Patient Data and Real-World Data for decision-making on Rare Diseases therapeutic approaches and health policies

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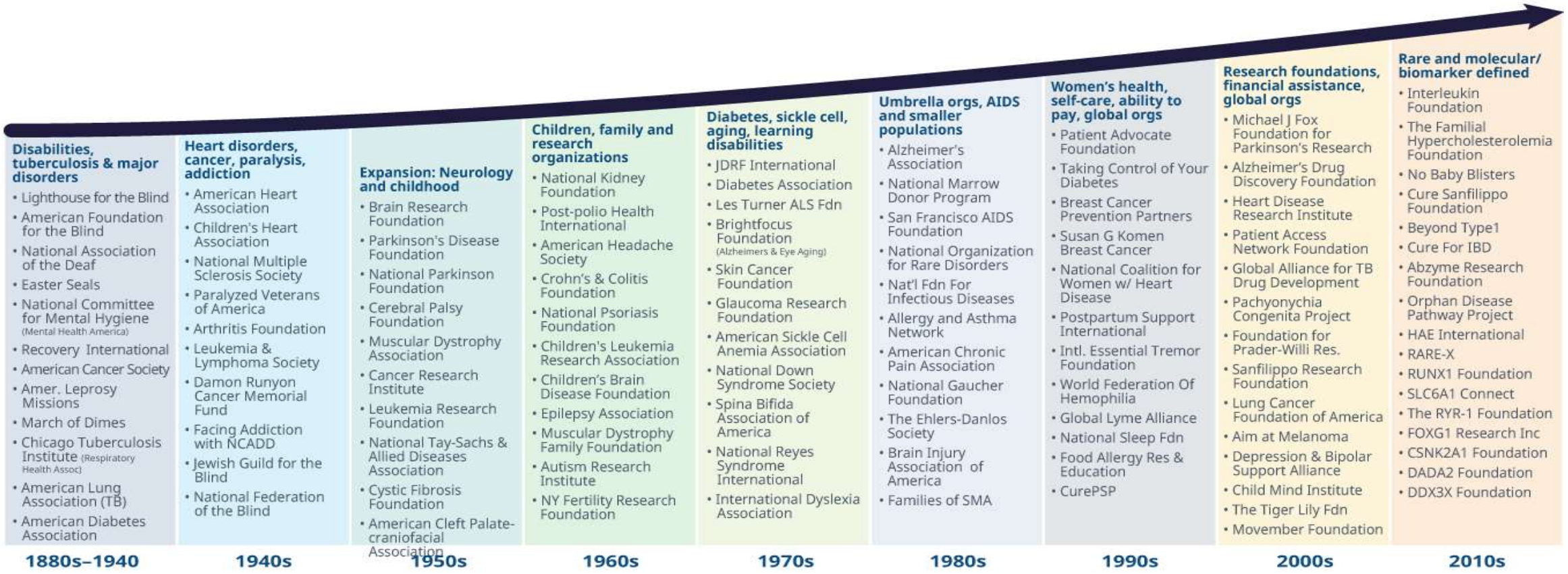
The creation of patient organizations over time



Source: Form 990 data from IRS Statistics of Income (SOI) program, filed 2015–2021.

Notes: Reflects only those patient organizations still in existence today. Uses ruling date from the IRS Business Master File as a proxy for creation and overrides have been applied in select cases where dates differ dramatically and were noted.

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Information shared by patients that helps to guide drug development

- Symptoms of their condition and changes experienced over time (natural history)
- Physical and psychosocial impacts of symptoms and/or care conditions on their quality of life and function both in care settings and clinical trials
- Experiences with therapies used to treat the condition and/or related clinical studies
- Input on whether changes in specific outcomes are meaningful to them
- Their view of tradeoffs between disease outcomes and treatment benefits and risks
- Views on unmet medical needs and available treatment options
- The relative importance of any issue or different possible outcomes as defined by patients
- Patient views on appropriate trial designs (burden, barriers, technology use) and views on patient support initiatives that can help facilitate trial access and participation

Helps to create:

- A greater patient focus in medical product development
- Meaningful patient centered endpoints
- Adjustments to clinical trial design to ensure accessibility and minimize burden on patients
- Trials that are attractive to patients, meet their needs and recruit well
- Treatments that are attractive to patients and will be utilized
- Educational materials that can be given to patients to aid their understanding of care programs and trial participation

Source: FDA definition of patient experience data from Patient-Focused Drug Development: Collecting Comprehensive and Representative Input Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders :2020 Jun.

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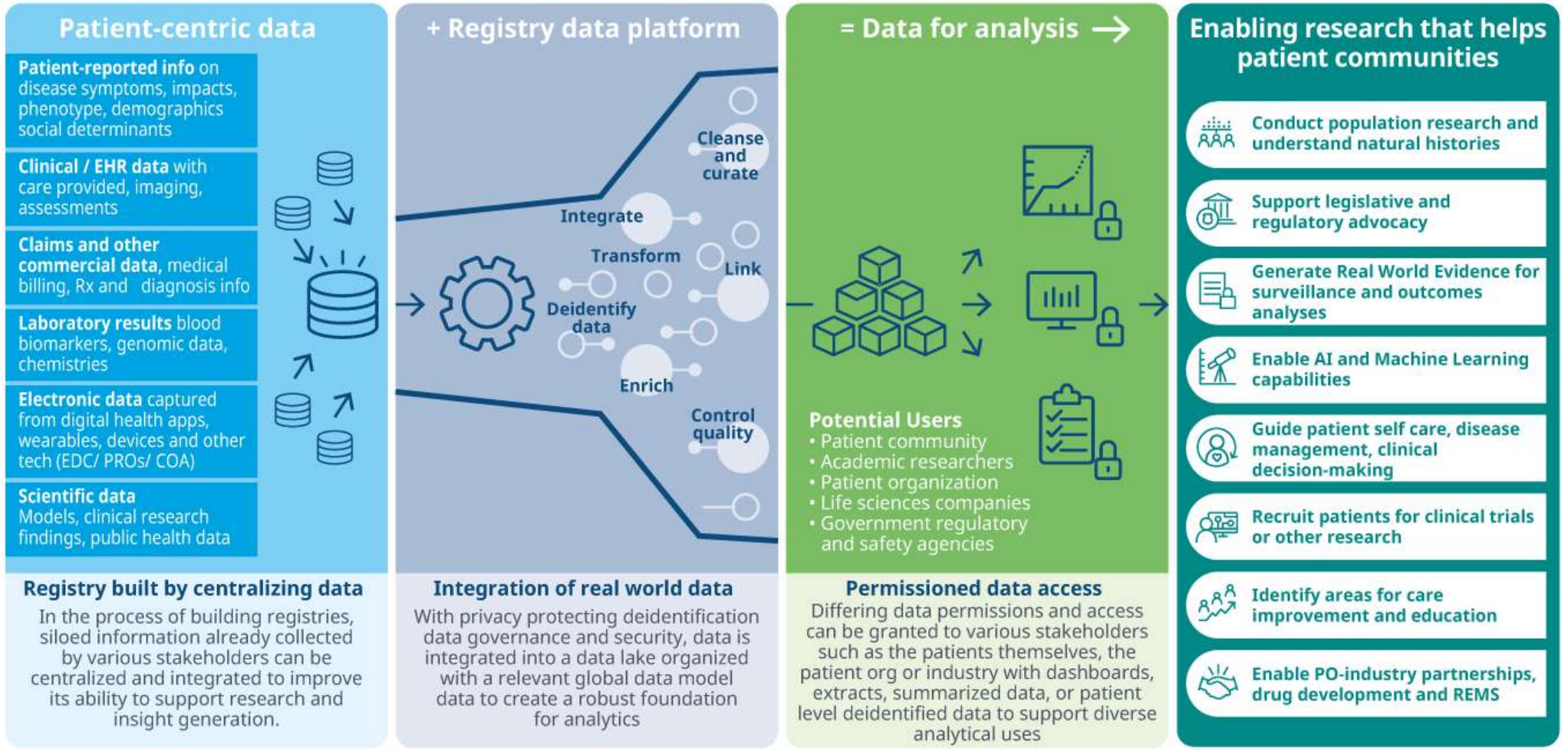
How patient-centric registries and data repositories enable research



