La Industria de los Ensayos Clínicos

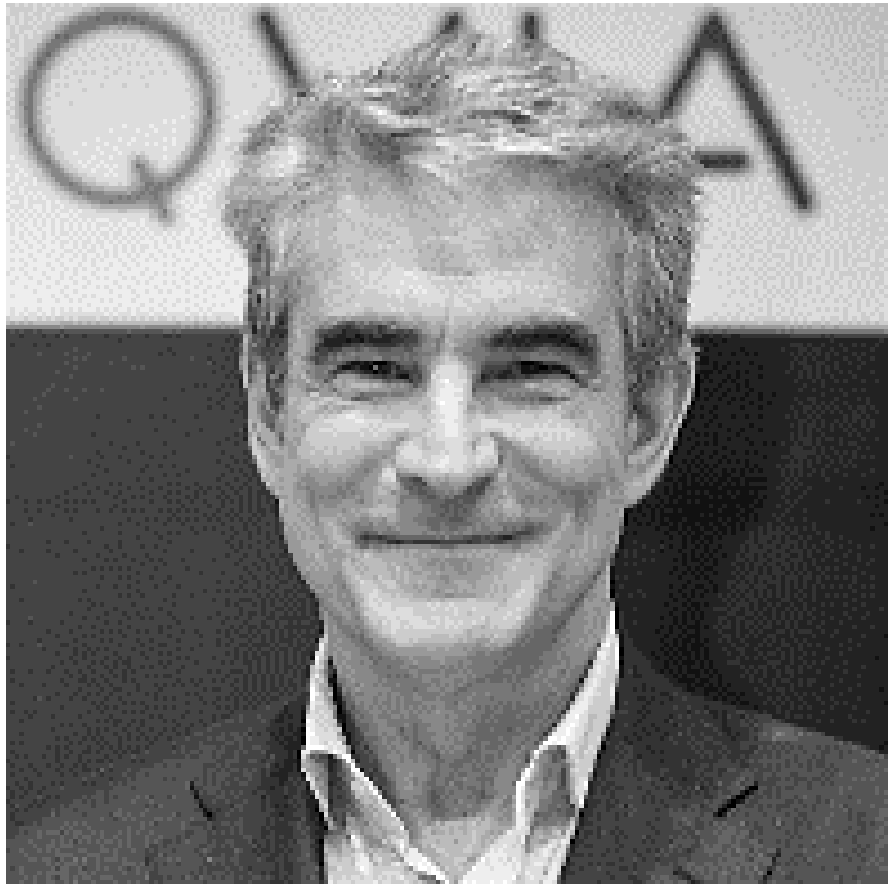
Sopar Tertúlia Claris – Salut
10Dec2020

John Ward - Prime Site Director, Spain (Ponente)
José Luis Fernández – Sr. VP & GM Spain, Portugal & Turkey (Contraponente)

Agenda

1. Presentaciones
2. ¿Que es IQVIA?
3. ¿Que hace IQVIA?
4. Los Ensayos Clínicos
5. Real World Evidence
6. El Futuro
7. Conclusión y Q&A

1. Presentaciones



José Luis Fernández

Sr. Vice President & General Manager
Spain, Portugal & Turkey

John Ward

Prime Site Director
Spain

2. ¿Que es IQVIA?

imshealth
INTELLIGENCE APPLIED.

Empresa líder en información de salud y “big data” con grandes capacidades analíticas

QUINTILES

Experiencia mundial en ensayos clínicos y datos en uso real en las principales áreas terapéuticas avaladas por expertos clínicos

IMS Health & Quintiles are now

IQVIA™

IQVIA - *Más que la suma…*

The Human Data Science Company™

IQVIA CORE ™ – How we do it…

Domain Expertise

+1,100 Medical Doctors

+1,400 PhDs

+2,500 Statisticians

+850 Epidemiologists / RWI experts

Transformative Technology

+100,000 users on our software platforms

e360 salesforce

Unparalleled Data

+530m non-identified patient records

+800,000 data sources

+75b healthcare records

+85% of global drug sales covered

Advanced Analytics

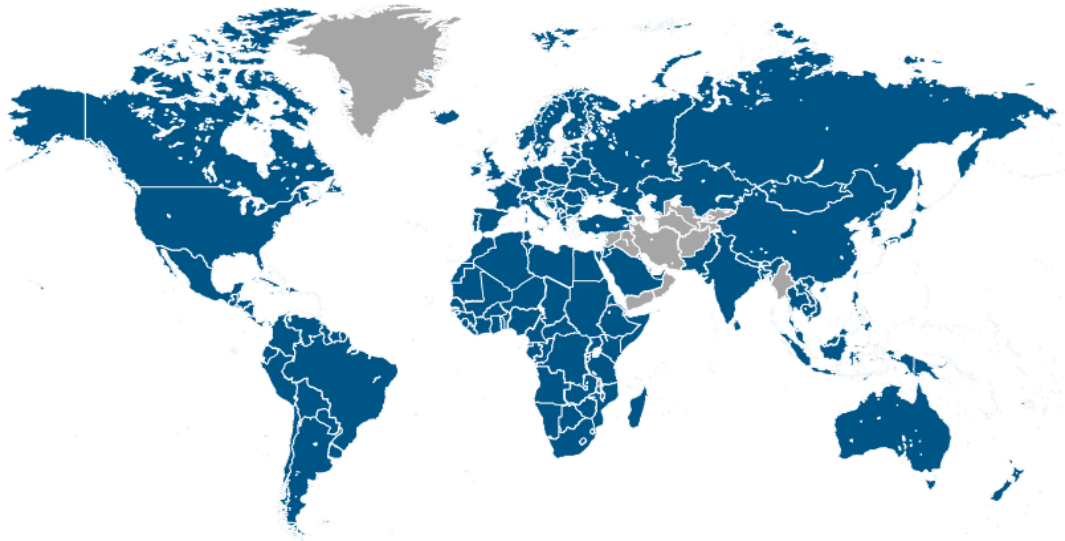
+200 Patents and patents pending

+6,000 client engagements / year

IQVIA - Líder global en consultoría, investigación, tecnología y análisis de datos para la industria de la salud

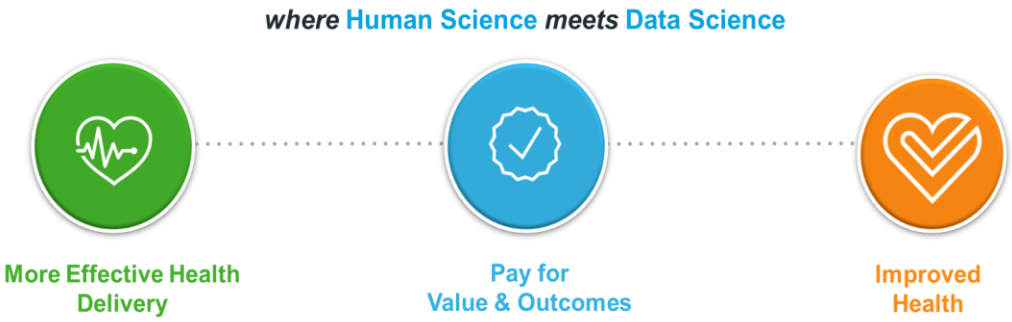
IQVIA - Global

- 100+ países en 6 continentes | 67,000+ empleados | $12bn+ en ingresos
- 1200+ expertos en salud | 4500+ expertos en clientes y la industria | 5000+ clientes
- 35,000+ proyectos. Proveedor, Pagador, Farmacéutico & Salud Público en los últimos 5 años



