



IRDiRC

INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

BOOSTING THE DEVELOPMENT OF RARE DISEASE THERAPIES: THE **IRDiRC** ORPHAN **D**RUG **D**EVELOPMENT **G**UIDEBOOK

Virginie Hivert

IRDiRC, Therapies Scientific Committee Vice-Chair

Therapeutic Development Director, EURORDIS – Rare Diseases Europe



Therapies Scientific Committee (TSC)

Diego Ardigò

IRDiRC, Therapies Scientific
Committee Chair

Global Rare Disease Unit,
R&D Head, Chiesi group
(Italy)



Virginie Hivert

IRDiRC, Therapies
Scientific Committee Vice-
Chair

Therapeutic Development
Director, EURORDIS – Rare
Diseases Europe

A BIT OF HISTORY...



IRDIRC

INTERNATIONAL
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Almost ten years later



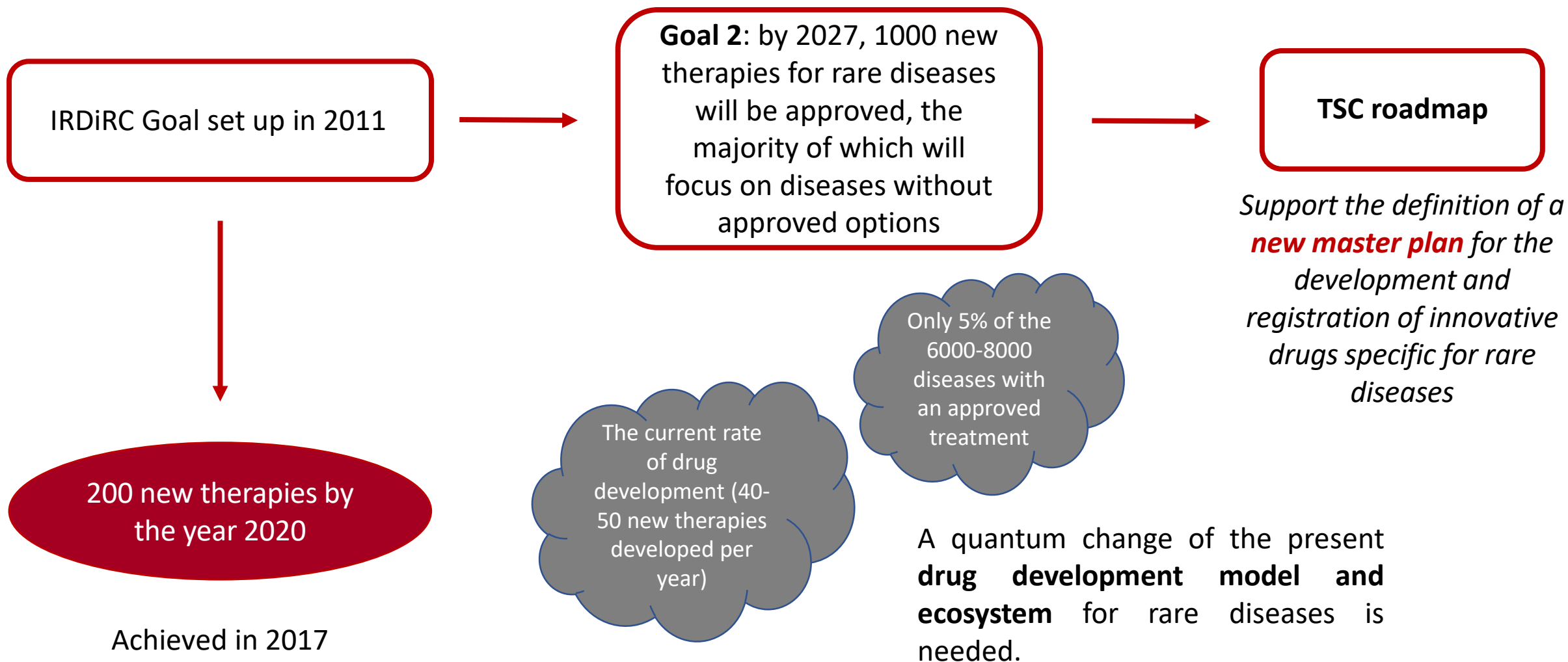
Officially launched in **2011** under the impulsion of the European Commission and the US NIH to accelerate medical breakthrough for People living with Rare Diseases (**PLWRD**) by creating a **network of research funders**



