Life Science Leadership Summit: *Use of Real World Evidence to maximize asset value*

20th June 2022

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Welcome

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IQVIA capabilities

100+ Countries

60+ Years of experience

Helped develop **50% of all FDA approved drugs** launched by **EBP companies in 2020**

79,000+
Employees

1,650+
MDs

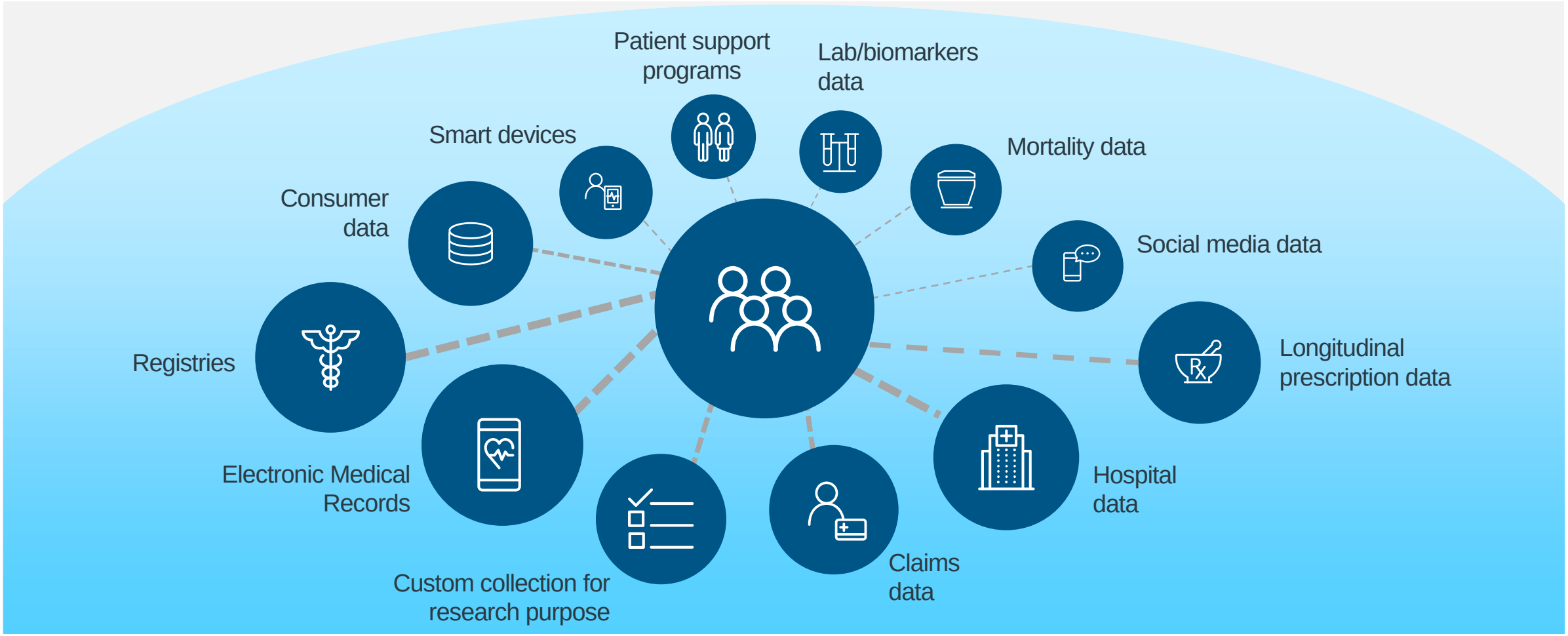
2,000+
PhDs

565 clinical studies in **77 countries**
for EBP companies in the last 5 years

4,600+
Data scientists

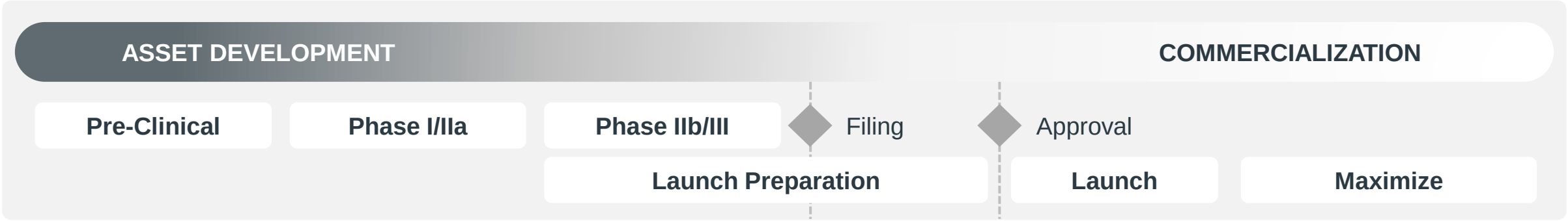
Real World Evidence (RWE)

RWE is a supplement to, not a replacement of, randomized control trials (RCT)



RWE maximizes value across the asset lifecycle

Accelerating clinical development and *supplementing RCT evidence package*



Natural History

External Comparators

Long term follow up (LTFU)

Data informed trial design

Expanded Access

Post authorization safety study (PASS)

AIML and RWD enabled site identification

Budget impact model and ongoing HEOR

Data-enabled patient recruitment

On-going medical evidence

Predictive analytics to inform physician and patient targeting and support

RWE accelerating clinical development

Proof not promises

Data informed protocol assessment

AIML and RWD site identification

Data-enabled patient recruitment

91% protocols revised to mitigate risks prior to startup[1]

33% Reduction in Site ID timeline vs historical benchmark[1]

34% faster recruitment rates vs historical benchmark[1]

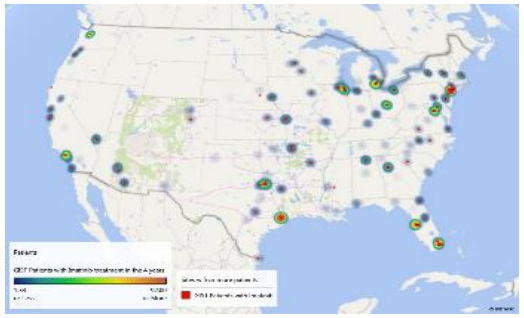
1. IQVIA trial data on file as of Sept 30,2021