IQVIA - Spain

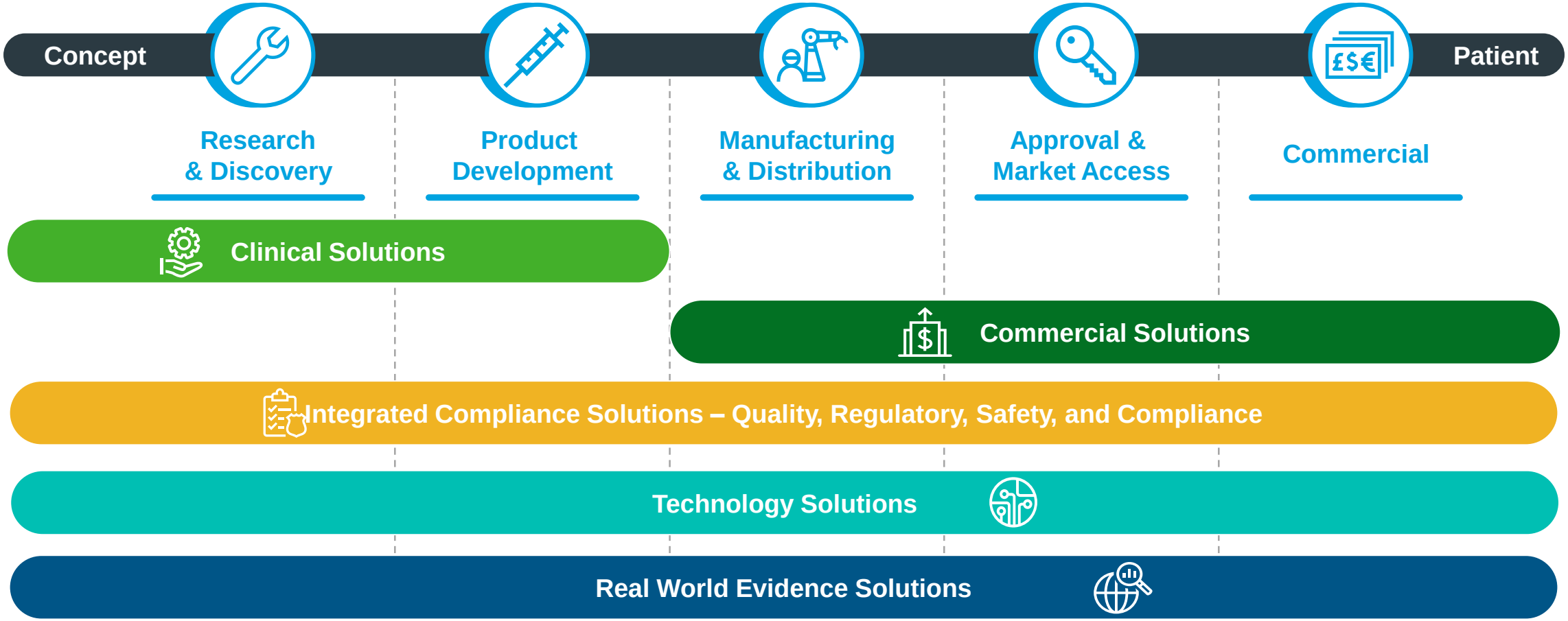
- 2 Oficinas: Barcelona y Madrid
- 1.600+ Empleados
- 120M€+ de facturación



3. ¿Que hace IQVIA?

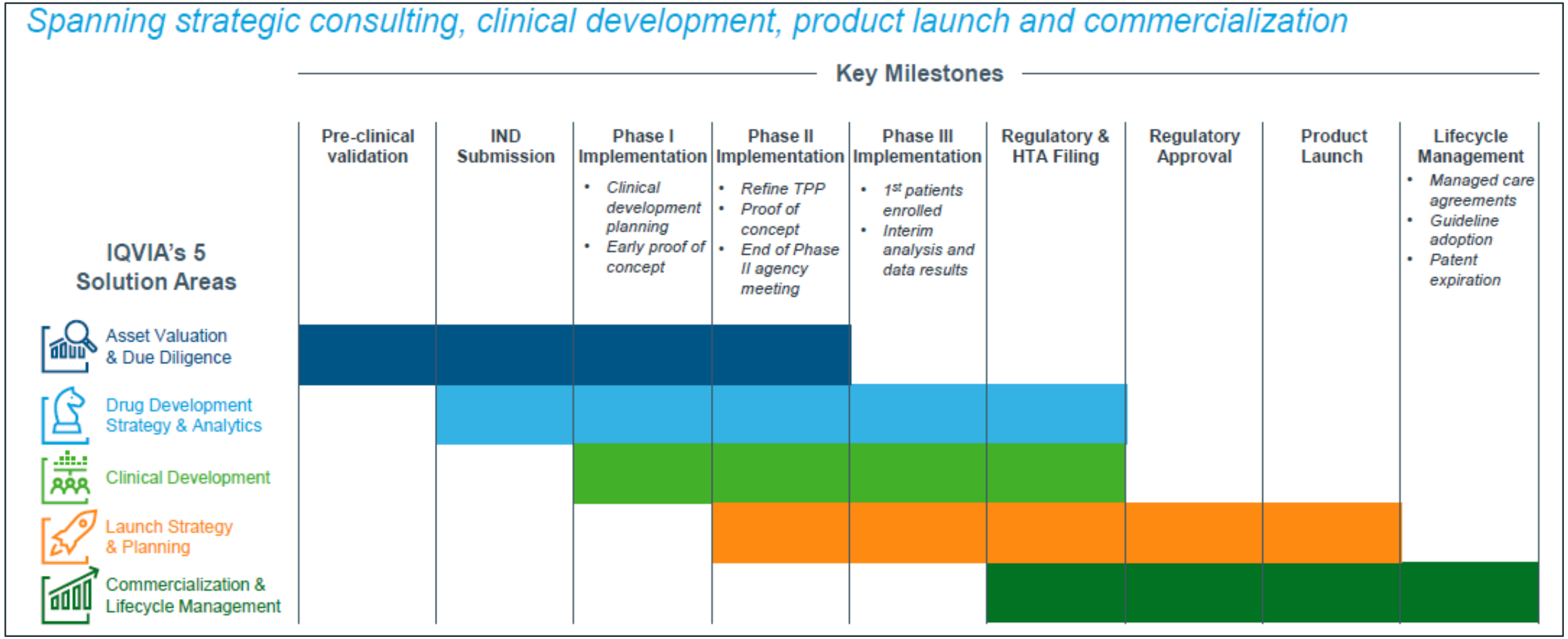
Product Lifecycle

Los distintos departamentos trabajan interconectados generando una visión global a nivel I+D y práctica clínica



Product Lifecycle

Soluciones desde la evaluación de los productos candidato hasta su comercialización



IQVIA participa en todas las áreas del sector salud

Los distintos departamentos trabajan interconectados generando una visión global a nivel I+D y práctica clínica

Contract Research Organisation

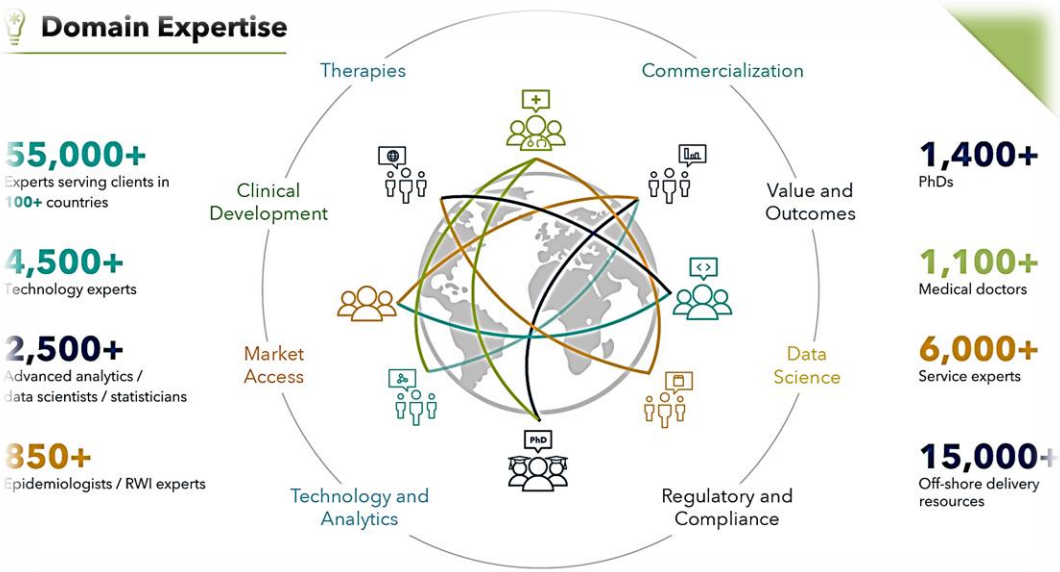
- EECC Fase I, II, III
- EECC Fase IV
- Otros estudios postautorización

RWE - Health Economics & Outcomes Research

- BBDD y registros para realización de estudios
- Consultoría especializada en ámbito de salud
- *Predictive Analytics*
- *Otros estudios en el ámbito de la salud*

Tecnología

- Big Data
- Seguridad de la información
- Anonimización/ Confidencialidad



¿Con quién trabajamos?

- Servicios de Salud, Hospitales, Centros de Salud, Farmacias…
- Industria farmacéutica
- Administración Pública y Agencias Reguladoras
- Organizaciones y Sociedades del ámbito de la salud
- Investigación académica

4. Ensayos clínicos

Ensayos Clínicos

¿Que es un ensayo clínico?

Un **ensayo clínico** es un estudio de investigación que se realiza en personas para saber como funciona un nuevo medicamento ante una enfermedad.
Sirve para saber si el nuevo medicamento es eficaz y seguro y que dosis proporciona el máximo beneficio para el paciente.

