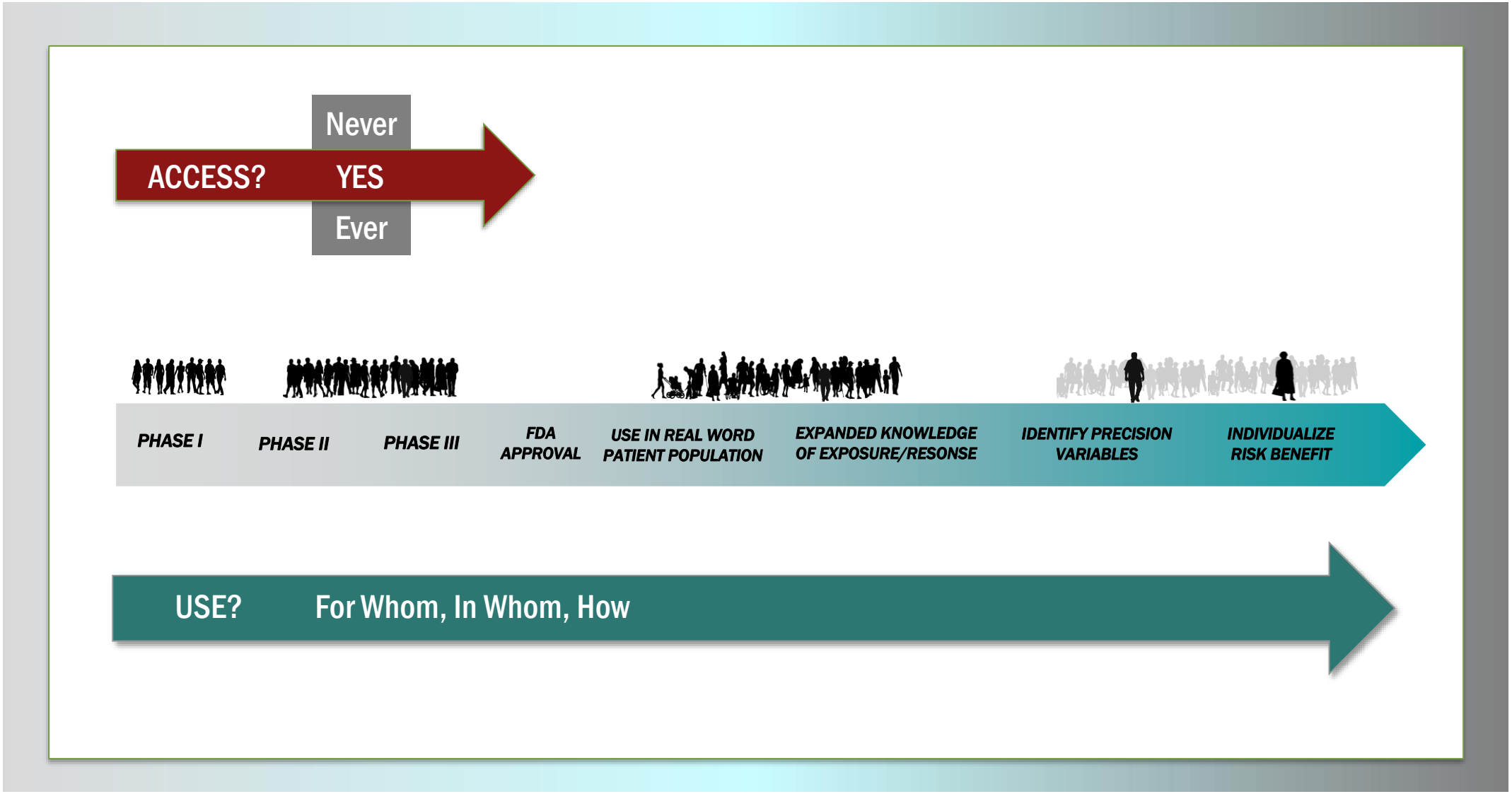
FDA OCP/MCERSI Workshop:

Clinical Pharmacology Guidances Advancing Drug Development and Regulatory Assessment: Role and Opportunities

May 8-9, 2024

U.S. FOOD & DRUG ADMINISTRATION

Patient-Centered Regulatory Decision Making



Is Pharmaceutical R&D in Crisis?

