

Overview of the RWD/RWE Landscape

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Disclaimer

I am an employee of F. Hoffmann La-Roche.

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Overview

- 1 What, where and how
- 3 The evolving global landscape
- 4 Summary and the future



What, where and how

RWD Definition; an evolving topic

EMA definition*

- *“Routinely collected data relating to a patient's health status or the delivery of health care from a variety of sources **other than traditional clinical trials**”*



FDA definition**

- *“Data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.
.....Under FDA’s RWE Program, **evidence from traditional clinical trials will not be considered RWE.***



*EMA publication “Real-World Data for Regulatory Decision Making: Challenges and Possible Solutions for Europe” April 2019 Publication in Clinical Pharmacology & Therapeutics

** FDA RWE Framework December 2018

RWD: The what and where

➤ **Depending on the source - RWD could include information about a patient's:**

- Demographics, age, gender etc.
- Diagnosis, disease, symptoms, health status
- Medications/treatments
- Disease progression
- Region of living
- Behavior
- Lifestyle

➤ **Collected in traditional settings:**



Physicians office notes Electronic
Health care records (EHR)



Insurance billing
and claims

➤ **But also outside of such settings:**



Wearables

Steps or
heart rate



Social media

e.g. home
tablets and
recording of AE



Diagnostics / omics databases

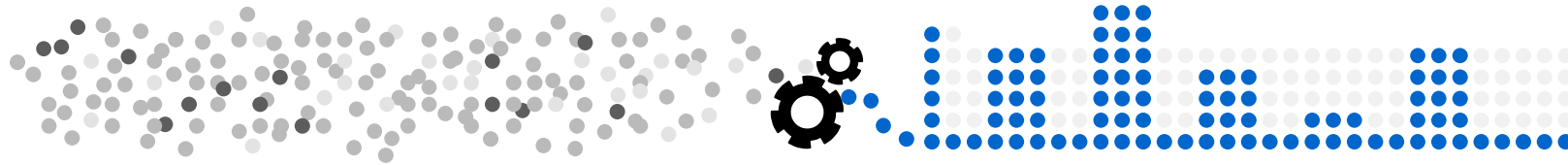
e.g. genetic and
Other biomarker data



Disease and product registries

From real-world data to real-world evidence

Passing through stringent curation, standardisation and analysis



Real-world data (RWD)

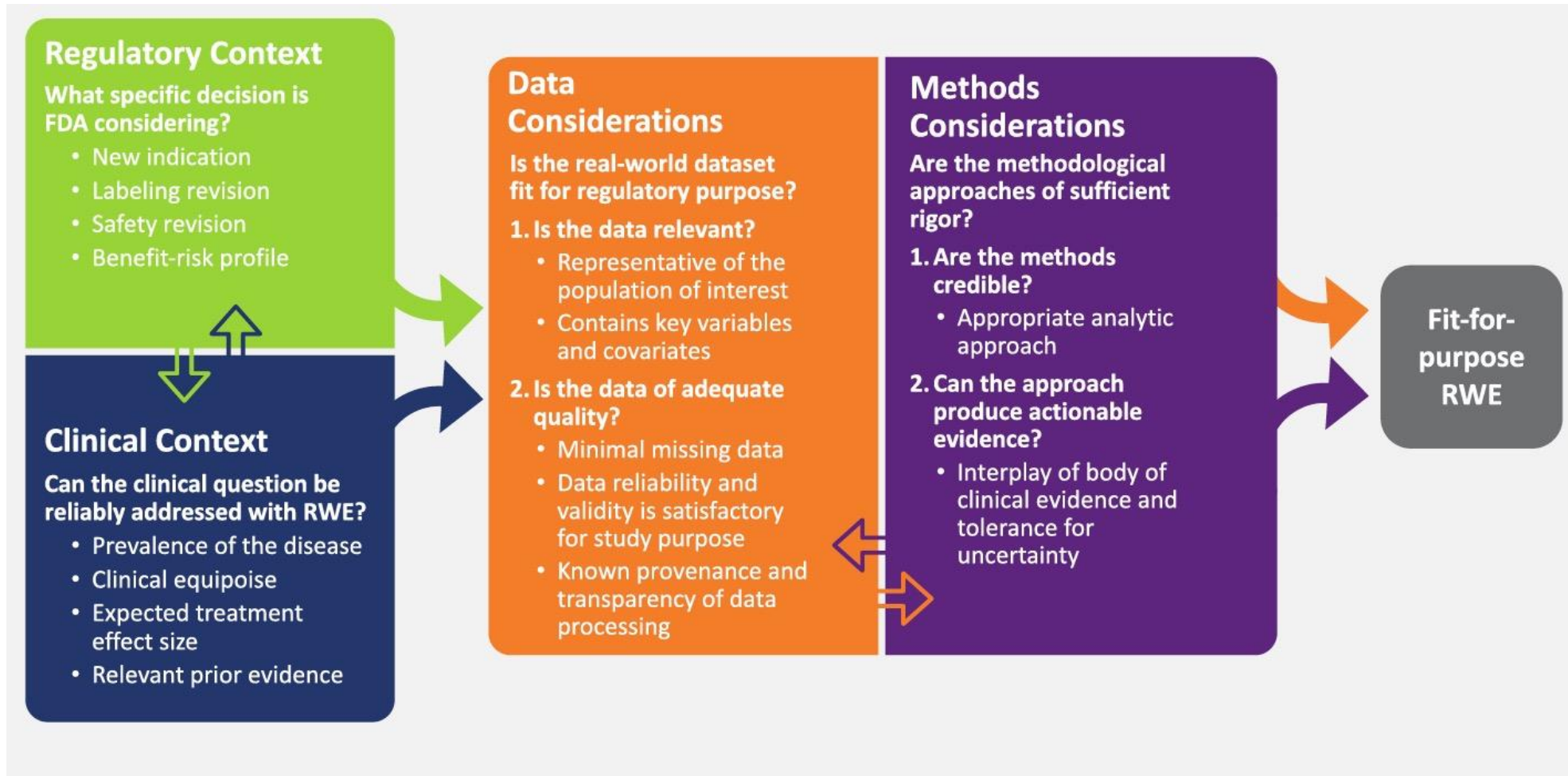
- Data relating to patient health routinely collected from a variety of sources **outside of traditional controlled clinical interventional trials**, under real-life clinical practice

