抗がん剤の新たな治験デザイン

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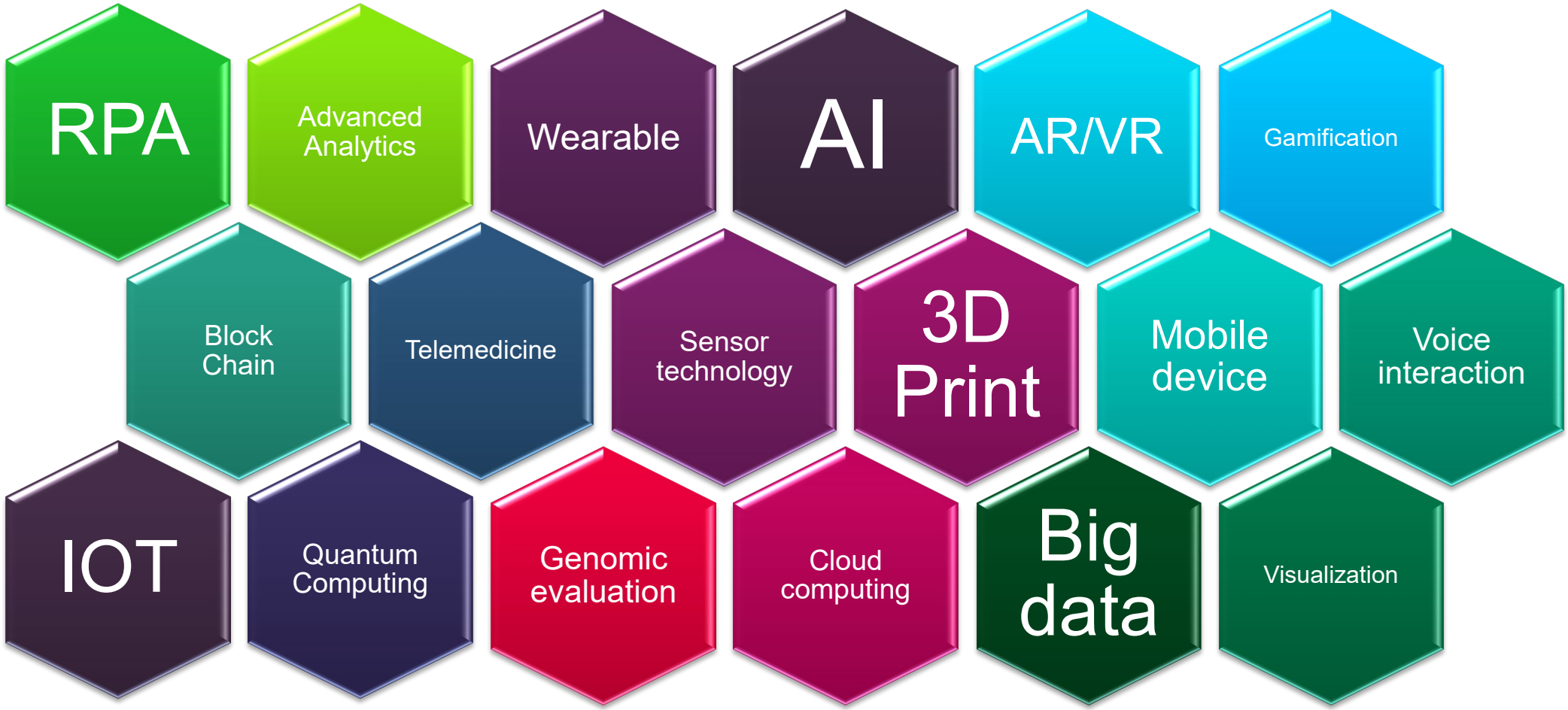
Evolution of technology provides reimagine Clinical Trials.



