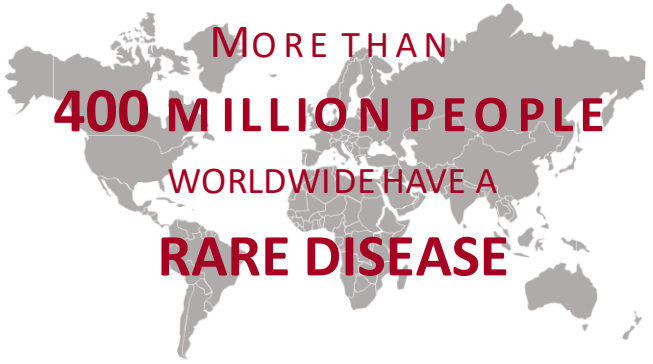
IRDiRC

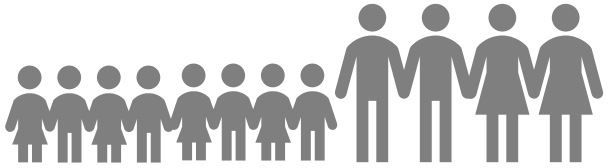
INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

ORPHAN DRUG DEVELOPMENT GUIDEBOOK

Rare Diseases landscape and Orphan Drug Development Trends



More than
400 million people
worldwide have a
rare disease



70-80% are Children

6,000 – 8,000
Diseases are classified as Rare

~80% considered **Ultra-rare**
~60% are serious and disabling
~50% are life threatening

~80% of **Rare Diseases are of genetic origin**

500 Drugs have reached the market
5% of rare diseases have an approved treatment
700-800 treatments in development

World Health Organization. Background paper 6.19 Rare Diseases. (2013); Orphanet; RARE Facts. Global Genes.

TREMENDOUS UNMET MEDICAL NEED

IRDiRC's GOAL

1000 new rare disease treatments by 2027

At the current rate of drug development (40-50 new therapies developed per year), it would take **500** years to get a treatment for all these conditions! Therefore a quantum change of the present **drug development model and ecosystem** for rare diseases is needed.

Austin CP, et al., Future of Rare Diseases Research 2017-2027: An IRDiRC Perspective. Clin Transl Sci. 2018 Jan;11(1):21-27

Orphan Drug Development Guidebook

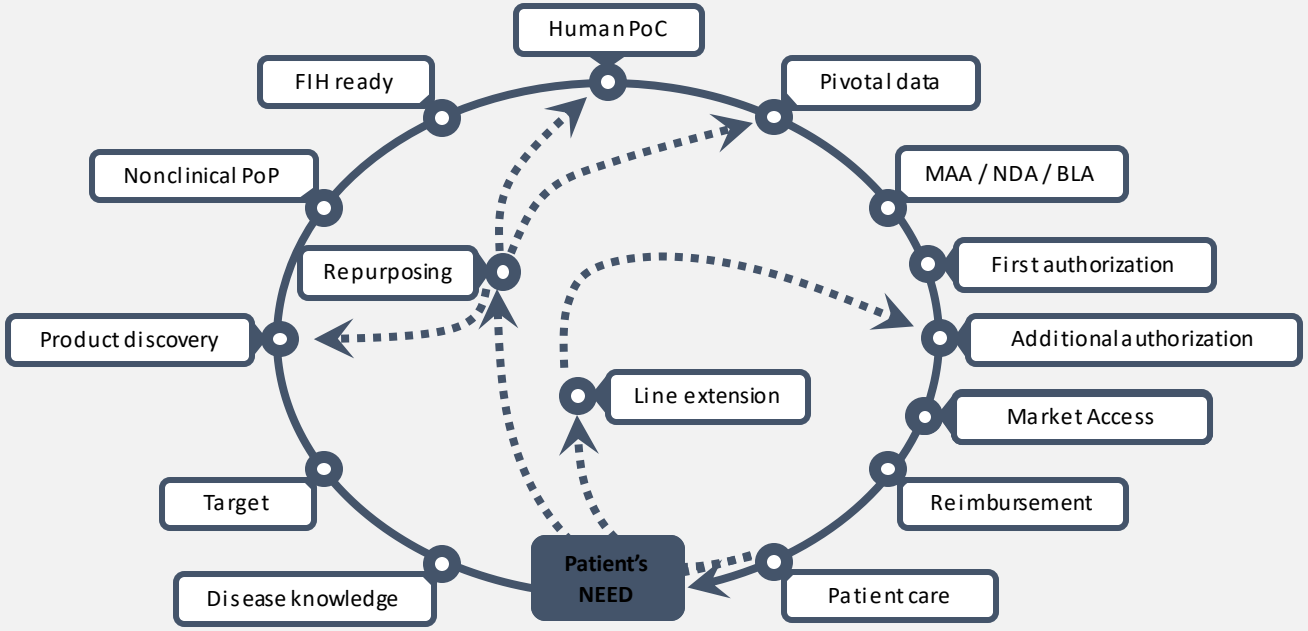
A patient focused guidebook that describes the available tools, incentives, resources and practices for developing traditional and innovative drugs/therapies for rare diseases and how to best use them. It can be used by academic, non-profit organizations, small and larger (innovative) biotechs and patient-driven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

