Konferencja z okazji
Międzynarodowego Dnia Badań Klinicznych 2017

BADANIA KLINICZNE:
porozmawiajmy o innowacjach

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Dostępność nowoczesnych terapii, – dokąd zmierzamy i co nas czeka w przyszłości?

Availability of modern therapies – where we are heading and what's the future?

Nick Sykes
Senior Director, Pfizer
(on behalf of EFPIA)

About EFPIA and CTR 536/2014

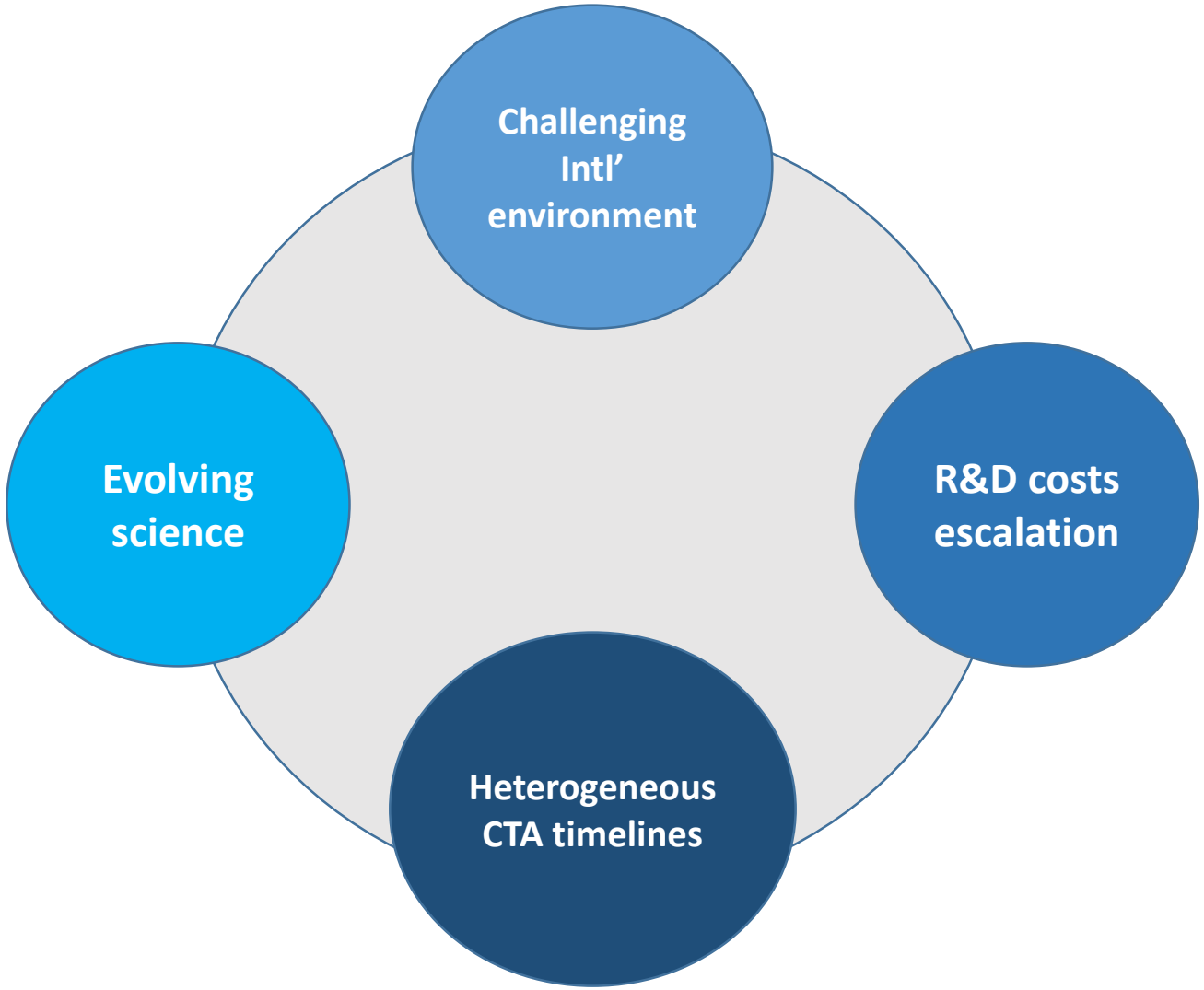
The European Federation of Pharmaceutical Industries and Associations (EFPIA) represents the pharmaceutical industry operating in Europe. Through its direct membership of 33 national associations and 40 leading pharmaceutical companies, EFPIA is the voice on the EU scene of 1,900 companies committed to researching, developing and bringing to patients new medicines that will improve health and the quality of life around the world.

> EFPIA sees the implementation of the Clinical Trials Regulation as an opportunity to demonstrate Europe's commitment to clinical innovation, scientific collaboration and transparency of clinical trials information. Successful implementation of EU CTR is one of EFPIA's priorities.