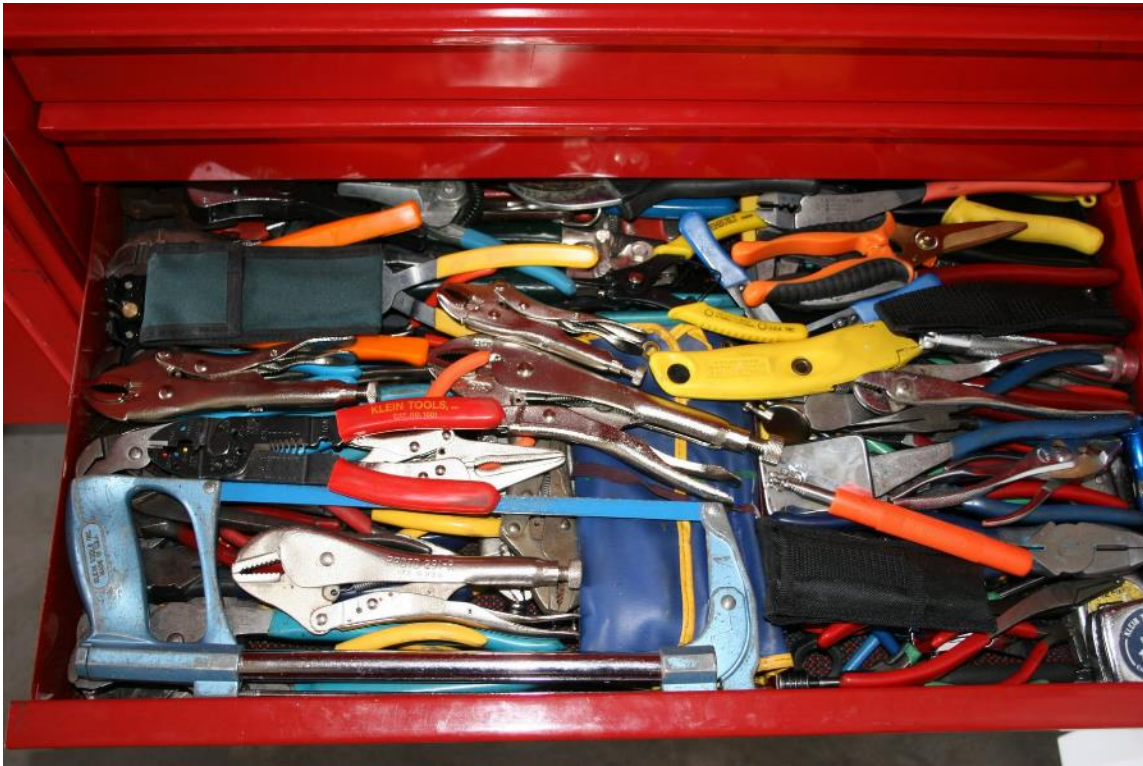
The network has expanded:

- **Funding organizations** (i.e., funding bodies, groups of funders (for small funders), companies) investing more than 10 million USD over 5 years in rare disease research
- **Umbrella patient advocacy organizations** representing broad patients' interests for all rare diseases in at least one country or larger area

ADDRESSING A TREMENDOUS UNMET MEDICAL NEED



From the current toolbox ...



... toward a new one.



ORPHAN DRUG DEVELOPMENT GUIDEBOOK

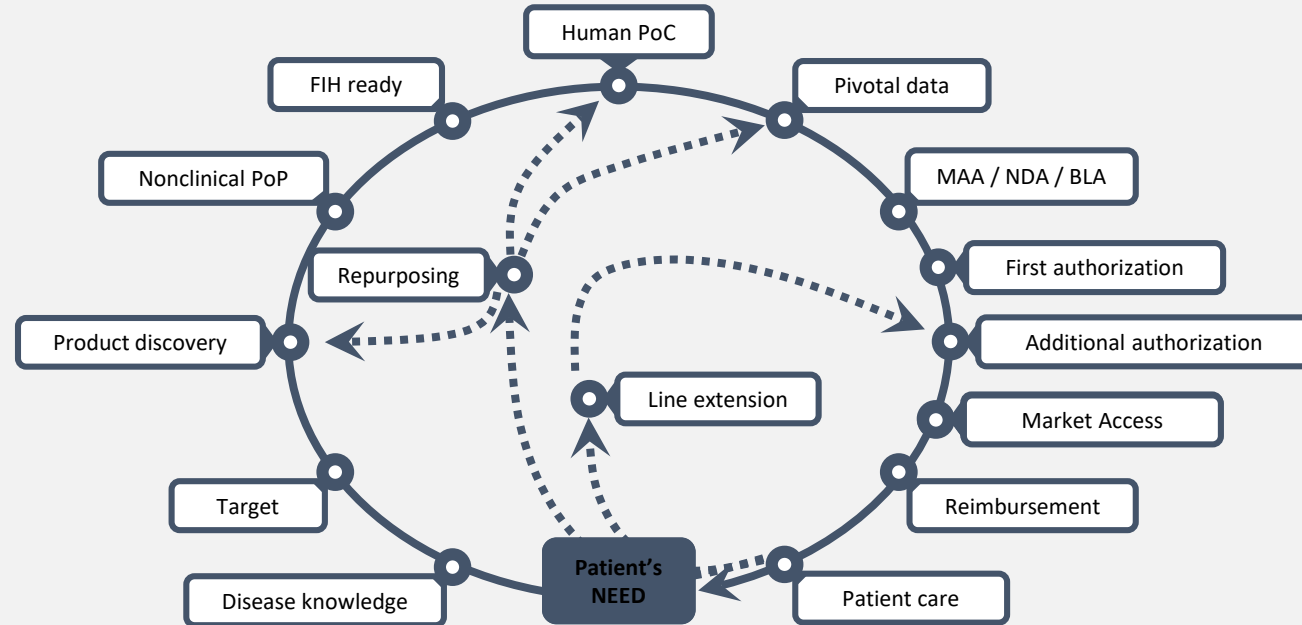
A patient focused guidebook that describes the available tools, incentives, resources and practices for developing traditional and innovative drugs/therapies for rare diseases and how to best use them. It can be used by academic, non-profit organizations, small and larger (innovative) biotechs and patient-driven drug developers.