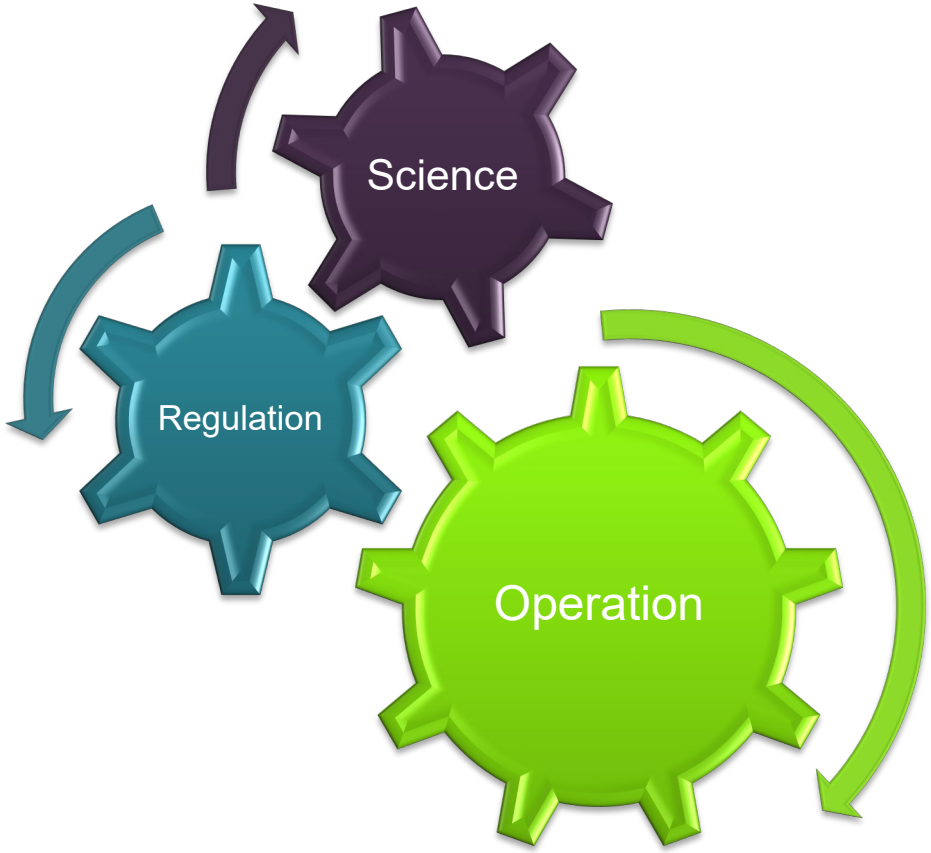
臨床試験で得られるもの（マクロ的にデータの解析を行う）

有効性と安全性評価

患者さん一例一例に対して処方

“新しい”タイプの治験のKeyword

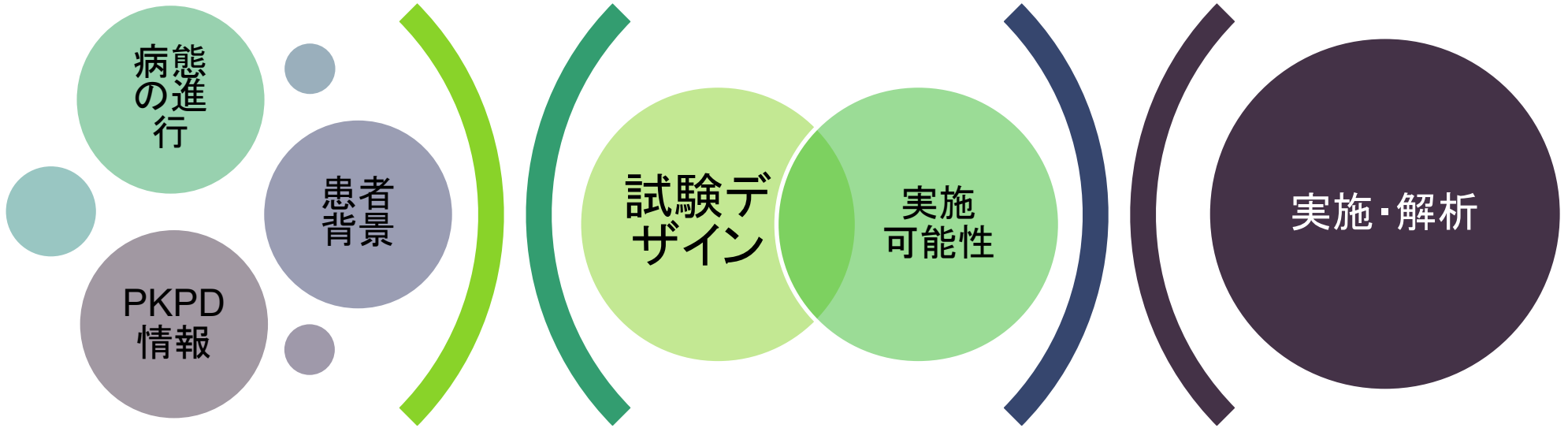
- Multi Regional Clinical Trial： Global trial
- Model Informed Drug Discovery and Development （MID3）： Pharmacometrics approach
- Complex study design: Master protocol
  - Basket trial
  - Umbrella trial
  - Platform trial
- New Statistical Approach: Bayesian Approach, Adaptive
  - // Innovative use of external data
  - // Formal incorporation of prior knowledge
- Biomarker, Companion Diagnostics
- Decentralized Clinical trial