Better than the Beatles

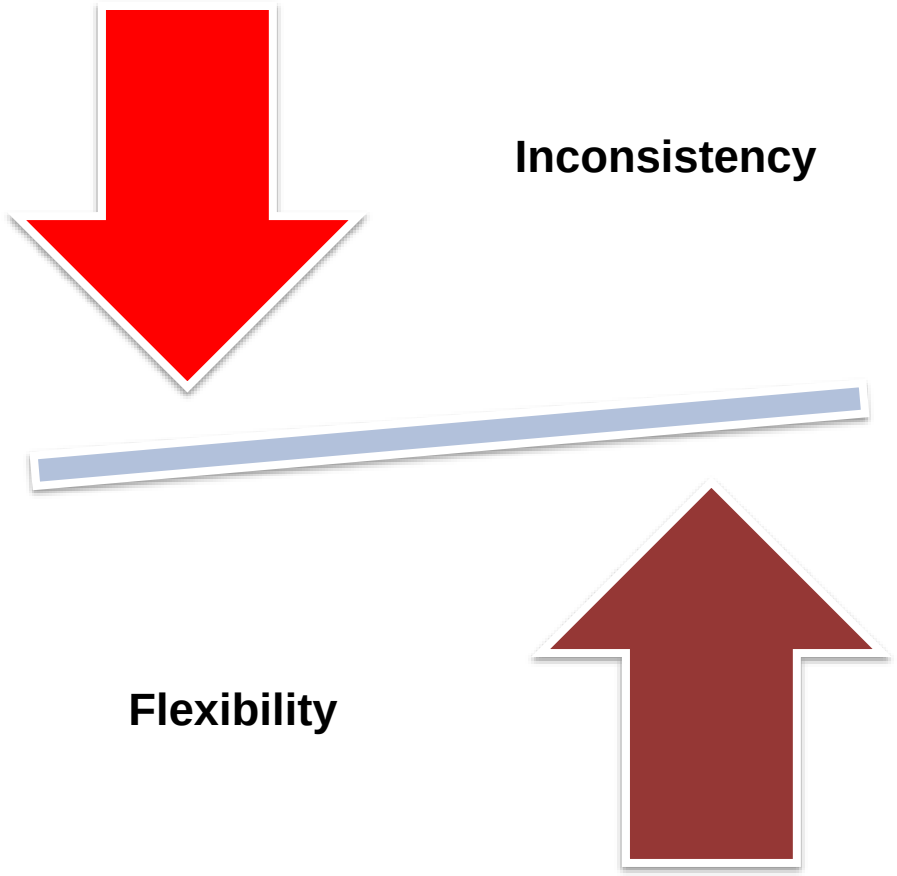
Basic Research / Brute Force

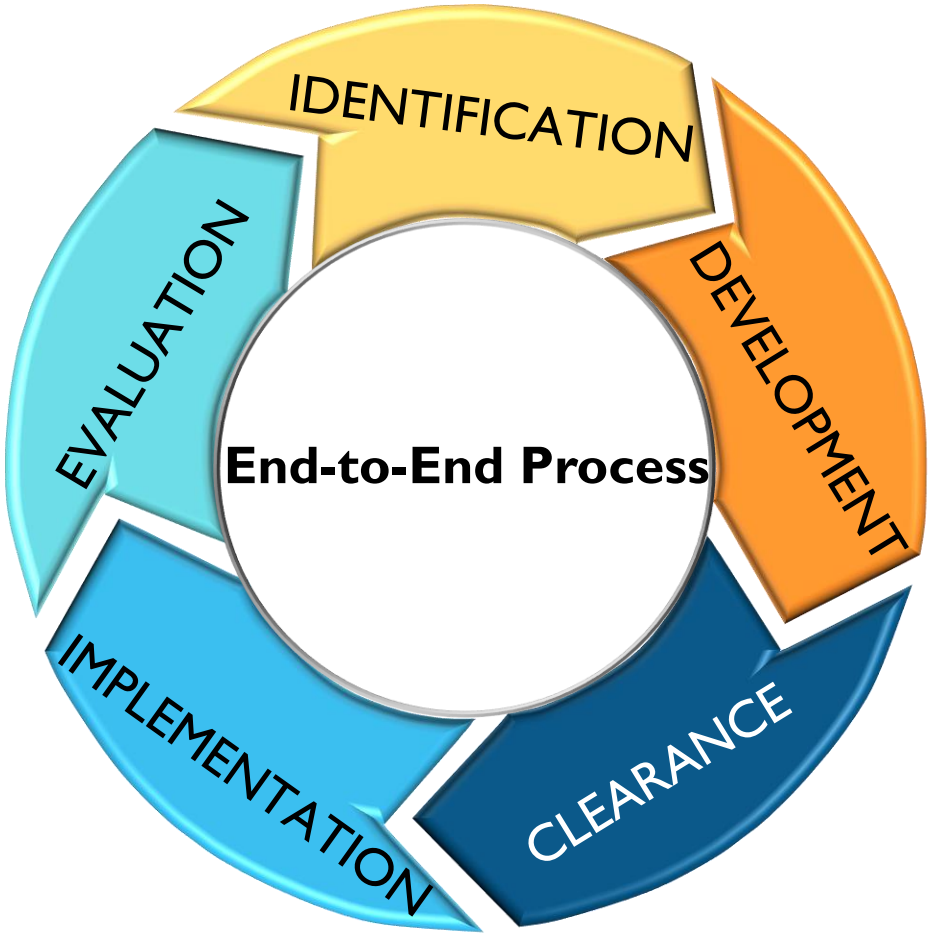
Throw Money at It

Cautious Regulator

Scannell et al. 2012 [PMID 22378269]