ODDG – PROJECT AT-A-GLANCE



- 1 **Workshop** with 20 drug development experts and stakeholders
- 1 milestone-based drug development **framework (gameboard)**
- 110 **Building Blocks** (BBs) i.e. tools, incentives, resources and practices used during development
- 3 **Case Scenarios**
- Use of building blocks across the different **phases** and **milestones** of drug development
- **Roadmap & Check-lists** of “what to do” and “when to do it”

ODDG - FRAMEWORK

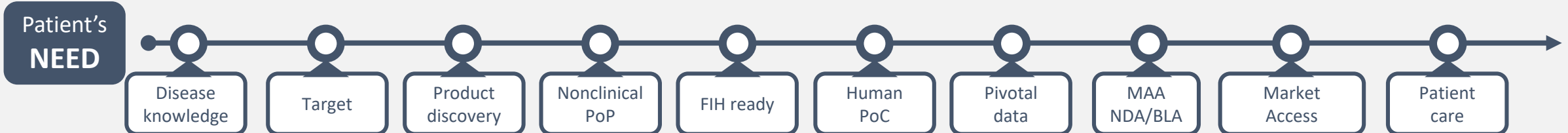


NOT SUITABLE FOR THE DEVELOPMENT OF MEDICAL DEVICES

DOES NOT TAKE INTO CONSIDERATION REPURPOSING AND LINE EXTENSION

LESS SPONSOR CENTRIC AND MORE STAKEHOLDER CENTRIC

POST-APPROVAL PHASES HAVE BEEN GROUPED AS MARKET ACCESS

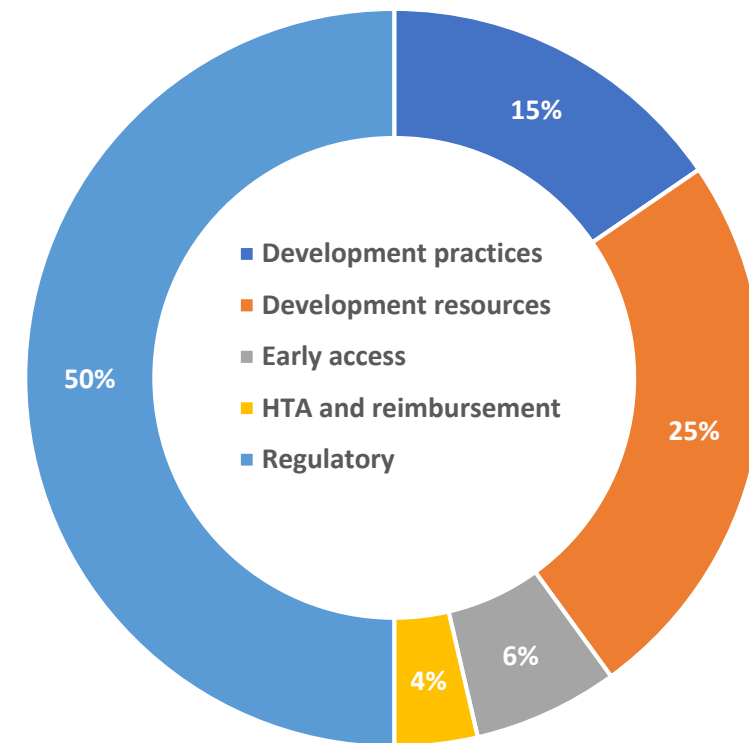


ODDG – BUILDING BLOCKS (BBs) CLASSIFICATION

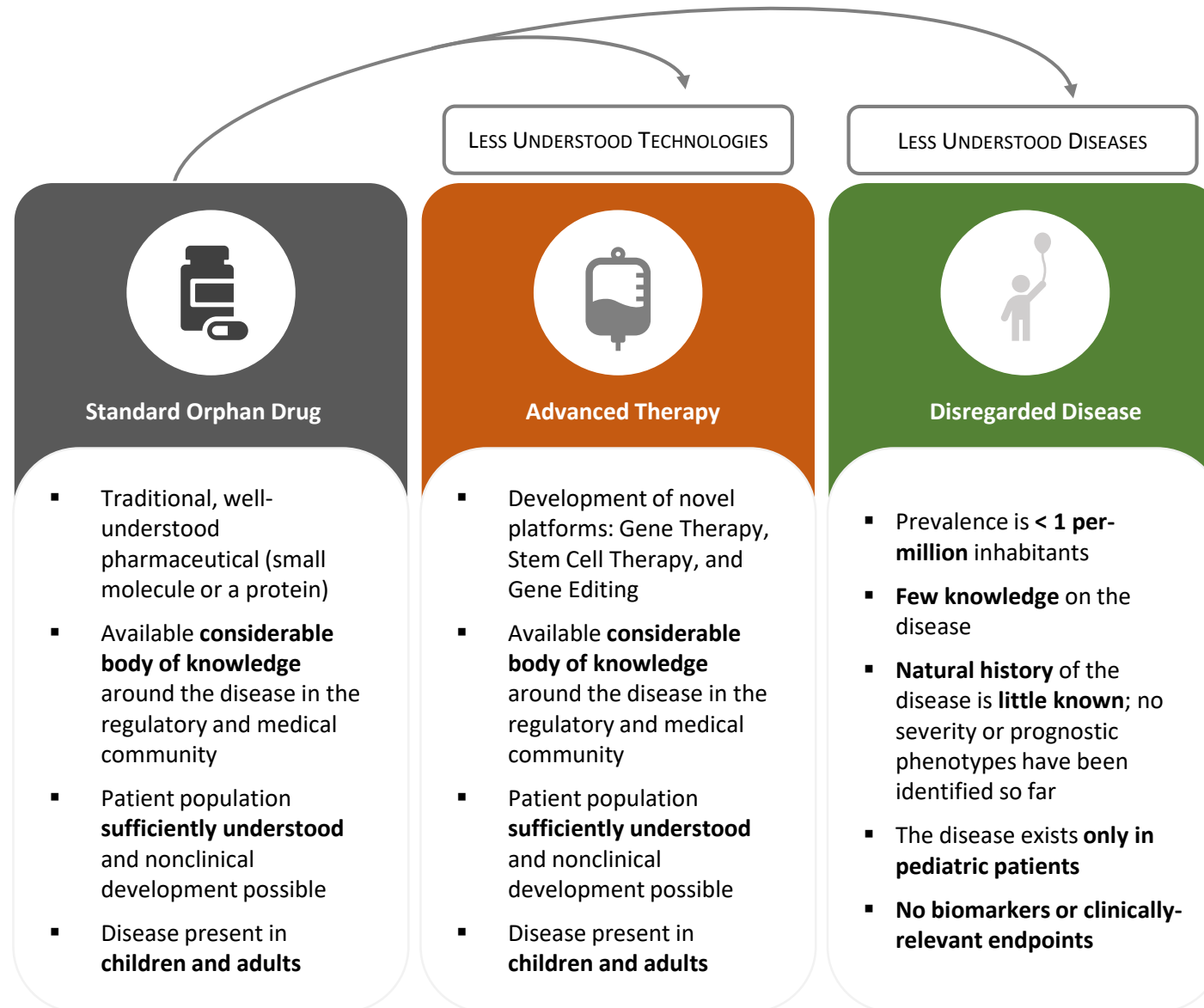
For each BB it was created a factsheet describing its relevance to rare disease drug development, availability, scope of use, output, pros and cons of usage, best time to apply, duration and costs.