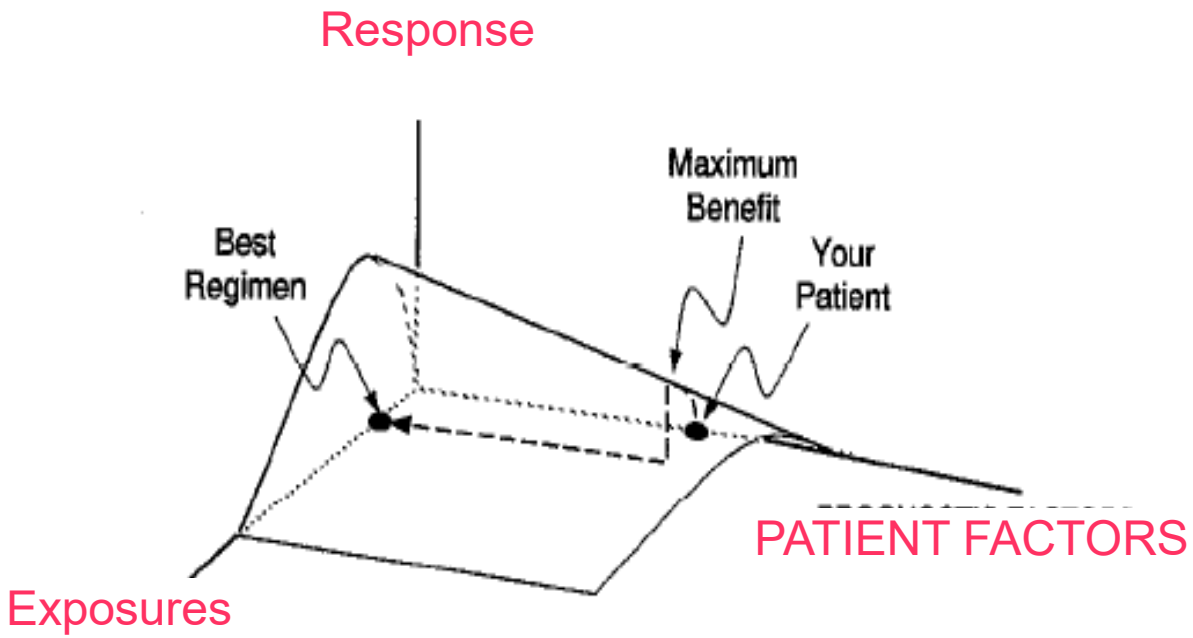
MID3を用いた“詳細な”推定を元にした試験

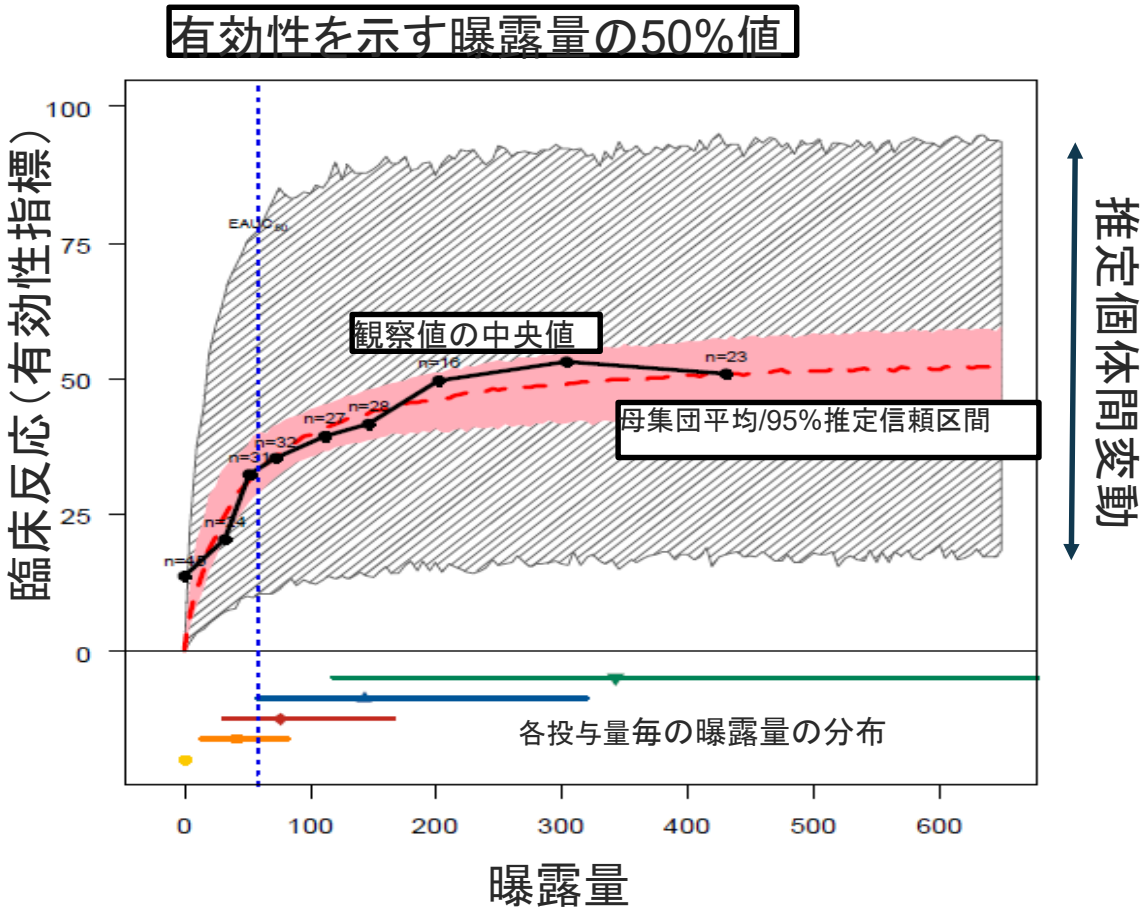
FiHの用量、相互作用試験などの用法用量をあらかじめ推定し、実施可能で評価可能な試験計画の立案と実施