Source: IQVIA Institute, Aug 2023.

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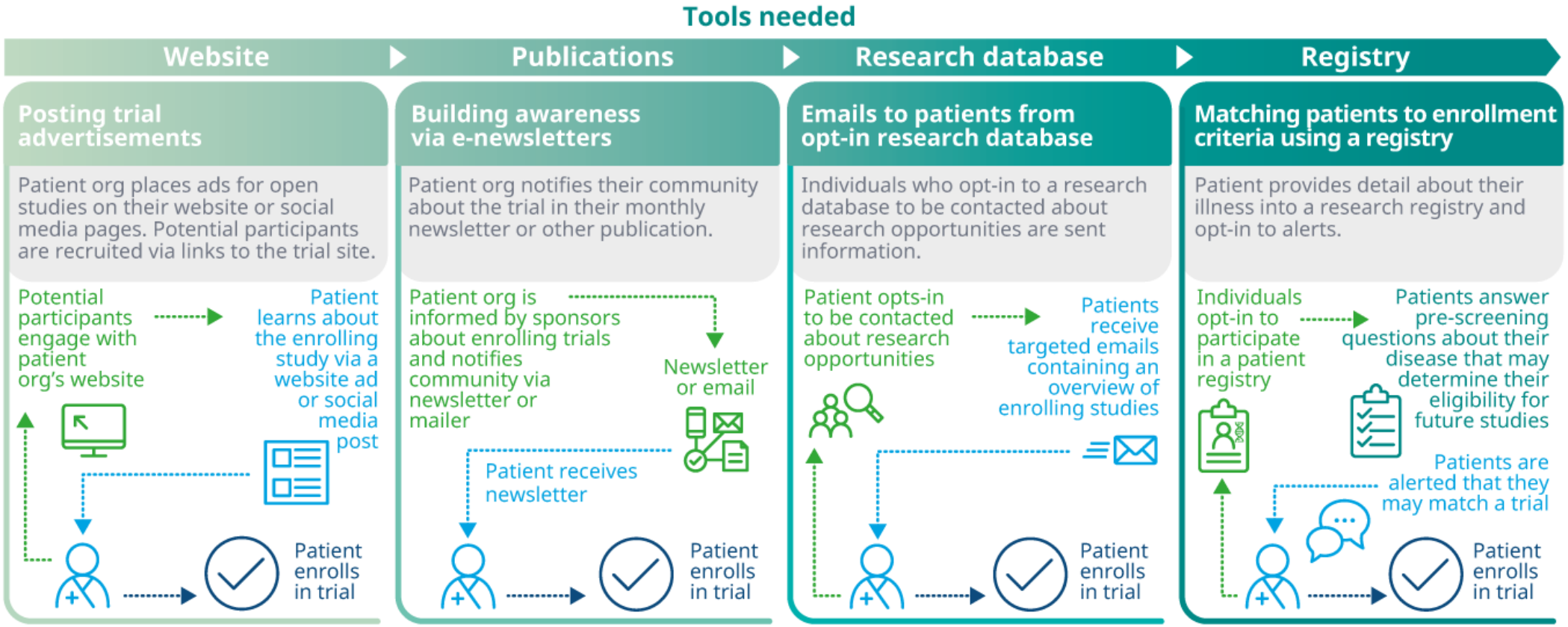
How patient organizations help trial enrollment succeed



Source: IQVIA Institute; Jul 2023.