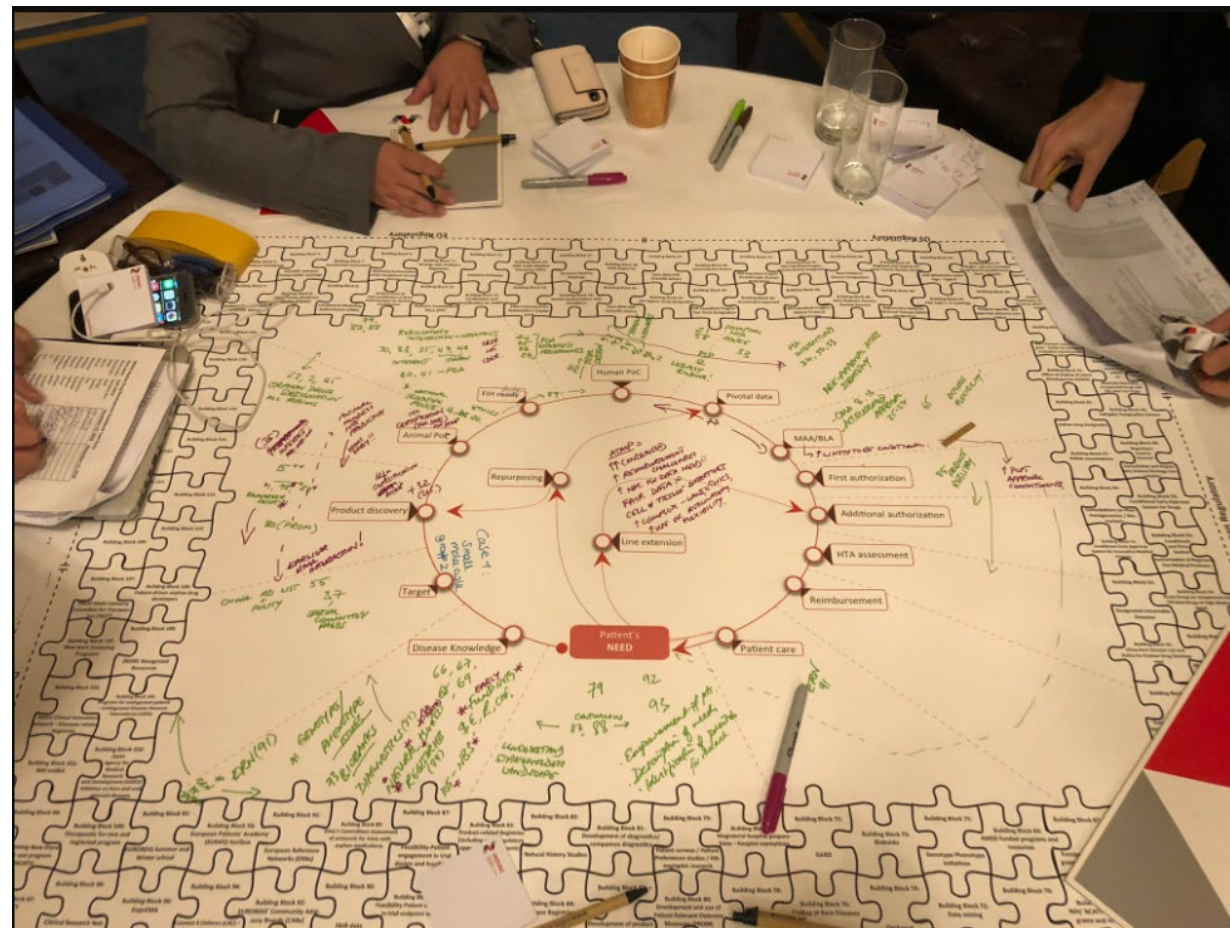
110 BBs were identified consisting of:

- **Regulatory** - pathways, designations and incentives for ODD in EU, US and Japan
- **HTA and reimbursement** - practices and procedures to support the economic value proposition and assessment, mainly focused on EU
- **Early access** - programs to enable patient treatment before regulatory license or local approval, either reimbursed or provided at no cost, according to the local regulation and practices
- **Development practices** - best-practice established by developers in the field of rare diseases, to improve orphan drug development in terms of speed, quality or efficiency
- **Development resources** - physical or practical existing accessible resource, to support drug developers in the orphan space