臨床試験を計画、検討するために予測を行い、効率的な治験および臨床開発計画

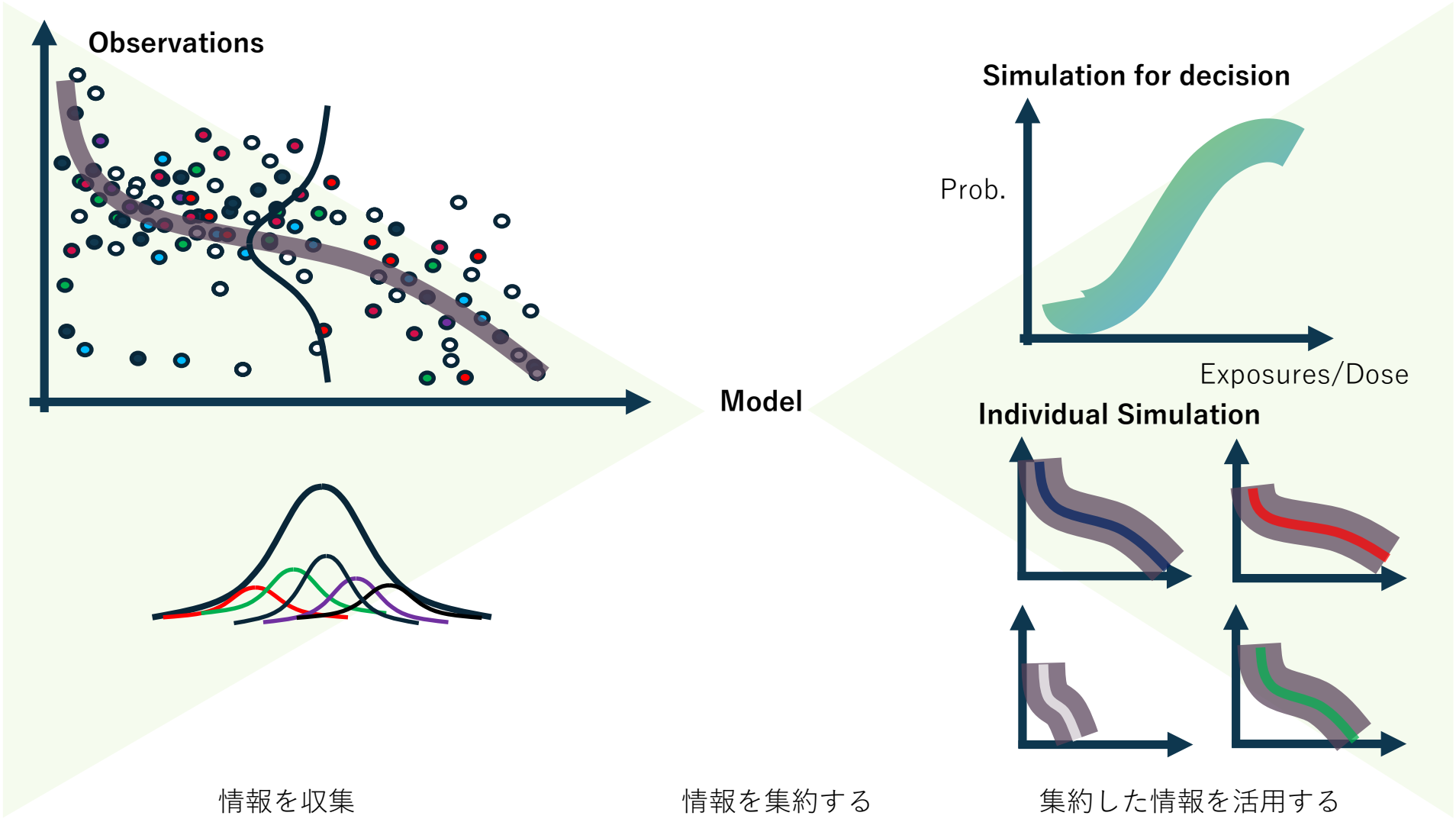


Exposure-response understanding





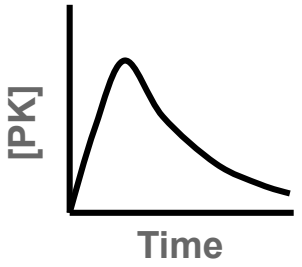
モデル解析の概念


Observations
Model
Simulation for decision
Prob.
Exposures/Dose
Individual Simulation
情報を収集
情報を集約する
集約した情報を活用する

