

## Meeting report series

# Report of the 3rd Teleconference of the Working Group on Chemically-derived products including repurposing

23 July 2014

## Organization

Organized by: Scientific Secretariat  
Teleconference

## Participants

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## Apologies

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## Agenda

- ▶ Review of the Therapies Scientific Committee recommendations for the IRDiRC Roadmap
- ▶ Repurposing Workshop

## REPORT

### Therapies Scientific Committee recommendations for the IRDiRC roadmap

The members of the Working Group (WG) reviewed the recommendations elaborated by the Therapies Scientific Committee (TSC).

#### Recommendations for funding

##### Stage of compounds

In the TSC recommendations, it is not well defined at what stage compounds should be in order to get funding. Members questioned if this should be defined or left as it is. The same question was raised during the 3<sup>rd</sup> Annual Drug Repositioning, Repurposing and Rescue Conference that took place in Boston on the 15-16 July: it was questioned at what stage a drug could be repurposed.

Some members of this WG proposed prioritizing repurposing of compounds which have at least completed Phase I (so data should be available on toxicity, efficacy, etc) and demonstrated efficacy. However, this raised the question if a compound should also be prioritized based on the completion of Phase I alone. In the field of rare diseases, it is often hard to prove the value of theoretical efficacy due to difficulty in running a long-term trial and/or the lack of funding in creating suitable animal model. Other exceptions also need to be accounted for e.g. the use of off-label drugs which have not gone through Phase I.

A broader definition might be more suitable than a narrow one, in order to include the diseases that need more help.

A repurposed drug is not necessarily one that has already been approved for another indication. For instance, if a company had taken a drug through Phase III but subsequently dropped because it did not show efficacy in that indication, there are still a lot of data there and it still can be repurposed, as long as there is a very strong theoretical argument in favour.

##### Regulatory agencies and receipt of Scientific Advice

The easiest starting point for funding projects with repositioning molecules might be for products that have contacted regulatory agencies to request Protocol Assistance/Scientific Advice (SA) for designing the studies needed for the development of the product. In support of this notion, it was pointed out that at least two regulatory agencies have published data indicating that compliance with SA correlates with a success in Marketing Authorization.

A further discussion on this will take place in the next teleconference as members could not agree if the advice from regulatory agency should be a pre-requisite for funding or not.

## Overall conclusion

The criteria for funding, either for a new or a repurposed compound, should be the availability of data on safety and efficacy, be it theoretical or experimental, that are convincing to the granting authority.

## **Recommendations for IRDiRC funding organizations**

Several points were raised during the discussion:

- ▶ International collaboration for rare disease is important but not mandatory.
- ▶ The commitment to apply for scientific guidance for products not yet having sought scientific advice should be defined on the diagram on page 3 (it links back to the discussion above about regulatory agencies projects assessment).
- ▶ The flexible additional shared criteria “Most life threatening, severe or debilitating diseases” should be taken out as it is too subjective (page 3).
- ▶ The flexible additional shared criteria “Unmet medical need/absence of alternative treatments” should be a mandatory shared criteria (page 3). Research should be encouraged in that domain.
- ▶ The Scientific Secretariat could assist the compilation of data on “Priorities for gap analysis funding: analysis of outcomes from previously funded projects (FP6, FP7, E-Rare, NIH...) in order to understand the reasons of success or failure” (page 5). This will give guidance for the future.
- ▶ The TSC could be asked to provide lists of off-label use of therapies to fulfil “Assessment of off-label use of current therapies that may be of relevance for the patient needs” (page 5) as many exist but are not freely available.
- ▶ Cross link data base from Agencies and funding organizations in order to rationalize the development in RD. Finding information of research works already performed is extremely time consuming.
- ▶ Repositioning is key in the IRDiRC race and has to be considered as top line for funding. It could be IT tools as well as empirical research made by clinicians or patient’s groups.

## **Recommendations related to regulatory processes**

It was suggested that regulatory processes for a molecule which is a new compound or an old compound already marketed should be different.

It was also mentioned that following one or two years of the designation, it could be required for the sponsors to return to the agency for a face-to-face discussion with the regulators if the conversion of designation is slowed or stopped by regulatory hurdles (e.g. financial reasons, lack of expected positive results).

## **Summary of roadmap**

In order to reach the goal of 200 new therapies by 2020, more funding should be made available. That point should be emphasized to the TSC. New funding mechanisms should be found to resolve the financing problem.

New methodological approach of clinical trials using telemedicine and e-health decreases the need of patients in hospitals as well as the cost and the duration of the trial. This could release more funding and increase the amount of drugs approved, although this approach is not well precedent for any diseases, let alone rare disease.

Investment is needed to “identify compounds” for designation and follow-up.

### **Metrics of progress**

Improving the attrition rate between designation and granting authorization could be a way to assess the accuracy of work. With over 2,000 designations not into authorization, question was raised on what is required to bring them into the market.

### **Repurposing Workshop**

The main purposes of this workshop would be to:

- ▶ Define the criteria for funding: at what stage compounds have to be to get funding, etc
- ▶ Understand why many orphan designations are not been converted into drugs.
- ▶ Create an opportunity to meet people from different domains and make them understand the value of their work to get drugs on the market.

The ideas of the WG on other topics for this workshop will be collected before being proposed to the TSC. The Scientific Secretariat will compile them and a proposal should be ready in September 2014. The workshop should be scheduled for early 2015.

### **Deliverables**

- ▶ Compile data on the analysis of outcomes from previously funded projects (FP6, FP7, E-Rare, NIH...) in order to understand the reasons of success or failure.
- ▶ Provide lists of off-label use of current therapies that may be of relevance for the patient needs.
- ▶ Circulate the article “Financing drug discovery for orphan diseases”. *Already done*
- ▶ Find out why so many orphan designations are not converted into drugs.
- ▶ Write two pages on the Repurposing Workshop for September.
- ▶ Write down some topics for the Repurposing Workshop for September.
- ▶ Compile the topics for the Repurposing Workshop.
- ▶ Plan the next teleconference to be held in October 2014.