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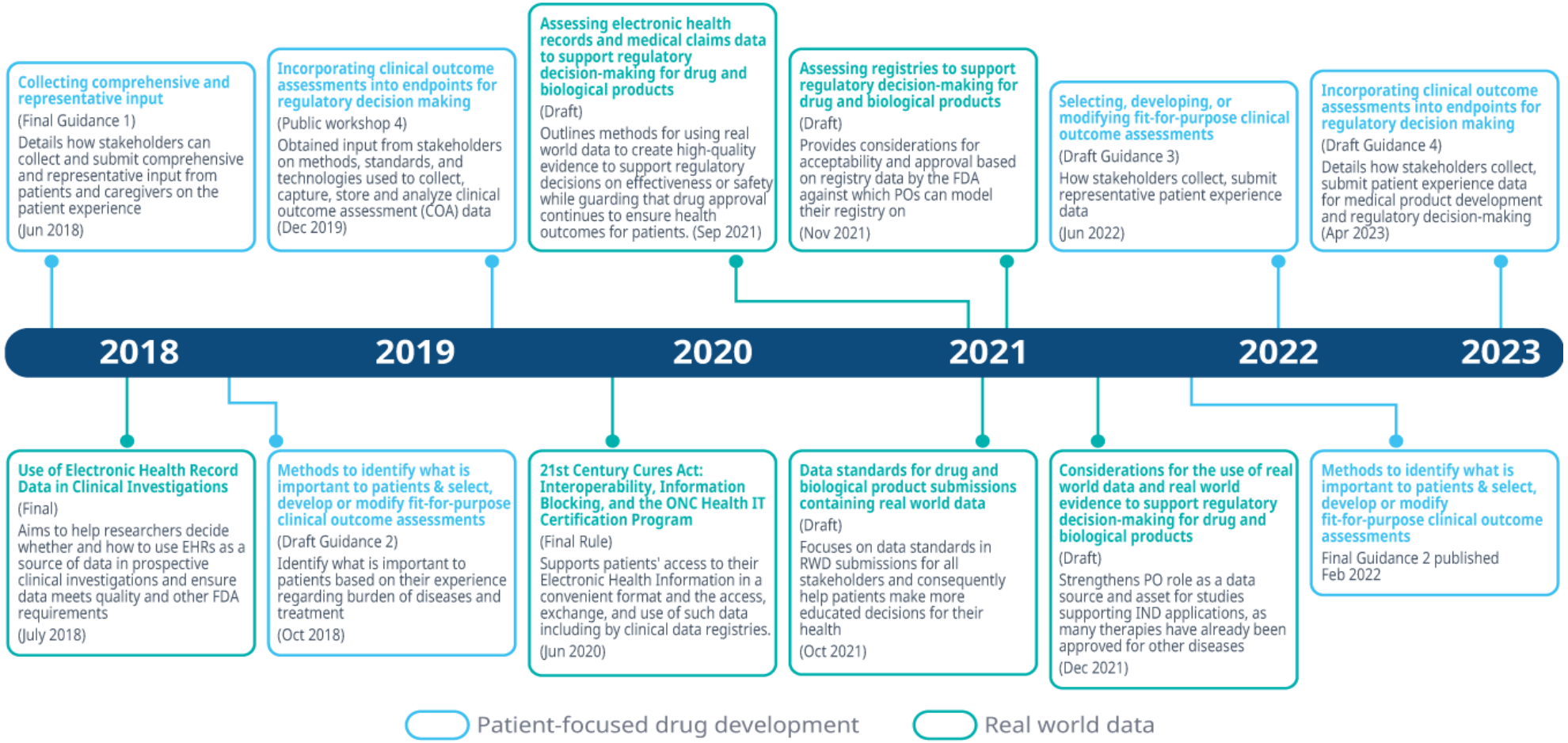
Timeline of patient-centric data guidance at the FDA



Source: FDA, 2023.
Notes: FDA = Food and Drug Administration; IND = investigational new drug application; NDA = new drug application; BLA – biologics license application.
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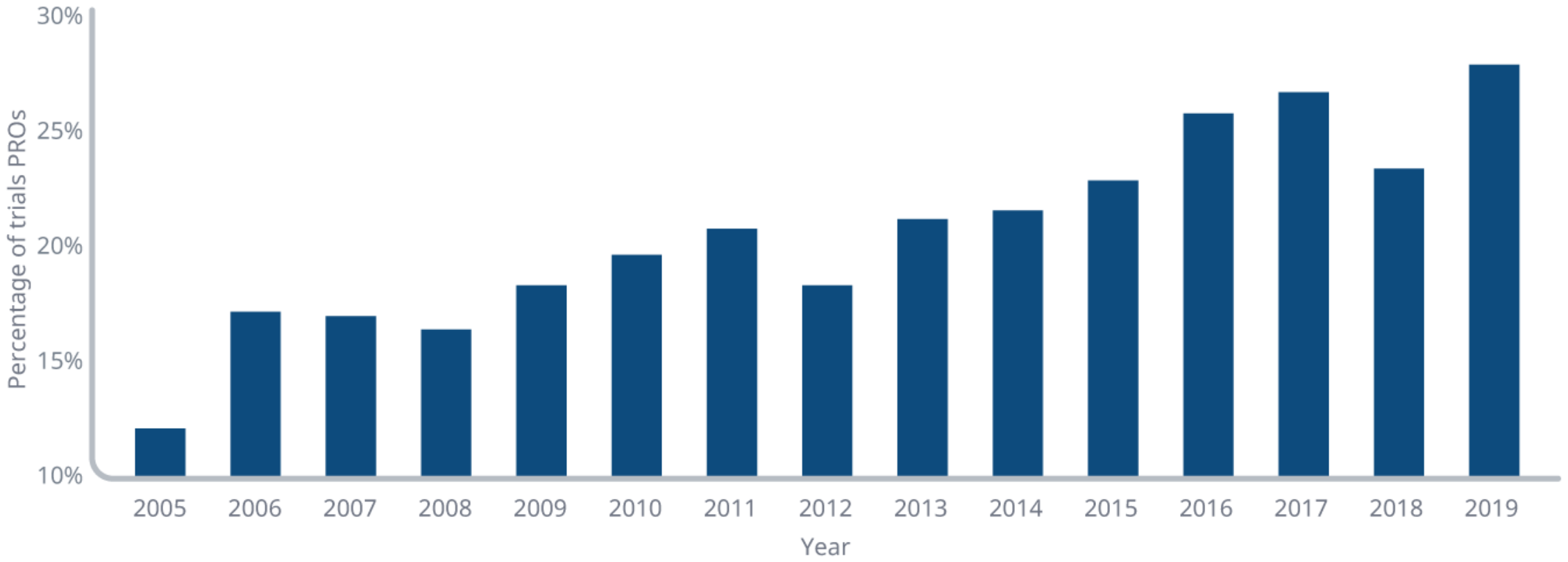
Inclusion of PROs in Phase III oncology rare clinical trials



Source: IQVIA PubMed review. 2019. Whitsett J, Reaney M, Lai L (2023). Integration of patient experience data (PED) into regulatory and payer decision-making. In Reaney M (ed.) Using Patient Experience Data to Evaluate Medical Interventions. Generating, understanding and using patient experience data within and alongside clinical trials. IQVIA. Pages 168–187.

