Rationale

The development of rare diseases therapies requires the right regulatory, economic and political ecosystem to ensure that investments are made in areas where research would not otherwise be done. The current European regulatory framework has brought enormous benefits to the rare disease community in terms of the number of orphan designations and indications, as well as paediatric medicines.

However, there is increasing political scrutiny of incentives for research and development, with some criticisms of the latter being improperly linked with the high price of innovative medicinal products. More importantly, the great majority of rare diseases are still without appropriate therapy. Although the current incentives systems have addressed many issues, some important ones remain unanswered.

Objectives and expected outcomes

This workshop will take a balanced look at the role of incentives in therapies development, address shortcomings of the current system and consider what the rare disease community can do now to fulfil important unmet medical needs.

In particular, the workshop will collect feedback, and explore interests and positions on the forthcoming joint evaluation of the legislative framework for orphan and paediatric medicines, as well as the current evaluation of the incentives system. Furthermore, the workshop will present the opportunity to discuss how to support therapies development for rare diseases and ensure access of the patients to these treatments. We expect this event to allow us to collectively reflect on better and innovative approaches, building on what is already working.
In the morning, we will take a close look at the current framework for the development of therapies. In December 2017, the European Commission launched a roadmap for the joint evaluation of the Orphan Medicinal Product Regulation and the Paediatric Medicines Regulation, on top of the ongoing evaluation of incentives in pharmaceutical product development. We will hear from DG SANTE what the results are so far and what policymakers expect from this evaluation. We know the road to discovery of therapies for rare diseases is long and complicated, but what is the role of incentives in this process? We will have an overview of the key role that incentives (economic and regulatory) play. To conclude the keynote speeches, we will hear if the current framework is really addressing the unmet needs of people with rare diseases, and what may be missing from discussions to date.

The cross-fire panel debate will reflect on the position expressed in the presentations of the morning and further elaborate possible solutions to ensure that the right incentives are in place to fulfil real unmet needs from all stakeholders’ point of view (patients, academics, policymakers, payers, and industry).

The three breakout sessions that will ensue will look at specific areas of development that could potentially fulfil real unmet needs for people with rare diseases. It is important to remember that up to 95% out of the approximately 6,000 identified rare diseases do not have an approved therapy. The breakout sessions will thus try to answer the following questions:

1. How can repurposing of existing medicines with potential to treat rare conditions be encouraged? Which kind of regulatory framework and what type of incentives are needed? Which business model(s) could be envisaged?
2. Are the current economic or regulatory incentives adequate to really foster R&D for rare diseases therapies? Are there other avenues in place to look at to encourage further research and to ensure improved fulfilment of the paediatric needs?
3. Are the scientific advancements represented by advanced therapeutic medicinal product (ATMPs) such as gene therapy sufficiently supported? Do we have the right regulatory and political environment in place to ensure that science is translated to access?

The rapporteurs of each session will open up the afternoon session, reporting back from their individual sessions, engaging the broader audience on the topics discussed in the morning, and highlighting ideas to move forward. The ensuing panel session will then broaden the horizon to look at what is happening in the rest of the world, what potential lessons can be learnt from other geographies and other areas, and what alternative and complementary models could be introduced.

The final session will reflect on the discussion of the day and conclude with concrete actions that must take place to ensure that we close the gap between development and access so that therapies are developed and available for the great majority of people with rare diseases who currently do not have the hope they deserve.