ODDG – PROJECT AT-A-GLANCE

- 1 Workshop with 27 drug development experts and stakeholders
- 1 milestone-based drug development framework
- 116 Building Blocks (BBs)
- 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”

TECHNICAL EXPERTS AFFILIATIONS

ODDG - FRAMEWORK

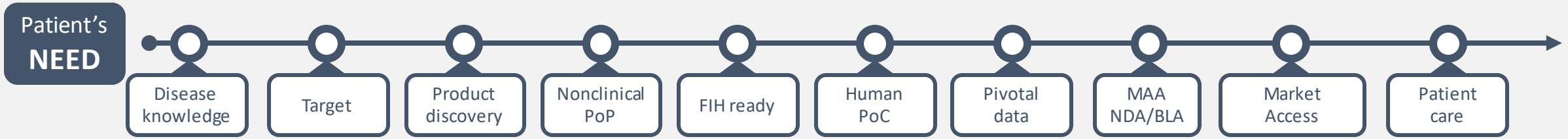


NOT SUITABLE FOR THE DEVELOPMENT OF MEDICAL DEVICES

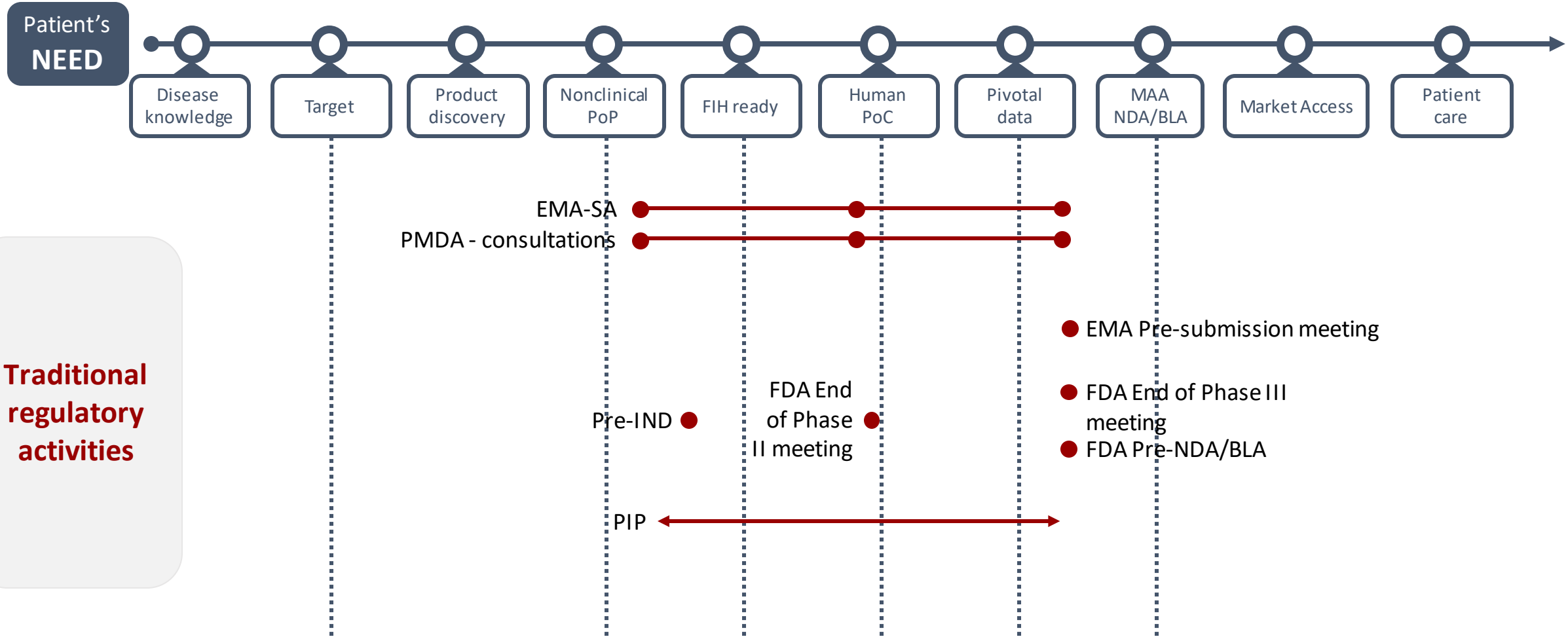
DOES NOT TAKE INTO CONSIDERATION REPURPOSING AND LINE EXTENSION

LESS SPONSOR CENTRIC AND MORE STAKEHOLDER CENTRIC

POST-APPROVAL PHASES HAVE BEEN GROUPED AS MARKET ACCESS



ODDG- FRAMEWORK



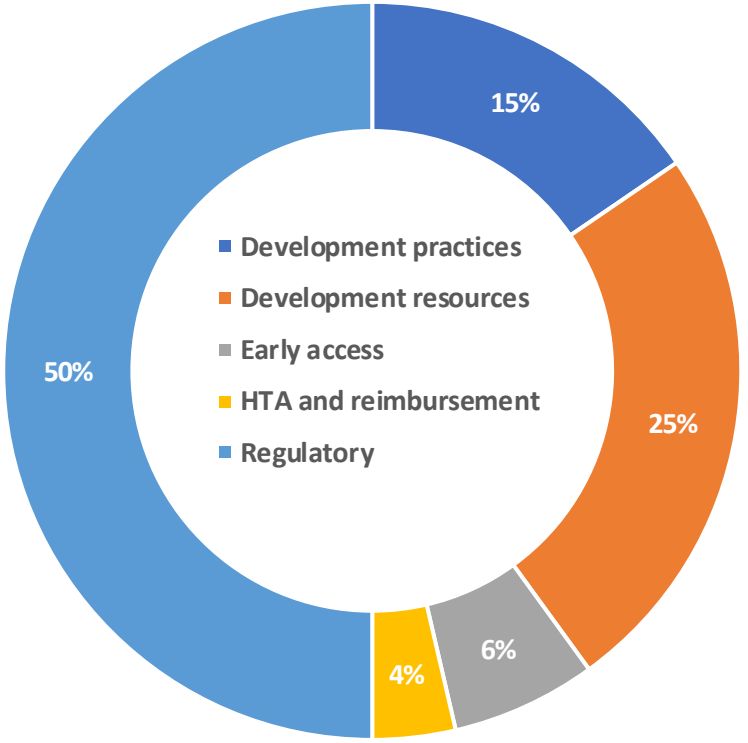