Real-world evidence (RWE)

- The evidence derived from the analysis and / or synthesis of RWD

Considerations for Generating RWE Fit for Regulatory Purposes

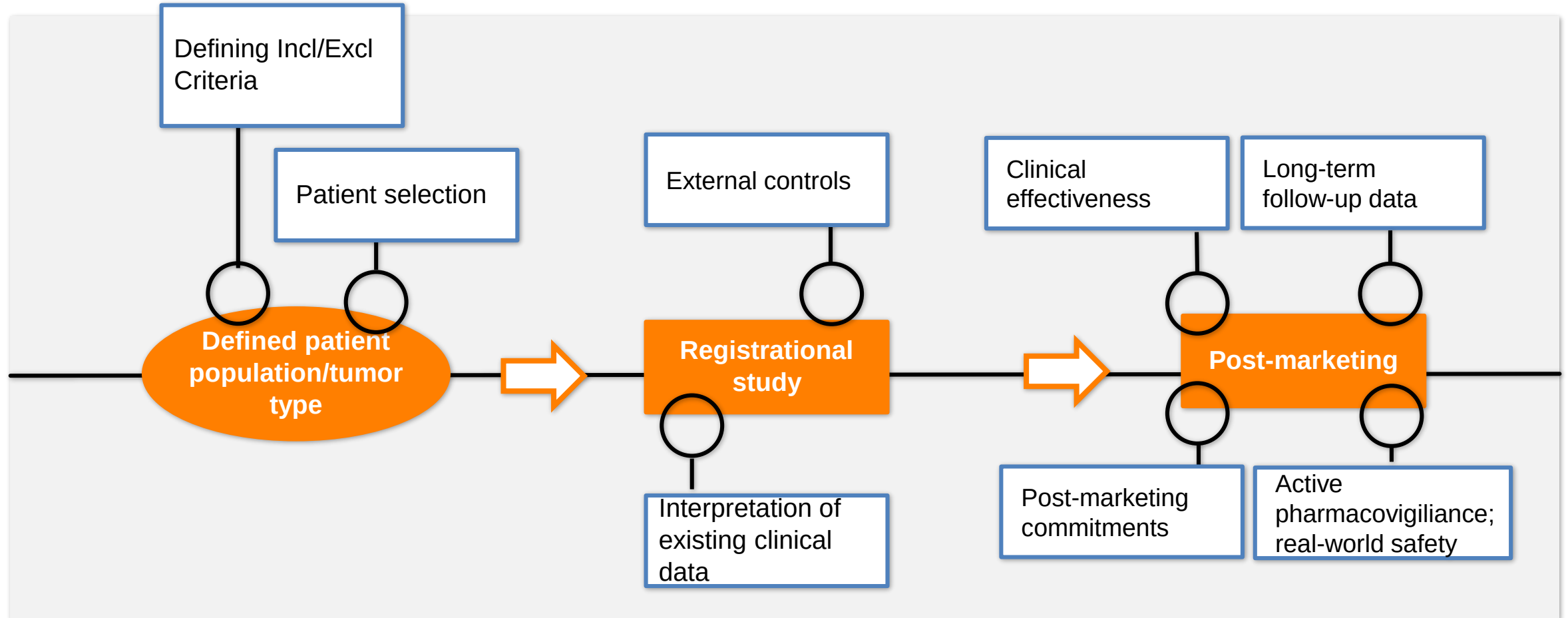
The Duke Margolis White Paper (2018) as an example