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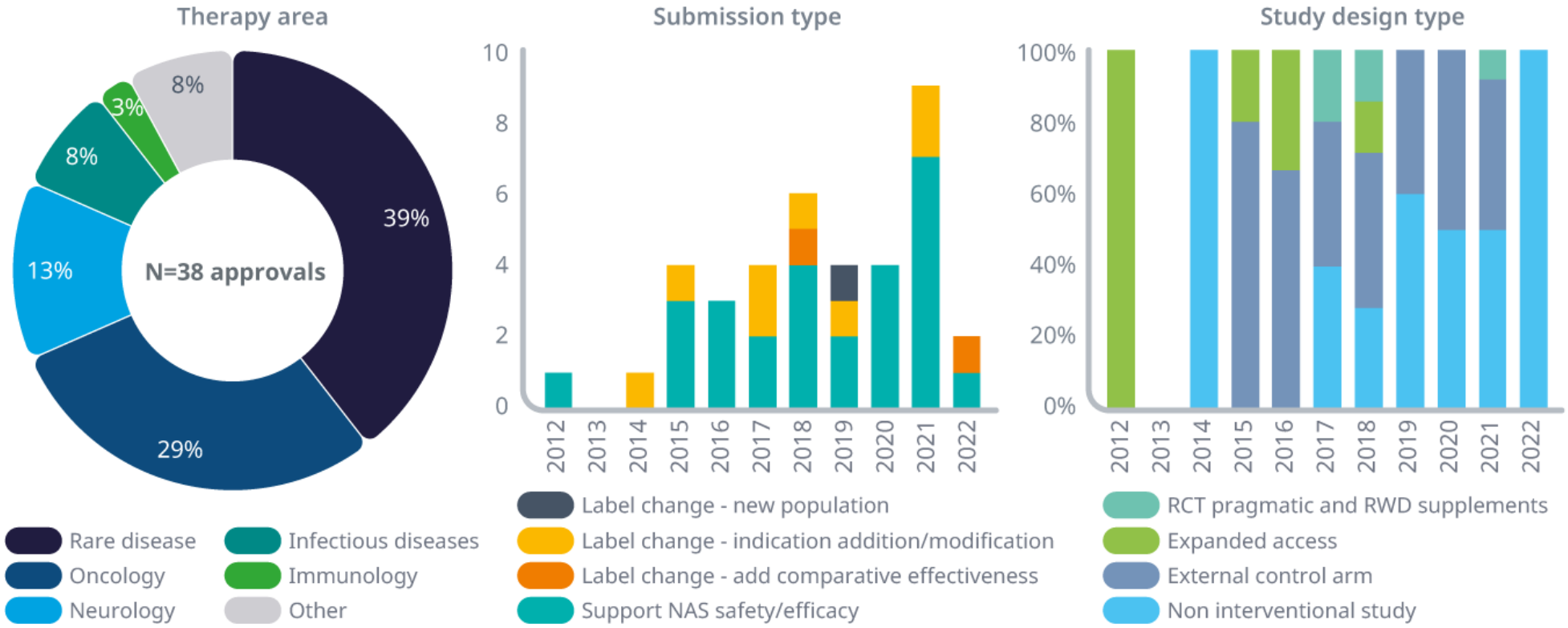
FDA approvals based on real world evidence (RWE), 2012–2022



Source: Global trends in R&D 2023: Activity, productivity, and enablers. Report by the IQVIA Institute for Human Data Science, Feb 2023; Citeline Trialtrove, IQVIA Institute, Jan 2023.

Notes: Collected from public sources relating to the approval trials for medicines. Data collected under a treatment IND or expanded access protocol has been considered a form of RWE by the FDA, such as in rare disease settings where there is little chance of a prospective trial. RWE approvals shown here include those granted after approval (e.g., carglumic acid 2010 RWE but drug was a 2006 launch). Analysis includes some double counting where a drug may have had more than one type of RWE design type or submission type.

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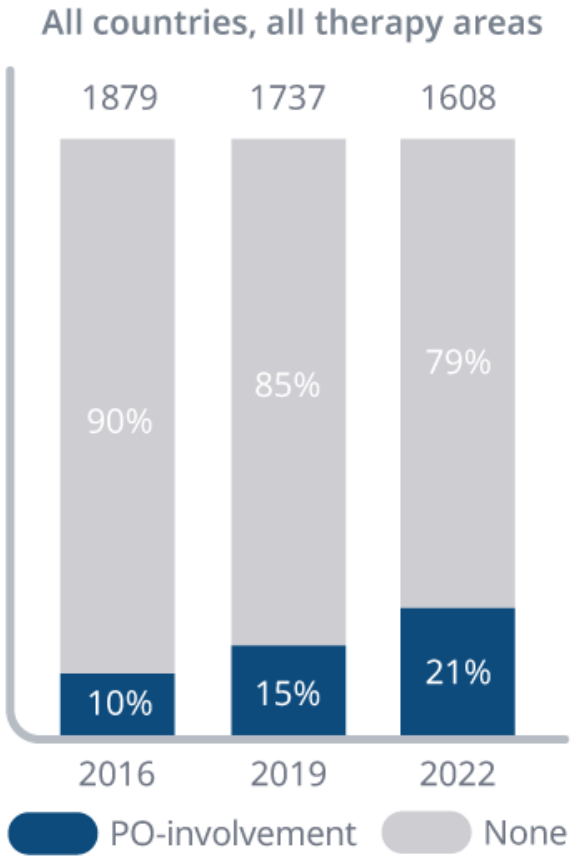
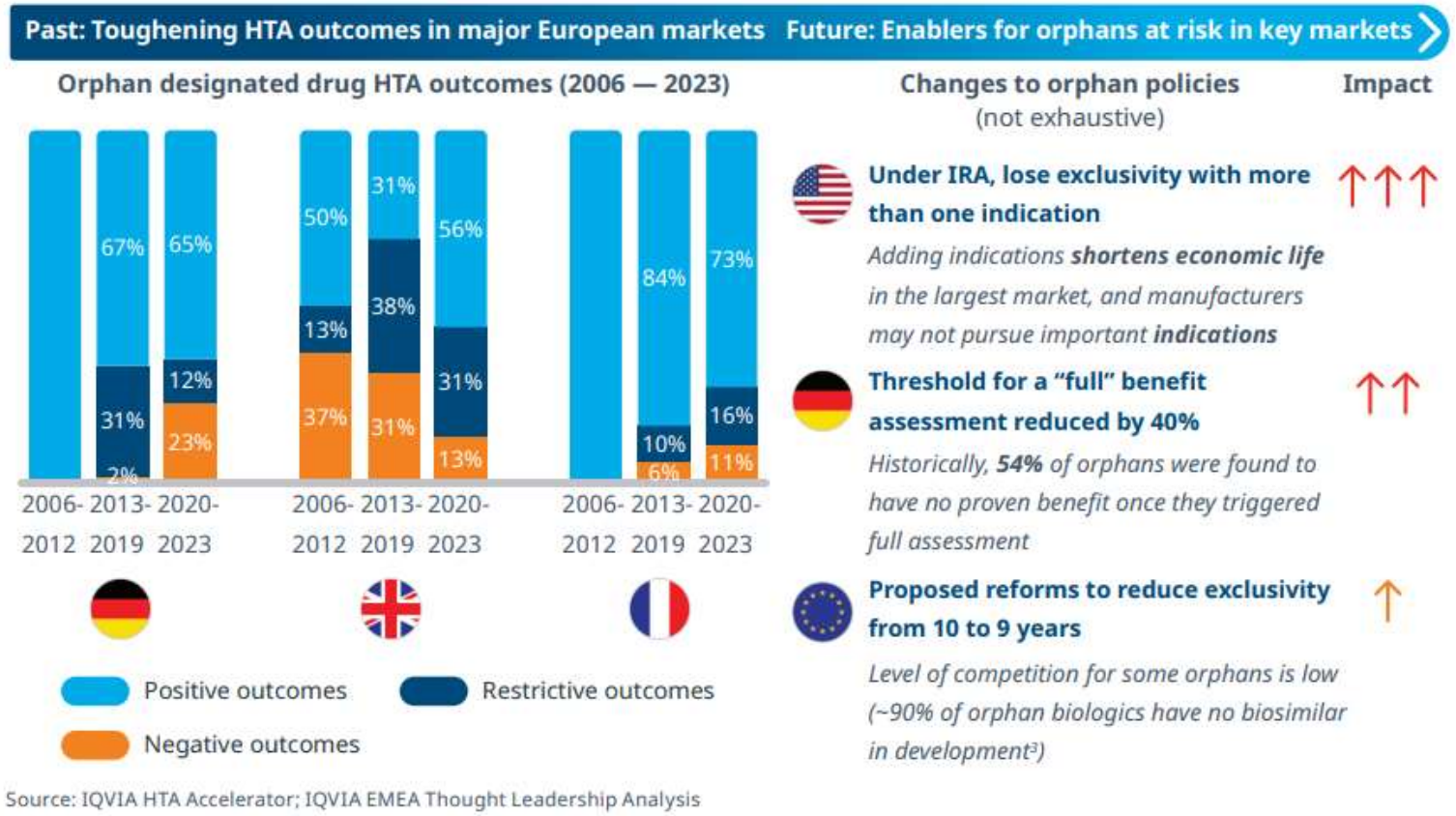
Patient organization contribution to health technology assessments across all countries and by therapy area in select EU and Commonwealth countries





