EVIDENTIQ

CLINICAL DATA SCIENCE GROUP

RWE: Utilising Patient Insights to Better Design and Conduct Meaningful RWE Studies

By Thierry Escudier– 29th of September 2022

Who are we?

EvidentIQ is a next generation technology-amplified data science group championing new standards in value creation and innovation driven relevance for customers.

The EvidentIQ offering brings a pioneering end to end eClinical solution that meets increasing customer demand across clinical operations and clinical data management with a suite of applications within a single cloud platform, including EDC, CTMs, eTMF, ePRO, coding, remote monitoring, RBM, RTMS, Payments and eFeasibility.

By combining its platform with a broad data science service portfolio such as patient recruitment, patient engagement media and a host of RWE late phase solutions EvidentIQ significantly helps customers optimize HTA submissions, pricing and reimbursement needs.

Over the past decade, patient centricity has become a pillar in product development and life cycle management in the healthcare industry.

Our mission is to engage our patients' online communities to uncover real-world evidence and advance medical research.

EvidentIQ patient platforms have a fast-growing cohort of 500k+ patients and caregivers across +1,200 therapeutic areas representing chronic and rare diseases.

Professional Services

RWE SERVICES

- Project Setup
- Protocol Development
- Questionnaire development (translations, etc.)
- Standard Analysis Plan
- Regulatory Management (EC and IRB)
- PV Management
- Data Collection (invitations sent to patients, reminders, screening, etc.)
- Data Analysis
- Report Preparation
- Advisory Board Management
- Publications (manuscripts, abstract, posters)

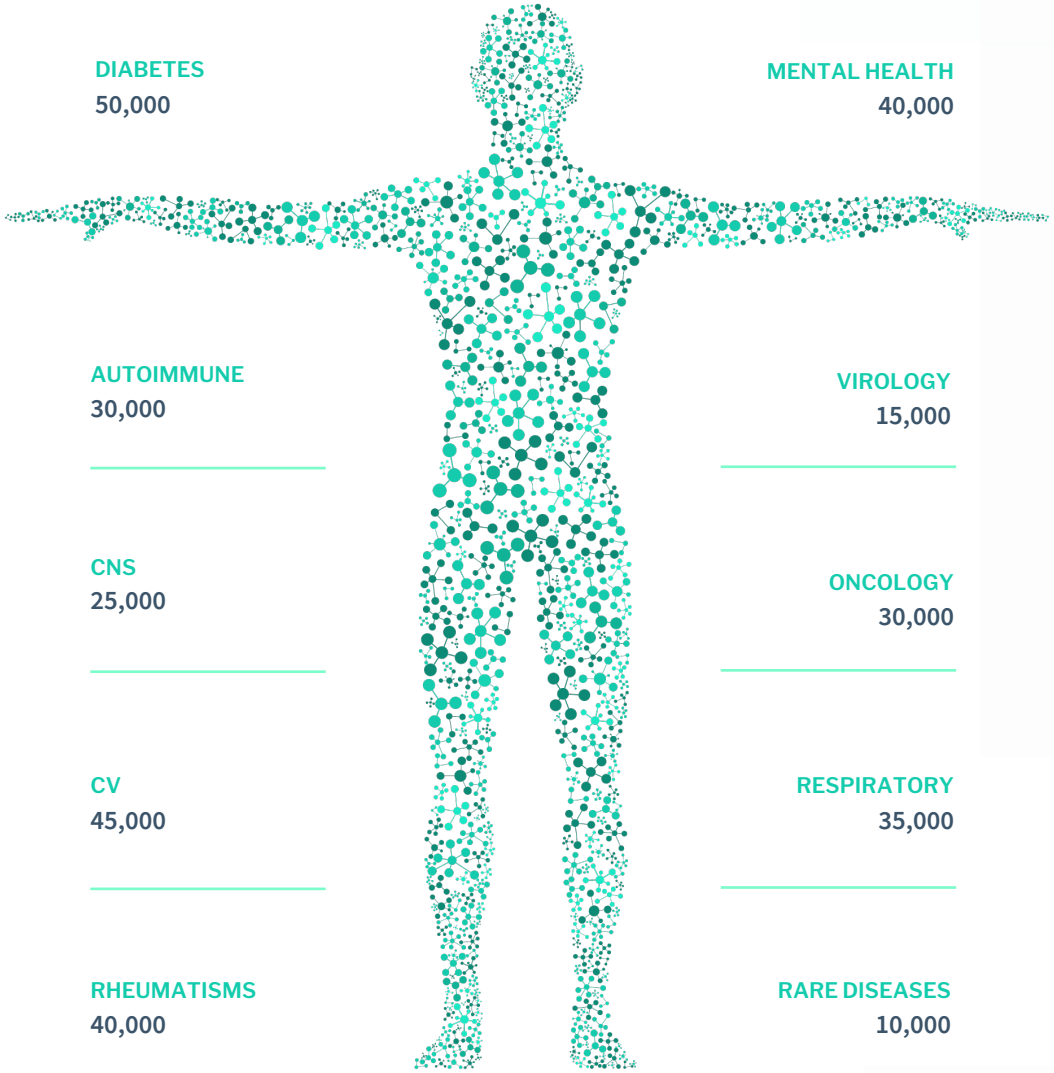
CLINICAL SERVICES

- eCRF Design
- eTMF Setup Services
- Data Management and Data Analysis
- Patient Recruitment
- Data Management Strategy Services
- Target Product Profiling
- Trial Feasibility Assessment
- Protocol Development