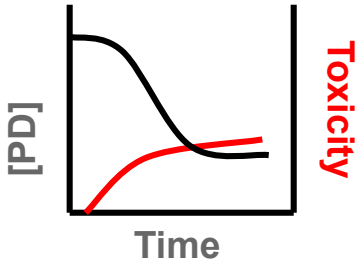
From modeling to clinical trial simulation

Drug Model

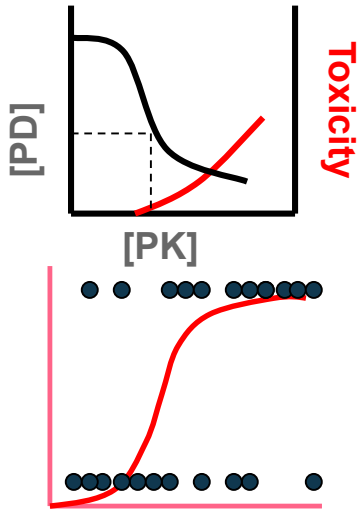
PK



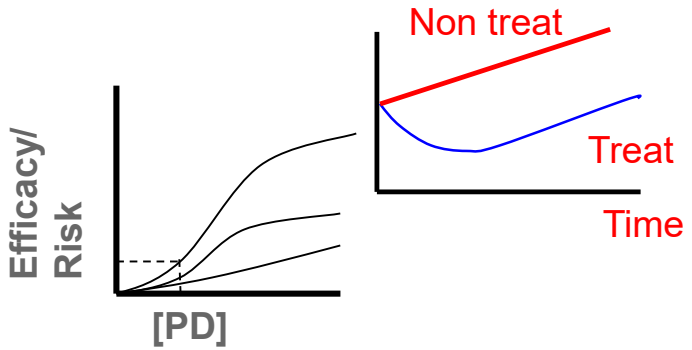
PD



PK/PD, Exp-Res



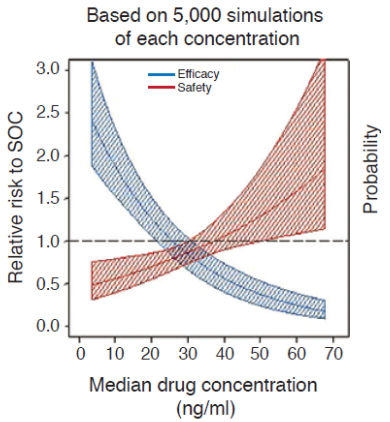
Disease Model



Trial Model

Other information