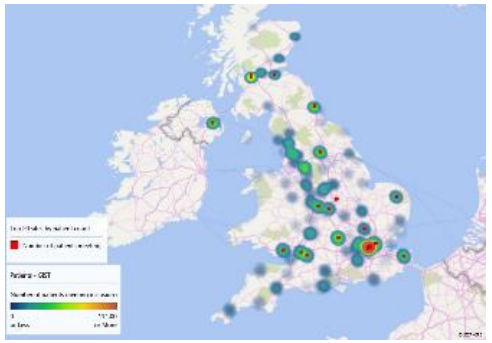
Acronyms: AIML, Artificial Intelligence and Machine Learning; RWD, real world data

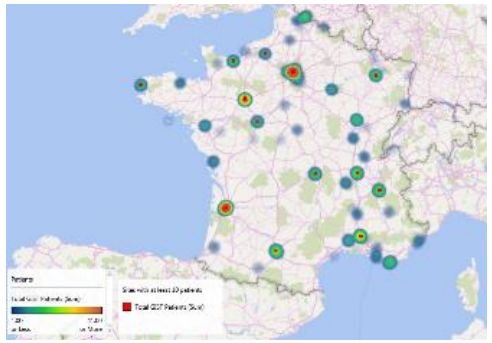
CASE STUDY
Phase 3 pivotal trial

Study benefits from Connected Intelligence

Productivity and efficiency improved through faster recruitment and fewer non-enrolling sites



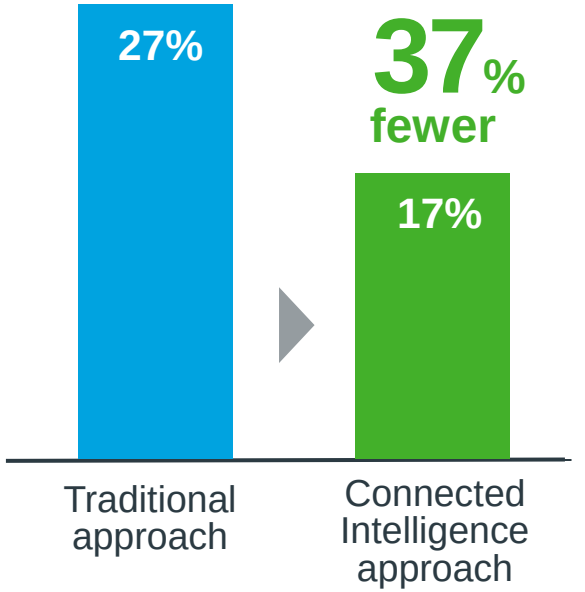




Patient density and site locations

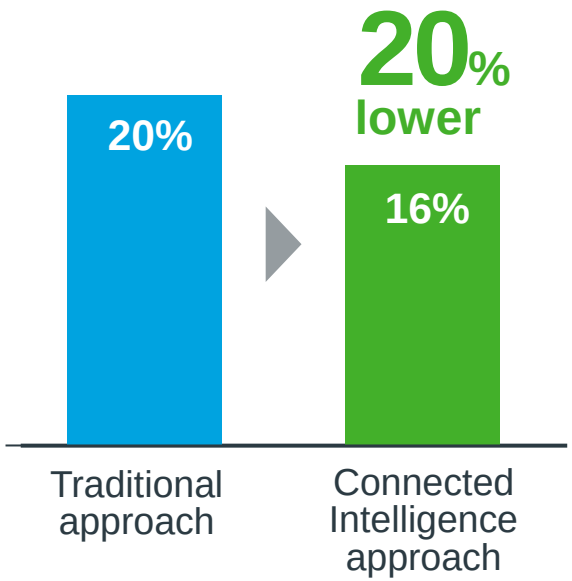
Fewer Non-Enrollers

Non-enrolling Sites



Lower Screen Failure

Screen Failure Rate (Patients / Site / Month)



Recruitment completed ~5 weeks ahead of schedule

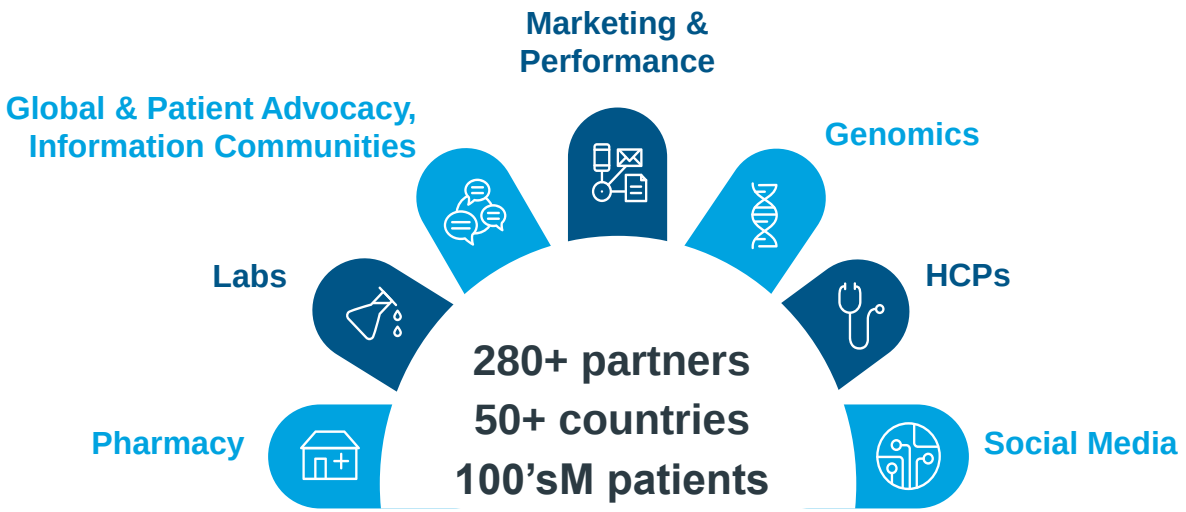
Data driven patient outreach to improve diversity in clinical trial participants

Pressure tested by COVID-19

Differentiated, connected solutions

- Global RWE, precision patient targeting
- Direct-to-patient recruitment
- Diversity in clinical trials
- Patient engagement portal
- Site tech enablement
- Decentralized trials