Traditional regulatory activities

ODDG – BUILDING BLOCKS (BBS) CLASSIFICATION

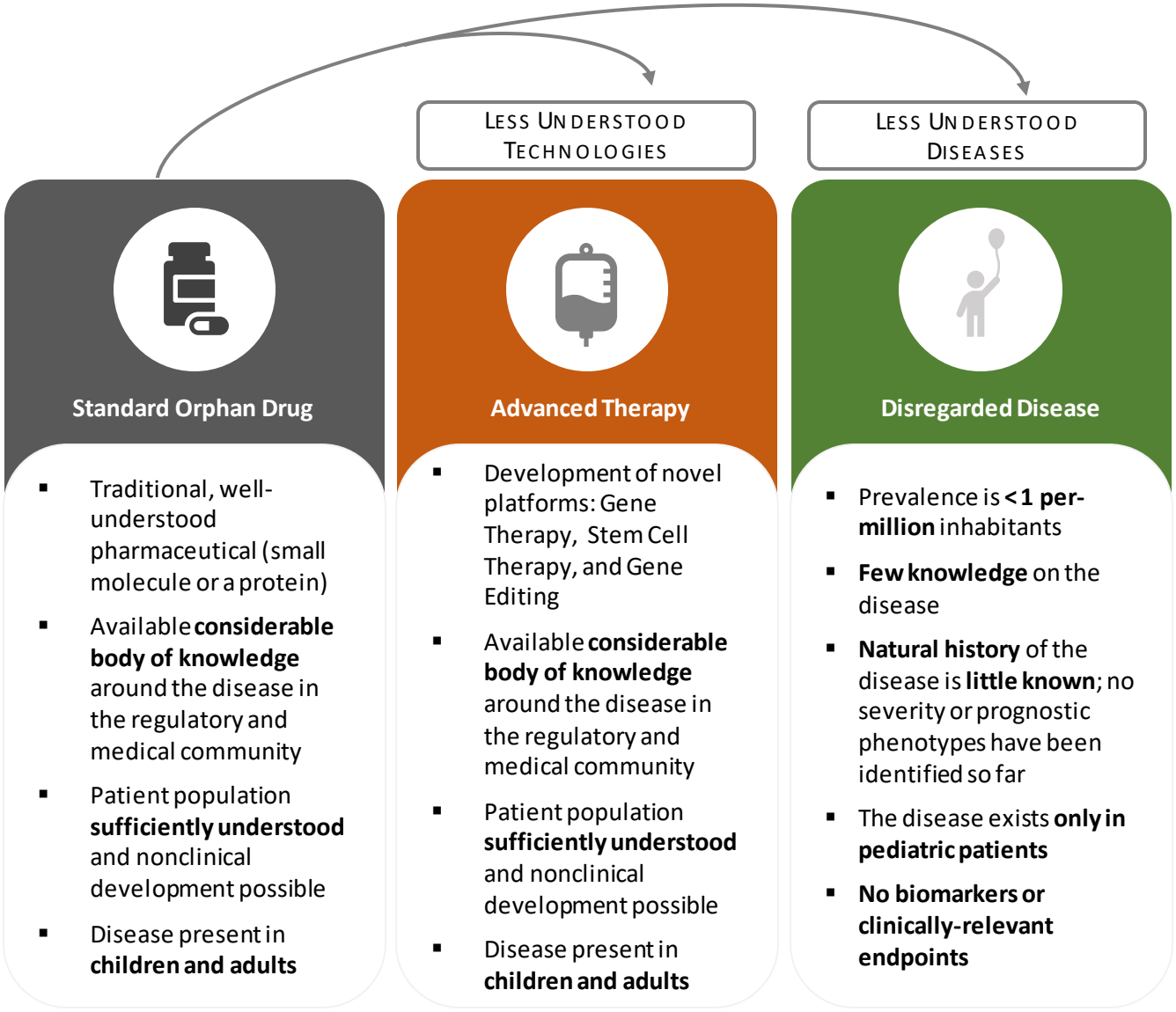
For each BB it was created a factsheet describing its relevance to rare disease drug development, availability, scope of use, output, pros and cons of usage, best time to apply, duration and costs ().