ODDG – CASE STUDIES







COMMENT · 20 APRIL 2020

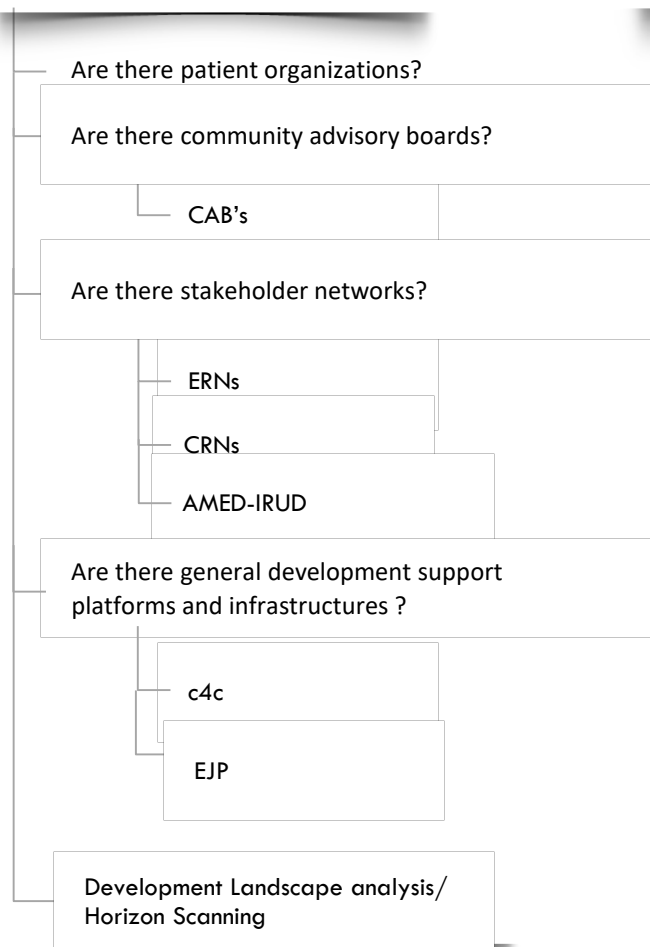
Boosting delivery of rare disease therapies: the IRDiRC Orphan Drug Development Guidebook

The International Rare Diseases Research Consortium (IRDiRC) has created a Guidebook to facilitate drug development for rare diseases by organizing available tools into a standardized framework.

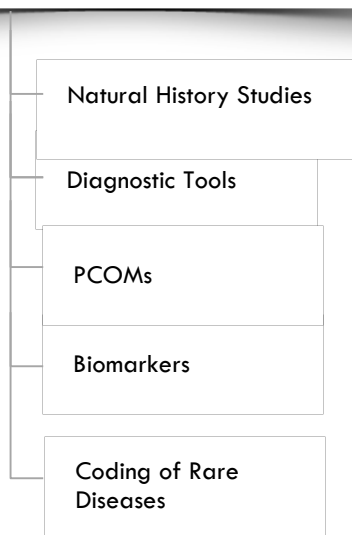
Anneliene Hechtelt Jonker, Virginie Hivert, Michela Gabaldo, Liliana Batista, Daniel