Source: IQVIA HTA Accelerator; IQVIA EMEA Thought Leadership Analysis

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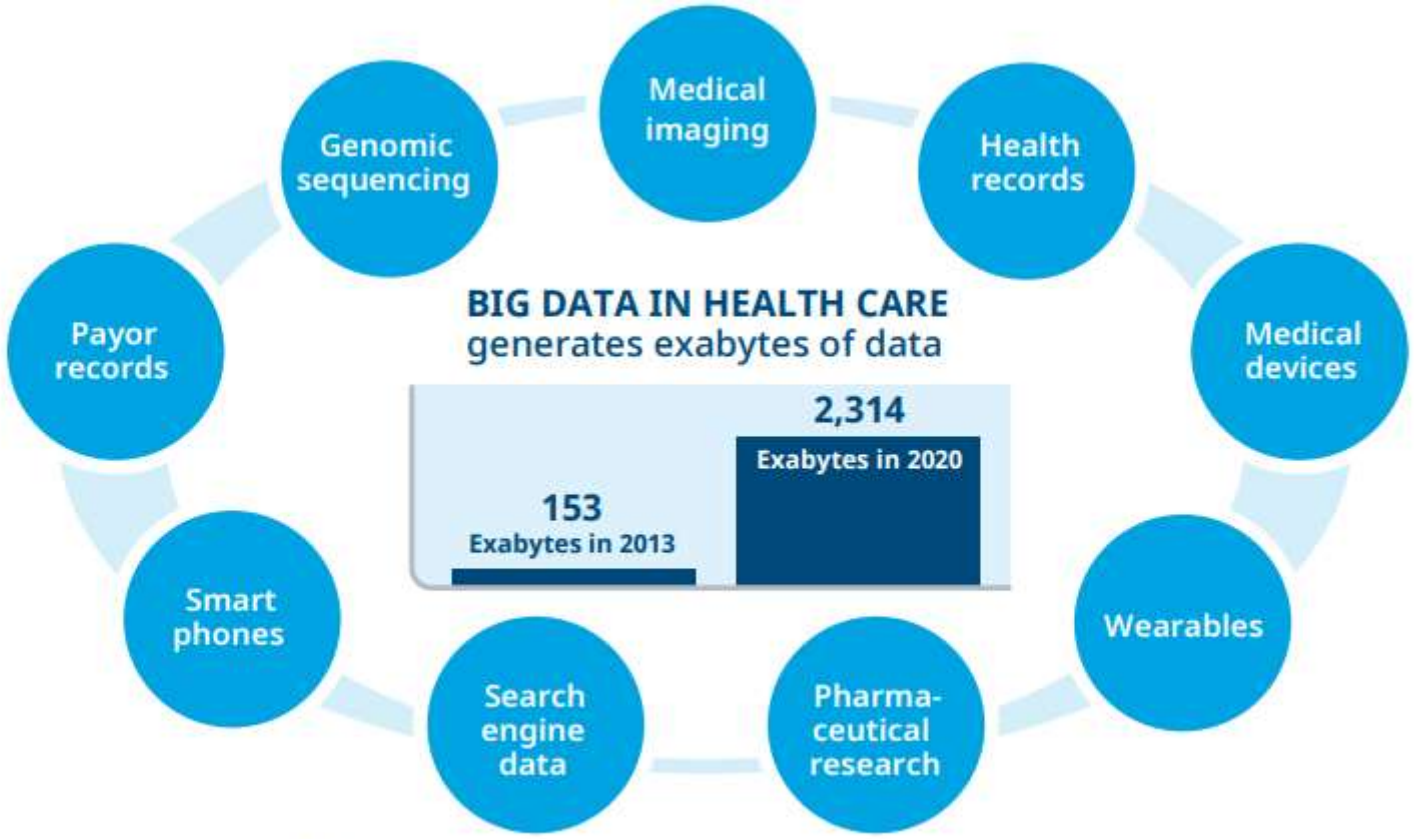
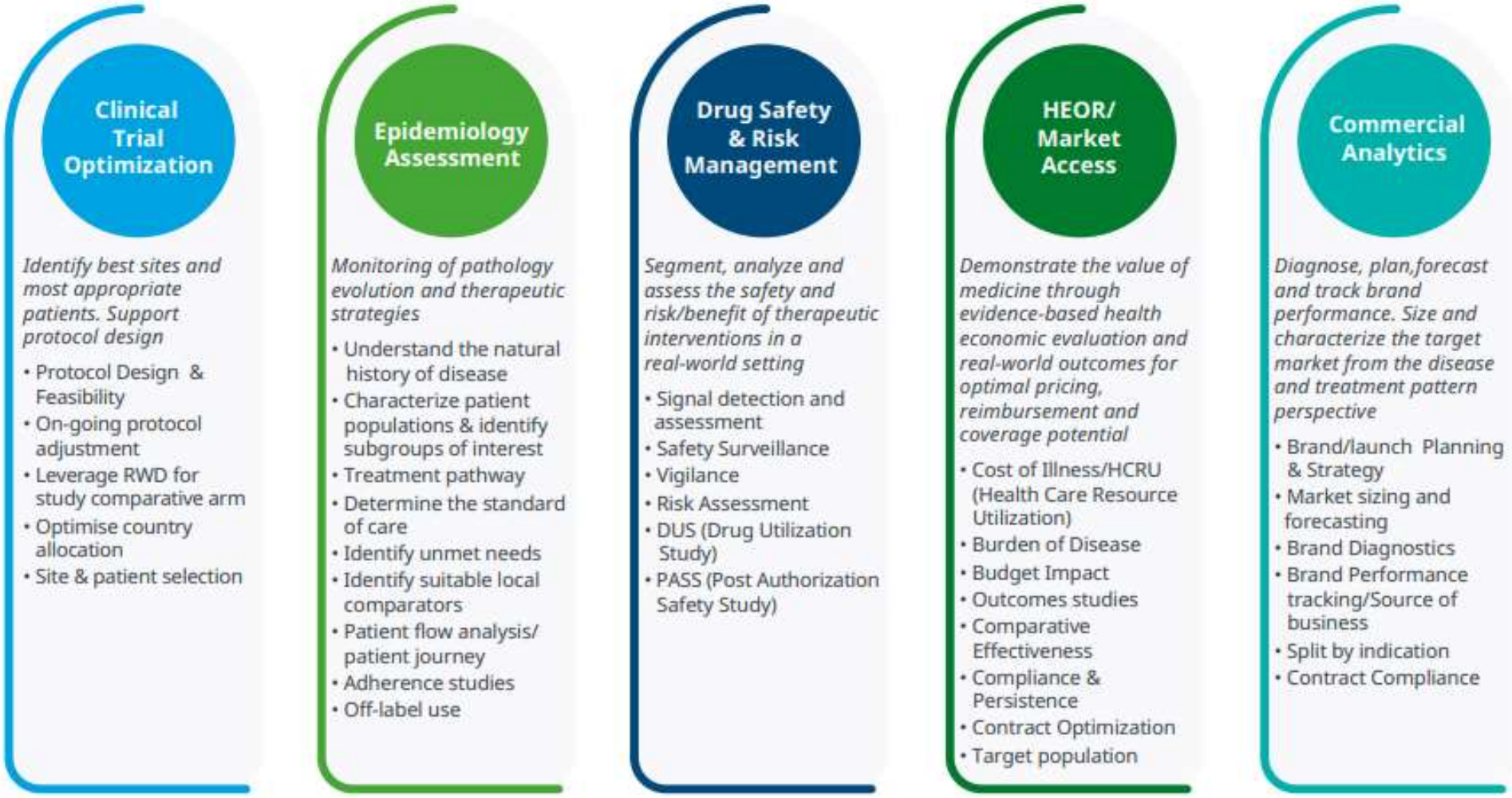
Real World Data sources



