SMART4FABRY

Smart Functional GLA—nanoformulation for Fabry disease

FACING RARE DISEASES BY PHARMA INDUSTRY

Rare diseases, like Fabry disease, mean a major health problem for a significant number of people in total, but affecting small amount of patients for each individual diseases.

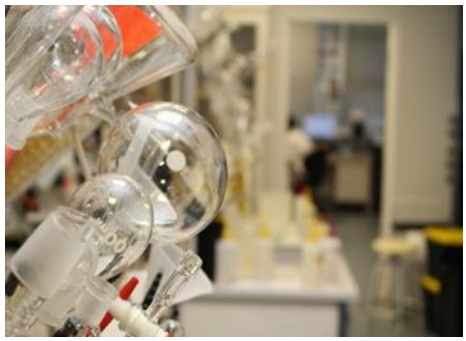
This situation leads to a deficit of medical and scientific advances. Rare diseases have been ignored as a global concept for a long time, as population is quite scattered and diagnosis tools are not available in many cases. This implies little or directly no research around rare diseases in many cases, due to this in the current moment there is no cure for many of the existent rare diseases. On the other hand, there have been significant advances for some of them, showing that with and adequate investment and innovation model, progress towards cure of many rare diseases is possible.

SOME FACTS ON RARE DISEASES

- In Europe, a disease is considered to be rare when it affects **1 person per 2000**
- In the current moment, around 60 to 70 thousand different diseases belong to this category
- Orphan Drug designation is a special mechanism to fight rare diseases with an increasing impact in the EU, USA and Japan

PARTNERS

THE NEED FOR OPEN INNOVATION



Consequently, efforts for development of novel treatments has been discontinued for decades, as Pharmaceutical industry and capacity for innovation were focused in well-known diseases, easier for business models and ready for "*blockbuster molecules"* The described situation leads to an "innovation gap", motivated by the lack of knowledge on rare diseases and the low purchasing power due to the small amount of patients, which should drive the investment in novel medicines to treat rare diseases. A cooperative framework between public and private institutions is needed to get new therapeutic alternatives, including academia, SMEs, industry and clinicians.

NEW PARADIGMS

Fortunately, during the last decade this situation is changing. Efforts from private and public initiatives (IRDiRC, the European Commission, patient associations, foundations and NGOs), together with a major compromise from the Pharma industry, is leading to the development of novel treatments and diagnosis tools for rare diseases. The industry is conscious of the demands of the world of rare diseases, and is getting more and more compromised with the problem. This is due to several factors, as social responsibility convincement, personal compromise of the major shareholders (as happens with Praxis Pharmaceutical and Fabry) and opportunities to face projects prioritized by unmet medical needs with a different concept of commercial benefit and more innovative business models. This new approach should be faced using open innovation paradigms, which means developing solutions through collaboration with different partners, sharing technology, intellectual property, knowledge, etc, allowing the obtaining of solutions with low cost and lower risk. SMART4FABRY project is a clear example of this strategy, putting together academics and industry in order to obtain a revolutionary, more efficient, cheaper and safer treatment for Fabry Disease.

ORPHAN DRUGS:

208 Orphan drug designations were granted in the EU in 2016. This number of designations constantly increases since 2002, with a cumulative number of 1824 designations in the EU.

7% OF THE WORLD POPULATION IS AFFECTED BY RARE DISEASES

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CONCLUSIONS

Rare Diseases are rare when considered in a case-by-case basis, but affects a significant amount of people Worldwide. Everyone has the right to be treated, and to benefit from innovation.

Small and medium enterprises and mid-caps are a key driver for these changes, but many times these companies cannot afford the whole development and/or they lack the needed expertise. A cooperative framework with all the other agents involved in the development chain, based on an open innovation framework will be useful to face the challenge of the development of novel therapies for rare diseases. Improved investigation in rare diseases must be an objective by itself, but it also opens new, innovative pathways, which may be used to develop treatments for disease s with higher incidence, meaning an increasing impact.

Support from public funding is also needed, in order to ensure that all unmet medical needs have the same chances to benefit from innovative treatments, despite their incidence or the pure economic potential of the proposed developments.

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