A fast-growing cohort of 500k+ patients and caregivers across 1,200 conditions

72%
PATIENTS

28%
CAREGIVERS

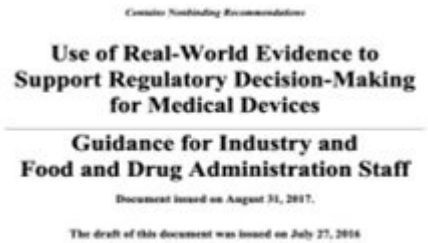


The rising importance of RWD/RWE for regulatory purpose

Even RCT still stays the gold standard for evaluating ratio benefit/risk and getting regulatory approvals, the main agencies are all suggesting to pharma to include RWD collection in their clinical development program.

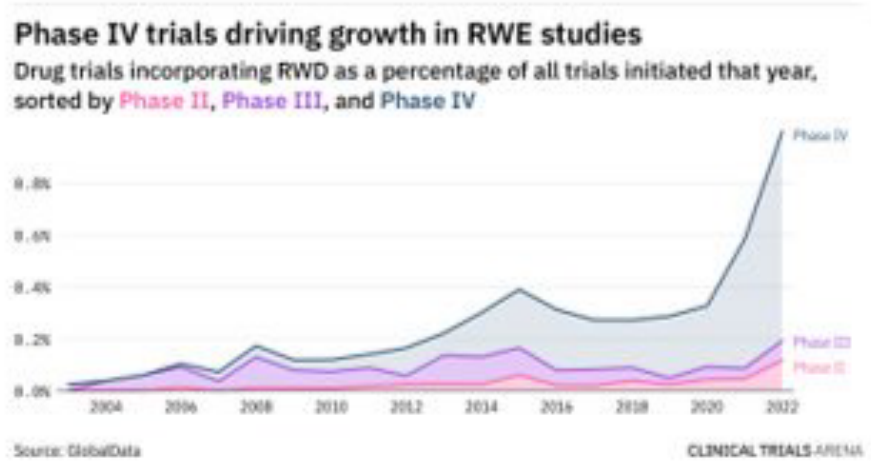
Following the 21st century Cure Acts of 2016, the FDA has recently published guidelines to illustrate the interest of implementing RWD during clinical development phase.


Submitting Documents Using Real-World Data and Real-World Evidence to FDA for Drugs and Biologics
Guidance for Industry
DRAFT GUIDANCE

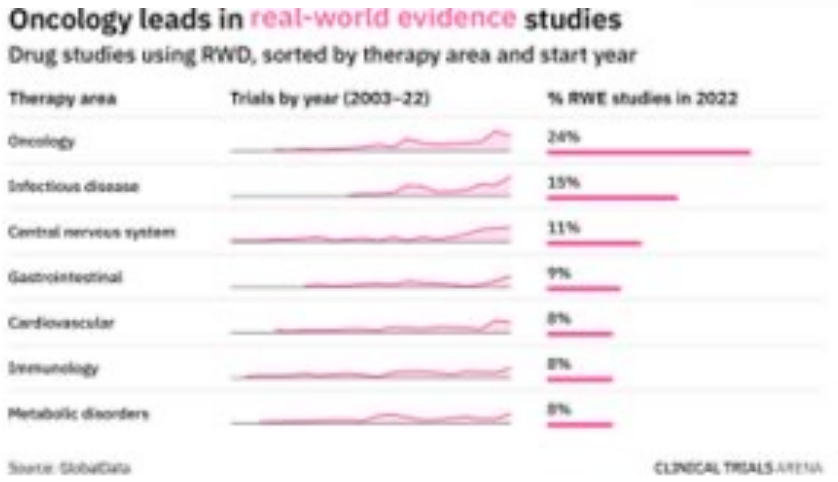

Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices
Guidance for Industry and Food and Drug Administration Staff
Document issued on August 31, 2017.
The draft of this document was issued on July 27, 2016

June 2022 - The most promissing news for the near future :

Recent survey suggest a slow but increasing uptake from pharma regarding RWD collection for regulatory purpose



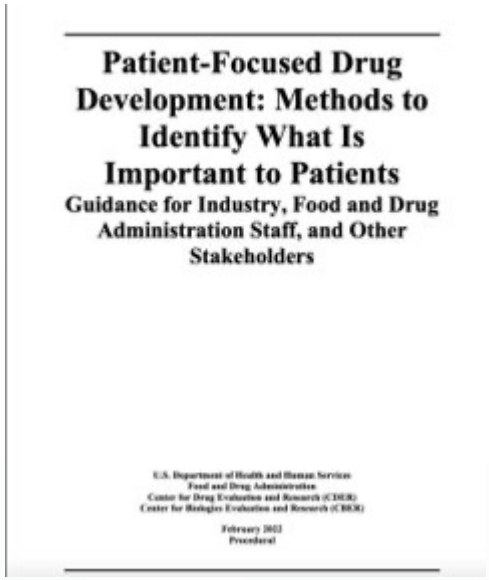
ONCOLOGY is the top TA for RWD COLLECTION