- BASELINE
- PLACEBO EFFECT
- DROP-OUT RATE
- ADHERENCE



Clinical Trial Simulation

→ Calculation of probability by repeated simulation

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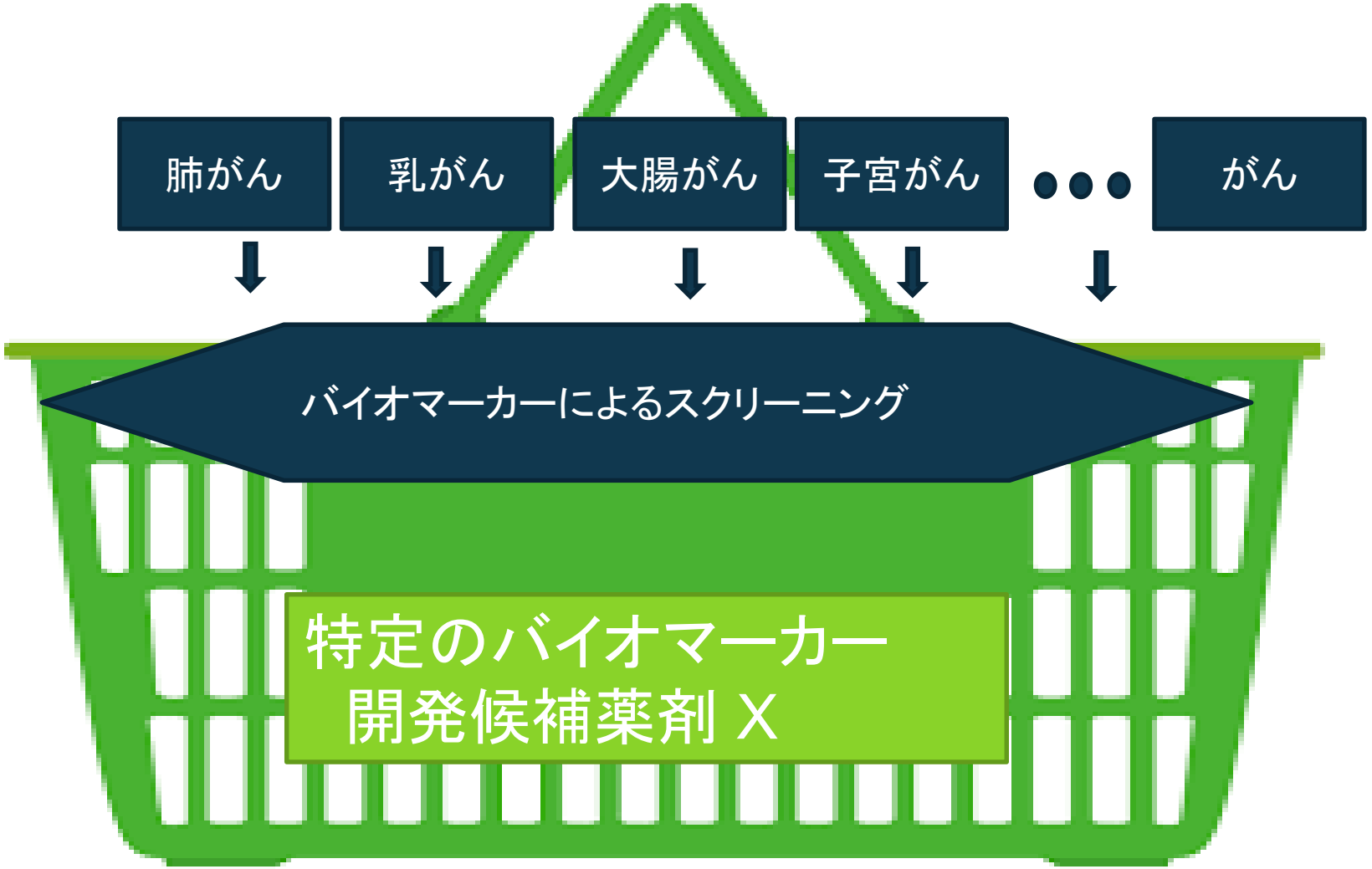
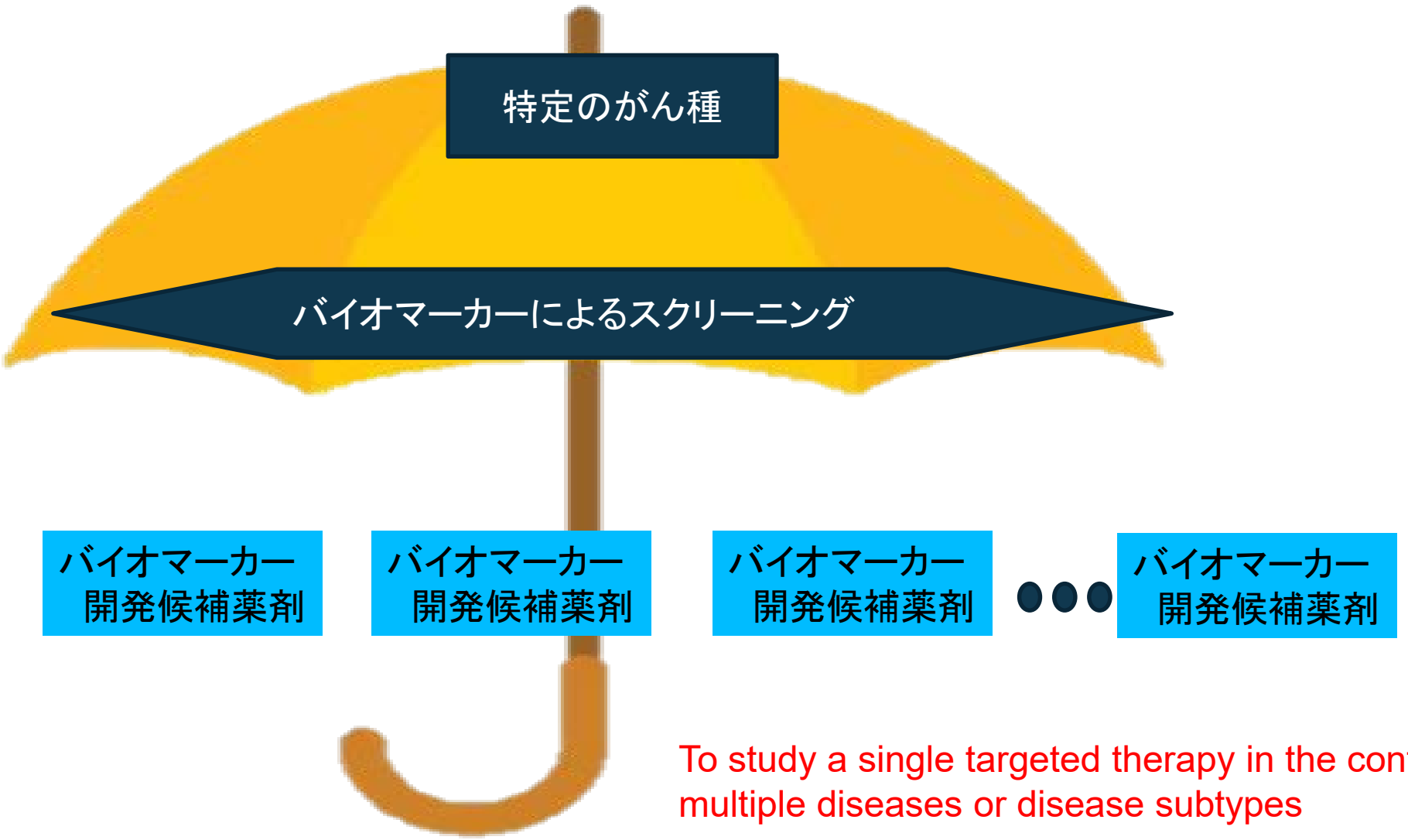
Master Protocol /バスケット試験