Aim of today's presentation

- Outlining challenges in clinical development of new drugs
- Providing an overview of some of the ways we are changing how we manage and run clinical trials
- Addressing some of the challenges and highlighting the opportunities

Challenging global medicine development environment



Rising to the Challenge

- Making crisp, objective decisions
- Allocating resources appropriately
- Looking for ‘early signs of clinical activity’ & “Killer” experiments
- Increasing Phase 2 success Rates
- Improving cycle time
- Seeking expedited pathways

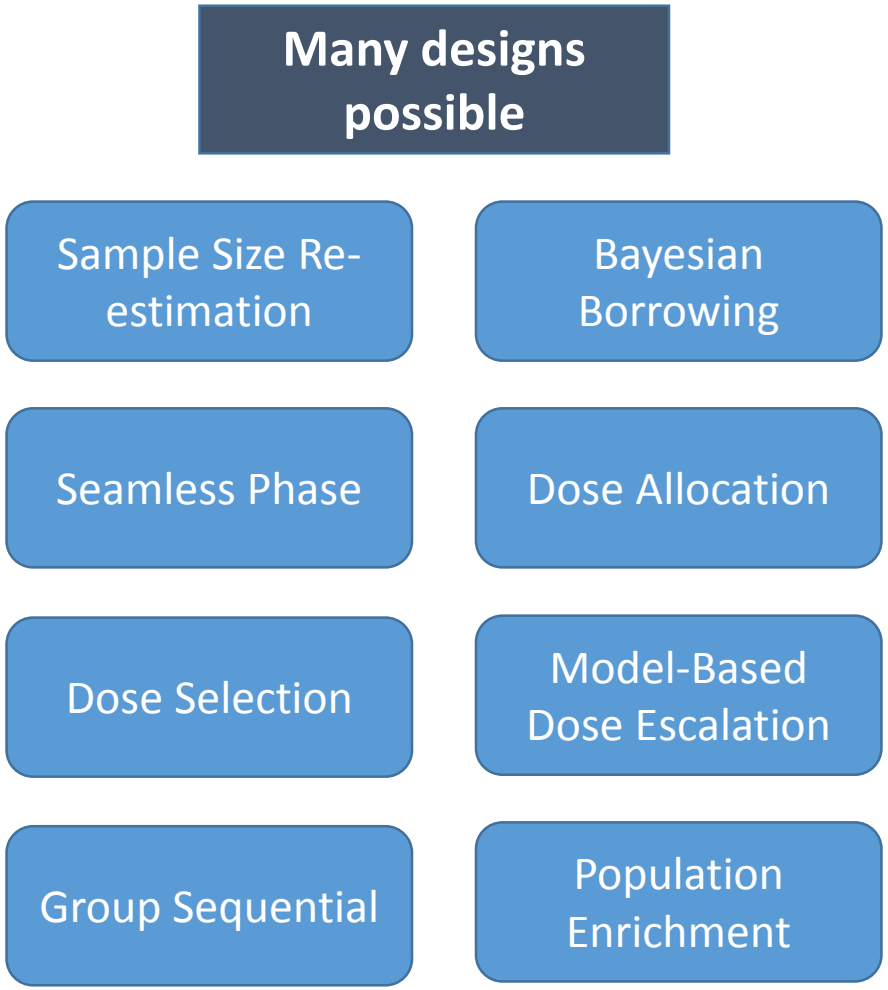
Results being seen: Recent big ‘game-changers’:

- Cures for Hepatitis C
- Immuno-oncology

Current Challenges in Clinical Development: Solutions being Adopted

- Greater efficiencies in running trials using innovative trial design approaches and technology
- Move towards a patient-centered approach to drug development
  - Personalized treatments
  - Drug-diagnostic co-development
- Research and care need to be better integrated, anticipating real life implementations