Promising adoption and generalization of RWD collection in clinical development phase should not hide the challenges

- Multiple data sources
- Meaningful data
- Standardisation
- Volume

Engaging Patient in early stage of your project will enable you to benefit from their expertise

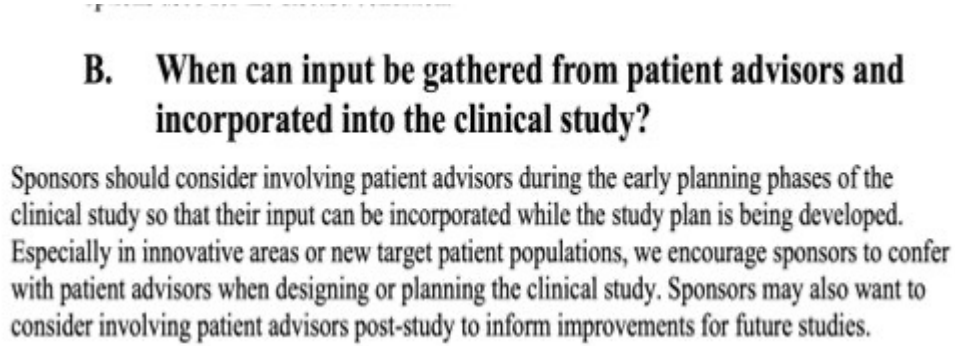


Patient-Focused Drug Development: Methods to Identify What Is Important to Patients

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

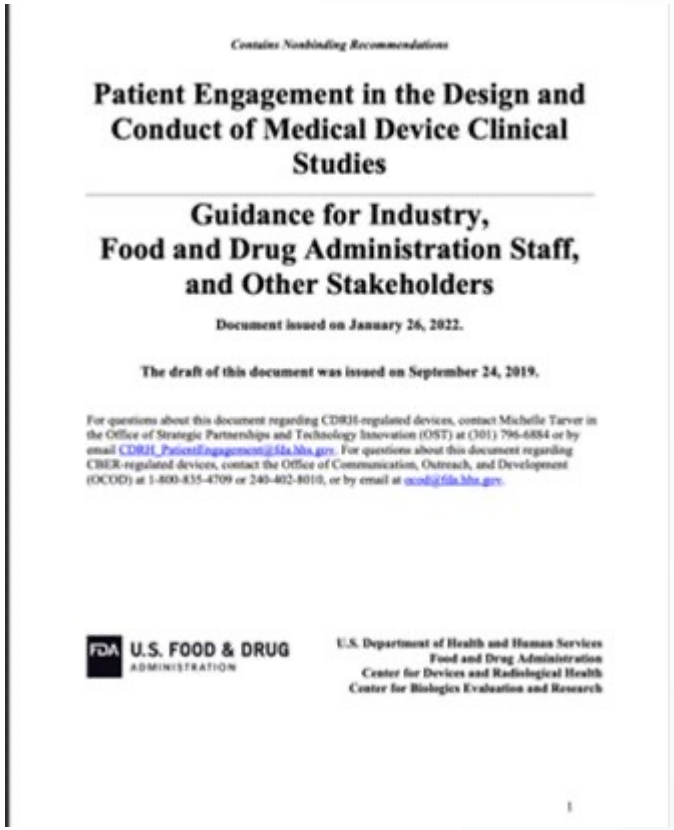
U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

February 2013
Procedural



B. When can input be gathered from patient advisors and incorporated into the clinical study?

Sponsors should consider involving patient advisors during the early planning phases of the clinical study so that their input can be incorporated while the study plan is being developed. Especially in innovative areas or new target patient populations, we encourage sponsors to confer with patient advisors when designing or planning the clinical study. Sponsors may also want to consider involving patient advisors post-study to inform improvements for future studies.



Contains Nonbinding Recommendations

Patient Engagement in the Design and Conduct of Medical Device Clinical Studies

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

Document issued on January 26, 2022.

The draft of this document was issued on September 24, 2019.