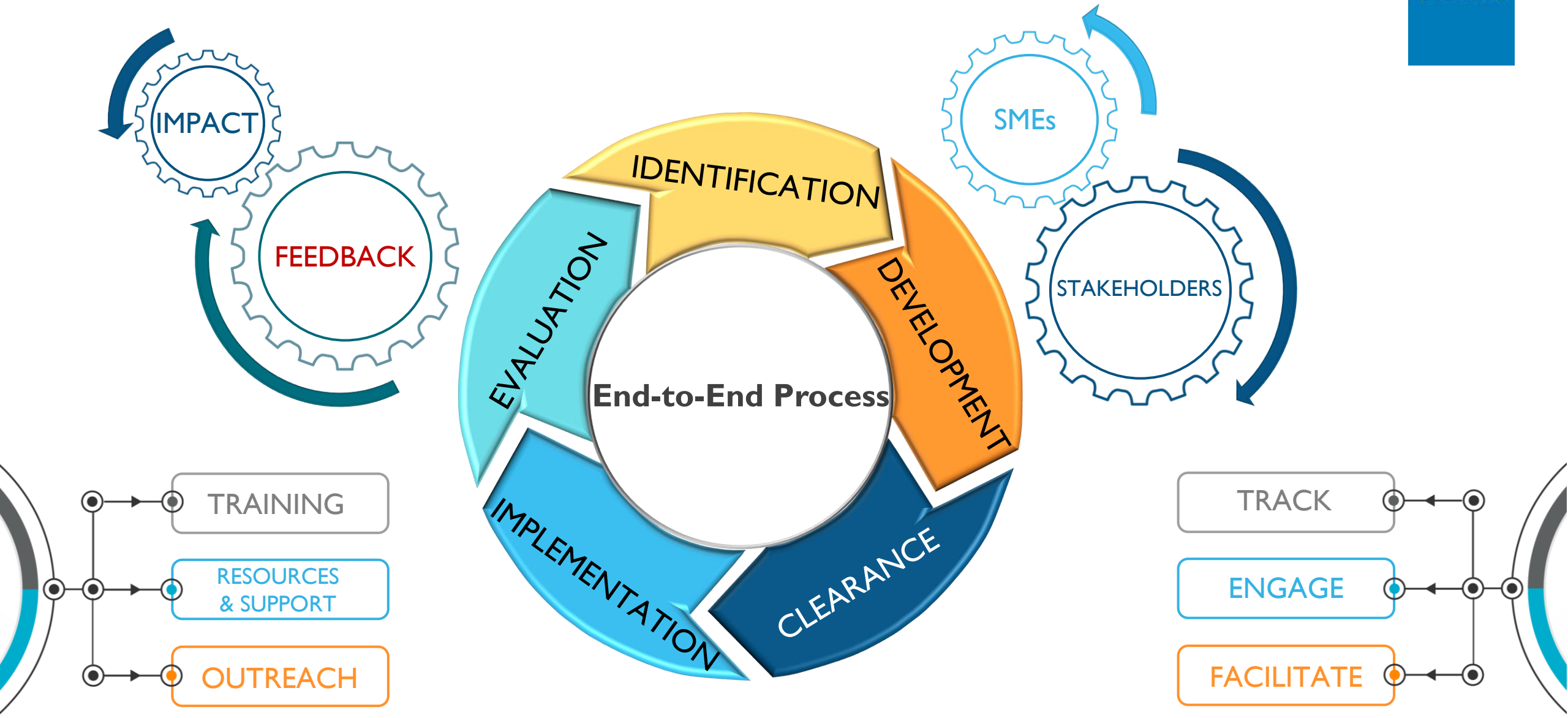
Guidance & Policy: Best Practices



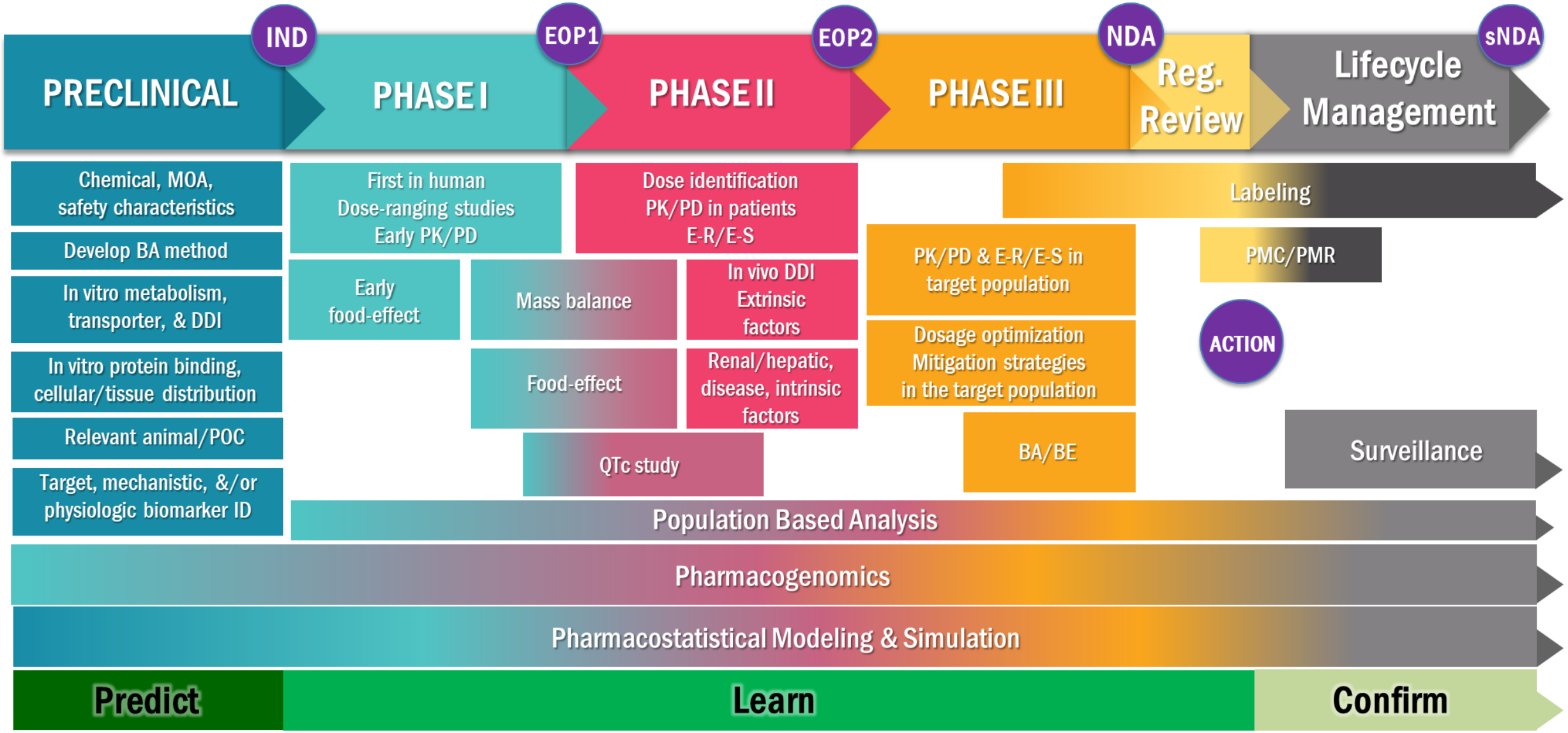


https://www.fda.gov/regulatoryinformation/guidances/#about