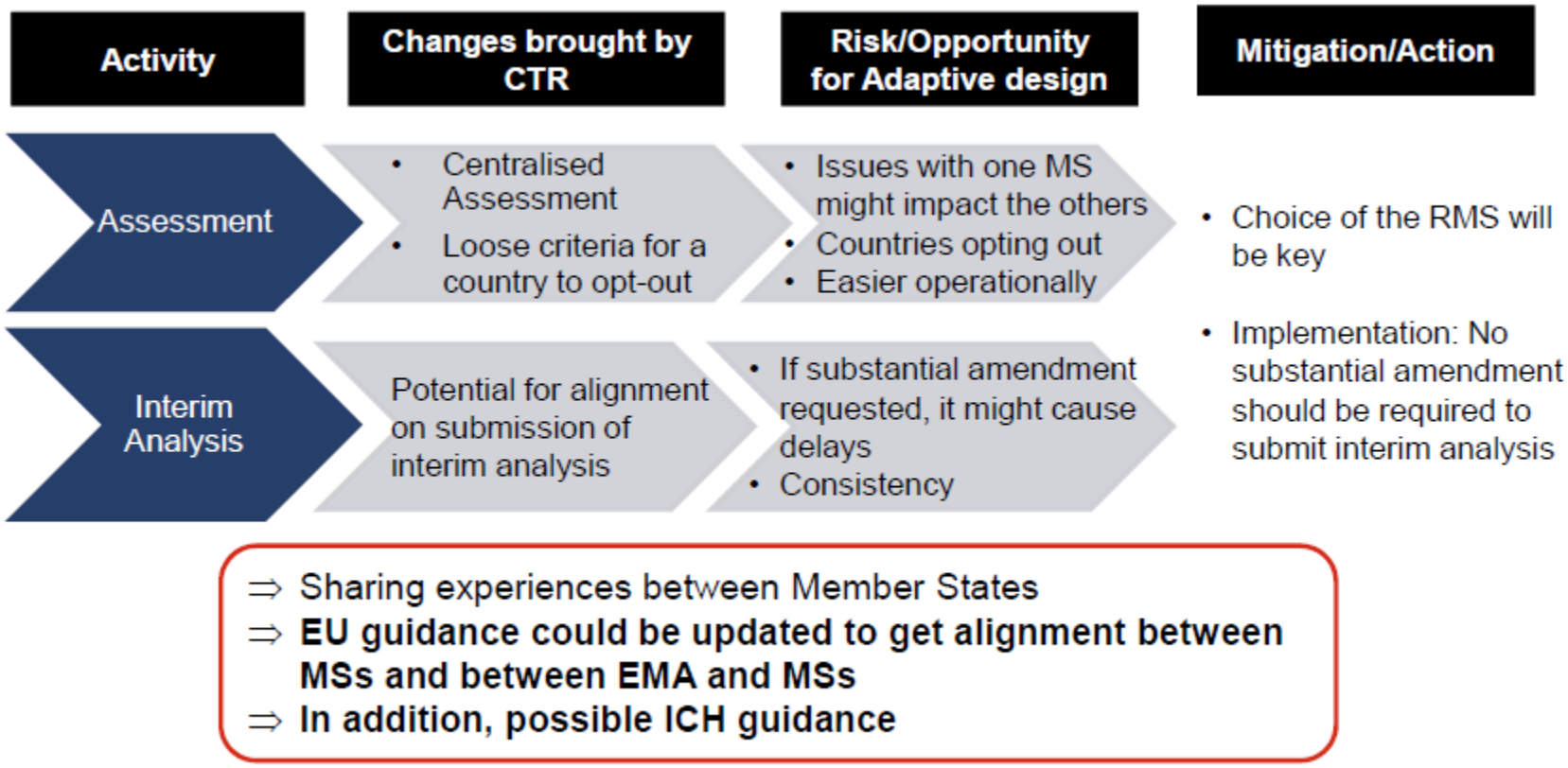
Adaptive Clinical Trial Design



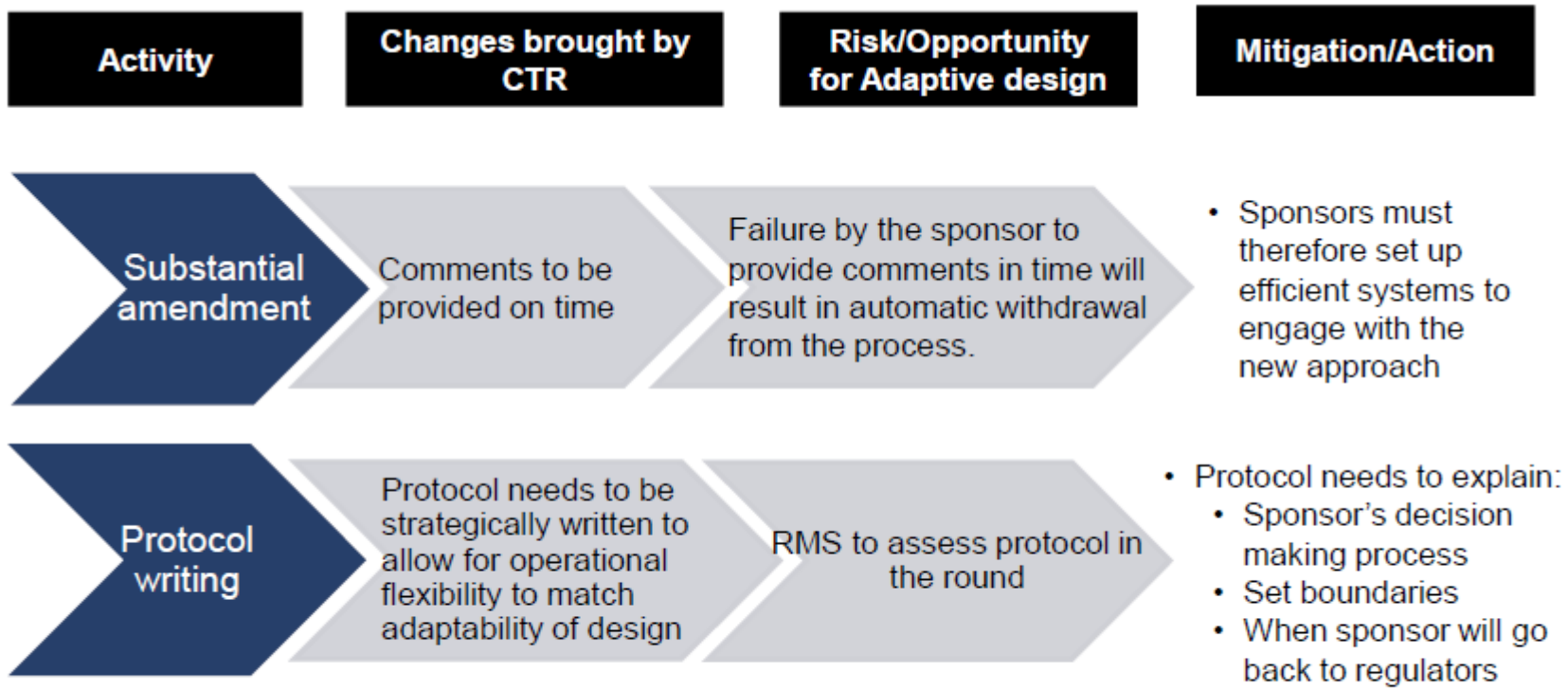
Paving the Way for Innovation

Will the EU Clinical Trial regulation meet its stated objective of quicker access to new and innovative medicines?