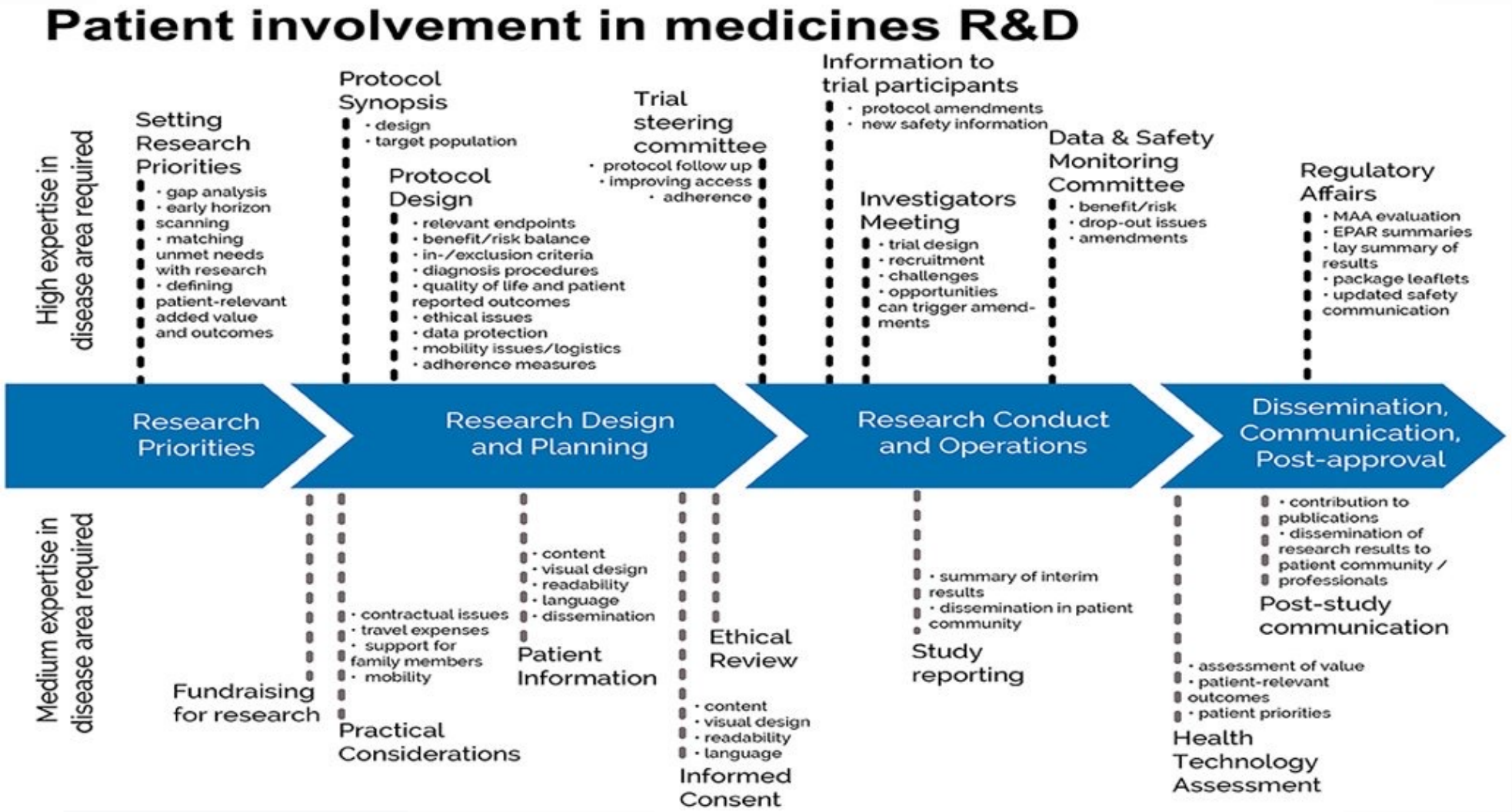
For questions about this document regarding CDRH-regulated devices, contact Michelle Tarver in the Office of Strategic Partnerships and Technology Innovation (OST) at (301) 796-6884 or by email CDRH_PatientEngagement@fda.hhs.gov. For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010, or by email at ocod@fda.hhs.gov.

FDA U.S. FOOD & DRUG ADMINISTRATION

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

1

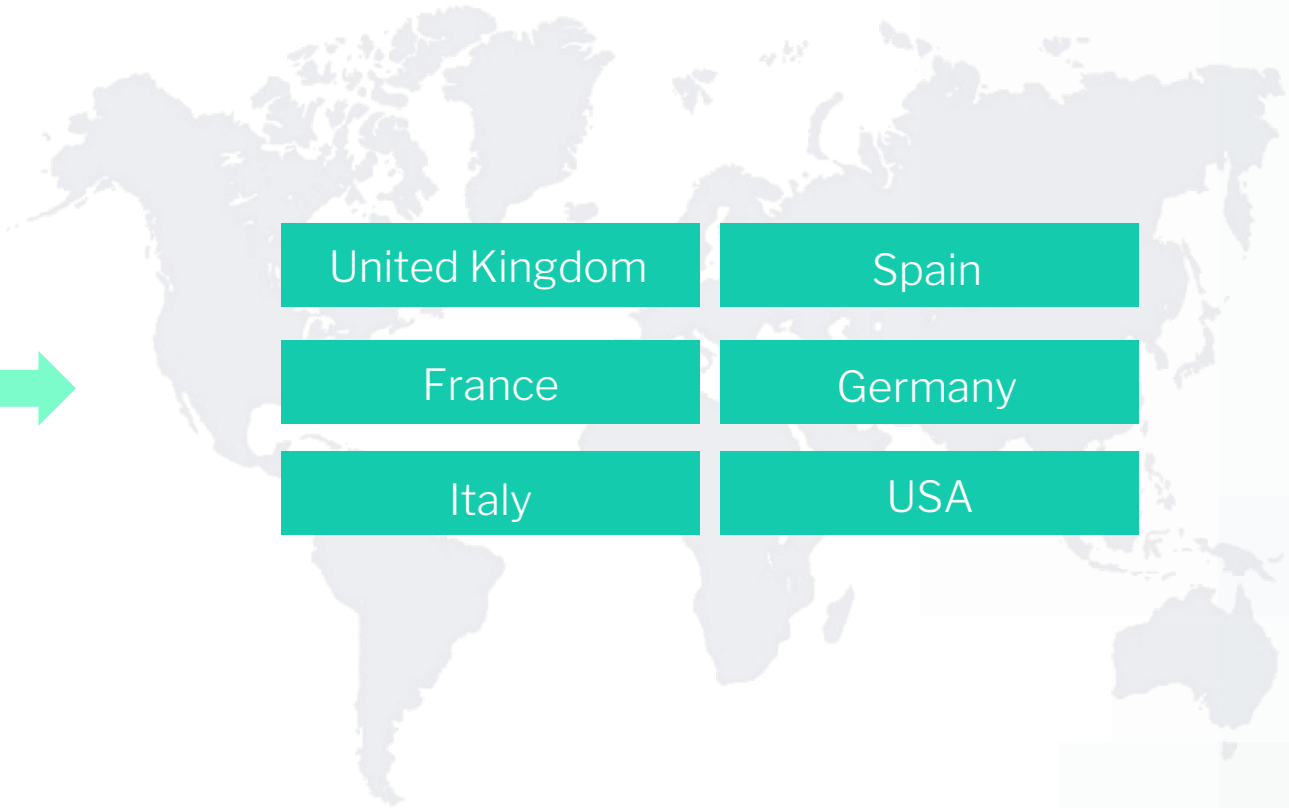
EUPATI, the roadmap of patient implication



