# IRDiRC
INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

## Drug Repurposing Guidebook

### Building Block I450

This document defines the content of the FACT SHEET to be created for each identified tool, incentives, initiative or practice (the Building Block) introduced by public bodies or used by developers to expedite drug repurposing in Rare Diseases (RDs).

| ITEM | DESCRIPTION |
|---|---|
| Building Block (BB) Title | Drug Discovery Platform |
| References | https://www.perlara.com <br><br> https://www.genfit.com/ <br><br> https://www.scilifelab.se/units/ddd-platform/ <br><br> https://www.nature.com/articles/s41586-020-2117-z |
| Description | Platform technologies, such as drug discovery platforms, are considered a valuable tool to improve efficiency and quality in drug product development. The fundamental objectives of such platforms are to support discoveries of drug molecules and provide bioanalytical support to the scientific community. A drug discovery platform brings together knowledge and expertise in the first steps of the repurposing and drug discovery process (target identification and validation, hit finding and lead optimization), as such shortening time in the repurposing process. It also can bring about new knowledge in biological research by providing access to cutting edge drug screening and evaluation resources with a particular emphasis on developing novel drugs to improve human health. <br><br> The basic idea is that such a platform, in combination with a risk-based approach, is the most systematic method to leverage prior knowledge for a given new molecule. Furthermore, such a platform enables a continuous improvement by adding data for every new molecule developed by this approach, increasing the robustness of the platform. |

| ITEM | DESCRIPTION |
|---|---|
| Category | Supporting tools |
| Type of BB | Development resource |
| Geographical scope | International |
| Availability | A number of drug discovery platforms are proposed as industrial platforms, where others are academically driven platforms. Overall, costs for the use of the platform, and the expertise of the platform's managers need to be paid. |
| Scope of use | To screen efficiently large number of drugs for the right genetic background. |
| Stakeholders involved | Preclinical researchers from academia and industry |
| Enablers/ Requirements | NA |
| Output | A list of drug repurposing candidates for a specific disorder that can be tested in further wet-lab studies. New knowledge based on existing datasets. |
| Best time to apply and time window | Discovery of drug candidates useable for repurposing |
| Expert tips | If you do intend to use drug discovery platforms, be sure you have the right genetic background for the disease for which you are looking for a drug candidate.<br><br>PROs: Allows you to systematically discover new candidates that you might have missed otherwise.<br><br>CONs: The drug identified via the platform might not be available for use in repurposing |