Cuando se compara un medicamento experimental con uno ya aprobado y utilizado en la práctica habitual permite conocer si el fármaco bajo investigación ofrece menos, igual o más beneficios respecto al medicamento ya existente.

* Un ensayo clínico no es un tratamiento..

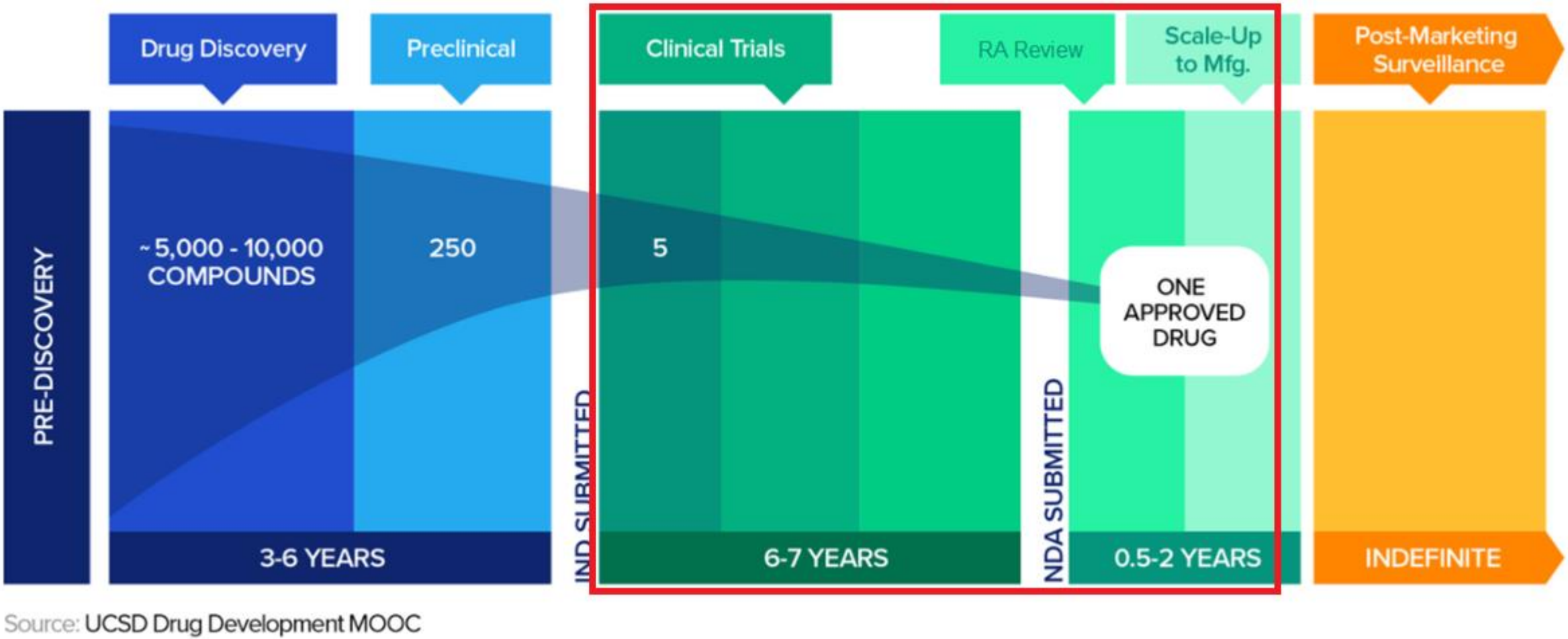
El desarrollo de un producto farmacéutico