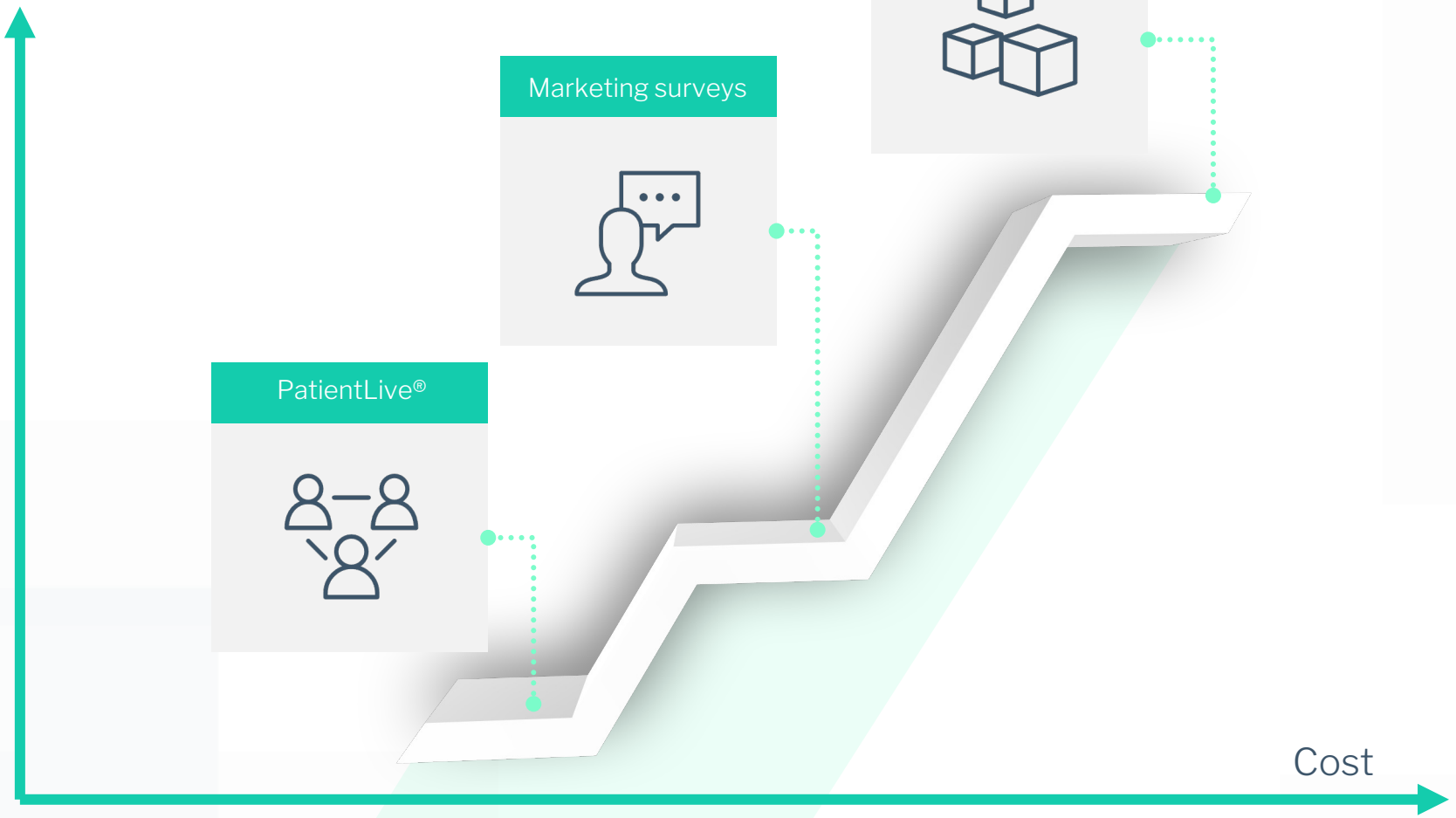
EviendtIQ Patient Digital Platform

+500K

PATIENT COMMUNITY
(Patients + Caregivers)