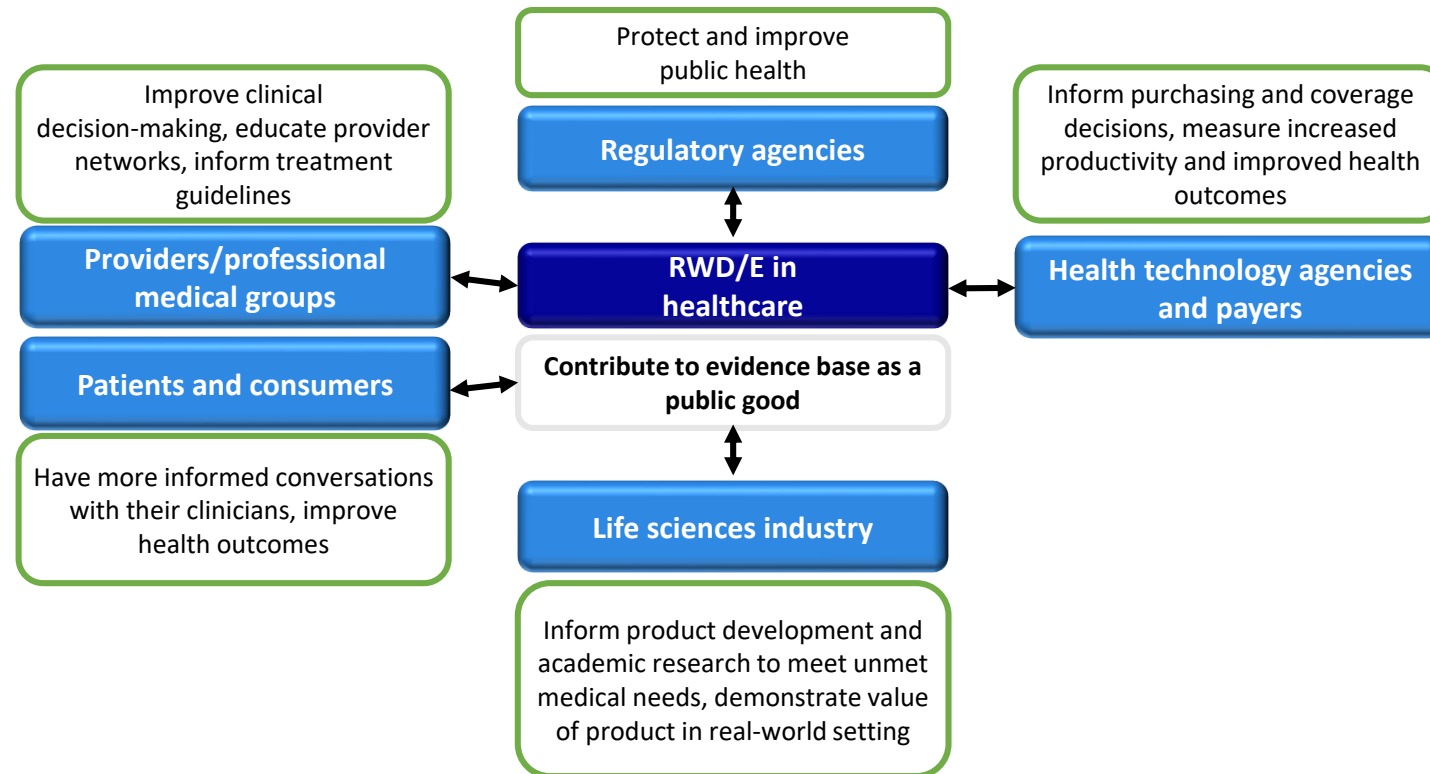
Why has RWD become of interest to stakeholders in the Healthcare space?