Source: "Harnessing the power of data in health".

Real World Data possible uses



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Examples of RWE throughout the product lifecycle

Increasing role of RWD to potentially generate insights or evidence through-out the product lifecycle

Examples uses in early development

- Evidence planning; Data strategy plan
- External Comparators
- Validate biomarkers and other potential outcomes
- Disease Registries
- Regulatory Intelligence to inform indication and dosage strategy

RWD for RWE post approval

- Demonstrate value & differentiation
- Labeling expansion
- Real-world safety & effectiveness
- Patient perspective/PRO
- Cost-effectiveness
- Implications for Rare Disease (Registry; SOC, Feed Recruitment of Ph2/3

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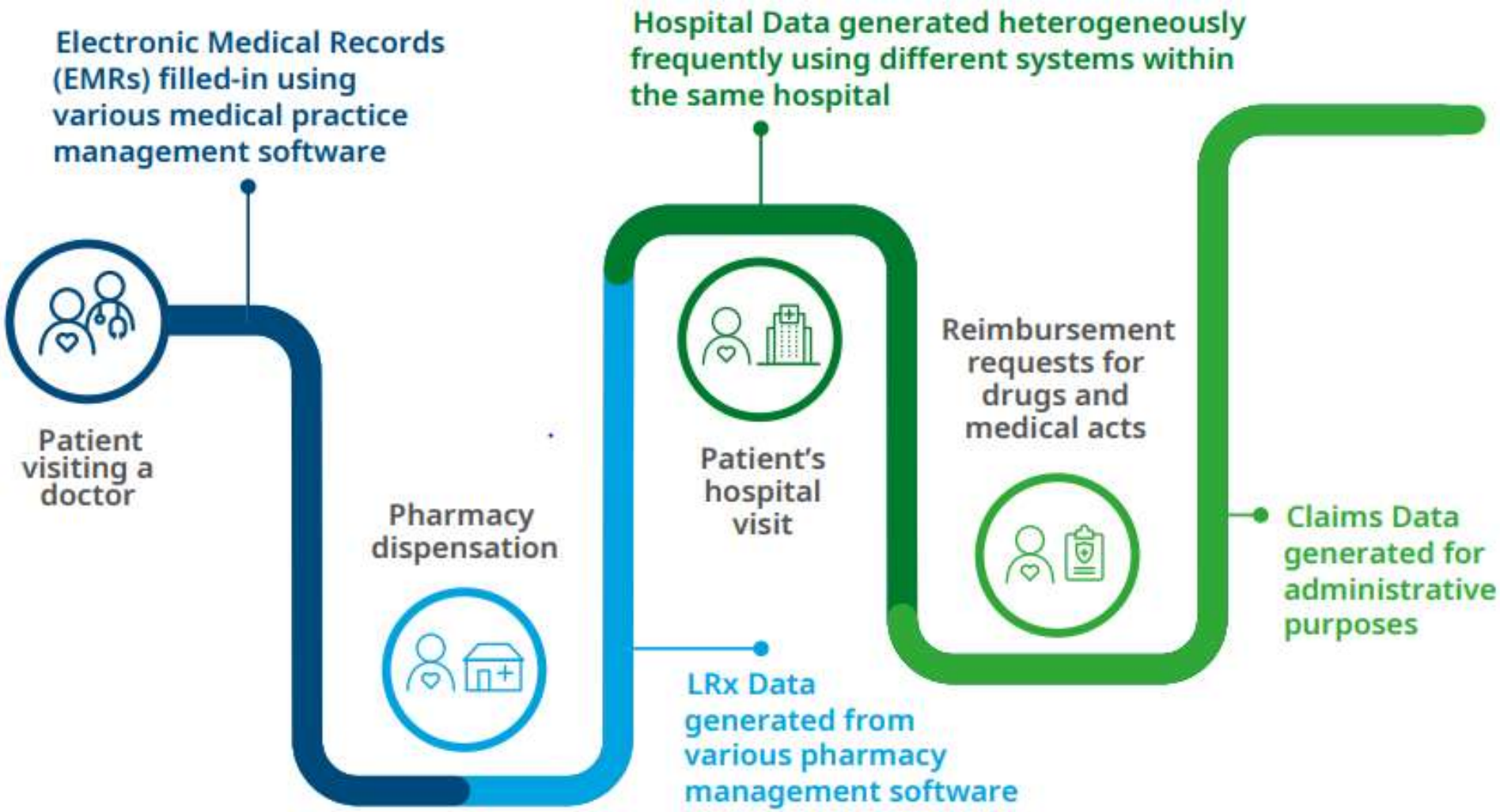
Data is generated for administrative-patient management purposes, not for analytical-scientific ones