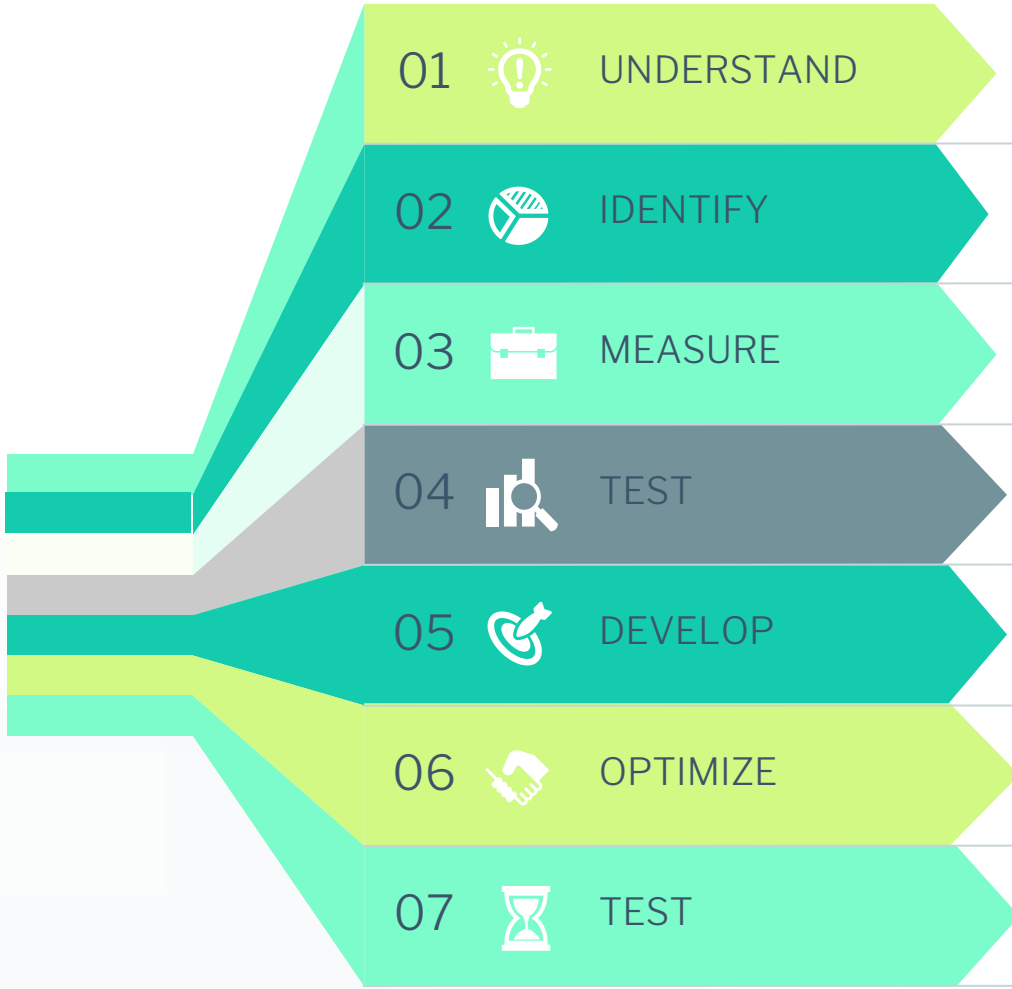


The fastest and easiest way to generate patient insights



Make patient centricity a reality: Optimize your projects with patient insights!

DIGITAL PATIENT PLATFORM



Patient experience: **disease/treatment burden**, quality of life.

Unmet needs in terms of product, services and information.

Patient's perception of your products, devices and services.

Concepts of services, patient tools and campaign messages (messages, visuals, videos...).

Patient-centric material for **clinical trials**.

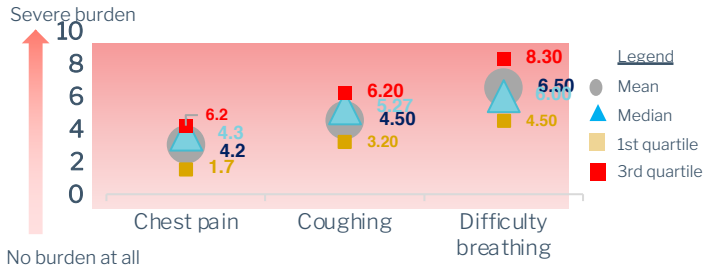
The **design** of your **real-world patient studies** and clinical trials.

Concepts of **services** and **tools** to increase patient retention in your clinical trial.