Un proceso largo

@Efpia 20May2019

Las etapas del desarrollo de un producto farmacéutico

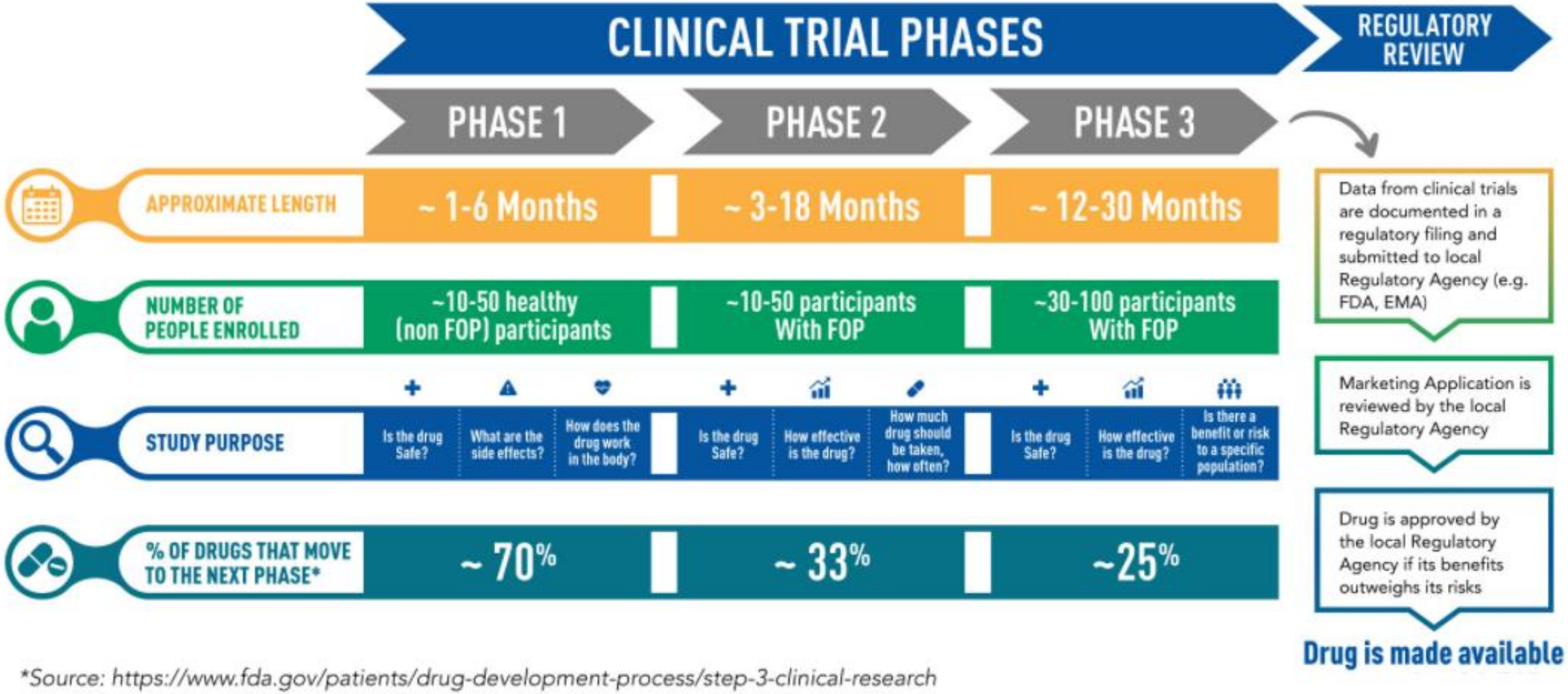
Un proceso largo y costoso



Source: UCSD Drug Development MOOC

Ensayos Clínicos

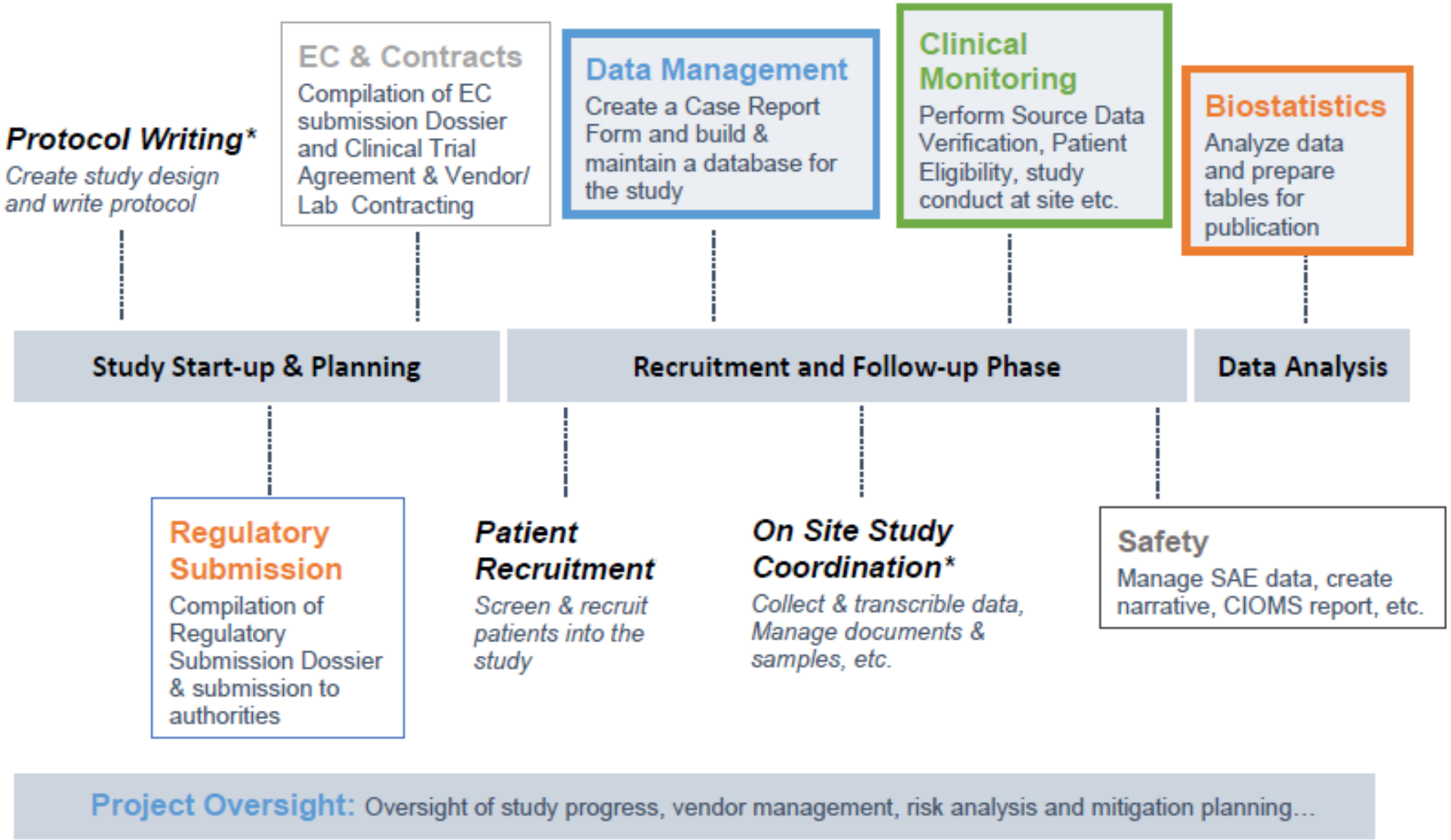
Las fases de un ensayo clínico



*Source: https://www.fda.gov/patients/drug-development-process/step-3-clinical-research

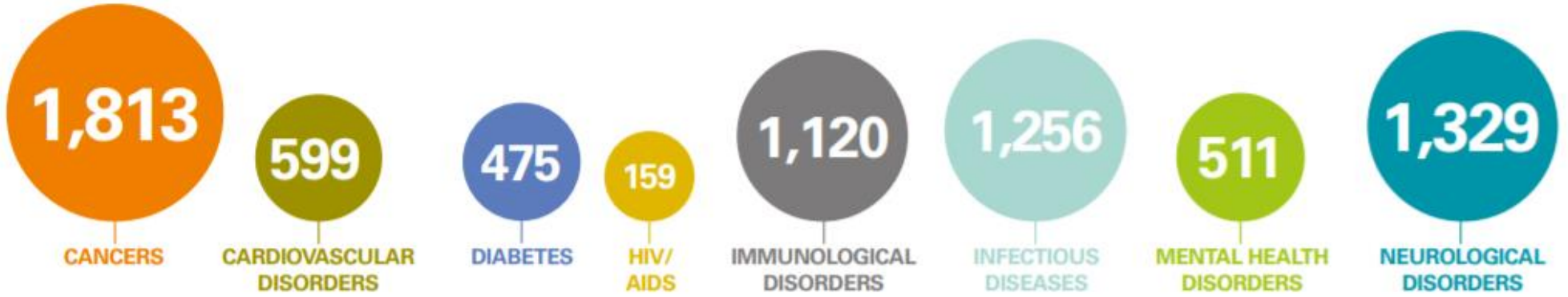
Ensayos Clínicos

Desde el protocolo hasta el análisis de los resultados



Ensayos Clínicos

La importancia de la investigación



Source: PhRMA

Source: EMA - Human Medicines Highlights 2018

Ensayos Clínicos

El impacto de la investigación

@Efpia 20May2019

Ensayos Clínicos

Los números en Europa

@Efpia 20May2019

Ensayos Clínicos

La Agencia Española de Medicamentos y Productos Sanitarios (AEMPS, Hitos 2019)

MEDICAMENTOS DE USO HUMANO

1.357
Medicamentos autorizados

47.345
Variaciones de medicamentos

833
Ensayos clínicos autorizados

3.144
Muestras analizadas por los Laboratorios Oficiales de Control de Medicamentos

33.875
Autorizaciones de uso compasivo

MEDICAMENTOS VETERINARIOS

1.579
Notificaciones de efectos adversos

109
Medicamentos autorizados

2.932
Variaciones de medicamentos

27
Ensayos clínicos autorizados

INSPECCIÓN Y CONTROL*

444
Inspecciones NCF/BPC/BPFV

56
Retiradas por problemas de calidad

609
Actuaciones contra problemas de suministro

700
Páginas web investigadas sobre medicamentos ilegales

PRODUCTOS SANITARIOS

5.850
Incidentes evaluados

521
Nuevas autorizaciones de empresas

994
Certificados de exportación

511
Casos evaluados en el control de mercado

COSMÉTICOS

760
Cosméticos y productos de cuidado personal revisados

1.921
Certificados de exportación

*Los indicadores reflejados en inspección y control incluyen las actuaciones en medicamentos de uso humano y veterinario.

5. Real World Evidence

What is Real World Evidence?

Clinical Evidence Derived from Analysis of Real World Data[1]

Real World Evidence (RWE) is the clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD

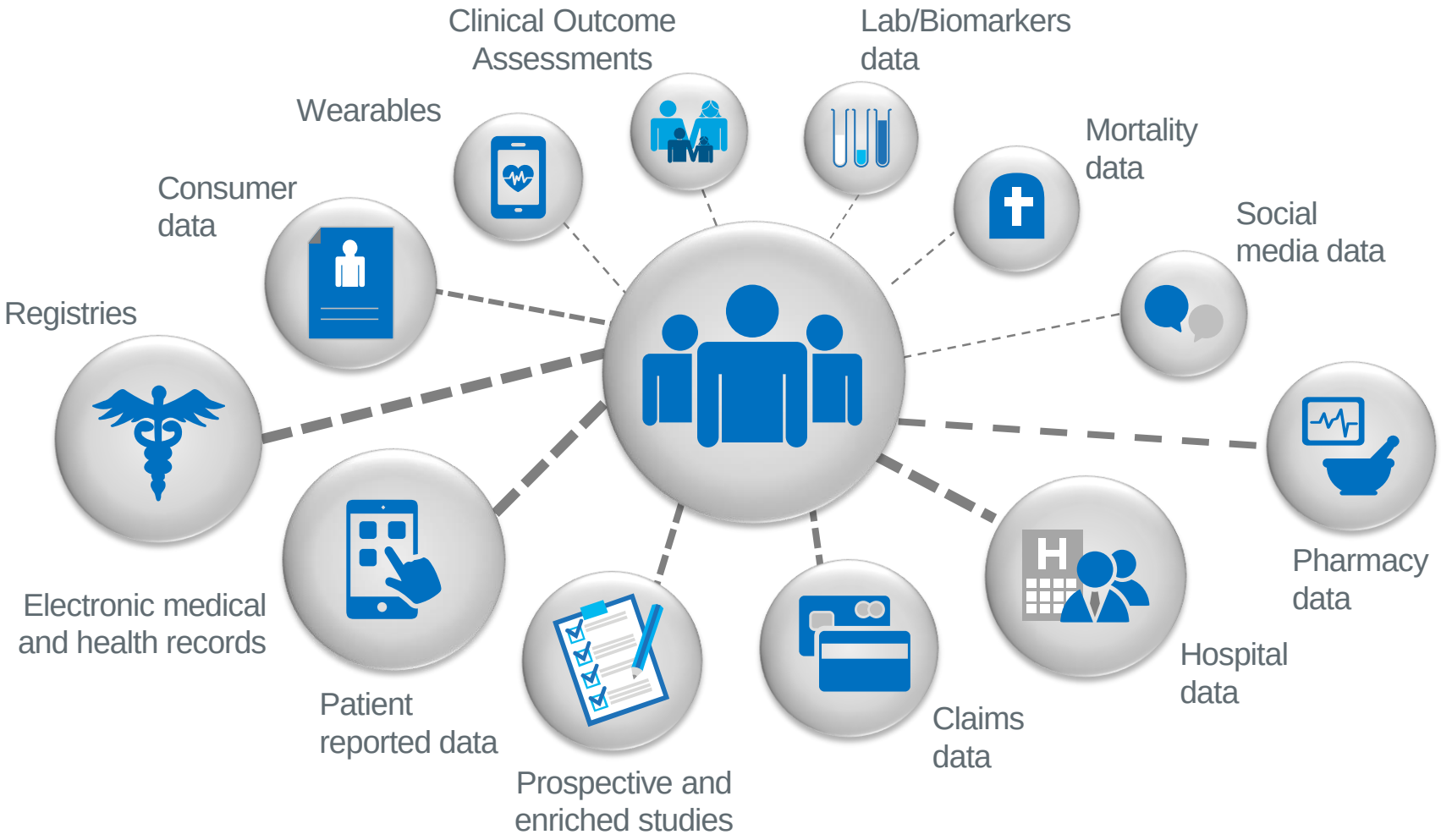
Real-World Data (RWD) are data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.

1. Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices Guidance for Industry and Food and Drug Administration Staff Document issued on August 31, 2017.

IQVIA MEDTECH

Examples of RWD sources

RWD is Patient- Level Data



RWD can answer a diverse set of business & research questions

Clinical Trial Optimization

Inform on Identification of best sites and most appropriate patients. Support protocol design

- Protocol Design & Feasibility
- On-going protocol adjustment
- Leverage RWD for study comparative arm
- Country Allocation
- Site & patient selection

Epidemiology Assessment

Monitoring of pathology evolution and therapeutic strategies

- Understand the burden of disease/natural history of disease
- Characterize patient populations & identify subgroups of interest
- Natural history of the disease/treatment pathway
- Determine the standard of care
- Identify unmet needs
- Identify suitable local comparators
- Patient flow analysis/ patient journey
- Adherence studies
- Off-label use

Device Safety & Risk Management

Segment, analyze and assess the safety and risk/benefit of therapeutic interventions in a real-world setting

- Signal Detection
- Safety Surveillance
- Vigilance
- Risk Assessment
- Post Authorization Safety Study

HEOR/ Market Access

Demonstrate product value through evidence-based health economic evaluation and real-world outcomes for optimal pricing, reimbursement and coverage potential

- Cost of Illness/HCRU (Health Care Resource Utilization)
- Burden of Disease
- Budget Impact
- Outcomes studies
- Comparative Effectiveness
- Compliance & Persistence
- Contract Optimization
- Target population

Commercial Analytics

Diagnose, plan, forecast and track brand performance. Size and characterize the target market from the disease and treatment pattern perspective

- Brand/launch Planning & Strategy
- Forecasting
- Brand Diagnostics
- Brand Performance tracking/Source of business
- Split by indication
- Contract Compliance

Advanced analytics, AIML (Artificial Intelligence & Machine Learning) or predictive modeling can be leveraged across all types of usage

(i.e. Disease detection, Predicting non-adherence, Predicting LOT and disease progression, Treatment response profiling)

IQVIA MEDTECH

The value of RWE to meet multi-stakeholder needs

RWE can be used to understand safety, performance and effectiveness of a product under real-life conditions

Hospitals and Physicians
- Inform decision-making
- Improve care
- Understand real-world treatment choices
- Gain opportunities for thought leadership

Payers
- Determine value and coverage
- Monitor usage within criteria

Patients & Advocacy Groups
- Understand treatment choices
- Understand risks and benefits
- Reflect the patient experience

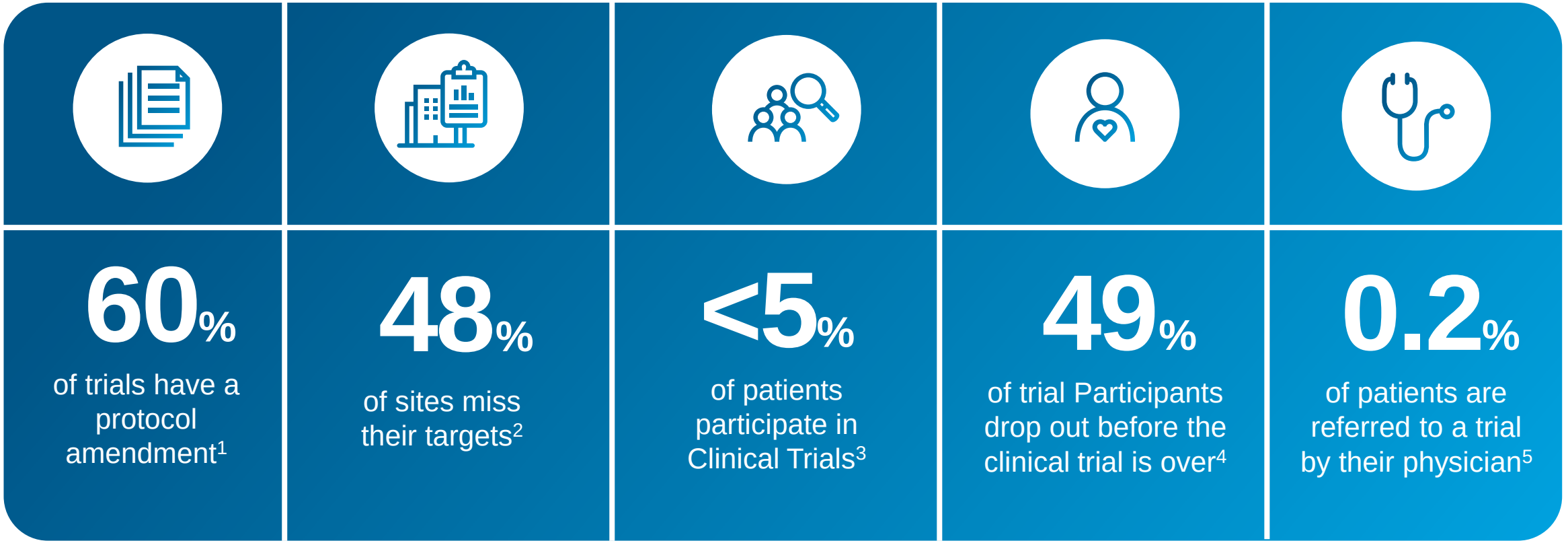
Regulators
- Evaluate safety signals
- Ensure product safety & performance
- Safety requirements

Industry
- Meet safety, performance & effectiveness commitments
- Establish value
- Secure reimbursement
- Generate publications

5. El Futuro

The Challenges

Ongoing Clinical Development challenges



[1] Getz, K. Protocol amendments: a costly solution. Applied Clinical Trials Online. Accessed September 21, 2016

[2] Getz, K. Changing Drug Development Landscape and its Anticipated Impact on R&D Operations. Accessed September 21, 2016

[3] E. Miseta. *Clinical Leader*. July 13, 2015

[4] Impact Report (2006) Tufts CSDD 8(5)

[5] Tufts Center for the Study of Drug Development Impact Report. 2017

Industry Challenges…

80%
of Clinical Trials are delayed

60%
R&D Expense growth (2007 – 2017)[1] vs. 40% revenue growth

+$1B
increase in cost to bring an asset to market vs 2013

4.8
years to accurate diagnosis for rare diseases

-23%
Industry return on R&D investment (2007 – 2017)

1. Top 25 Innovator Pharma companies
Source: Company Financial Reports; IQVIA Analysis

Datos & Tecnología

The Present & Beyond

Pharma Industry Trends

Eroom's Law	• R&D costs consistently outpace new drug approvals, **trials are growing less and less efficient.**
Targeted Therapies	• **Targeted therapies narrowing population** eligible for the trial- smaller sample sizes, difficult to find patients
Shorter Trials	• Regulators are now becoming **more flexible and allowing companies to use surrogate endpoints** (for example, 'progression-free survival') to gain approval.
Predictive Analytics	• Pharma **employing more predictive analytics to identify key usage patterns and look for treatments targeted to a specific set of patients**.
Technology Adoption	• Drug discovery will continue to **test the benefits technologies such as artificial intelligence (AI), the Internet of Things (IoT) and machine learning (ML)**
Mobile Patient Data	• Increased use of **connected devices to collect patient endpoint data** • Growth of ePRO on mobile devices leading to explosion of diary assessments.
Real World Data	• Pharma and CROs, to **increase staff for collection and analysis of real world data** by 25% between now and 2020

System demands and trends point towards the need for greater adoption of Data and Technology to enable Clinical Trial performance