Integrated healthcare network



450K+

direct-to-patient referrals

105K+

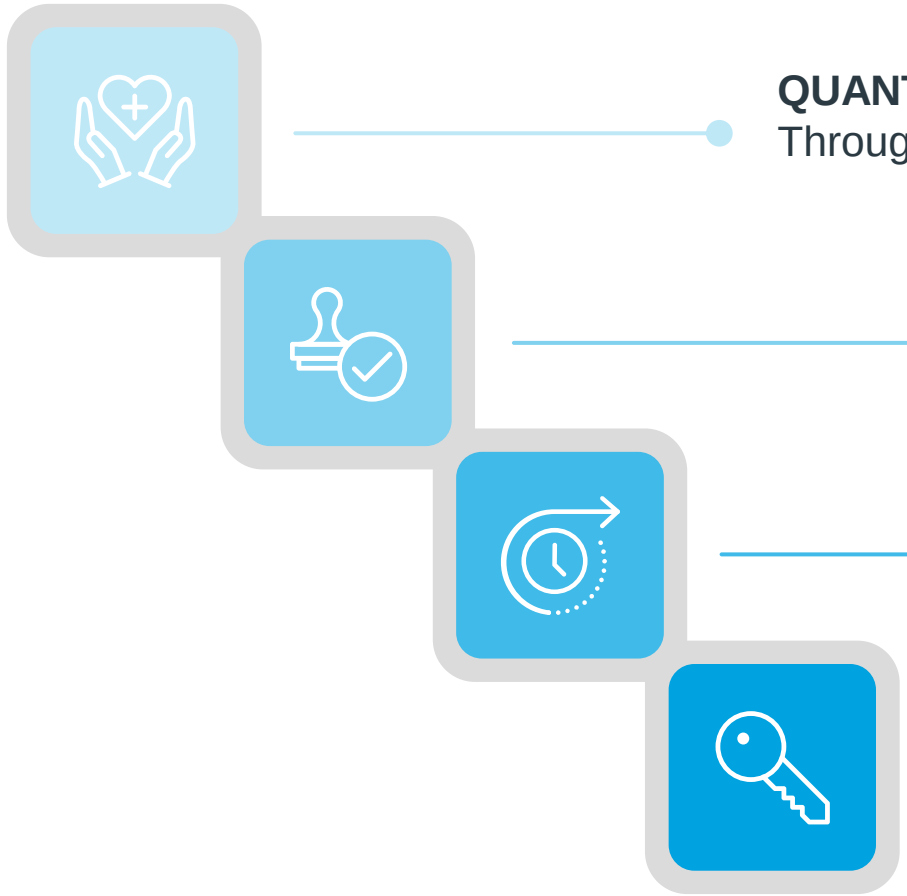
patients enrolled in vaccine trials within 4-6 weeks per trial

49%

Meaningfully larger diverse populations enrolled in COVID-19 vaccine trials versus peers[1]

1. Emergency Use Authorizations (EUAs) Filings for Vaccines and Related Biological Products Advisory Committee Meetings.

RWE supplementing RCT evidence package



QUANTIFY UNMET NEED AND BURDEN OF DISEASE
Through natural history of disease studies

SUPPORT REGISTRATION OR LABEL EXPANSION
With external comparator

ENABLE LONG-TERM FOLLOW-UP OF NOVEL TREATMENTS
Particularly for cell and gene therapy

IMPROVE PRICING, ACCESS AND LAUNCH
Through RWE input into HTA submissions

CREATE A STRONGER EVIDENCE PACKAGE TO MAXIMIZE ASSET VALUE

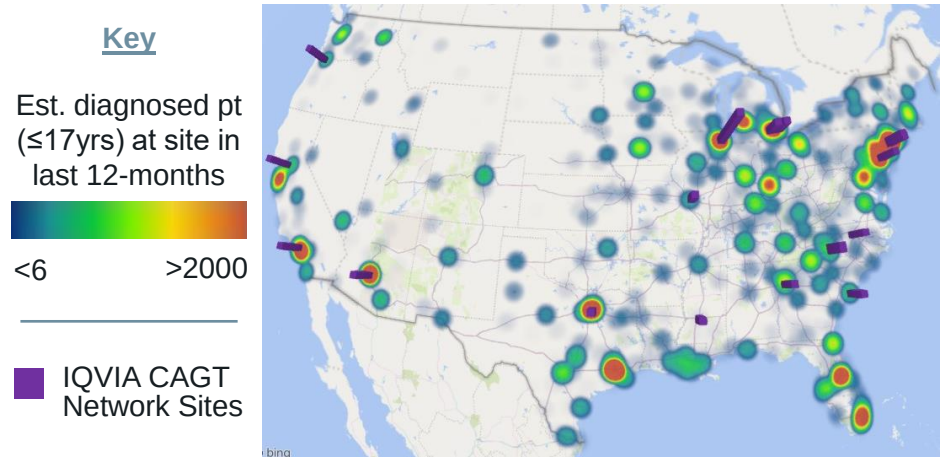
CASE STUDY
Natural History of Disease

Combined phase I and natural history of disease study

500 patients
Natural History program

10 patients
Phase 1/2 CAGT study

Site Targeting with Data-Driven Insights and IQVIA CAGT network



Challenge:

Client launching phase 1 gene therapy trial wanted to better understand their target population and their burden of disease

Solution:

A combined natural history of disease (NHOD) and Phase 1/2 study program

Value:

- NHOD describes the patient population and their disease burden globally
- Combined program provides resource efficiencies
- NHOD informs the design of the pivotal phase 3 trial
- Patients can be rolled over from the NHOD study to the Phase 3 trial

Regulatory decisions informed by RWE[1]