110 BBs were identified consisting of:

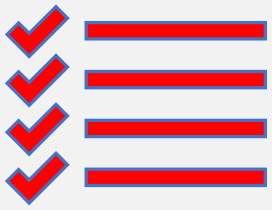
- **Regulatory** - pathways, designations and incentives for ODD in EU, US and Japan
- **HTA and reimbursement** - practices and procedures to support the economic value proposition and assessment, mainly focused on EU
- **Early access** - programs to enable patient treatment before regulatory license 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



ODDG – How do you start the development of your product?



S
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STakeholders mapping

Available information on the disease

Financial **R**esources

Target Patient Value Profile

START

STAKEHOLDERS MAPPING

- Are there patient organizations?
- Are there community advisory boards?
  - CAB's
- Are there stakeholder networks?
  - ERNs
  - CRNs
  - AMED-IRUD
- Are there general development support platforms and infrastructures ?
  - c4c
  - EJP
- Development Landscape analysis/ Horizon Scanning

AVAILABLE INFORMATION ON THE DISEASE

- Natural History Studies
- Diagnostic Tools
- PCOMs
- Biomarkers
- Coding of Rare Diseases

FINANCIAL RESOURCES

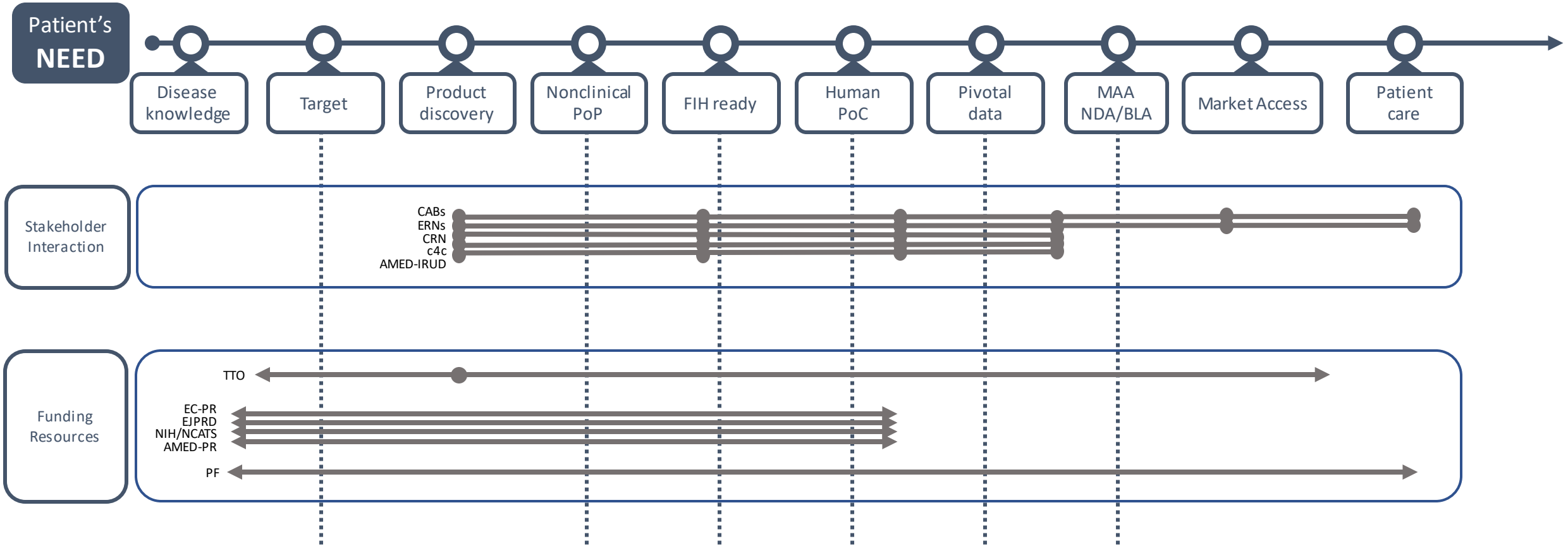
- Public Funding
- Private Funding

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

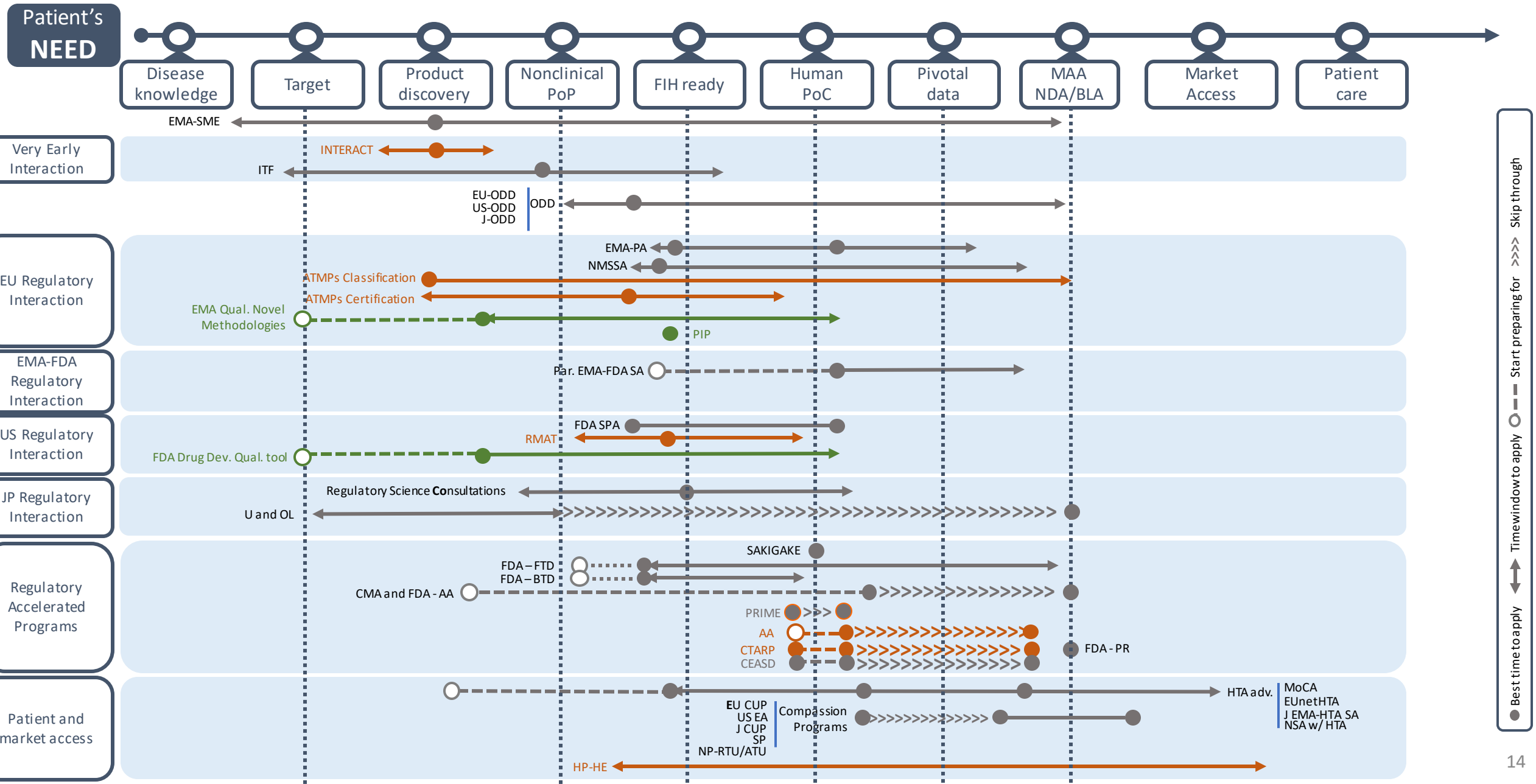
ODDG – START CHECKLIST



ODDG – REGULATORY & ACCESS

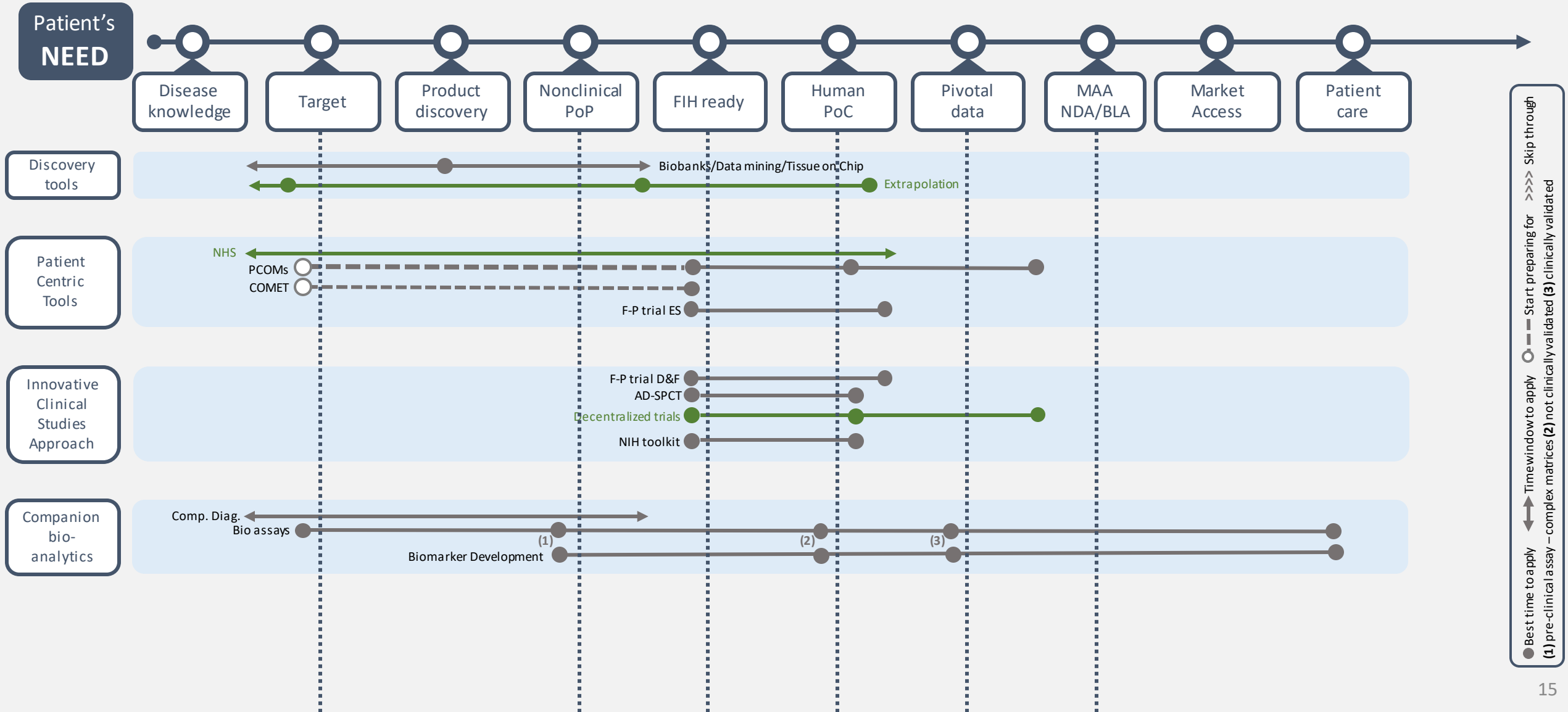
STANDARD ORPHAN DRUG DISREGARDED DISEASE ADVANCED THERAPY

