Includes:
 - Includes (not exhaustively):

- Description of the tool
 - PROs and CONs
 - Best timing to use it
 - Timelines and procedures
 - How to do it...
 - Etc
- ▶ Standard cases and output
- Will be put in the skeleton, for illustration
 - What do you have to do prior to a trial, when is the good time for that?
 - Will lead to checklists that will be made available to the community
 - Will be made suitable for small molecules, biologicals and advanced therapies, and available for multiple geographical regions
 - Will have to show the continuum of evidence generation from early clinical phase to real life medical use
- ▶ Timeline
- March 2018: Drafting of development framework, list of building blocks, fact sheet template
 - Will be prepared by TSC chairs and send to TSC members
 - April 2018: Review and approval by TSC members
 - May 2018: Drafting of fact sheets by individual TSC members
 - Followed by review by the group via TC
 - Q3 2018: Formation of a Task Force of external experts with series of TCs to:
 - (1) finalize development framework and fact sheets
 - (2) define the standard cases to be discussed in the F2F workshop
 - Q4 2018: TF workshop to develop the standard cases
 - Q1 2019: Preparation workshop report, Guidebook preparation, checklist preparation, and dissemination of results

Main action points

- ▶ Draft paper drug repurposing
- ▶ Finish analysis of drugs 2010-2016 and draft paper
- ▶ Volunteer for review ongoing TSC publications
- ▶ Send in questions for analysis drugs EMA and FDA
- ▶ Send ideas of building blocks or initiatives for the Guide to drug development