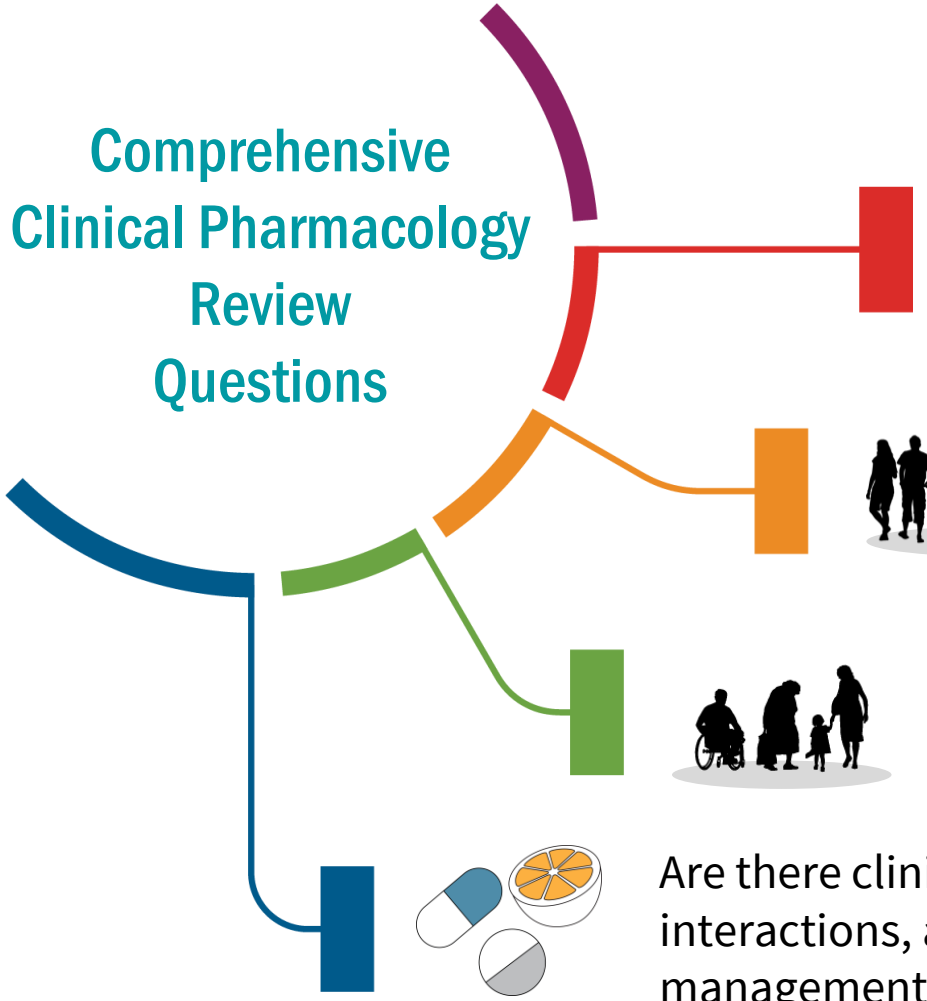
Guidance & Policy: A Community Effort

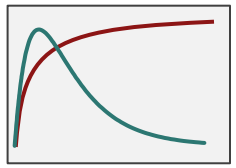


“Classical” Clinical Pharmacology



Clinical Pharmacology: Key Questions





To what extent does the available clinical pharmacology information provide pivotal or supportive evidence of effectiveness?