Regulatory Guidance



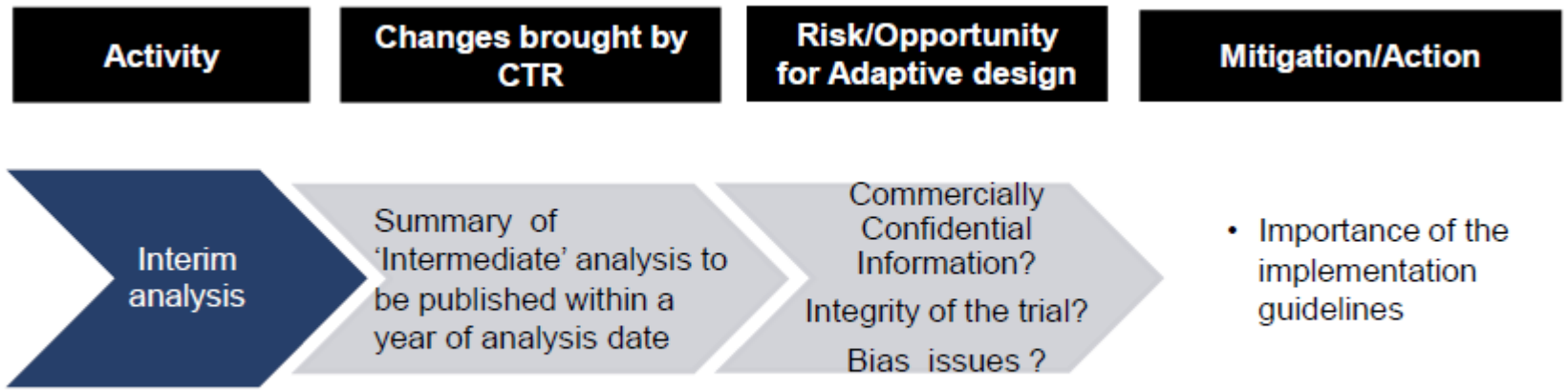
Strategic Planning



Timing for Changes

Activity	Changes brought by CTR	Risk/Opportunity for Adaptive design	Mitigation/Action
Adding a country & Substantial Amendments: new sites etc.	• Adding a country cannot be in parallel of initial approval & no substantial amendment possible in parallel • Potentially longer assessment timelines	Risk of delay 	As those are key enablers of the adaptability of the design, Sponsors could: • Strategically plan within protocol • Take advantage of the sequential submission of part I and part II

Transparency



Adaptive Trials: To Sum Up

Many designs possible

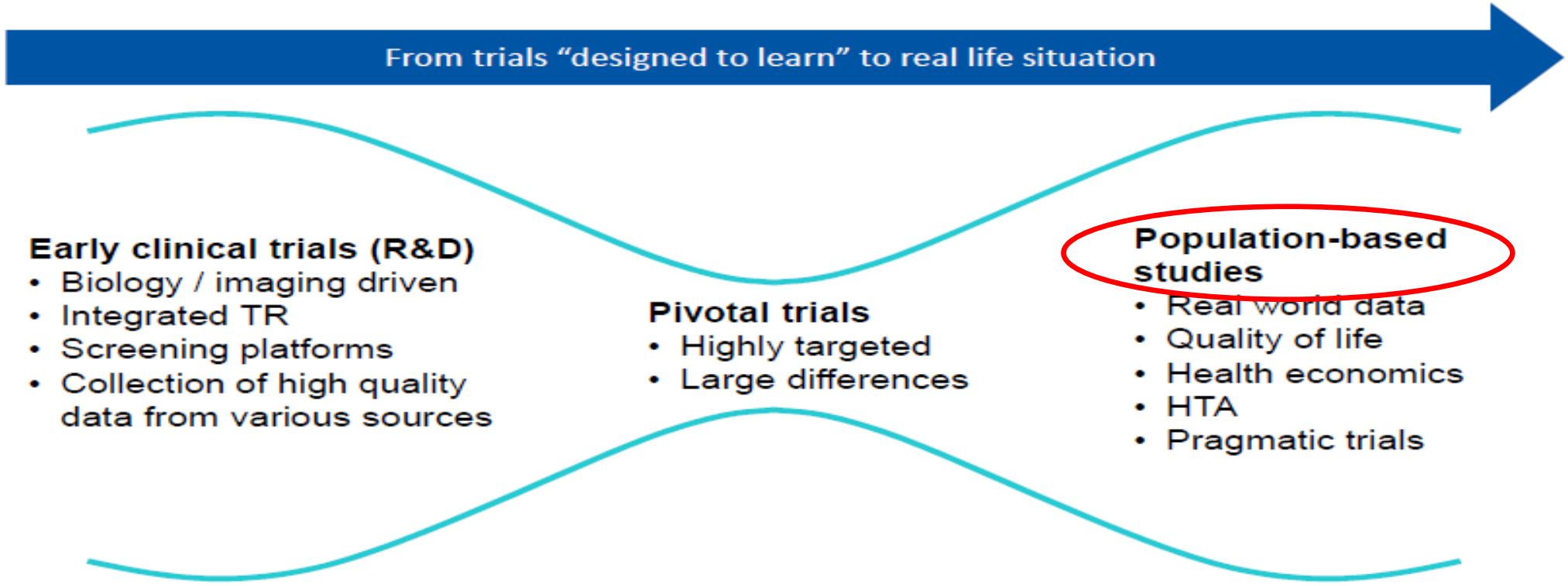
- Sample Size Re-estimation
- Bayesian Borrowing
- Seamless Phase
- Dose Allocation
- Dose Selection
- Model-Based Dose Escalation
- Group Sequential
- Population Enrichment

All requiring flexibility

- Centralized assessment
- Strategic protocol writing
- Adding countries/sites
- Substantial amendments
- Interim analysis
- Transparency