Selected examples

				RWE	FDA		EMA	NMPA	PMDA
					Approval	Expansion	Approval	Expansion	Expansion
Pragmatic Randomized	Janssen	Paliperidone palmitate	Schizophrenia	Primary data collection on time to relapse		2018			
External Comparator	AMGEN	Blinatumomab	B-cell precursor acute lymphoblastic leukemia in 1st/2nd complete remission w min. residual disease	EMR		2018	2019		
	AMGEN	Denosumab	Post menopausal women with osteoporosis at high risk of fracture	RWE from Taiwan and Hong Kong				2020	
		Abatacept	Prophylaxis of acute Graft vs Host disease in combo w CNI and MTX in hematopoietic stem cell transplant	Registry-based effectiveness study submitted w RCT		2021			
	Genentech	Bevacizumab	Metastatic or recurrent squamous NSCLC in combination with platinum-based chemotherapy	Medical record review in 3 hospitals				2020	
	MERCK Pfizer	Avelumab	Metastatic Merkel cell carcinoma	European Registry and US EMR	2017 Accelerated		2017 Conditional		
	NOVARTIS	Onasemnogene abeparvovec-xioi	Pediatric (<2 yo) spinal muscular atrophy	Natural history comparator	2019 Fast Track				
Linked Data	astellas	Tacrolimus	Adult and pediatric lung transplant recipients	Transplant registry linked to SSA death records		2021			
	CHUGAI	Pertuzumab+ trastuzumab	HER2+ colorectal cancer progression	Nationwide cancer genome screening project + ex-Japan linked EMR					2022
Other RWE	Pfizer	Palbociclib	HR+, HER2- advanced/metastatic breast cancer in males	AE reports, EMR, health insurance claims		2019			

1. Medical products approved with the requirement of post-approval RWE safety studies not shown here

External comparator during clinical program to unlock further funding

CASE STUDY
Investment Support

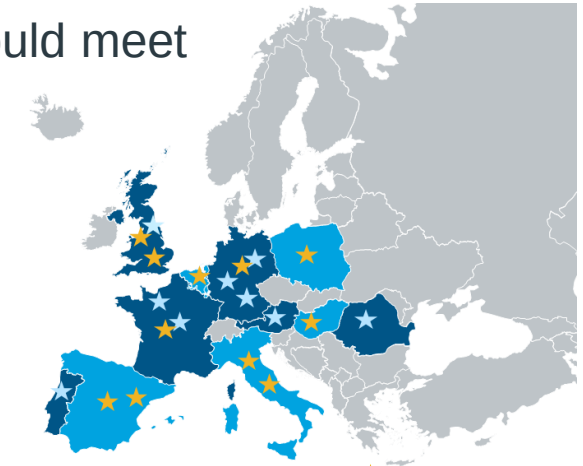
Situation:

- Client was a small biopharma company
- Key asset is in phase 2 single arm trial scheduled to run until 2029
- Wanted to demonstrate value of asset to investors to unlock additional funding

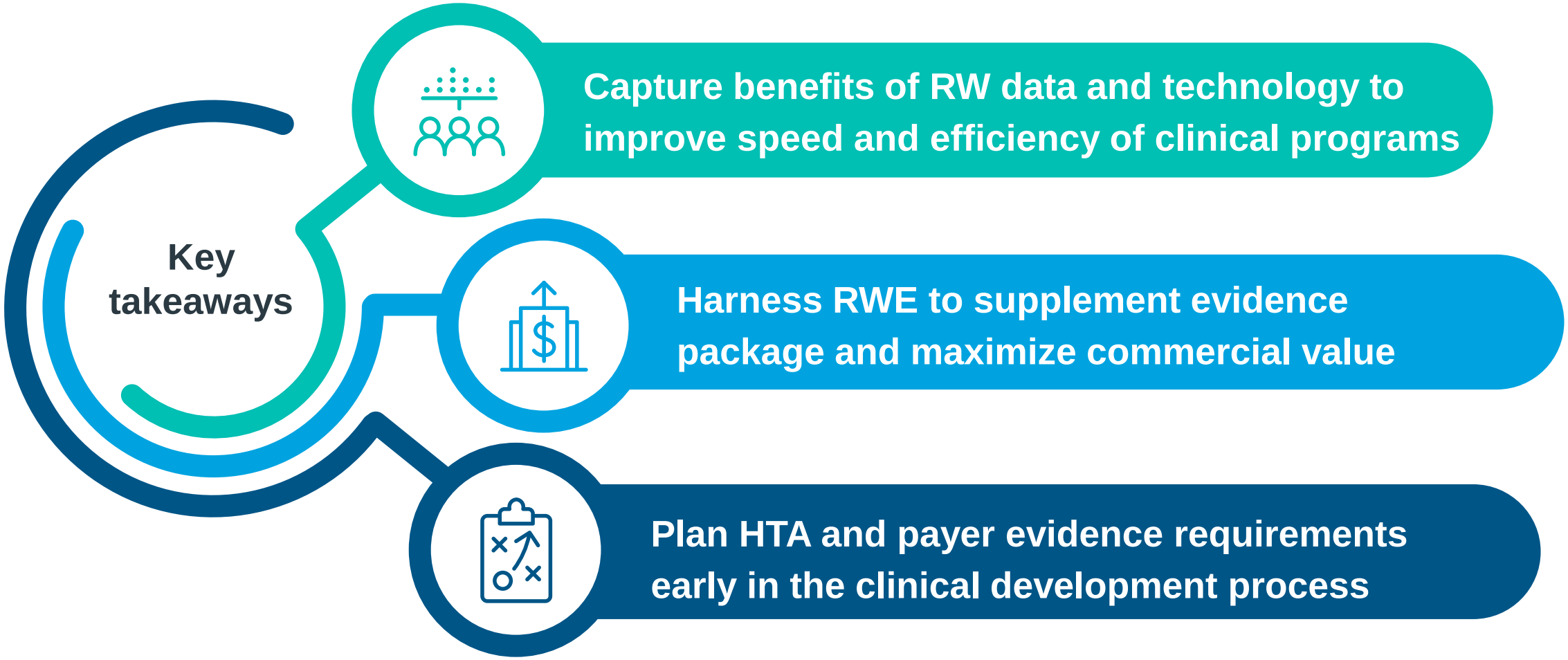
Solution:

- An External Comparator study using IQVIA's Oncology Evidence Network (OEN) provided a fast and cost effective way to demonstrate value
- Two hospitals within IQVIA's OEN were selected as data sources
- Study design was developed in close partnership with client team to ensure the outputs would meet their specific requirements