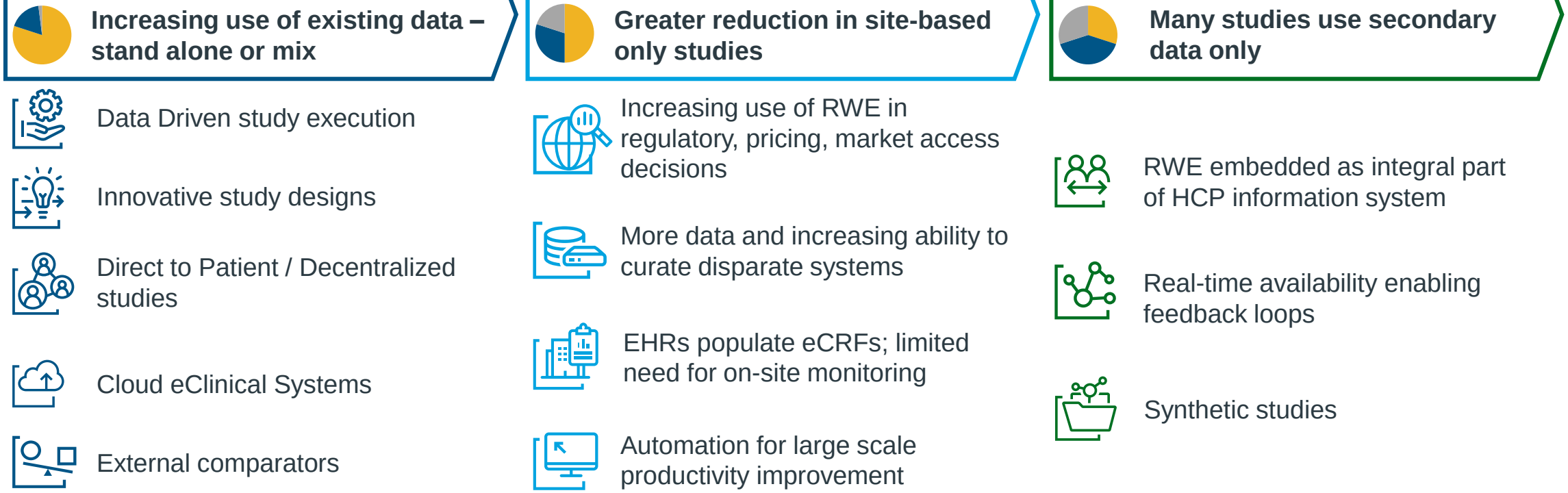
Research System Demands

- **Recruit & retain a patient** as a trial subject
- Meet the longitudinal requirements of **complex & new diseases**
- **Site demands** for efficient technology solutions
- **Sponsor interest** in new models e.g. virtual trials, RWD comparators, synthetic controls, enriched studies, database extensions

Healthcare System Trends

- **Regulatory & payer acceptance** of novel technology & infrastructure
- **Protocolization of treatments** for evidence driven patient care
- **Digitization of care** through Health IT infrastructure investment
- **Consumer & patient engagement** investments & market disrupters

Data & Tech: Transforming the Clinical Trials Paradigm



Inteligencia Artificial y Aprendizaje Automático

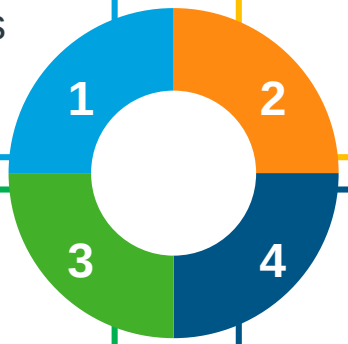
Para abordar las necesidades cada vez más complejas derivadas del Big Data que se centran en cuatro áreas principales

Detección de la enfermedad

- Ayudas a los médicos a identificar pacientes
- Identificar pacientes en riesgo de desarrollar o tener una enfermedad no diagnosticada o no codificada
- Ayudar a cuantificar mejor grupos de pacientes para planificar la distribución de recursos

Progresión de la enfermedad

- Predecir la progresión de la enfermedad.
- Identificar indicadores clave de pacientes que pronto serán candidatos para una terapia en particular.
- Desarrollar alertas para avisar a los médicos responsables de estos pacientes en "tiempo real”



Predicción del comportamiento del paciente

- Identificar los factores clave de no adherencia.
- Orientar estrategias de adherencia específicas
- Señalar a los pacientes que probablemente suspendan el tratamiento antes de tiempo

Perfiles de respuesta al tratamiento

- Predecir proactivamente la respuesta al tratamiento en pacientes individuales
- Ayudar a los médicos a tomar decisiones de tratamiento.
- Optimizar los acuerdos innovadores de financiación al identificar a los pacientes con más probabilidades de responder bien a una terapia

Ensayos Descentralizados

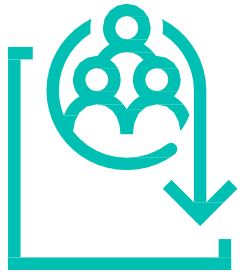
Industry Challenges Drive Innovation

<5%

of patients participate in clinical research[1]

48%

of sites miss enrollment targets[2]



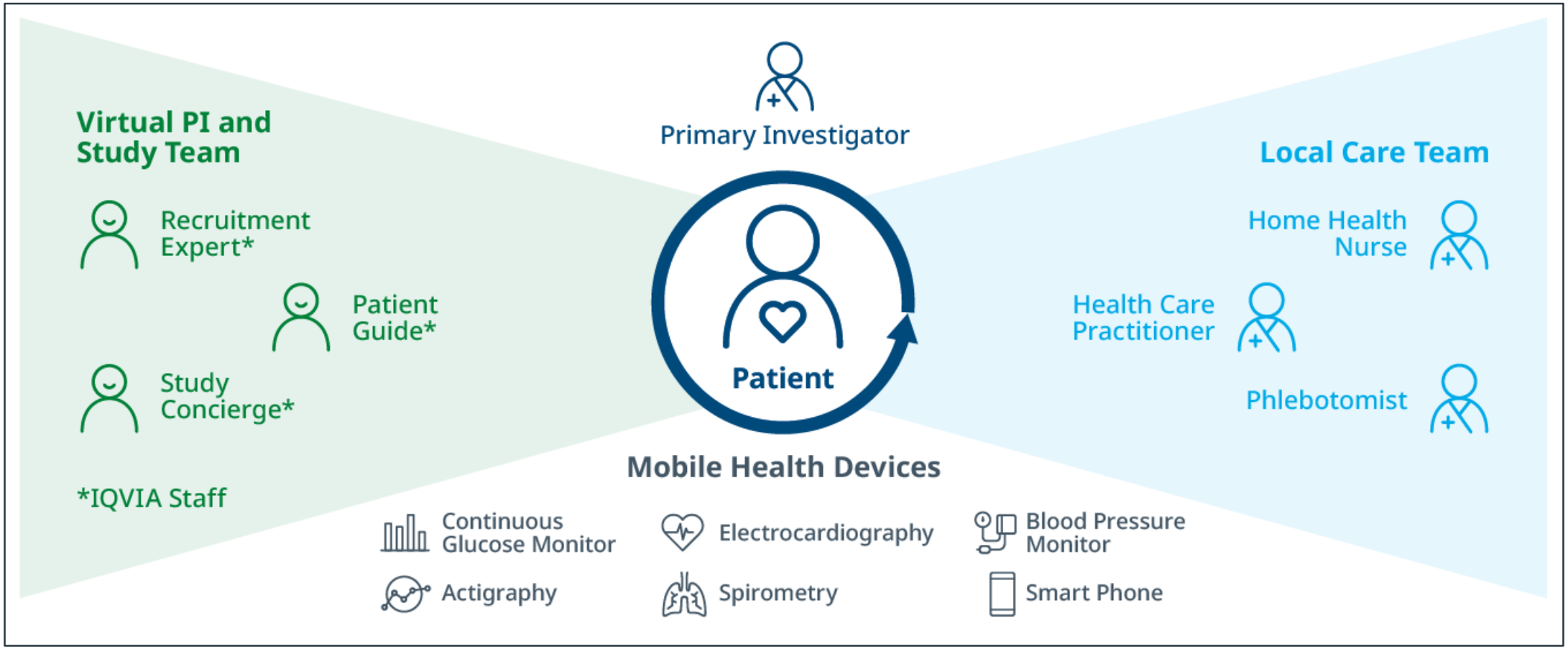
49%

of trial participants drop out before study ends[3]

[1] E. Miseta. *Clinical Leader*. July 13, 2015 [2] Impact Report (2013) Tufts CSDD 15(1) [3] Impact Report (2006) Tufts CSDD 8(5)

