IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

Meeting report series

Report of the 11th Therapies Scientific Committee Teleconference

Teleconference
May 22 2017

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
Dr Michela Gabaldo, Milan, Italy
Dr Adam Heathfield, Sandwich, UK
Mr Yann Le Cam, Paris, France
Dr Maurizio Scarpa, Wiesbaden, Germany
Prof Josep Torrent i Farnell, Barcelona, Spain

Dr Lilian Lau, Scientific Secretariat, Paris, France
Dr Valerie Salamon, Orphanet, Paris, France
Dr Takeya Adachi, AMED, Tokyo, Japan

Apologies

Dr Seng H. Cheng, Framingham, USA
Dr Shuling Guo, Carlsbad, USA
Dr Sandrine Marreaud, Brussels, Belgium
Dr Akifumi Matsuyama, Osaka, Japan
Dr Karin Rademaker, Utrecht, the Netherlands

Agenda

1. Welcome and introduction
2. TSC membership
3. Updates on ongoing Task Forces
4. Updates on new Task Forces
5. New rules for the therapy counter

6. Proposals for the strategic plan based on new goals

REPORT

1. Welcome and introduction

The Chair welcomed all participants of the teleconference call; each member and invited observer briefly introduced themselves.

2. TSC membership

2.1 Mandate of the TSC

Among the most important elements:

- Develop policies and guidelines
- New ideas of actionable projects to contribute towards achieving IRDiRC's objectives
- Expertise contribution, e.g. review IRDiRC Recognized Resources, input in State-of-Play report

To keep up engagement and momentum:

- About 4 meetings per year: 1-2 face-to-face, 2-3 teleconferences
- Mandatory participation in 50% of these meetings
- Chair actively checking compliance and rules if possible to host a meeting in Parma

2.2 Expanding membership of the TSC

A number of renewal and new nominations will be put through to the CA for approval.

Wish to increase presence of missing actors:

- Academic groups involved in drug development
- Regulators
    - EMA: uncertainties due to Brexit thus unable to commit to long term engagement; willing to contribute to Task Forces
    - PMDA: have been approached via AMED (+ Ministry of Health), awaiting nominations
    - FDA: may invite to participate

In the future, in developing policies, the question on involvement of payers or investors should be systematically posed prior to making recommendations.

3. Updates on ongoing Task Forces

- Patient-Centered Outcome Measures (PCOM)

- Met about 1.5 years ago in Paris, France
- Recommendations available on IRDiRC website
- Publication: article writing in progress, led by Thomas Morel; will ask PCOM and TSC members to review prior to submission

- Small Population Clinical Trials (SPCT)
  - Met about a year ago at the EMA in London, UK
  - Recommendations available on IRDiRC website
  - Article writing led by Simon Day; submitted but not successful
  - Working on addressing issues raised by reviewer; will resubmit

- Data Mining and Repurposing (DMR)
  - Met about six months ago in Barcelona, Spain
  - Scientific Secretariat working with Task Force leaders on finalised recommendations
  - Article writing being planned
  - Recommendations will be published on IRDiRC website at the same time as article

4. Updates on new Task Forces

- Patient Engagement in Rare Diseases Research
  - Joint TSC/ISC initiative, together with Patient Advocates Constituent Committee (PACC)
  - Two focus points to tackle through best practices and guiding principles
    - Patient-industry partnership in medical product development
    - Patient engagement to improve quality of research
  - First leadership call held to discuss work plan
    - Q3 2017: approach Steering Committee members (8-10 people); member to help provide information for the writing of background document; prepare workshop and invite additional members
    - Q4 2017: 1-2 pre-workshop teleconferences
    - Q4 2017/Q1 2018: workshop of Task Force
    - Q1 2018: writing of recommendations and article(s) for publication
  - Important not to duplicate work done by a number of initiatives but not RD-specific (e.g. CTTI, National Health Council, EUPATI)
  - Profiles of Steering Committee members
    - PACC representative
    - Industry representative
    - Patient/EUPATI liaison
    - CTTI representative
    - National Health Council representative
    - RegulatorsClinicians/research-based stakeholders
    - IFPMA representative

- Clinical Research Network
  - Not yet formally approved by the IRDiRC Consortium Assembly (CA) although lengthy discussion was held in Catania last year and received great interest from CA members
    - Terms of reference to be defined
  - Basic premise: take existing initiatives (e.g. US's Rare Disease Clinical Research Network, the newly launched European Reference Networks in the EU, etc) and use their experience to identify common features, build common objectives and develop global framework to facilitate clinical research (e.g. though quicker patient identification, patient recruitment, scaling up of development of natural history studies)
  - Profiles of Steering Committee members
    - Coordinator of E-Rare/EJP
    - NIH/NCATS (funder of RDCRN) representatives
    - ERN representatives
    - Representatives of existing networks, e.g. TREAT-NMD, ECRIN, NeuroNEXT, Asian Academic Research Organization Network, EnprEMA
  - Note re ERNs: a working group is looking into the topic of research, so there is unique opportunity for critical mass to help identify patients, new trials, designs, endpoints, etc to help speed therapy development up; keen to collaborate with IRDiRC

5. New rules for the therapy counter

IRDiRC has set a new, bolder therapy goal. The TSC is mandated to keep track of the therapy counter as metric to measure the success of this goal.

Primary count remains the same in order to maintain continuity from the work done to date. A number of interesting secondary counts are being proposed (please see Document 2 circulated for the teleconference). Members should:

- Review these secondary counts
- If have additional ideas/feedback, please send to the Sci Sec
- To vote/approve on secondary counts (a survey will be sent out next week)

Members may also be asked to assist in discerning borderline cases, whether a therapy be counted or not. For now:

- Please review the proposed resolution measures
- Please send any comments/feedback to the Sci Sec

6. Proposals for the strategic plan based on new goals

Apart from the upcoming Task Forces, the Chair asked members to brainstorm on gaps in the therapeutic development system that are barriers to reaching the new IRDiRC therapy goal.

A questionnaire will be circulated to all members in order to guide this effort. Members should contact the Chair and Vice Chair with their ideas of actions, ahead of the questionnaire being sent, so prioritisation of actions can be developed. A teleconference may be organized in the next couple of months to fine tune the strategic plan for the TSC.