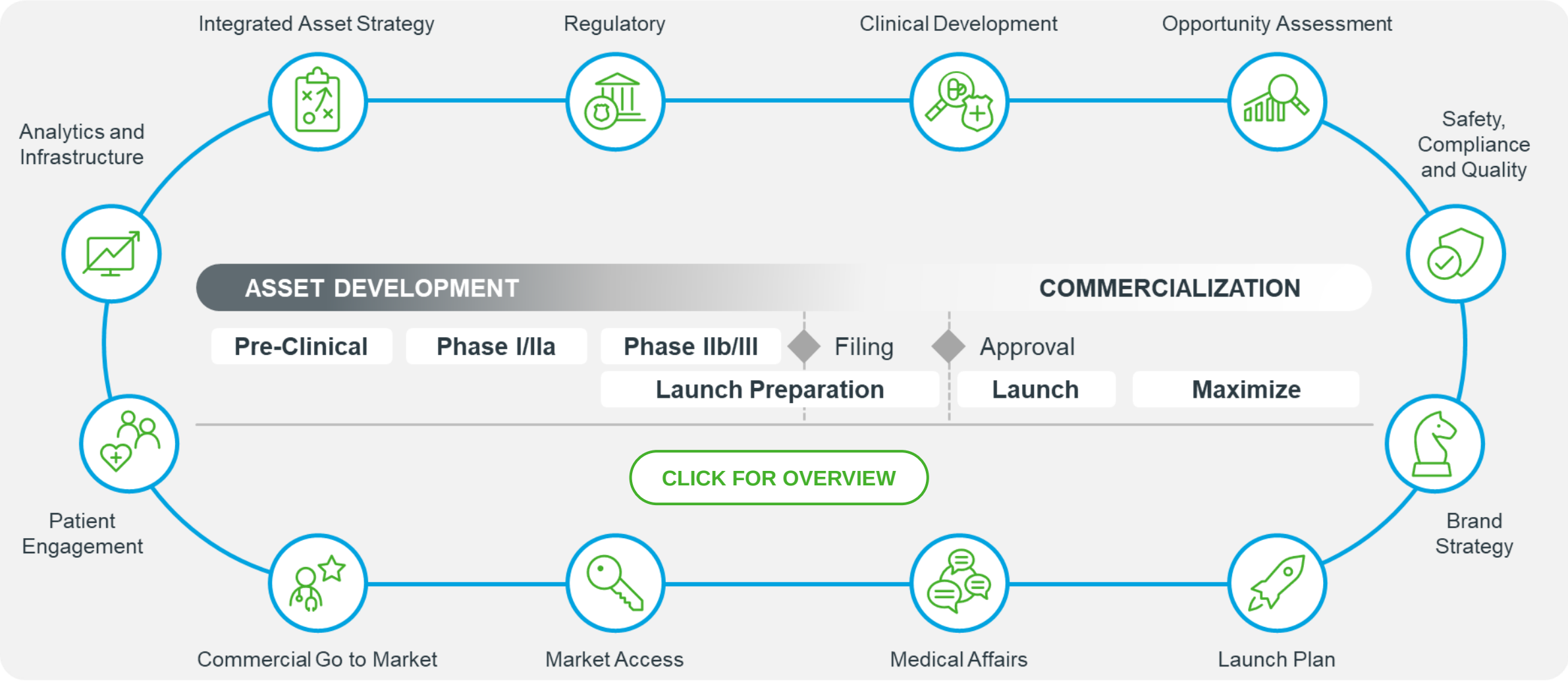
IQVIA's Oncology Evidence Network



- Less than 1 year from initiation to delivery of study report
- Less than €250k study budget
- Enable fundraising discussions
- Contextualize phase 2 trial results



IQVIA Asset Maximizer



Appendix

IQVIA, unique capabilities at scale

4,600+
Advanced analytics / data scientists / statisticians

1,900+
Epidemiologists/RWE experts

1,650+
Medical doctors

8,200+
Software development / support

1.2B+
Non-identified patient records

85%
Global pharma sales tracked

56+
Petabytes of unique data

300+
Life sciences-specific analytic libraries

100+
Countries

50K+
Pharmacy and wholesaler partners

5M+
Clinical trial investigators

100M+
Patient network for trial recruitment

2,000+
Hospital partners

100B+
Records searched in real-time

150+
Patent-pending methodologies

30+
Predictive disease detection solutions

IQVIA Real World Solutions industry-leading capabilities

Unmatched domain expertise

5,000+
Real World Specialists[1]

5,700+
publications / articles[2]

16
therapeutic centers of excellence[3]

Unparalleled data assets

1B+
unique RWD records[4]

30M terms[5]
coded in
200+
ontologies[6]

Operational excellence and scale

Voted #1
preferred Phase IV provider[7]

1M+
patients enrolled in Real World studies[8]

Connectivity with healthcare

23M
HCPs[9]

2,000+
hospitals in networks

20+
Countries with direct healthcare businesses

Best-in-class technology and analytics

Leading eCOA
Platform[10,11]

Award winning
Natural Language Processing[12]

120+
validated AIML models[13]

(1) IQVIA RWS Headcount - Sep'21.
(2) IQVIA Bibliography portal.
(3) IQVIA RDS Therapeutic COEs.
(4) IQVIA Global patient counts Jan'21.
(5) UMLS 2021AB statistics & IQVIA analysis.
(6) OHDSI vocabulary statistics & IQVIA internal analysis.
(7) Industry Standard Report, CRO Quality Benchmarking – Phase IV Service Providers, May 2020.
(8) IQVIA internal analyses.
(9) IQVIA One Key Jul'21.
(10) Winner of Fierce Innovation Awards – Life Sciences Edition 2020 in the Digital Health Solutions category
(11) Industry Standard Report, eCOA/ePRO Market Dynamics and Service Provider Performance, 2020.
(12) Questex's 2019 Fierce Innovation Awards — LifeSciences Edition in the Data Analytics/Business Intelligence category.
(13) IQVIA AIML inventory Sep'21.

Why is RWE important?