IRDiRC INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM



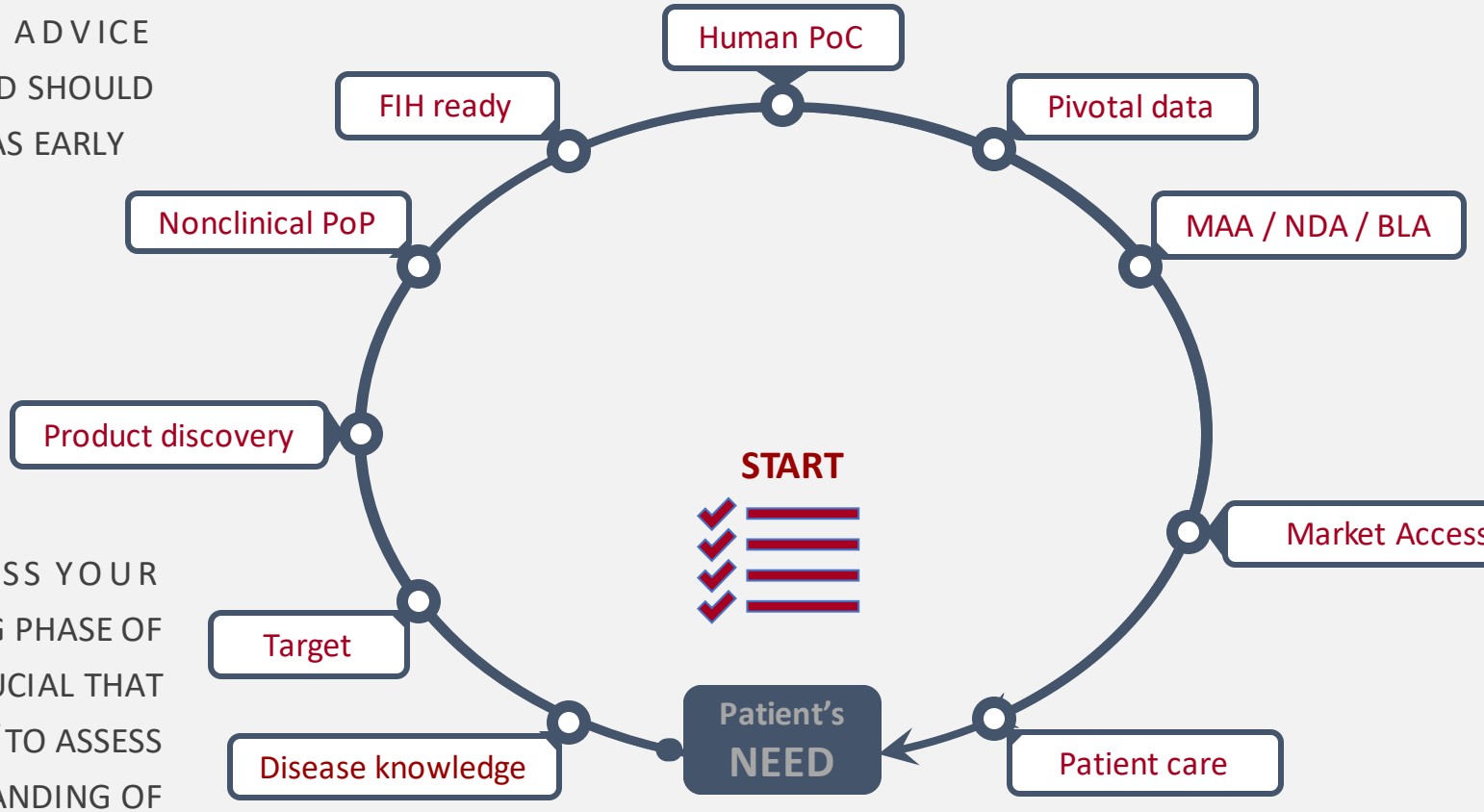
ODDG – Development Resources and Practices

Standard Orphan Drug | **Advanced Therapy** | **Disregarded Disease**



ODDG – Take Home Messages

Regulatory advice is essential and should be requested as early as possible.



Consider early on a strategy to apply for regulatory acceleration programs

Keep in mind an early interaction with HTA or payers is important

Regardless your starting phase of development it is crucial that you begin with START to assess the level of understanding of the disease

Starts with Patient's NEED rather than Idea

Ends with Patient's NEED rather than Patient Care