O'Connor, Annemieke Aartsma-Rus, Simon Day, Ken Sakushima & **Diego Ardigo** 

START

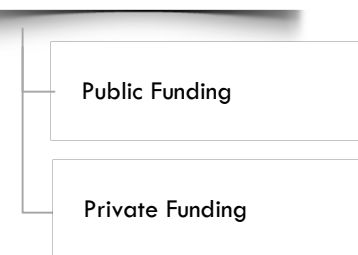
STAKEHOLDERS MAPPING



AVAILABLE INFORMATION ON THE DISEASE



FINANCIAL RESOURCES



TARGET PATIENT VALUE PROFILE

KEY TAKE AWAYS:

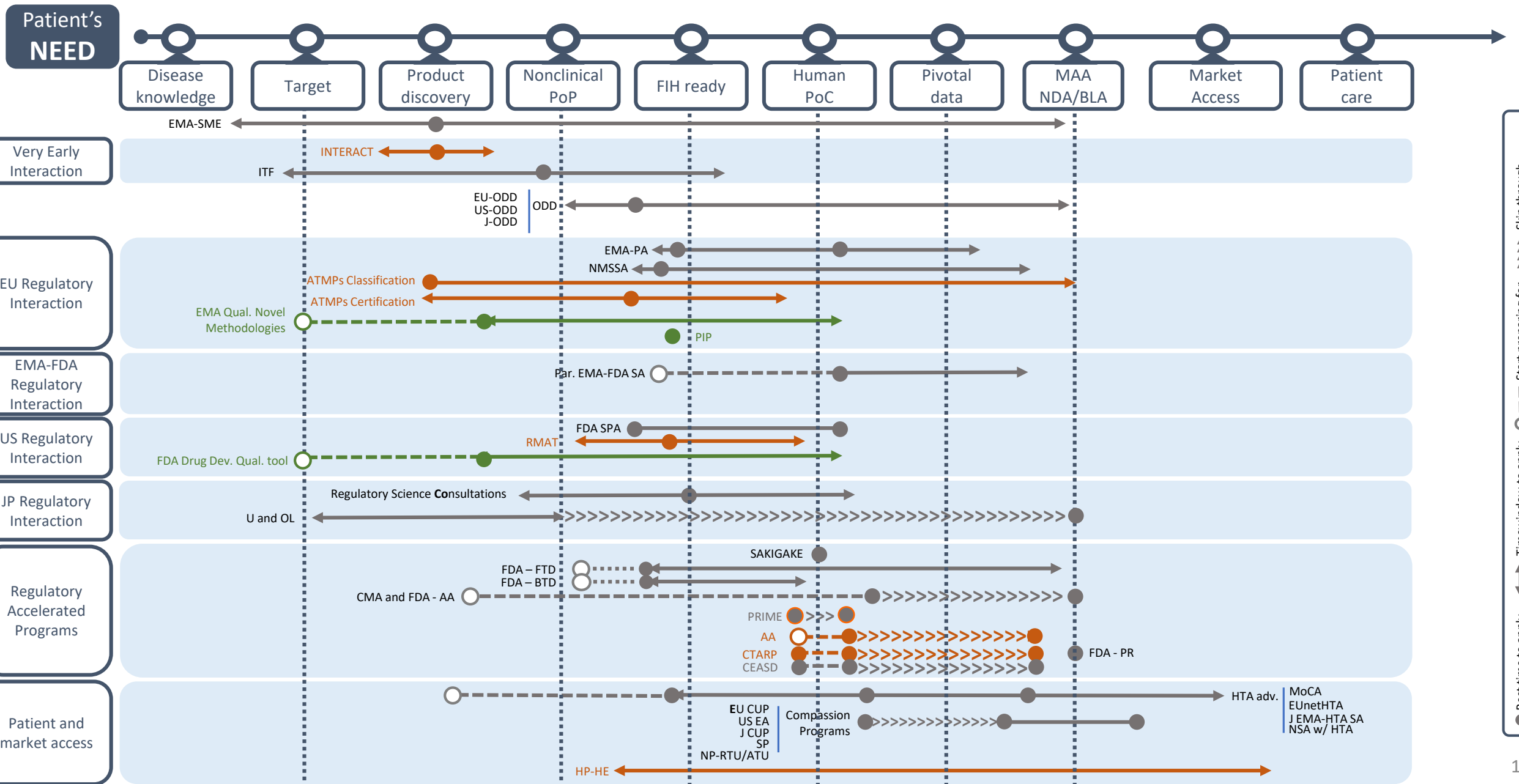
- Missing info on the disease need to be generated ASAP
- If not pre-existing, a solid stakeholder network has to be created
- Stakeholder engagement is a constant activity throughout development