Ensayos Descentralizados

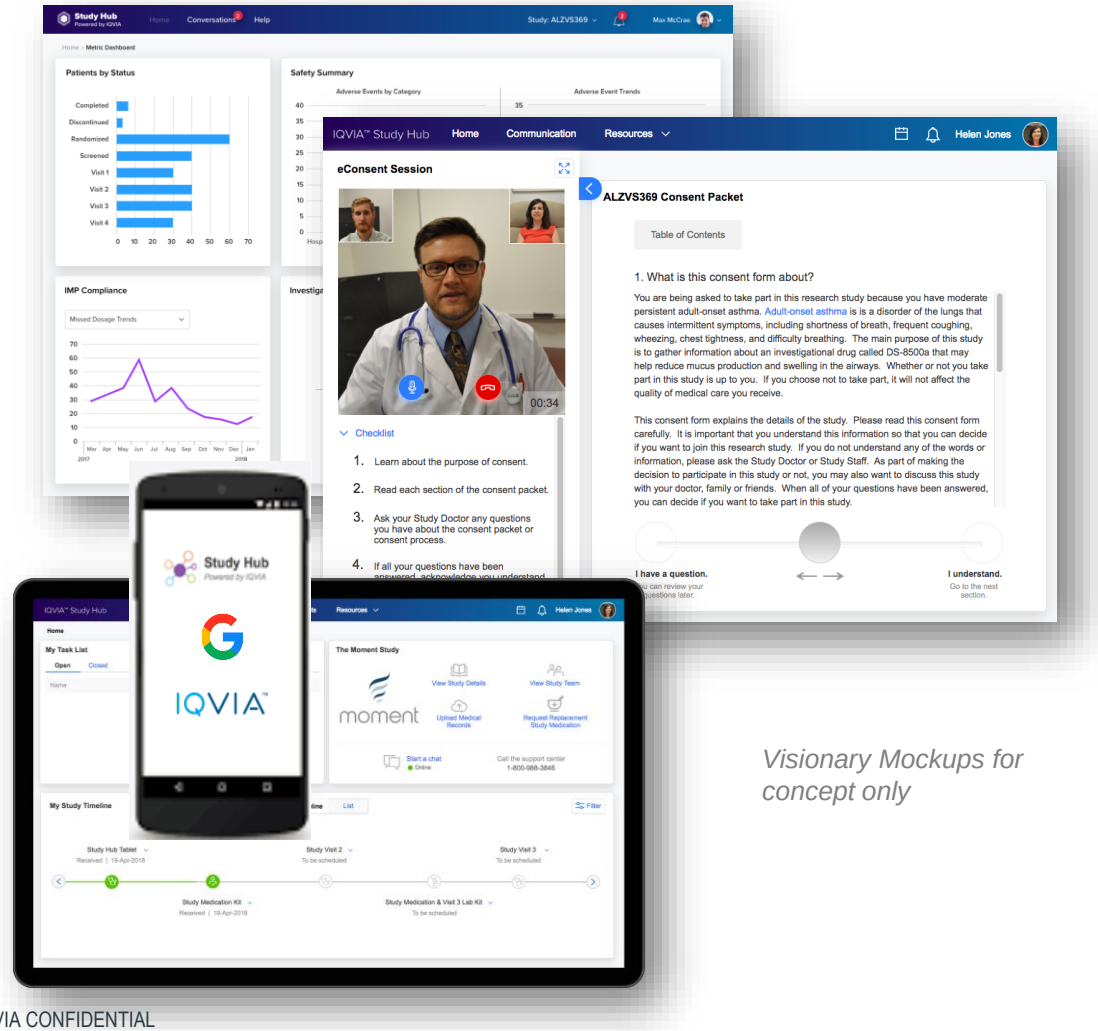
Un nuevo enfoque



Virtual Trial Technology Platform – IQVIA™ Study Hub

Comprehensive solution components linked to deliver fast and high quality results

Built on the World's Leading SaaS Platform



Visionary Mockups for concept only

- Direct to Patient Recruitment
- Integrated Televisit Technology
- Digital Communication Platform
- Direct-to-Patient Shipment of IMP
- Connected End-Point Devices
- Mobile Data Collection
- Virtual eConsent
- Patient Reported Outcomes (ePRO)
- Safety and Centralized Monitoring
- Medical Records Management
- Trial Database
- Fully Scalable

Investigator Initiated Trials

Investigator Initiated Trials - Benefits and Support

IITs are the seedlings of future medical advances, the testing grounds for concepts and innovation.

IITs provide the data for Label Extensions and full New Device dossier submissions as well as additional scientific data for Sales & Marketing and Medical Affairs.

Conventional CRO vs Academic Investigator Research

Aligning support to the specific needs of the Investigator and the Study

Conventional CRO model

- Complex structures
- Interdependent services
- Integrated preferred platforms
- Complex cost structure
- Designed for commercial research
- Pricing unrealistic for academic

Academic Investigator Research

- Consultative input and support
- Suite of modular services
- Fully matched to study outcomes
- Simple to contract and start up
- Simple to execute
- Controlled, unitized pricing

Academic Investigator Research: Model

Modular, focused, simple & controlled

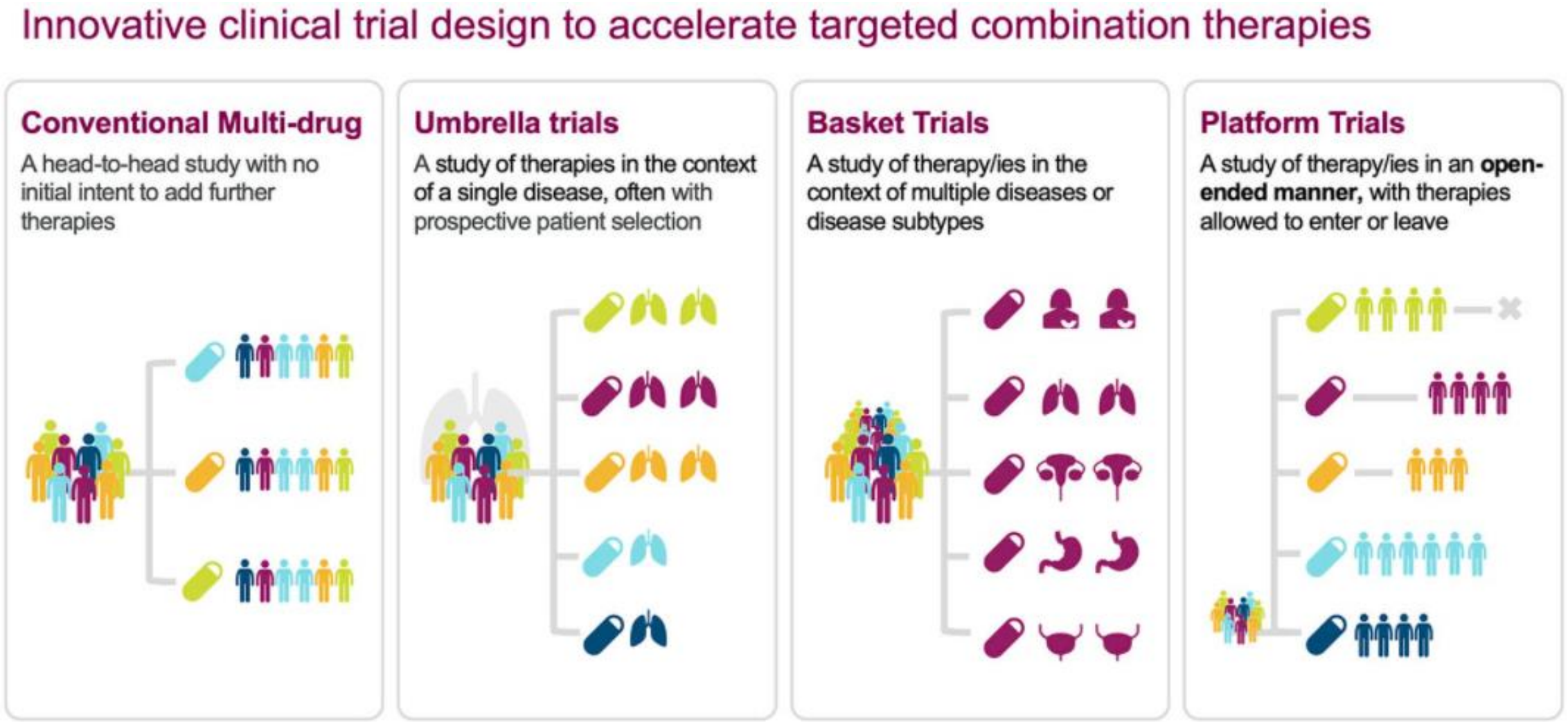
- Research Goals
- Key Endpoints
- Intended Data Use