Is the proposed dosing regimen appropriate for the general patient population for which the indication is being sought?

Is an alternative dosing regimen or management strategy required for subpopulations based on intrinsic factors?

Are there clinically relevant food-drug or drug-drug interactions, and what is the appropriate management strategy?

Important Trends

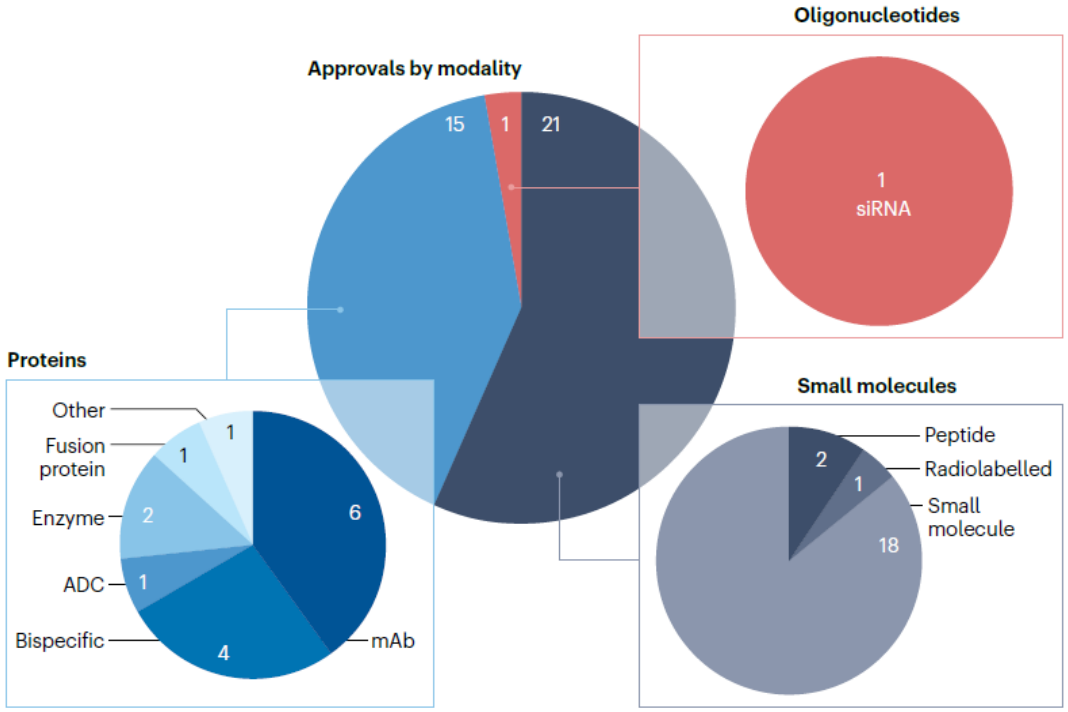
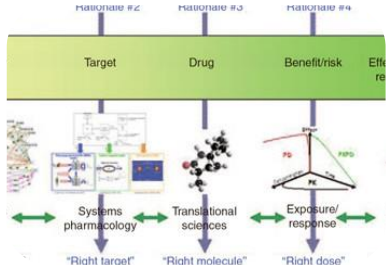
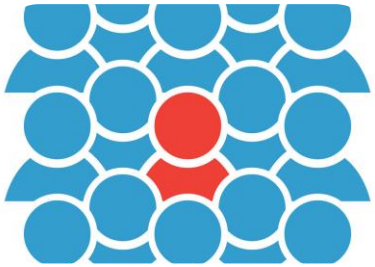


Fig. 3 | CDER approvals by modality. Small molecules, including peptides of up to 40 amino acids in length, and oligonucleotides are approved as new molecular entities (NMEs). Protein-based candidates are approved through biologics license applications (BLAs). ADC, antibody–drug conjugate; mAb, monoclonal antibody; siRNA, small interfering RNA. Source: *Nature Reviews Drug Discovery*.