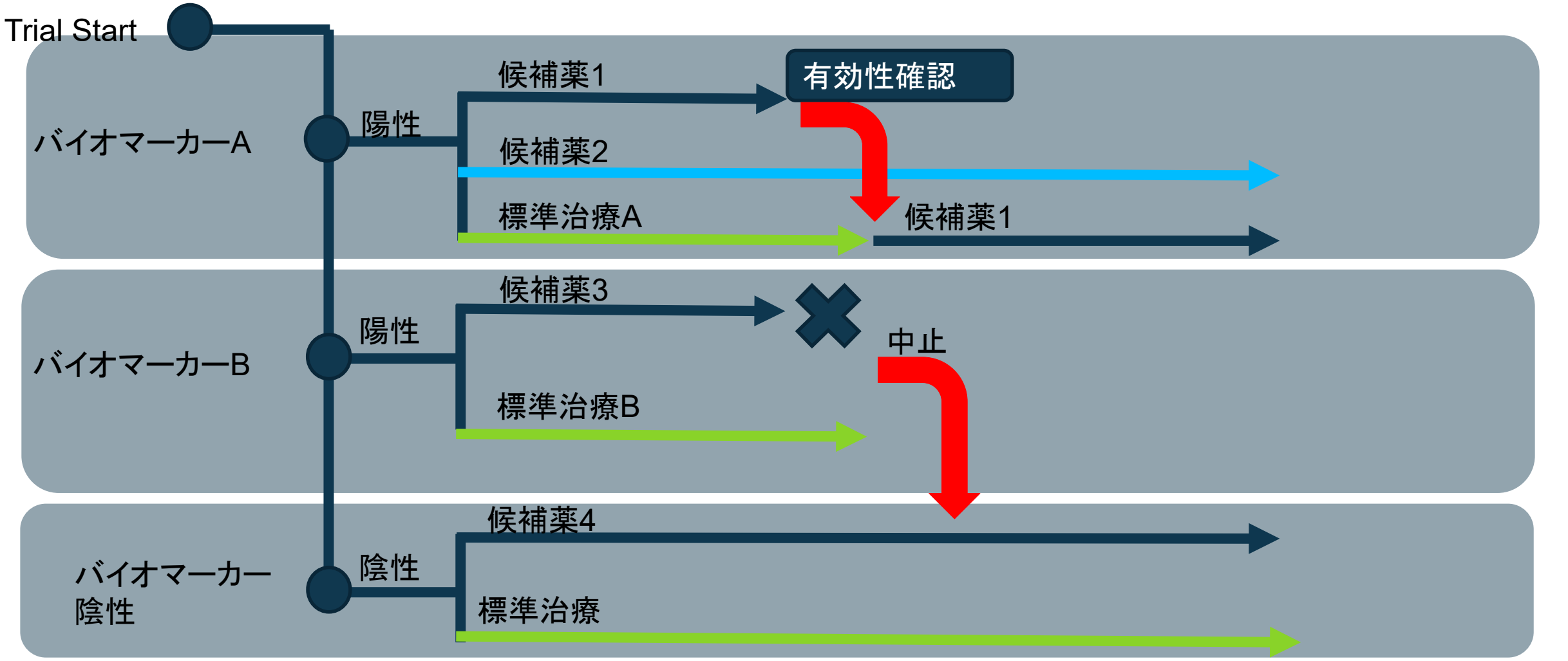
To study multiple targeted therapies in the context of a single disease