- RWD as a concept is not new , nor are methodologies!
- What is new
 - Newer sources RWD
 - Availability of highly sophisticated electronic tools to access, analyze and link data sources
 - Awareness of RCT limitations:
 - Only ~4% of all patients take part in clinical trials
 - RCT populations rarely reflect “real world” populations
 - Occur within a limited time frame
 - Not large enough to detect rare treatment effects
 - RCTs may not be generating evidence on endpoints useful to patients, providers, or payers
 - Not always ethical to have patients on placebo or not enough patients to sufficiently power a trial
 - Multitude of questions remain unanswered at the time of Regulatory approval

Examples of how RWD can be used in Clinical Development and Post-Marketing Setting

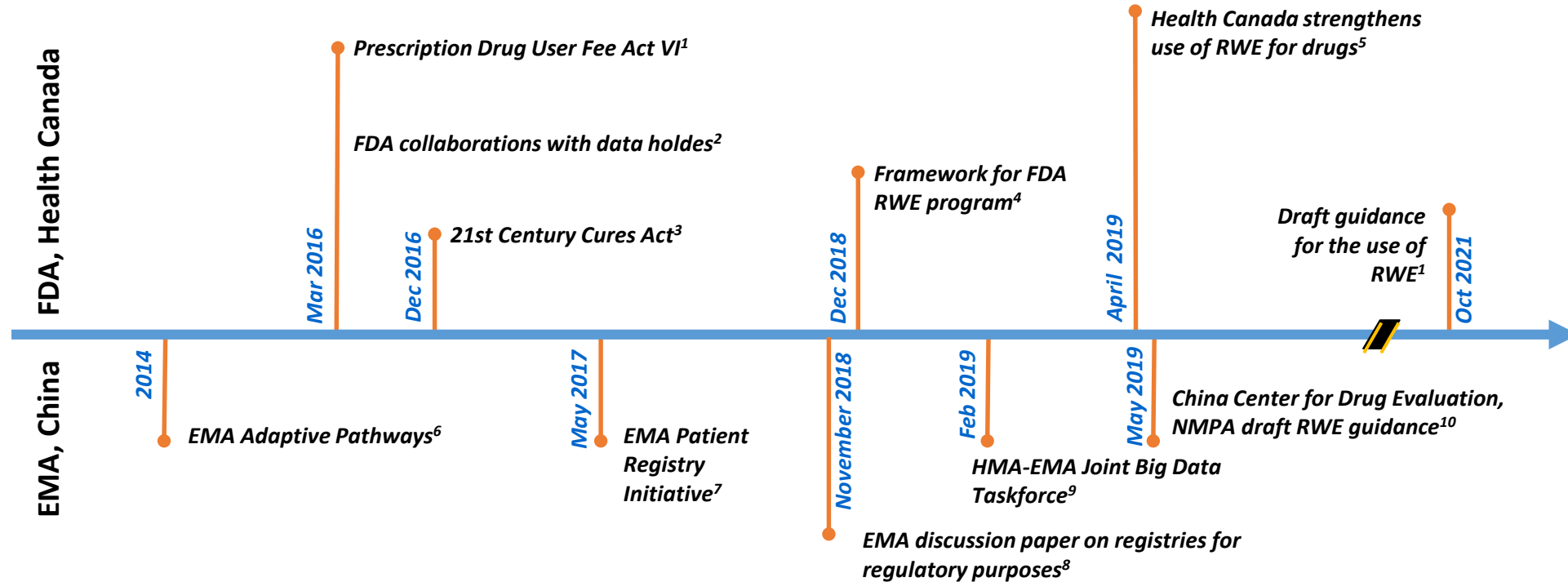


Who is interested in RWD/RWE and why?