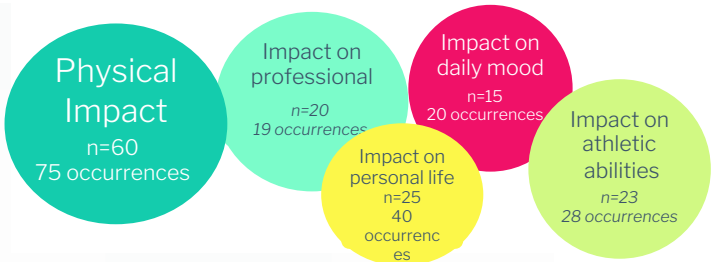
R&D
Respiratory - Asthma
Real-world Patient Insights

Objective: Understand the unmet needs that can be addressed by a future treatment, in order to optimize the inclusion of PROs in a phase 3 clinical trial.
Sample size: n=100 patients affected by asthma and on a biologic
Country: US

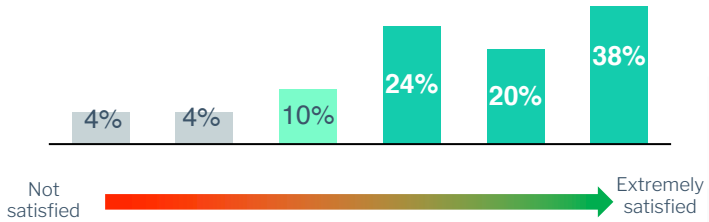
01 BURDEN OF CORE ASTHMA SYMPTOMS



02 IMPACT OF ASTHMA ON PATIENTS' QUALITY OF LIFE



03 OVERALL SATISFACTION WITH CURRENT BIOLOGIC TREATMENT



04 EXPECTATIONS REGARDING BIOLOGICAL TREATMENT - VERBATIM

Improve asthma control
Reduce all asthma symptoms (25)
Better breathing control (10)
Fewer attacks (9)

Easier access to injections
Ease of administration (7)
Self-administration (4)

Improve quality of life
Improve ability to engage in activities (14)
More stability (13)
Increase energy (9)

Reduce need for medical care
Reduce frequency of administration (8)
Reduce need for rescue medication (inhaler) (7)

Preferred route of administration for control medication

KEY INSIGHTS

- Negative impact of asthma on QoL due to **burdensome symptoms**
- Patients' primary concern is to **improve** treatment **efficacy** on **symptoms**, in particular shortness of breath
- Patients **would prefer to receive an injection**, however most would accept oral treatment

USE OF DATA

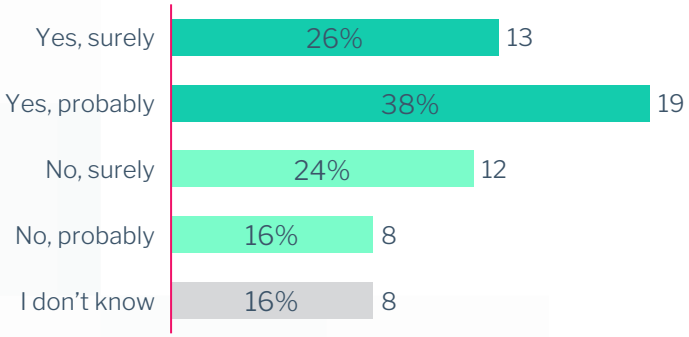
- **Adaptation of future clinical trial design** thanks to patient insights
- **Comparison of asthma patients' expectations** with the product in development
- **Optimization** of key indicators for the **clinical trial**

Protocol feasibility assessment
Clinical Operations
Autism

Objective: Test protocol of a future Ph. III clinical trial for children with Autism to optimize patient recruitment
Sample size: n=50 families of children and adolescents with Autism Spectrum Disorder aged from 2 to less than 18 years
Country: France

01

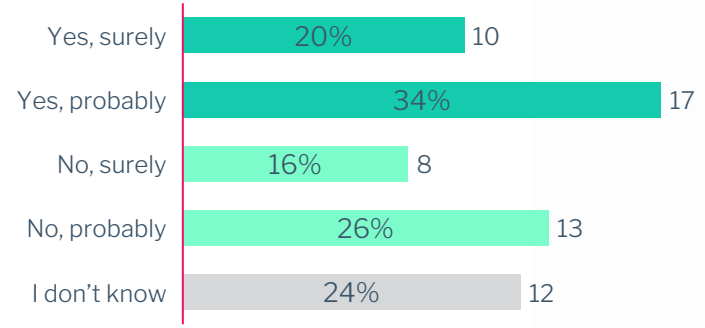
Clinical trials are conducted to demonstrate the efficacy of a future treatment. Would you be willing to accept your child's participation in this type of clinical trial?



Examples

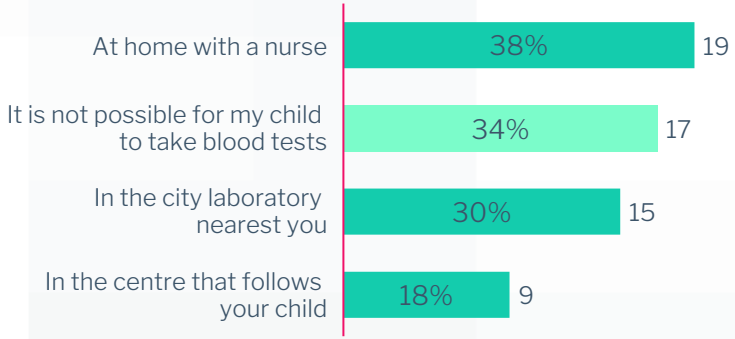
02

As part of a clinical trial you must go with your child every 15 days for 4 months to the investigator site for various medical examination for about 2 hours. Is that possible for you?