Master Protocol /アンブレラ試験

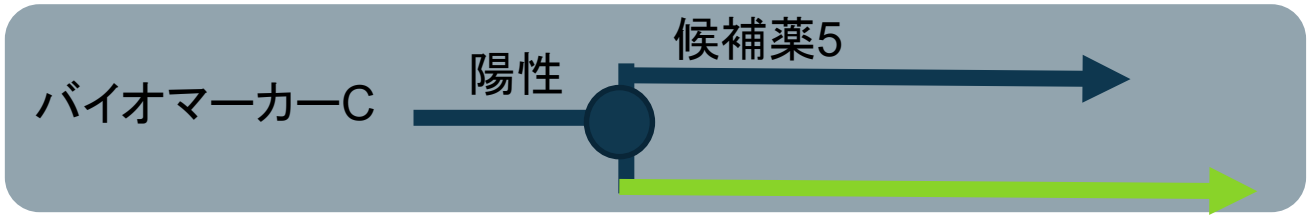


To study a single targeted therapy in the context of multiple diseases or disease subtypes

Master Protocol /プラットフォーム試験（例）



To study multiple targeted therapies in the context of a single disease in a perpetual manner, with therapies allowed to enter or leave the platform on the basis of a decision algo- rithm



Decentralized Clinical Trials　分散型治験

“Decentralized Clinical Trials are those conducted with the patient located *outside of clinical research centers* for either some or all of the required trial procedures“

治験開始

治験終了

治験開始

治験終了

テクノロジーの進化；管理システムやツール、AIなどのソフトウエア、ウエアラブルデバイス、パッチなどのIoTデバイスの登場

デメリット；被験者・家族との信頼関係構築の難しさ、デジタルのリテラシー、高齢者の参加の難しさ、データのセキュリティー上の懸念など

To modernize drug development, improve efficiency, and promote innovation, US FDA is running a **Complex Innovative Trial Designs** pilot program

Interacting with the FDA on Complex Innovative Trial Designs for Drugs and Biological Products

Draft Guidance for Industry

This guidance document is for comment purposes only.

Submit one set of either electronic or written comments on this draft guidance by the date provided in the *Federal Register* notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov/. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. You should identify all comments with the docket number listed in the notice of availability that publishes in the *Federal Register*.

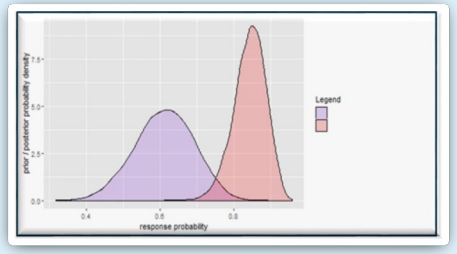
For questions on the content of this guidance, contact Center for Biologics Evaluation and Research (CBER), Office of Communication, Outreach, and Development (OCOD) at 240-402-8010 or 800-835-4709, or email ocod@fda.hhs.gov.

For questions about this document concerning products regulated by Center for Drug Evaluation and Research (CDER), contact Scott N. Goldie at 301-796-2055, or email druginfo@fda.hhs.gov.

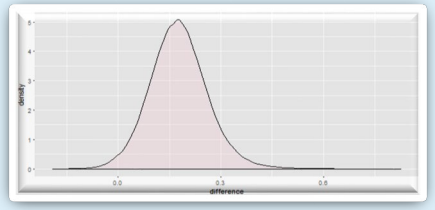
U.S. Department of Health and Human Services
Food and Drug Administration
Center for Biologics Evaluation and Research
Center for Drug Evaluation and Research
September 2019

EX. Bayesian Approach

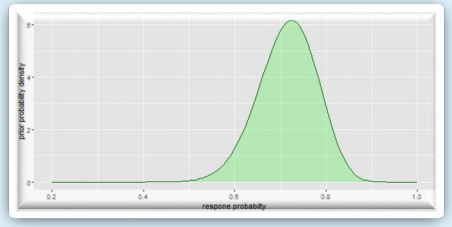
Previous trial with X and SoC

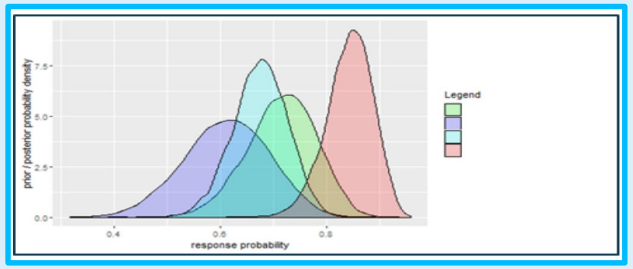


Evidence synthesis trial



Bayesian meta analytical prediction





ICH E6 (R3) Good Clinical Practice — Topic endorsed: June 2019

ICH E20 Adaptive Designs (new) — Topic endorsed: June 2018

より効率的な試験を求めて