Significantly reduced costs and added value

Ensayos Clínicos –Diseños Innovadores

Ensayos Clínicos – Nuevos Diseños

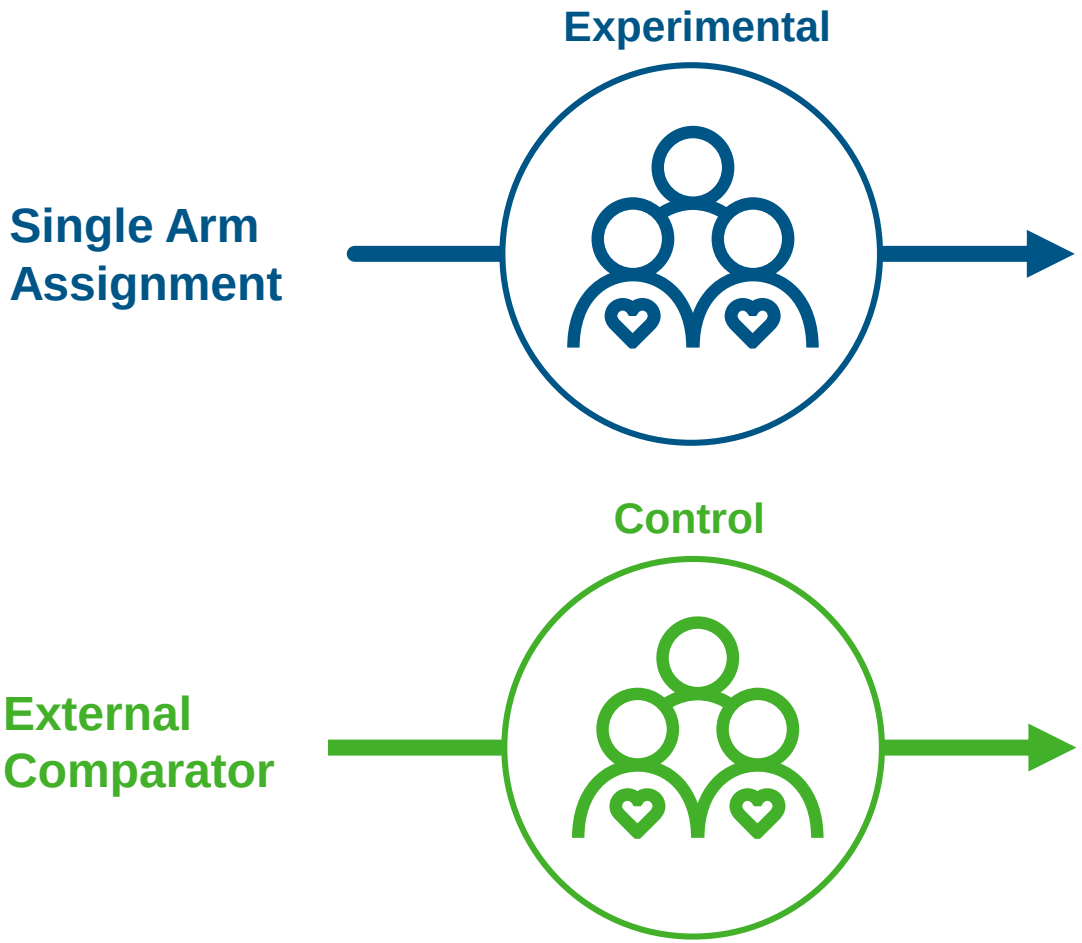
Estándar e Innovadores



Acerta

Augment with External Comparators

Control group derived from real world data



What it is and how it works

- External Comparator simulates the control arm in a trial
- Control group derived from real world data
- Patient cohort derived from real-world data (RWD) (e.g. registries, EMR, chart reviews, claims)
- RWD collected retrospectively or prospectively used to provide historical or contemporaneous comparators.
- Patients mirror the inclusion / exclusion criteria for the trial
- Trial outcomes are examined in the RWD external comparator cohort

External Comparators key takeaway

Useful when randomization is not feasible or patients unable to participate or healthcare resources not available e.g. during COVID-19 outbreak

6. Conclusión y *Tertulia*

Gracias por su atención

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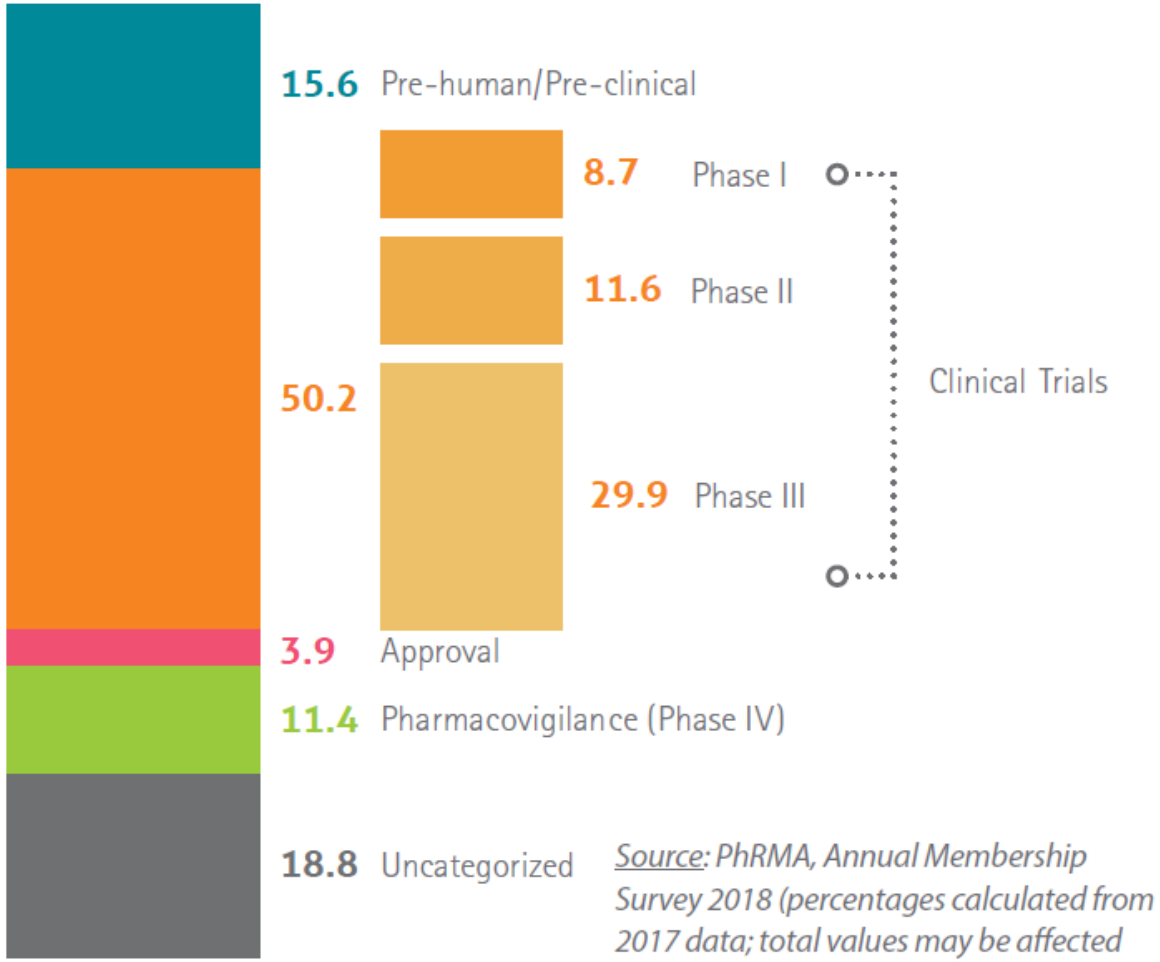
Extra Slides

Pharmaceutical Industry R & D in Europe

EFPIA 2017	€ million		€ million
Austria	294	Latvia	n.a
Belgium	3,508	Lithuania	n.a
Bulgaria	n.a	Malta	n.a
Croatia	40	Netherlands	642
Cyprus	85	Norway	126
Czech Rep.	77	Poland	340
Denmark	1,632	Portugal	100
Estonia	n.a	Romania	101
Finland	201	Russia	856
France	4,451	Slovakia	n.a
Germany	6,918	Slovenia	180
Greece	42	Spain	1,147
Hungary	176	Sweden	1,104
Iceland	n.a	Switzerland	6,105
Ireland	305	Turkey	66
Italy	1,530	U.K.	5,292
TOTAL			35,318

Allocation of R&D Investment by Function (%)



Source: PhRMA, Annual Membership Survey 2018 (percentages calculated from 2017 data; total values may be affected by rounding)