The IRDiRC Drug Repurposing Guidebook –creating an efficient and visible pathway for rare diseases

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Introduction

Over 400 M people with a rare disease worldwide

Over 80% are children

6000-8000 diseases are classified as rare

Repurposing can be more efficient, cheaper and innovative way of developing drugs

Development of the Guidebook

Gap analysis of tools, incentives for repurposing: BBs

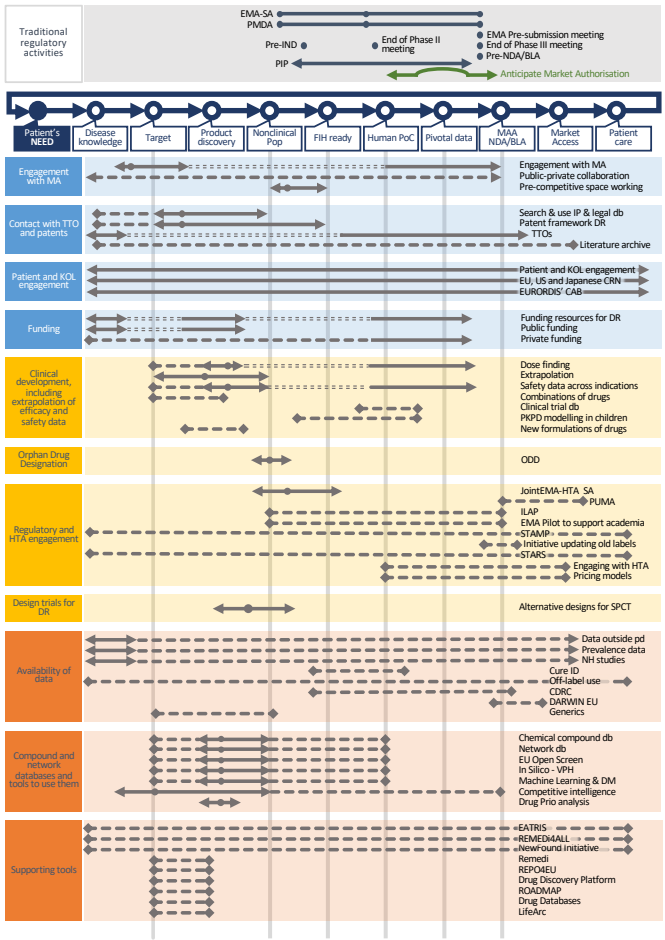
46 fact sheets for the BBs with tips and tricks for usage

Checklists with questions to start

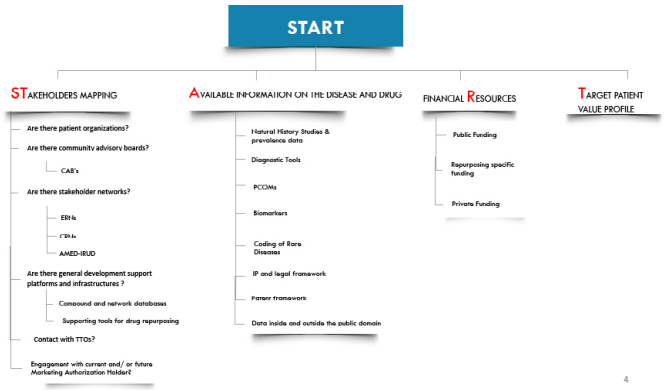
Gantt chart for optimal timing of each BB

11 clusters:
- Engagement with marketing authorization holders
- Contact with tech transfer offices and patents
- Funding
- Availability of data
- Clinical development, including extrapolation of efficacy and safety data
- Orphan Drug Designation
- Patient and Key Opinion Leader engagement
- Regulatory and HTA engagement
- Design trials for drug repurposing
- Compound and network databases and tools to use them
- Supporting tools

Results



Starting a Repurposing Trajectory



For each repurposing trajectory, there is the need to create a strategic plan, and reflect on stakeholder mapping, available infromation, financial resources and a target patient value profile.

Conclusions

IRDiRC's DRG Task Force has created a guidebook that provides key information in the form of building blocks and helps developers navigate an efficient pathway to patient access via a checklist and Gantt chart. This guidebook can support a wide group of stakeholders, i.e. academics, clinicians, small and medium enterprises, and patient-led groups wanting to start a repurposing program for rare diseases

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