THE 2ND INTERNATIONAL CONFERENCE ON
SYSTEMS MEDICINE, AI & DRUG REPURPOSING

The IRDiRC Drug Repurposing Guidebook: Creating an efficient and visible pathway for rare diseases

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Abstract

Drug repurposing is an exciting topic in the world of rare diseases, and it has often been suggested as a key approach for developing more therapies for the estimated 6000-8000 rare diseases. This strategy can be an attractive option because it often involves developing therapies in an efficient, potentially cheaper, and innovative way, building on previous knowledge and experience. Drug repurposing can be defined in several ways but in broad terms, can be considered as developing an existing drug in an indication outside the scope of the original indication, with the ultimate purpose of obtaining a new regulator-approved indication. Several tools and incentives have been developed to stimulate and ease the approach for repurposing for rare diseases. Nevertheless, the field still sees quite some challenges, such as intellectual property issues, lack of knowledge on regulatory requirements, the need for additional (re)formulation or obtaining additional safety-efficacy data that may be difficult to collect, and difficulties in commercialization due to the lack of sustainable business models. Consequently, repurposing approaches for rare diseases have, until now, not been as impactful as anticipated.

We will present the work of IRDiRC's Therapies Scientific Committee Task Force, following the previously launched Orphan Drug Development Guidebook. We set out to develop a Drug Repurposing Guidebook. This Guidebook is developed for researchers and developers involved in drug repurposing in the rare disease space, specifically academics, startups, small and medium enterprises, and patient-led groups. This Drug Repurposing Guidebook gathered and reviewed tools and created a roadmap to help deliver an efficient development program. This roadmap is integrated with a Gannt chart, highlighting the key repurposing activities for each development phase with checklists to consider the necessary steps to be implemented before starting a repurposing project. As such, this

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Guidebook can help researchers and developers who want to optimize a repurposing project for rare diseases. By allowing an understanding of the available tools, by asking the developer essential questions at different stages and directing them to the available resources, repurposing for rare diseases can be faster and more efficient, and more aligned with the regulatory processes.

Keywords

Drug repurposing, Rare dseases, Guidebook, Academics, SMEs, Patient-led repurposing, Regulatory processes

References

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