The evolving global landscape

Increasing regulatory focus globally



1. Khosla, S., et al. (2018) *F1000Res* 7:111 (Version 2); 2. genomeweb website, available at: <https://www.genomeweb.com/cancer/flatiron-health-fda-partner-cancer-research-project#.XE7RxExFyy9>;

3. FDA website, available at <https://www.fda.gov/downloads/RegulatoryInformation/LawsEnforcedbyFDA/SignificantAmendmentstotheFDCA/21stCenturyCuresAct/UCM562852.pdf>; 4. FDA website, available at:

<https://www.fda.gov/downloads/ScienceResearch/SpecialTopics/RealWorldEvidence/UCM627769.pdf>; 5. Health Canada website, available at <https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/announcements/optimizing-real-world-evidence-regulatory-decisions.html>; 6. EMA webpage, available at: <https://www.ema.europa.eu/en/human-regulatory/research-development/adaptive-pathways>;

7. EMA (2017): Patient Registry Initiative (EMA/180341/2017), available at: https://www.ema.europa.eu/documents/other/patient-registry-initiative-strategy-mandate-cross-committee-task-force_en.pdf; 8. EMA webpage, available at:

<https://www.ema.europa.eu/en/human-regulatory/post-authorisation/patient-registries>; 9. EMA (2019): HMA-EMA Joint Big Data Taskforce - Summary report, available at: https://www.ema.europa.eu/documents/minutes/hma/ema-joint-task-force-big-data-summary-report_en.pdf; 10. Draft Guidance available at: <http://www.cde.org.cn/news.do?method=viewInfoCommon&id=314865>

EMA RWD Initiatives (not exhaustive)

EMA/HMA Big Data Task Force

Formed to describe big data landscape from a regulatory perspective and to ensure the EU regulatory system has the capability and capacity to guide, analyse and interpret these data



Building a RWD Ecosystem

- A common data model for Europe
- A 'learning healthcare system', based on electronic health records and other routinely collected healthcare data, would allow RWD to be continuously fed into the system, ensuring that with every new patient treated, we know more overall about the practice of medicine....



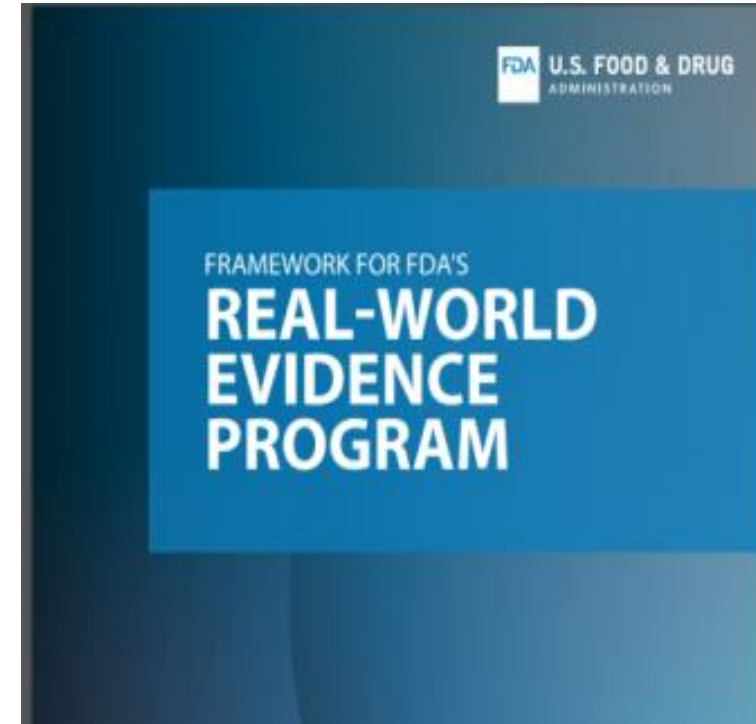
Patient Registry Initiative

- Aim to address challenges in using existing registries or establishing new ones
 - Discussion paper on methodological and operational considerations in the use of patient disease registries for regulatory purposes (Feb 2019) and introduction of the "Good Registry Practice" concept