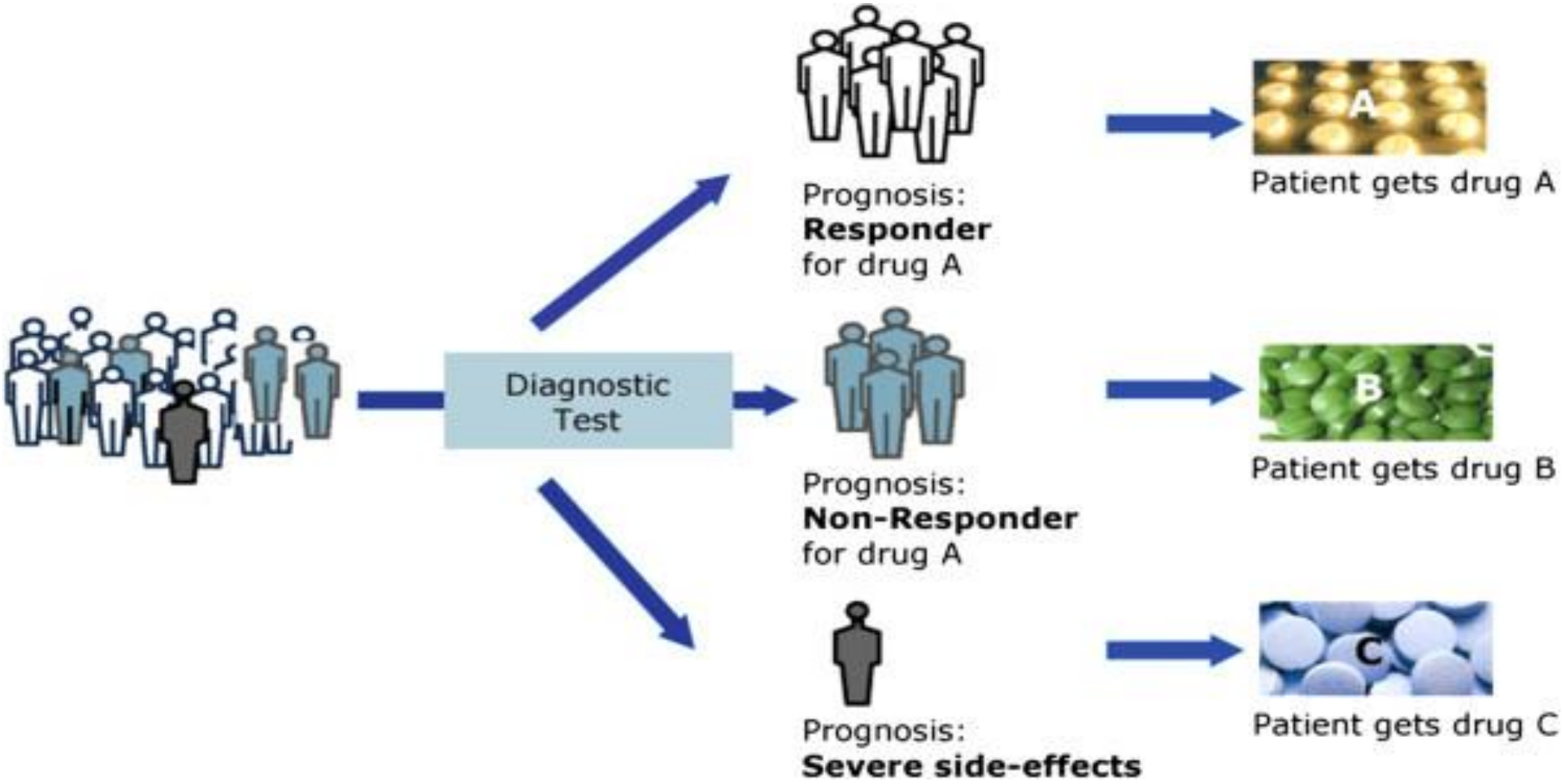
Implementation of the CT Regulation must allow enough flexibility to enable the adaptability required for a timely access by patients to innovative treatments

The Changing Clinical Research Pathway: Towards a Life-Cycle Approach to Evidence Generation