Recent examples of stakeholder acceptance of RWE

Feb 2021: Germany HTA (G-BA) issued first mandate for product to collect RWE

May 2021: Spain's MoH launches Valtermed initiative, a shared information system within the National Health System that collects RW data to allow measuring health results of drugs

Aug 2021: EMA-HMA BDSG Workplan 21-23 to incorporate RWD into reg work

Oct 2021: EMA Guideline on registry-based studies

May 2022: EC proposal for regulation for EHDS incl. secondary use of electronic health data

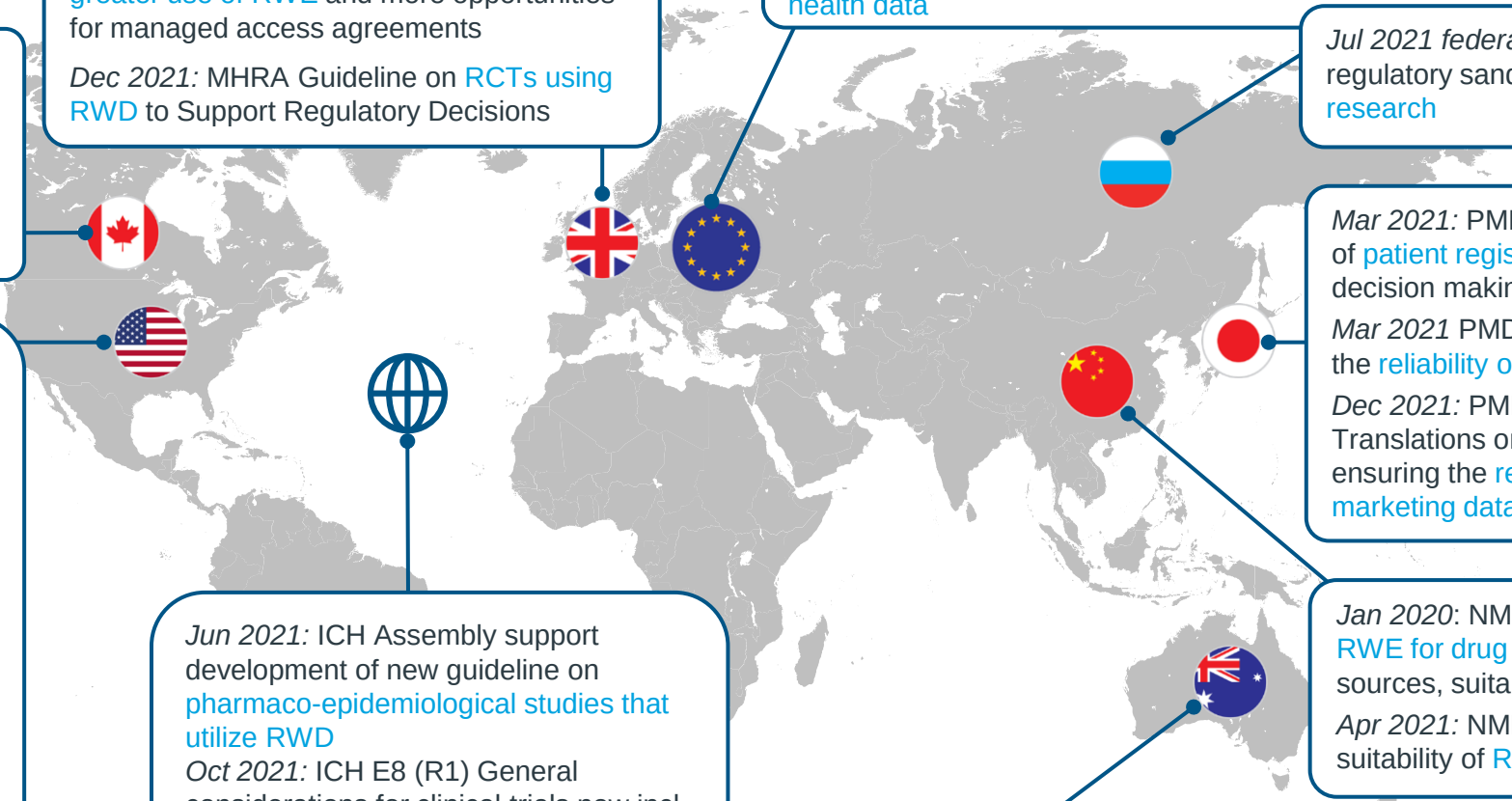
Aug 2021: NICE proposes several changes to its methods and processes including greater use of RWE and more opportunities for managed access agreements

Dec 2021: MHRA Guideline on RCTs using RWD to Support Regulatory Decisions

Mar 2019: HC Framework: Elements of RWD/RWE Quality Throughout the Product Life Cycle

Apr 2019: HC Framework: Optimizing the use of RWE to Inform Regulatory Decision Making

Jul 2021 federal law establishes regulatory sandbox to enable RWE research



Mar 2021: PMDA guidance: The use of patient registry data for regulatory decision making

Mar 2021 PMDA guidance: Ensuring the reliability of registry data

Dec 2021: PMDA Provisional Translations on points to consider for ensuring the reliability of post-marketing database studies

Sept 2021: RWD: Assessing EHR & Medical Claims Data to Support Regulatory Decision-Making

Nov 2021: FDA draft Guidance Assessing Registries to Support Regulatory Decision-Making

Nov 2021: US Congress Cures 2.0 Act draft Sec 304. Establishment RWE Task Force / Sec 309. Post-approval study requirements for accelerated approval using RWE

Dec 2021: FDA draft Considerations for the Use of RWD/RWE To Support Regulatory Decision-Making

Jun 2021: ICH Assembly support development of new guideline on pharmaco-epidemiological studies that utilize RWD

Oct 2021: ICH E8 (R1) General considerations for clinical trials now incl. use of RWD, external controls, and enriched studies

Jan 2020: NMPA Guidance: use of RWE for drug dev covering RWD sources, suitability & reliability

Apr 2021: NMPA Draft principles on suitability of RWD to generate RWE

Nov 2021: TGA Real world evidence and patient reported outcomes in the regulatory context

FDA use of RWE in New Drug Applications (NDAs) and Biologic License Applications (BLAs)

January 2019 - June 2021

- 116 approvals among the 136 (85%) included RWE in any form
- Increased approvals including an RWE study from 2019 to 2021, from 38 in 2019 to 25 in H2 2021
- A high proportion supplied RWE with the intent to provide evidence of product safety or effectiveness (65%)
- 83 of 136 (61%) used RWE studies with the intent to provide therapeutic context

Included NDAs and BLAs	2019 *n* = 51 approvals	2020 *n* = 59 approvals	2021 through June 30 *n* = 26 approvals	Total *n* = 136 approvals
Incorporated RWE for any purpose	38 (75%)	53 (90%)	25 (96%)	116 (85%)
Used RWE to provide therapeutic context	25 (49%)	36 (61%)	22 (85%)	83 (61%)
Used RWE to support safety and/or effectiveness	27 (53%)	46 (78%)	15 (58%)	88 (65%)
Safety only	17 (33%)	21 (36%)	5 (19%)	43 (32%)
Effectiveness only	7 (14%)	6 (10%)	2 (8%)	15 (11%)
Safety and effectiveness	3 (6%)	19 (32%)	8 (31%)	30 (22%)

Categories are not mutually exclusive.