FDA RWE Framework; December 2018

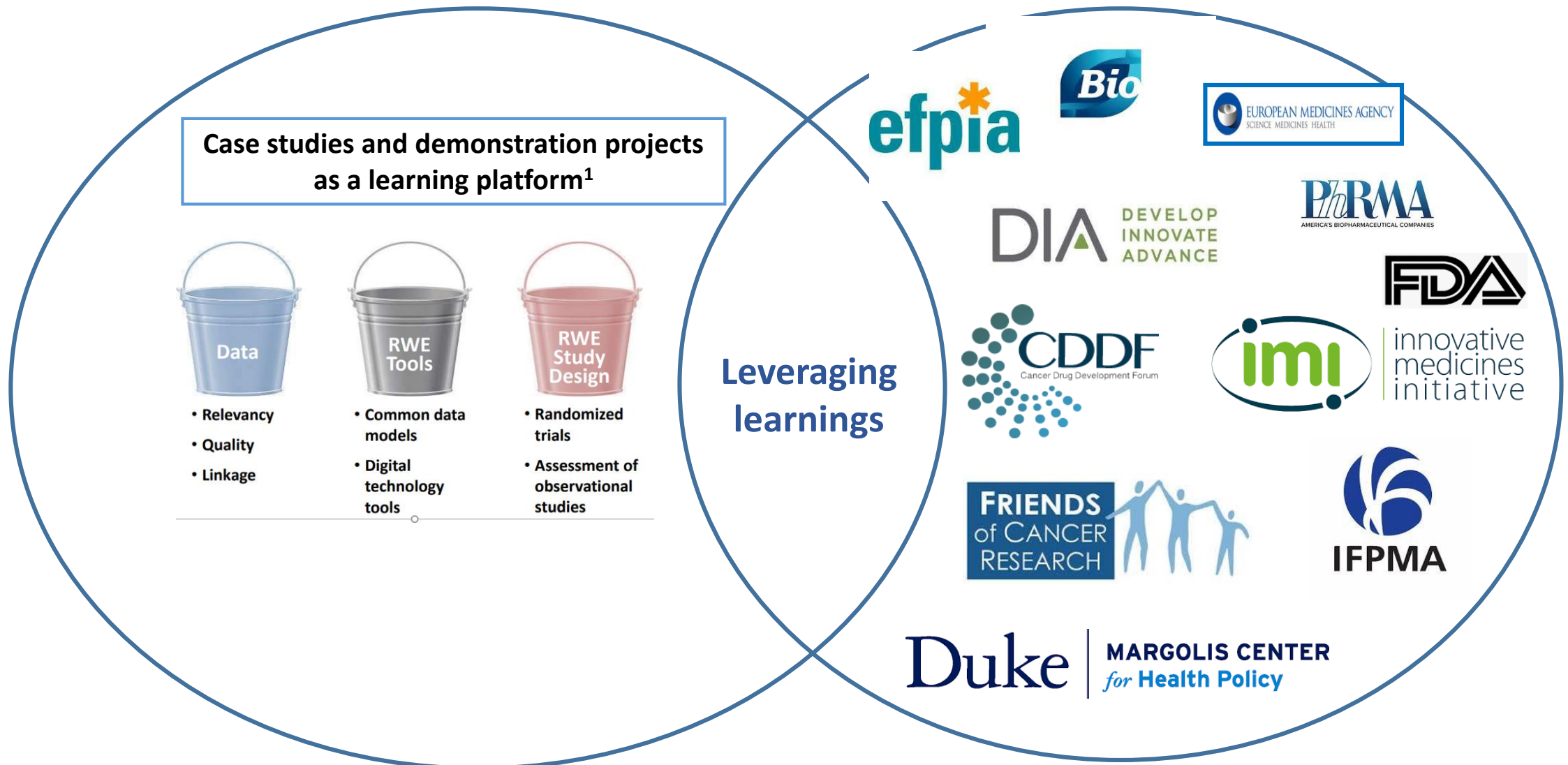
How is FDA informing RWD/E guidance?

- **Stakeholder engagement**
 - Conferences, workshops, soliciting comments
 - **Internal engagement**
 - RWE Subcommittee, tools for reviewers, consistency across reviews
 - **Demonstration projects**
 - Research projects and academic collaborations
 - Data Quality, RWE Tools, Study Design
- **Guidance development**
 - Have stated will have guidance out on a variety of topics by 2021



Summary and the future

Advancing the Use of RWD-RWE in Drug Development and for Regulatory Decision Making



¹Source of image: Jacqueline Corrigan-Curray, FDA, 'Framework for FDA's Real world Evidence Program', webinar on March 15, 2019

Summary

- RWD/RWE has the potential to transform how we do drug development
- Numerous challenges remain
 - Need for relevant and high quality RWD
 - Need to develop the right tools and methods to analyze the data
 - Generation and harmonization of approaches globally
- Early discussion with relevant stakeholders and prospective planning for collecting the right data for the right purpose is important
- Collaboration across healthcare stakeholders will be important in identifying solutions

Thank you!