Real World Data challenges - FAIR

Find

Need for:

- Assets profiled as per a Data Quality Framework
- dynamically updated / orchestrated syndicated catalog
- Robust processes to select fit for purpose data
- Data orchestration capabilities

Interoperate

Enable data sources comparability and interchange with clear understanding of applied data transformation and dissemination processes

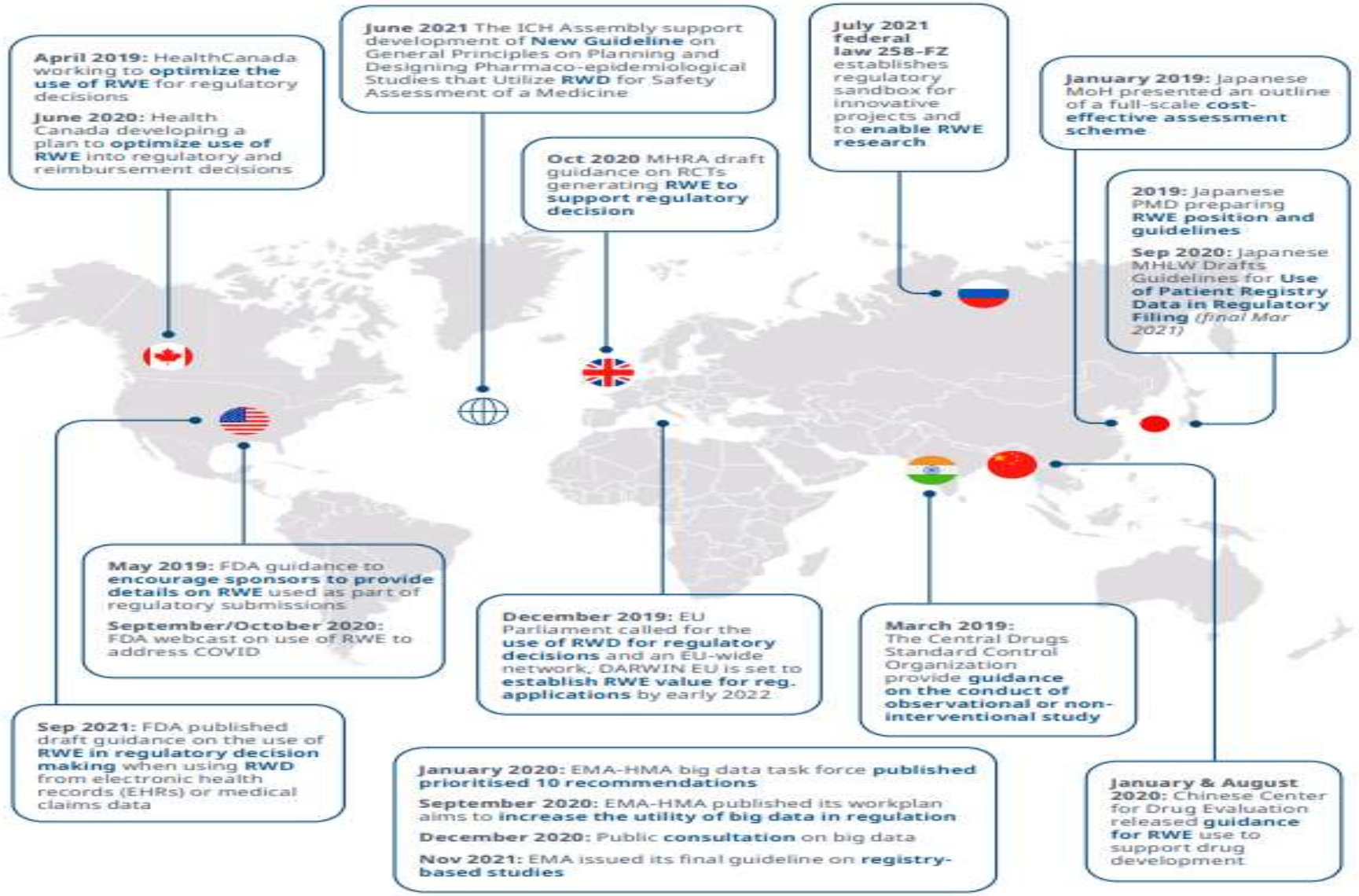
Access

Facilitate data access accross multiple settings in a transparent and compliant manner