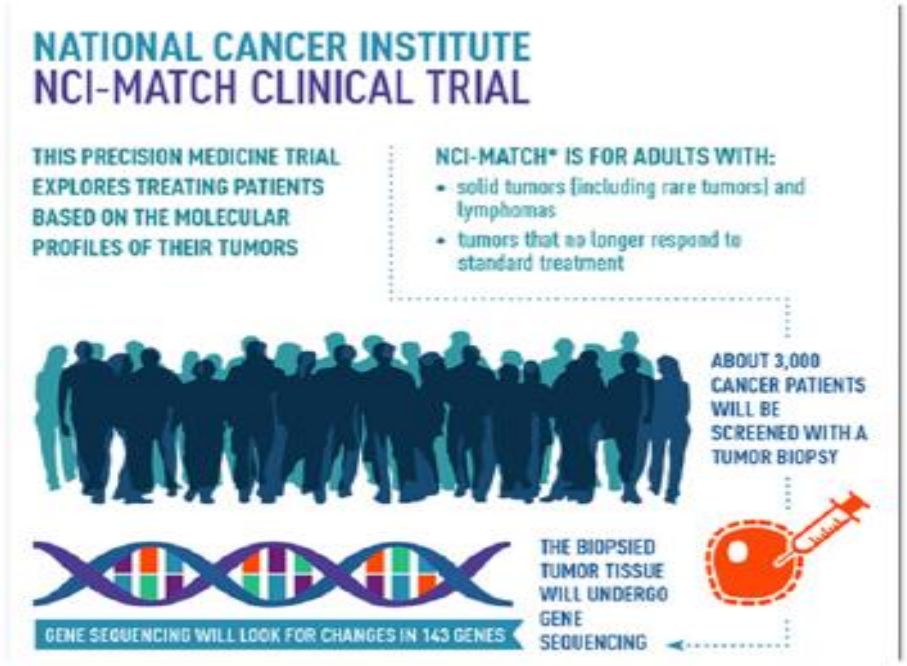


Burock et al. Eur.J.Cancer (2013), http://dx.doi.org/10.1016/j.ejca,2013.05.016

Sustainable Model of Drug Development – Efficiencies through Patient Selection

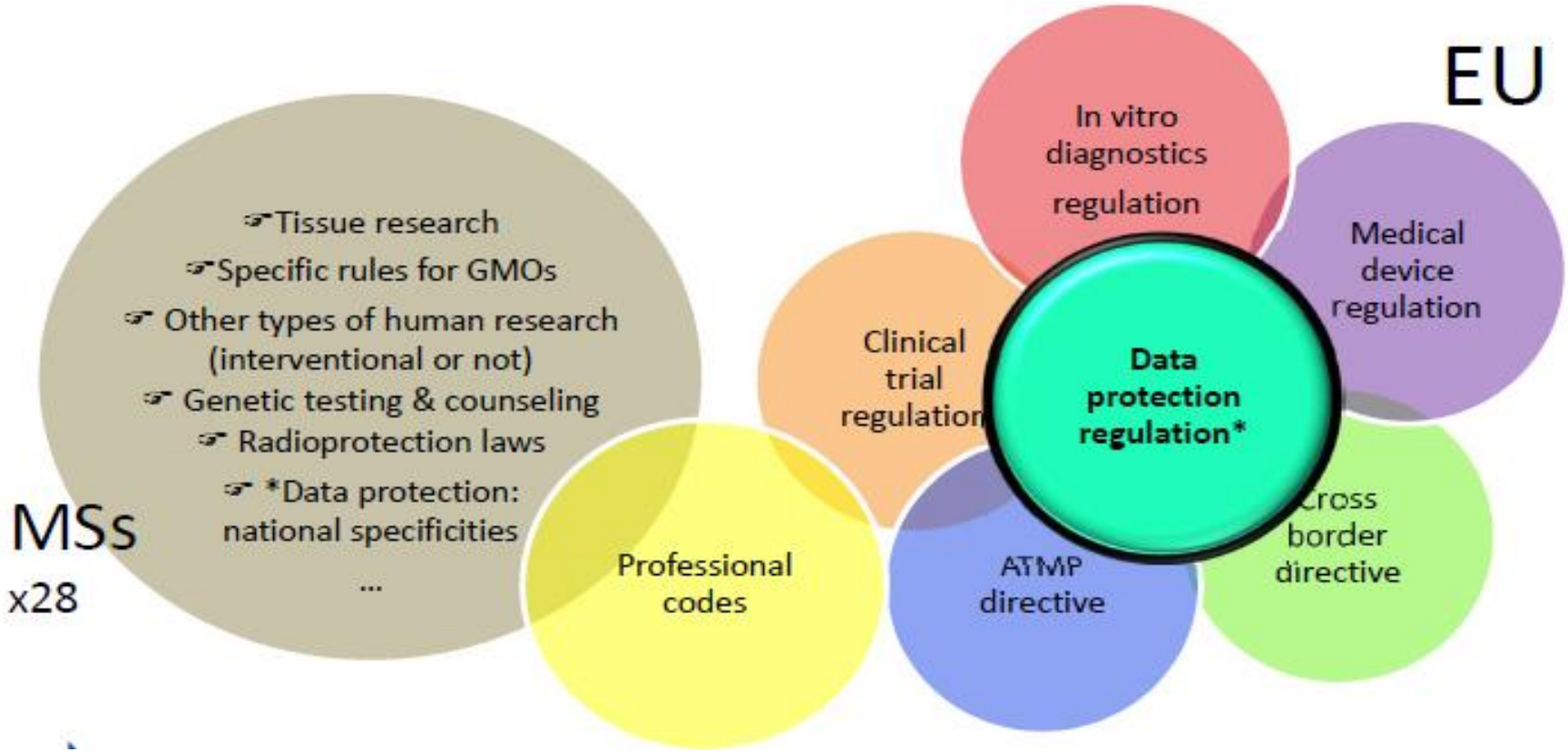


Example of 21st century personalized medicine trial



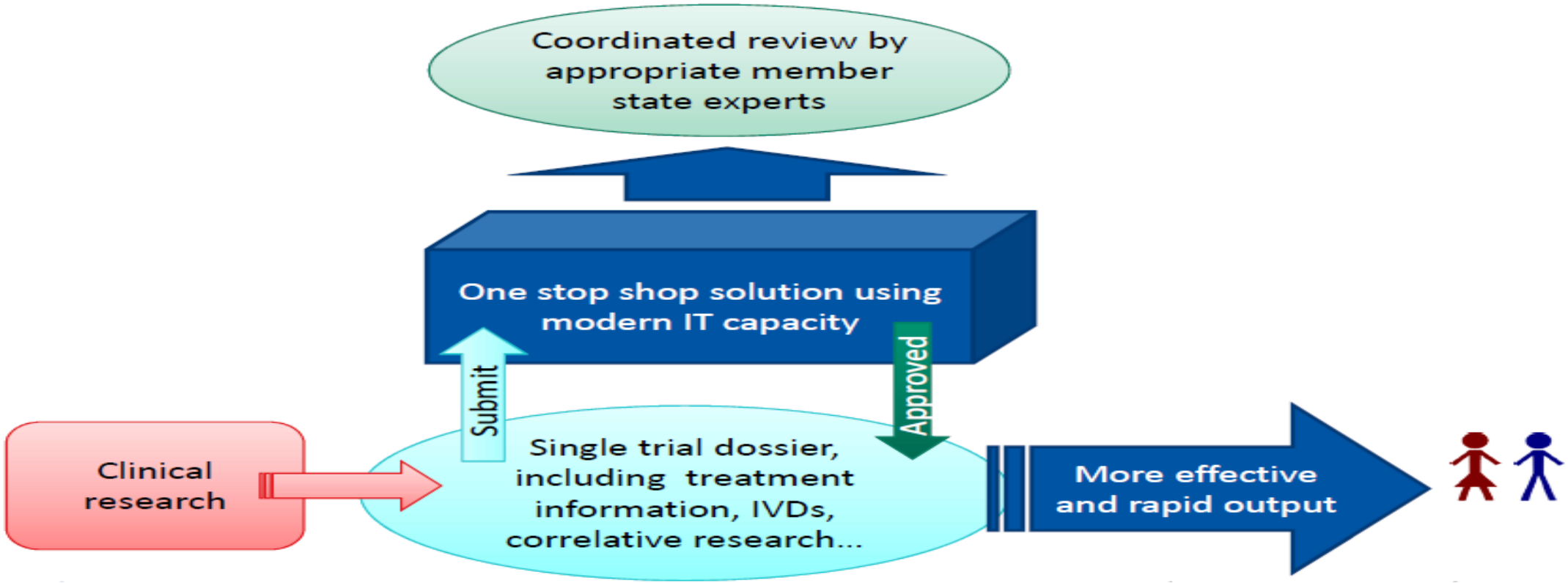
Such projects are at the edge of several regulations:
Clinical trials, data protection and IVD

Fragmentation of the regulatory framework in Europe: a major bottleneck



Streamline - Simplify - Harmonize

Europe must build an integrated and harmonized legal and ethical framework to foster relevant international clinical research



The Potential for Real World Data

Scrip Pharma intelligence

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ASK THE ANALYST

Takeda Enlists Real World Evidence To Boost Marketed Entyvio

09 May 2017 | NEWS

by John Davis

john.davis@informa.com

Executive Summary

Japan's Takeda says it has confirmed the benefits of its best-selling inflammatory bowel dise
therapy Entyvio in US medical practice through the collection of real world evidence.

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EMAIL

Relvar/Breo "Real World" Asthma Benefits Help GSK – But Can't Remove Generic Threat