ODDG – REGULATORY & ACCESS

STANDARD ORPHAN DRUG

DISREGARDED DISEASE

ADVANCED THERAPY



ORPHAN DRUG DEVELOPMENT GUIDEBOOK — FREELY ACCESSIBLE ON IRDiRC WEBSITE



Search this website

ABOUT

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Orphan Drug Development Guidebook Materials

Table of Contents [-]

NEW! [ODDG tutorial](#) & [Interactive Section](#) are available (see below)

Introduction

For the creation of the guidebook, different types of documents were created:

1. A list of all Building Blocks
2. A fact sheet form for each Building Block
3. A power point presentation of the Guidebook, including different figures
4. A tutorial, explaining the Guidebook in detail

Building Block List

A list of building blocks (BBs) (tools, incentives, resources, initiatives, and practices) either specific to rare diseases, or more often used in rare diseases but also available for common diseases were collected and analyzed. The full list of BBs available in Europe, United States of America and Japan is shown below (please note that this list might not be complete and it will be regularly updated).

List of BBs

Building Block Forms

Based on a systematic review of websites, literature search and the expertise of the Taskforce members, we have created fact sheets describing each BB by including key information on its use, duration, pros and cons, and among other aspects the TF's advice on the best time to use the BB. All fact sheets can be found below. You can access to the BB forms by type or by geographical scope.

By type

- BB Forms - Development practices
- BB Forms - Development resources
- BB Forms - Early access
- BB Forms - HTA and reimbursement
- BB Forms - Regulatory

By geographical scope

- European BB Forms
- United States BB Forms
- Japan BB Forms
- International BB Forms

Guidebook Presentation

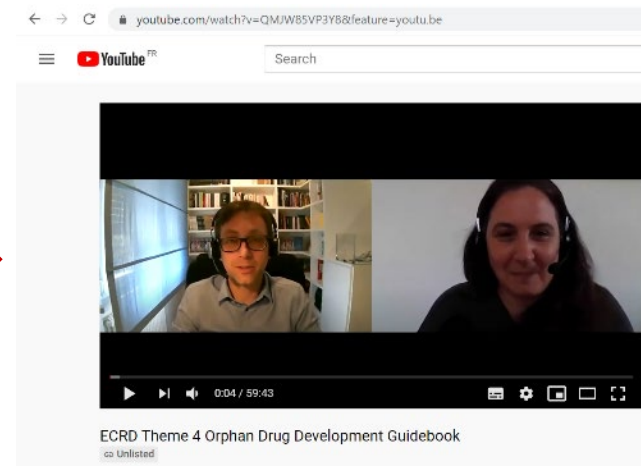
A more comprehensive representation of the guidebook and its methodology has also been created in following PDF file:

[IRDIRC_ODDG_Guidebook-Methodology-Representation](#)

Publication in Nature Reviews Drug Discovery

[Boosting delivery of rare disease therapies: the IRDiRC Orphan Drug Development Guidebook](#)

Including a video tutorial & an interactive website

youtube.com/watch?v=QMjW85VP3Y8&feature=youtu.be

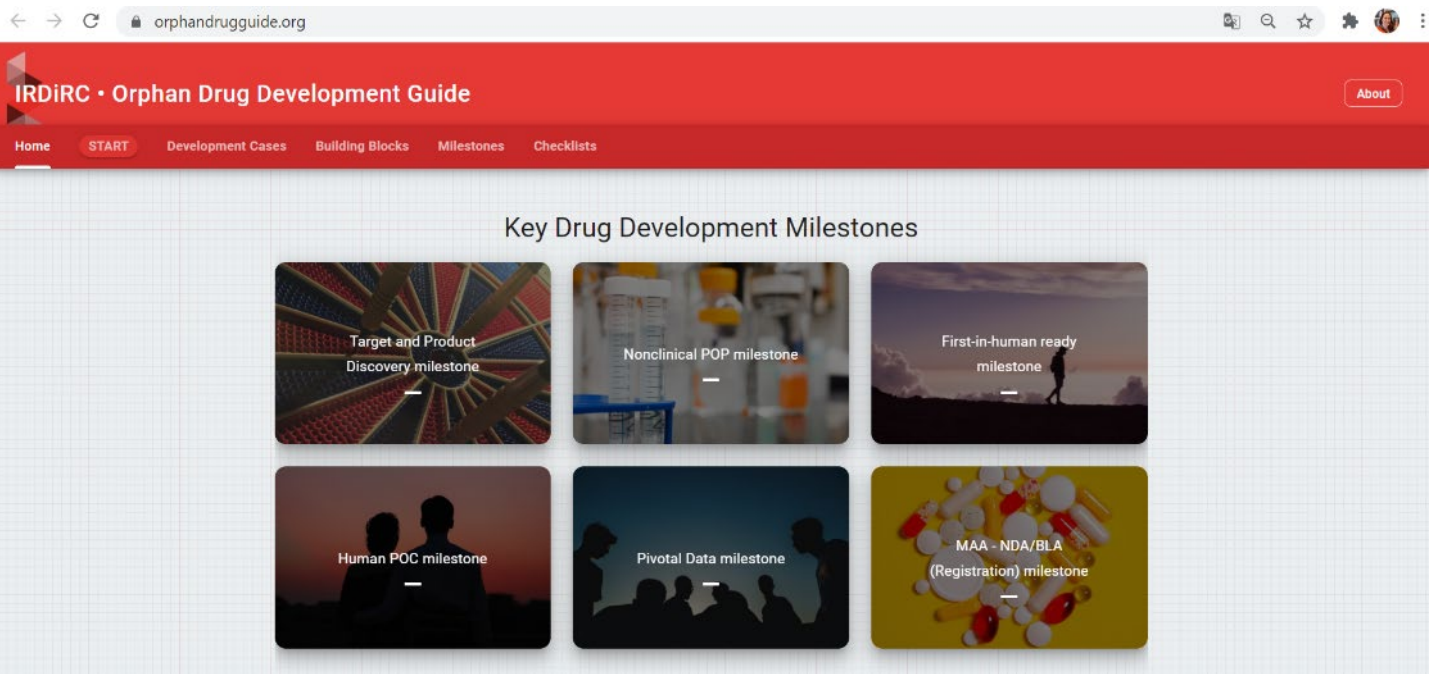
YouTube

Search

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ECRD Theme 4 Orphan Drug Development Guidebook

Unlisted



orphandrugguide.org

IRDIRC • Orphan Drug Development Guide

About

Home | START | Development Cases | Building Blocks | Milestones | Checklists

Key Drug Development Milestones

- Target and Product Discovery milestone
- Nonclinical POP milestone
- First-in-human ready milestone
- Human POC milestone
- Pivotal Data milestone
- MAA - NDA/BLA (Registration) milestone

HOW TO USE THE ORPHAN DRUG DEVELOPMENT GUIDEBOOK IN YOUR OWN SETTING

- The materials produced and displayed onto the interactive website can serve different purposes:
 - **Expert platform resource**: from accessing information on a single Building Block to gaining knowledge on the RD therapeutic development landscape as a whole
 - **Educational purposes**: the content and the Gameboard/framework can be used for trainings
 - **Strategic development planning**: the Gameboard/framework and the Building Blocks can be used to elicit a real case scenario for reflecting on a real development plan
 - **Long term sustainability** ensured by transitioning of the ODDG to EJP-RD [‘Innovation & Clinical Trials Support’](#)