DDT Qualification



MIDD/CID



Rare Diseases

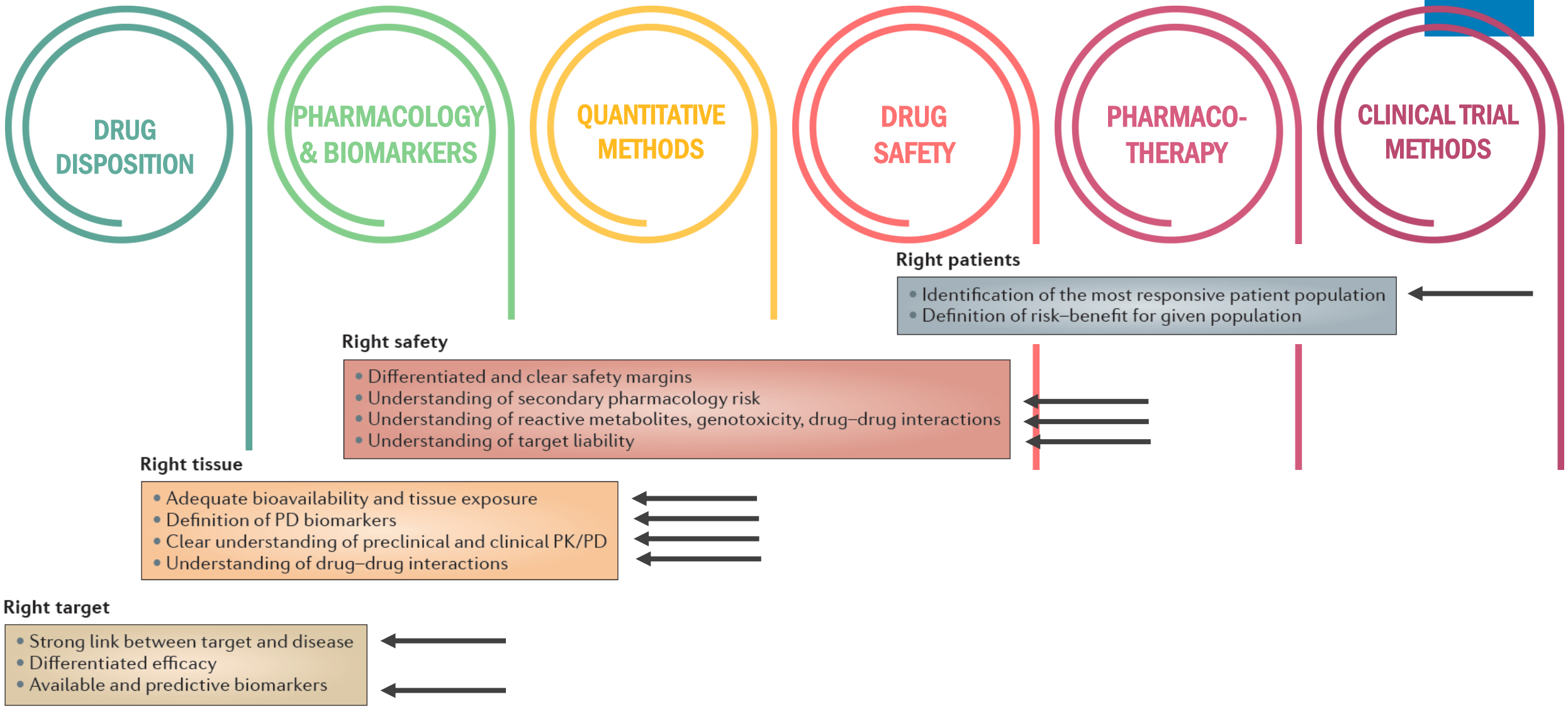
Precision Medicine

Patient Voice

RWE

Mullard NRDD 2023

A Translational Approach



Cook 2014 [PMID 24833294]

Our Charge

TRIAL PLANNING

- Patient Selection
- Dose Selection
- Response Measure Selection
- Trial Duration

INTEGRATION & REFINEMENT

Data, Information, Knowledge

DECISION SCIENCE

- Development Trajectory
- Regulatory Action

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