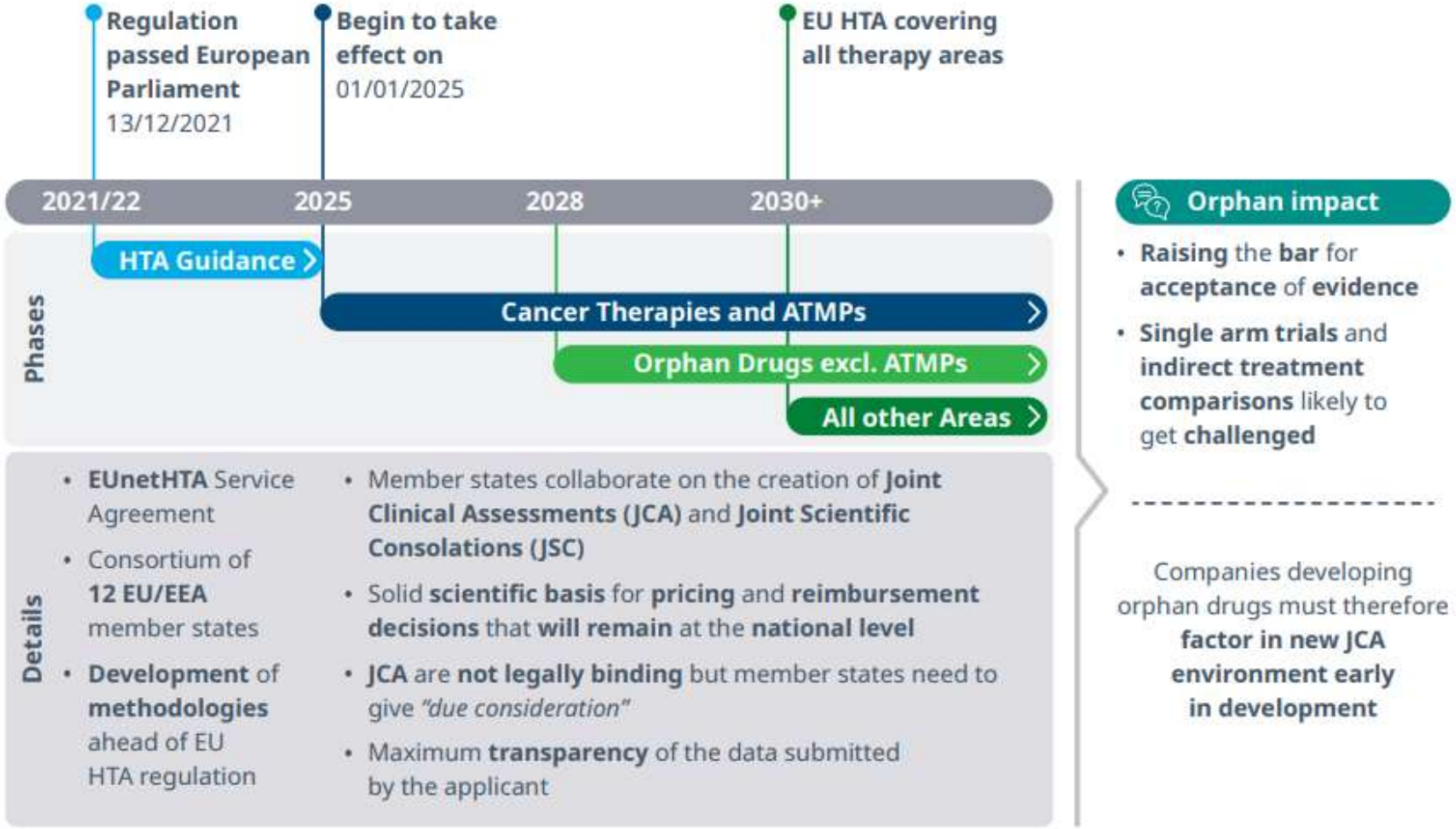
Reuse

After initial collection, reuse data in a compliant/transparent fashion via all statistical/data science to produce better outcomes

International examples



EU HTA and its orphan impact



EUnetHTA 21 consortium ended in September 2023 and the EU HTA coordination group and subgroups were set up, now awaiting the first of the four implementation acts to be put out for public consultation

Source: IQVIA EMEA Thought Leadership; IQVIA HTA Accelerator

Three pillars of rare disease launch excellence

Health system readiness

Understand the **patient journey** in the context of the **health system**:

- **Identify patients** and their doctors/caregiver early as they are **influential** for **treatment decision making**
- Consider **holistic access** to **healthcare**, including e.g., partnerships for transportation to hospitals or access to specialists
- **Improve diagnosis** by running e.g., **screening programs** and provide supporting materials for physicians
- **Leverage RWE** for patient finding, educating systems, doctors, payers, and HTA bodies about the disease

Stakeholder engagement

Great relationships with patients, caregivers, POs, external experts, treatment centers, regulators, and payers even more important:

- **Involve** and **engage key stakeholders early** for a **deep understanding** of patient and disease, which is crucial for therapeutic development and care pathway preparation
- **Collaborate** with **patients** and **PAGs** in early development for trial recruitment, designing patient-centered tools, and advocating for policy/reimbursement
- **Ensure medical-led engagement** from early discussions with regulators & payers to HCPs

Value and evidence

Start **evidence planning early**, particularly if few data sources, and **leverage RWE**:

- Develop a deep understanding of the **disease natural history**
- Use to inform and educate all stakeholders, including **early regulator** and **payor discussions**
- **Focus RCTs** on **patient-relevant endpoints** and **outcomes**, and include **PROs/QoL** as key metrics
- For **patient finding** (every patient matters)
- Inform and supplement **clinical trials** (e.g., with a control arm)

Source: IQVIA EMEA Thought Leadership

Addressing the three populations of rare disease opportunity

Start with the end in mind to maximise the treated population

Label population

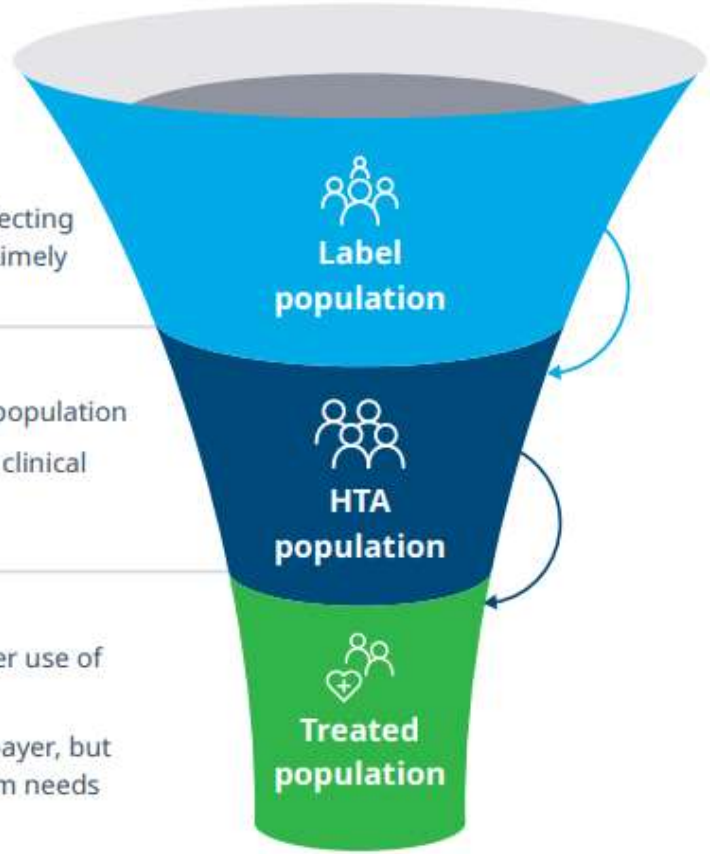
- Clinical development effectiveness, and label
- Getting an optimal label approved, reflecting the clinical potential of a product, in a timely and efficient fashion

HTA population

- Minimise the gap to ensure large HTA population
- Early stakeholder engagement around clinical trial design

Treated population

- Integrated evidence strategy with better use of health system data
- Focus on satisfying the regulator and payer, but also patient, HCP and healthcare system needs



Source: IQVIA EMEA Thought Leadership

Thank you!