BLA, biologics license application; NDA, new drug application; RWE, real-world evidence.

Source: Purpura CA, Garry EM, Honig N, Case A, Rassen JA. The Role of Real-World Evidence in FDA-Approved New Drug and Biologics License Applications. Clin Pharmacol Ther. 2022 Jan;111(1):135-144. doi: 10.1002/cpt.2474. Epub 2021 Nov 22. PMID: 34726771.

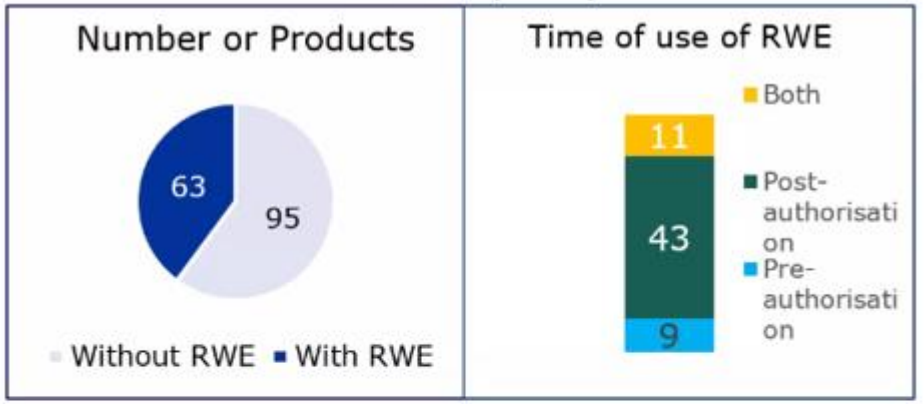
EMA use of RWE in Marketing Authorization Applications (MAAs)

2018-2019

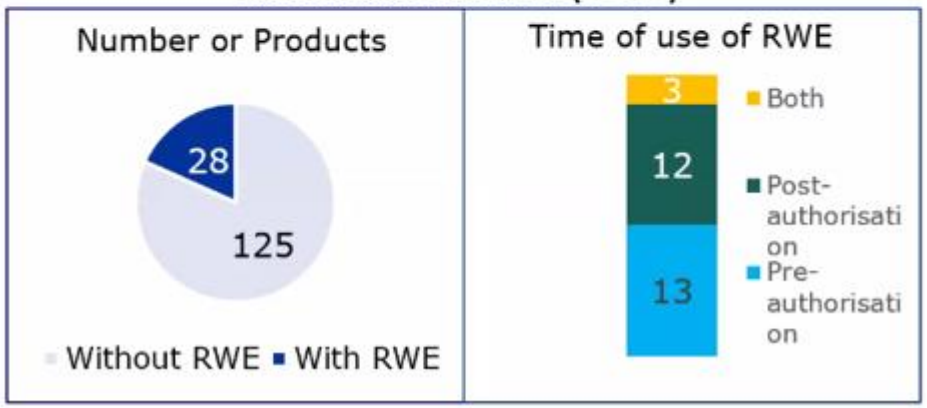
Results

- RWD/RWE used in **40% of MAAs** (mainly post-authorisation) and in **18% of EoIs** (mainly pre- or post-authorisation)
- Majority of products: **Antineoplastic** and **Immunosuppressants** (35% MAA and 42% EoI)
- When used pre-authorisation: mainly **supporting** study looking at **efficacy/effectiveness**
- When used post-authorisation: mainly **RMP Category 3** (for studies included in RMP) looking at **safety**

Initial MAA (n=158)



Extension of indication (n=153)



Slide from EMA Webinar on RWE 30 Nov 2021

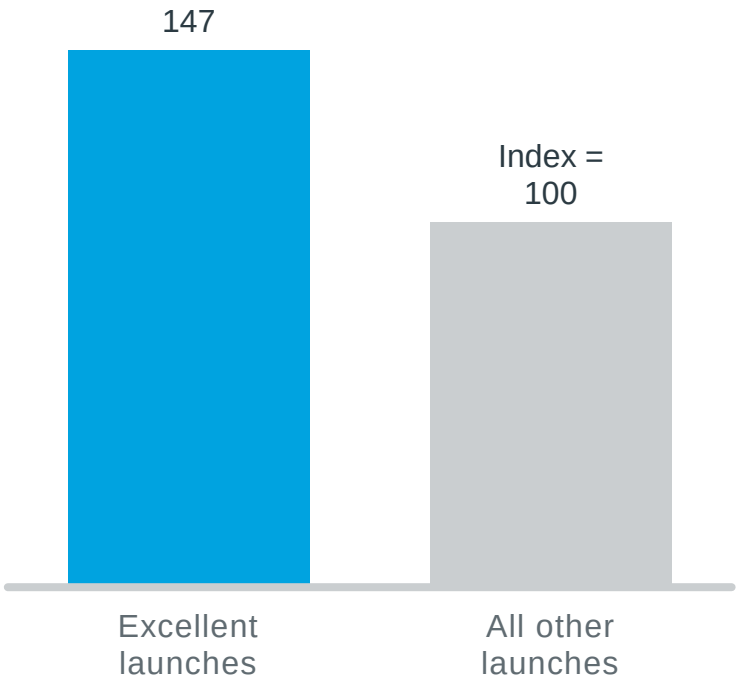
Source: Flynn, R., Plueschke, K., Quinten, C., Strassmann, V., Duijnhoven, R.G., Gordillo-Marañon, M., Rueckbeil, M., Cohet, C. and Kurz, X. (2022), Marketing Authorization Applications Made to the European Medicines Agency in 2018–2019: What was the Contribution of Real-World Evidence?. Clin. Pharmacol. Ther., 111: 90-97. https://doi.org/10.1002/cpt.2461

