

## Meeting report series

### Report of the 17th Therapies Scientific Committee Meeting

Teleconference  
September 10, 2018

#### Participants

Diego Ardigo, Chiesi Farmaceutici S.p.A., Italy – Chair  
Virginie Hivert, EURORDIS-Rare Diseases Europe, France – Vice Chair  
Michela Gabaldo, San Raffaele Telethon Institute for gene therapy, Italy  
Yann Le Cam, EURORDIS-Rare Diseases Europe, France  
Sandrine Marreaud, EORTC, Belgium  
Anne Pariser, National Center for Advancing Translational Sciences (NCATS), NIH, USA  
Ken Sakushima, PMDA, Japan

Marlène Jagut, Scientific Secretariat (Sci Sec), France  
Anneliene Jonker, Sci Sec, France  
Lilian Lau, Sci Sec, France

#### Apologies

Annemieke Aartsma-Rus, Leiden University Medical Center, The Netherlands  
Seng Cheng, Sanofi, USA  
Robin Conwit, National Institute of Neurological Disorders and Stroke (NINDS), NIH/ Georgetown University, USA  
Karin Rademaker, University Medical Centre, The Netherlands  
Maurizio Scarpa, Horst Schmidt Klinik / University of Padova, Germany/ Italy  
Josep Torrent i Farnell, Autonomous University of Barcelona, Spain

#### Agenda

- ▶ Activity C - Galaxy Guide
  - Creation of the Organizing Team
  - Review of Building Block forms
  - Choice of the workshop venue
  - Organization of the workshop
- ▶ Update other IRDiRC Activities
  - Draft proposal Activity D
  - Next steps Activity G

- ▶ Any other business
  - Any other business or initiative to be proposed/ discussed

## REPORT

### 1. Activity C - Galaxy Guide

#### 1.1 Creation of Organization Team

In order to direct this Task Force, an Organization Team will be set up. This team will effectively lead the Task Force. Members of the TSC that are interested in taking part in the team are asked to inform the Sci Sec.

→TSC members interested in taking part in the Organization Team are asked to inform the Sci Sec

#### 1.2 Review of Building Block forms

The Sci Sec has worked extensively on filling out Building Blocks:

- ▶ Review of the forms
  - Each member is asked to pick 5 Building Blocks of their interest to review. In order to do so:
    - Member fills out their name in the Building Block list that they will review these Building Block
    - Member reviews the Building Block (in file BB to review), and puts the reviewed file in the dedicated folder (BB forms reviewed). In particular, pros and cons and personal experience for each BB is needed
  - If no volunteers step forward to review, the Sci Sec will determine a “volunteer” for each Building Block

→TSC members to review all Building Block forms

#### 1.3 Choice of the workshop venue

Two venues are currently under consideration: Miami(Florida) and Dublin (Ireland)

- ▶ Dublin is “in between” the US and Europe
  - Well connected to US and Europe regarding flights
  - Rains in December, but not likely to snow and therefore limited chance to cancelled flights
  - Already an option provided by Orphanet Ireland
  - Preferred option from Task Force members so far
- ▶ Miami is on the east coast in the US
  - Well collected to other US cities and relatively well connected to Europe regarding flights
  - Sun in December, so no cancelled flights due to snow

As most members prefer Dublin, Sci Sec will work with Ireland Dublin team to confirm the venue. Requirements are a room for 20-25 people and a second smaller room for a breakout.

→Sci Sec to confirm the venue in Dublin

#### **1.4 Organization of the workshop**

In order to successfully take on the next steps in the Activity C – Galaxy Guide, a plan has been set out. The steps:

- ▶ By today
  - Finalize list of experts and TSC attendees - done
  - Define location of the WS - done
- ▶ By end of the week
  - Confirm list of BB form reviewers
  - Define the Organization Team (OT)
  - Plan approach to WS
    - Organization team meetings
    - Steering group TCs
  - Send additional invitations to experts and collect responses
- ▶ By mid-October
  - Arrange travels
  - Review of BB forms
  - Define WS agenda and methodology
  - Define standard cases

In addition, two teleconferences for Galaxy Guide Task Force members are organized. They will take place on October 24 and November 20, from 2-4 PM CEST/ 8-10 AM EDT/ 9-11 PM JST.

## **2. Update other IRDiRC Activities**

### **2.1 Draft proposal Activity D – Natural History and Registries Platform for use in RWE data collection**

This Activity is led by the ISC, but with strong participation from the TSC, FCC, and CCC

- ▶ Draft proposal is written by the current ISC Vice Chair
- ▶ Only the concept idea has been approved by the CA, the full proposal not yet
- ▶ Comments are very welcome, to improve the proposal. Members are asked to send in comments by September 24, 2018

→ Interested TSC members are asked to provide comments on Activity D draft proposal

### **2.2 Next steps Activity G – Clinical Research Networks for Rare Diseases**

This activity is led by the ISC, but with strong participation from the TSC and FCC

- ▶ Proposal has been approved by the CA in May 2018
- ▶ Background document will be written by Sci Sec, upon instruction of Chairs involved
- ▶ Original timeline will be extended a bit

### **Next steps and actions**

- ▶ Sent in suggestions regarding industry members and regulators for Activity C by September 19, 2018
- ▶ Sent out the invitations to selected nominees
- ▶ Inform Sci Sec if interested in taking part in the Activity C Organizing Team
- ▶ Review all Building Blocks
- ▶ Confirm venue for Activity C – Galaxy Guide Task Force
- ▶ Provide comments on Activity D draft proposal