08 May 2017 | ANALYSIS

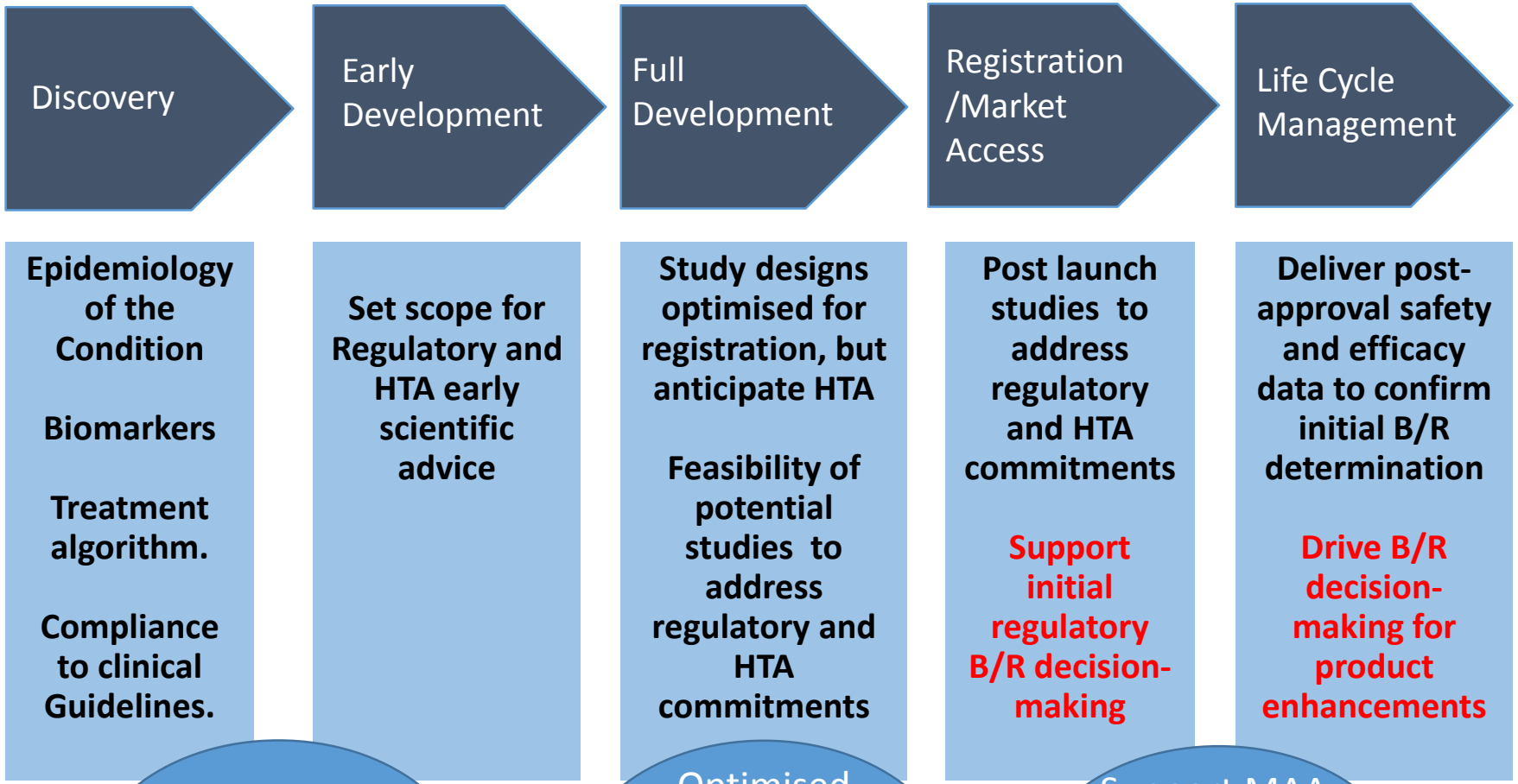
by John Davis

john.davis@informa.com

Executive Summary

Top-line data from a second patient group, asthmatics, in the Salford Lung Study indicate that switching from usual therapy to GSK's's Relvar Ellipta improves symptoms, but the results may not be enough to encourage switching before generic Seretide/Advair takes hold.

Real-World Data/Evidence is relevant throughout the drug development lifecycle



Technology can help….

Advances such as electronic data capture, and prevalence of wi-fi connectivity are driving changes in how clinical trials are conducted and analysed

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Pfizer to initiate study of smartphone app to help patients with lupus report symptoms

(Ref: Pfizer)
May 9th, 2017
By: Katie Bell
Tags: Top Story Apple Pfizer Lupus Internal Medicine Clinical Research (R&D)

Pfizer announced Tuesday that a new smartphone app, which aims to enable patients with lupus to report on their fatigue and other symptoms in real time, will be evaluated through the non-interventional VALUE study. The trial is due to begin at the end of the month, with results expected in December. Freda Lewis-Hall, Pfizer executive vice president and chief medical officer, said "the current paper methods of collecting patient-reported data can be cumbersome and inconvenient...VALUE holds promise for 'in the moment' reporting of symptoms and may open the door to similar tools being used in Pfizer-sponsored clinical studies for lupus and other diseases."

Pfizer said it jointly developed the mobile app with the Lupus Research Alliance and Tata Consultancy Services using Apple's ResearchKit platform and "building on existing research tools that have been validated in lupus." Kenneth M. Farber, co-CEO of the Lupus Research Alliance, noted that the "app may enable more frequent and consistent reporting from patients, thus providing better information for care teams and empowering patients to take a larger role in developing future therapies."

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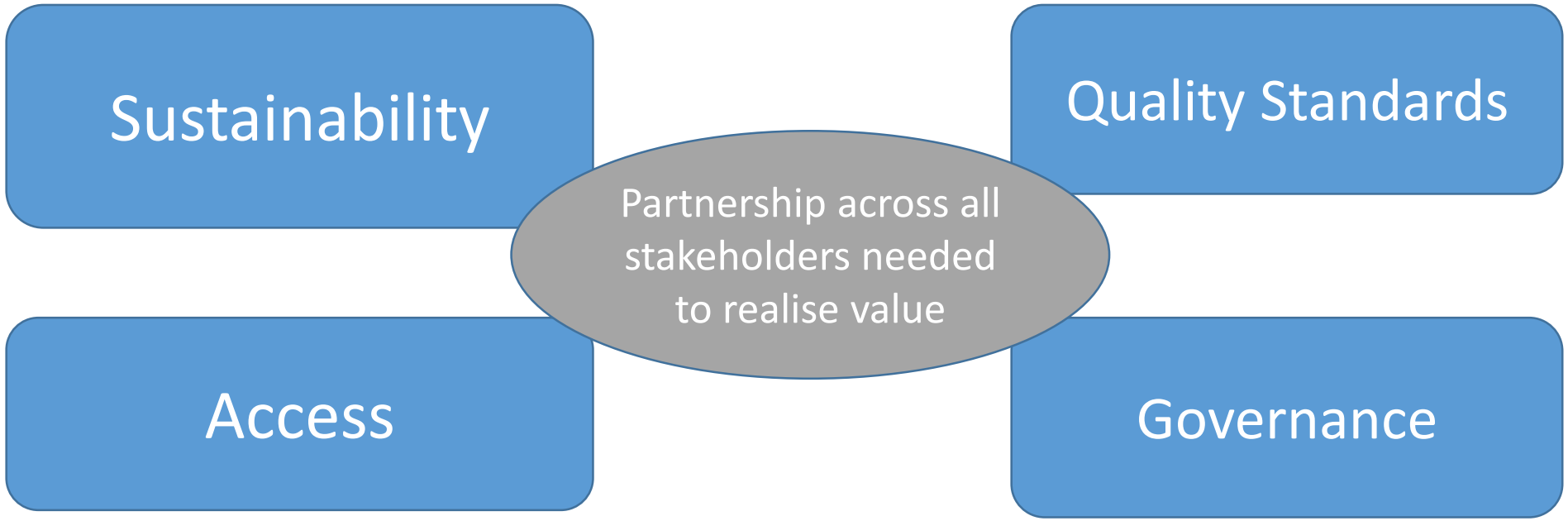
Trends in Technology Helping in Clinical Trials

- Using patients own health data from other systems
  - Electronic health records
  - Data captured on their own smartphone
- Making research participation more accessible
  - Self-reporting and tracking using technology in their pocket
  - Wearable sensors capturing data and transmitting the data to a remote location

Challenges remain…

Reaching the potential :



In Summary – Many opportunities to maximise efficiency

- Novel approaches to running/managing clinical trials are being adopted
  - Innovative trials designs
  - Patient-focussed approaches
  - Use of real-world data
  - Advances in technology
- Hurdles still to overcome
  - Legislative
  - Ensuring quality
  - Subjective

Questions