RWE is a critical element of launch success

The most launches with the highest index of RWE productivity in three highly competitive areas were also the most commercially successful

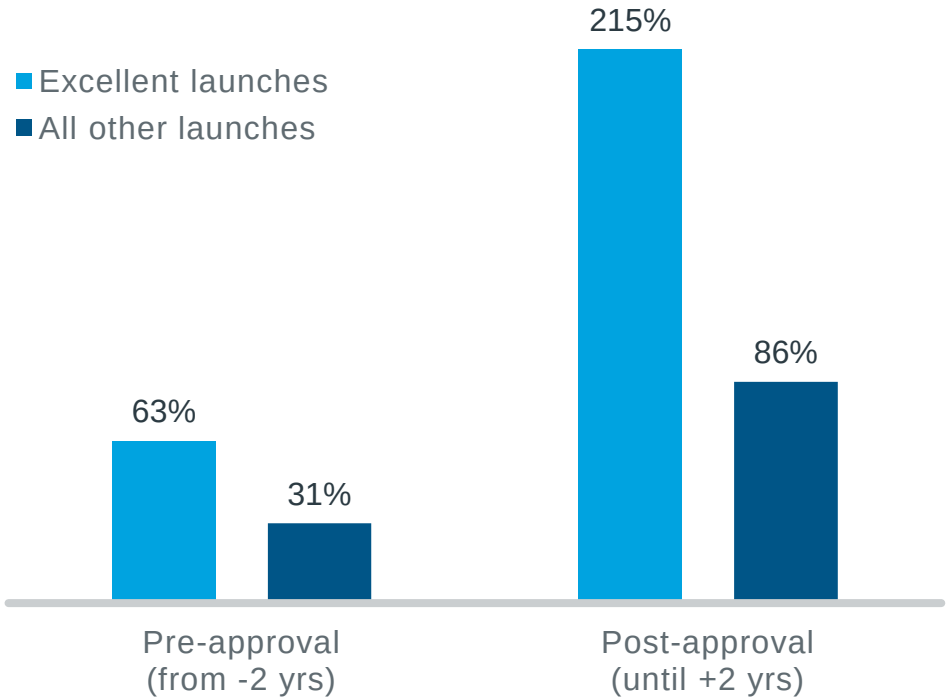
Normalised RWE lifecycle productivity*

(Indexed: average all non-excellent launches = 100)



Growth in RWE volume over product lifecycle

(2-yr CAGR for annual published RWE volume, by launch type)



RWE for commercial differentiation joins growing list of reasons to increase strategic investment in RWE, including:

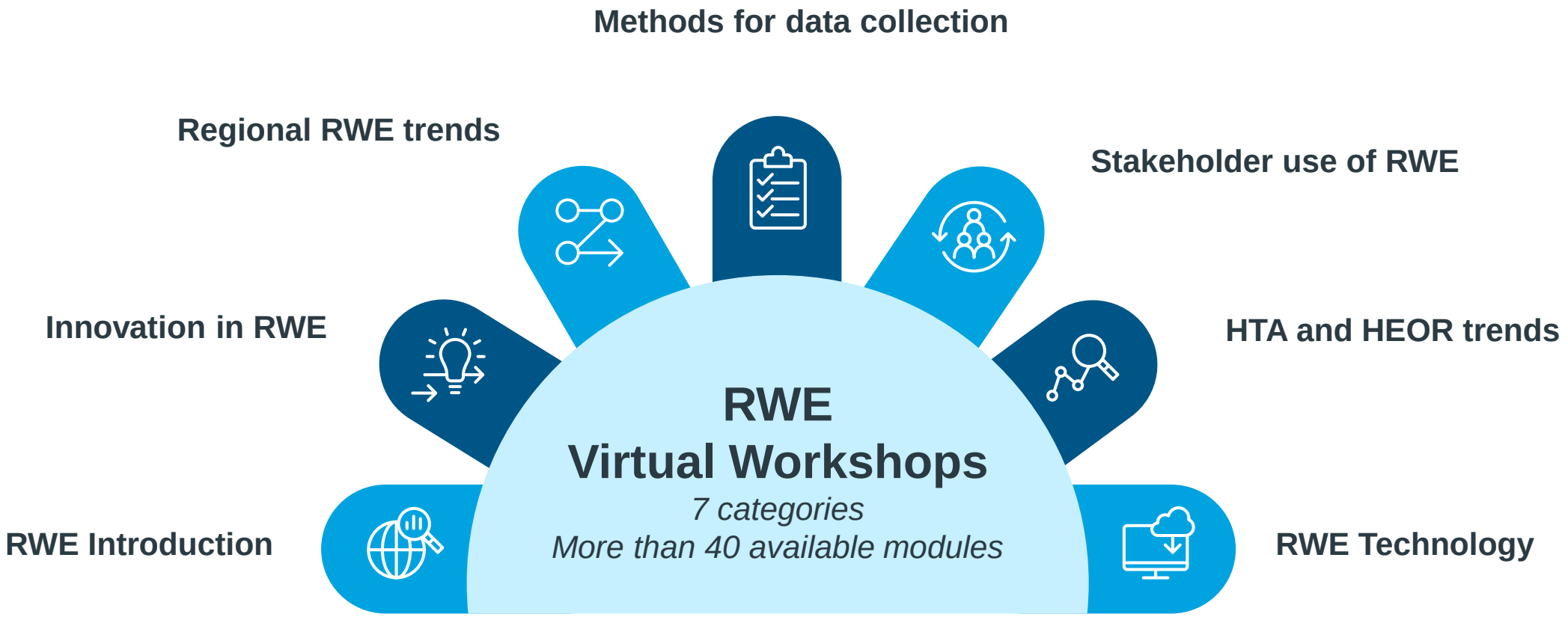
- Inherent limitations of RCTs
- Evidence gaps in the post COVID world
- Rising burden of proof
- Stakeholder reassurance
- Increasing competition
- Scientific education

* Total number of publications / number of indications approved >6month ago / normalised for time on market

Source: IQVIA European Thought Leadership

IQVIA’s RWE Academy - Customized Virtual Workshops

Discover the value of RWE across the product lifecycle



Click here for more information

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