03

If blood tests were to be carried out frequently on your child. What would be the ideal location?



Base: n = 50 respondents

KEY LEARNINGS

- **64%** of parents would be **willing to let their child participate** in a clinical trial
- The design of the clinical trial is suitable for **54%** of parents.
- The ideal place for **blood tests** is at home with a nurse.

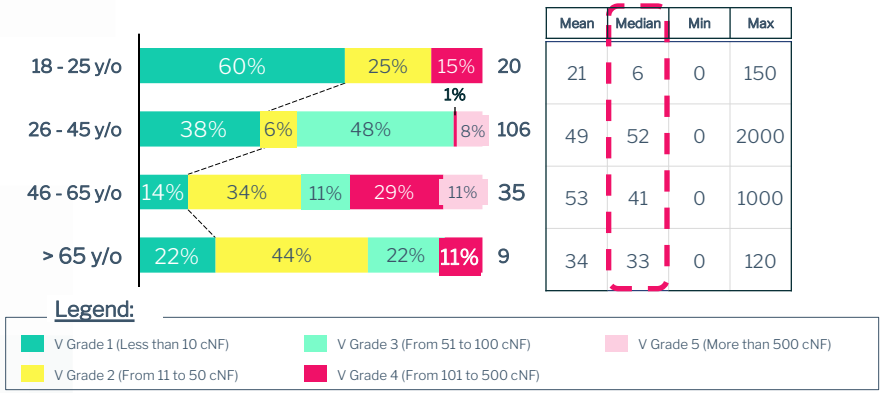
USE OF DATA

- Anticipate potential blocking points for patient recruitment and alert global team.
- Adapt the design of the future clinical trial thanks to patient insights.

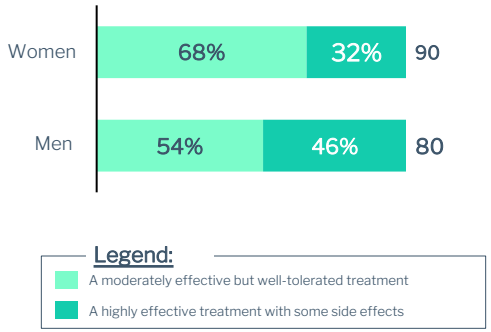
Target Product Profile
Neurofibromatosis
R&D

Objective: Define the burden of neurofibromatosis and patient expectations in terms of new treatment
Sample size: n=170 respondents – Patients with neurofibromatosis type 1
Country: EU5 - with Advisory Board

Examples

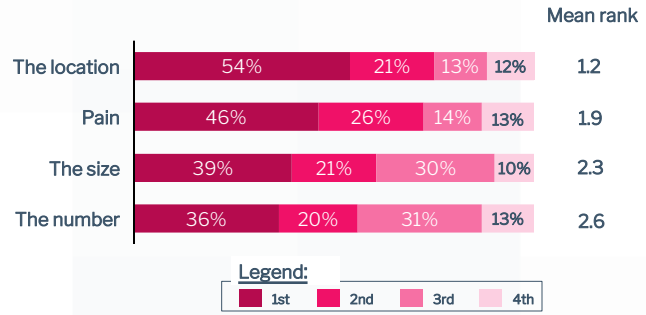


Mean	Median	Min	Max
21	6	0	150
49	52	0	2000
53	41	0	1000
34	33	0	120



1 Number of visible cutaneous neurofibromas

2 Inconveniences caused by cutaneous neurofibromas



3 Efficacy criteria of a future treatment
Depending on the country

Mean rank	Prevent the appearance of cNF	Block growth of cNF	Reduce the itching or pain of the cNF	Reduce the size of cNF
	2.1	4.7	3.0	4.2
	2.8	4.6	2.9	3.9
	2.9	4.7	3.2	3.6
	2.3	4.3	3.4	3.7
	2.1	4.4	3.2	3.9

Ranking question: 1= this criterion is the most important. 6= this criterion is the least important

4 Risk Benefit Balance
Depending on the gender

Base: n = 170 respondents

KEY LEARNINGS

- Patients with neurofibromatosis suffer from the visibility of their disease. The location of cNF is the main disadvantage caused by the disease followed by pain, size and number.
- Patients expect the future treatment to prevent the appearance of cNF and reduce itching and pain.
- A moderately effective but better tolerated treatment is also preferred.

USE OF DATA

Compare the expectations of patients with neurofibromatosis type 1 with the product under development.

Publication of results: preparation of an article to be published in a **scientific journal**.

Take away messages

- Patient are true expert of their pathology and the burden they experienced

- Getting their insights can improve :
  - protocol study design
  - accuracy of symptom evaluation,
  - quality of data
  - increase study acceptance therefore patient recruitment and retention

- Accessing quickly to a digital patient community facilitate to get those patient insights and therefore performing RCT and RWD Clinical studies

EVIDENTIQ

CLINICAL DATA SCIENCE GROUP

CARENITY – Paris (FR)

1 rue de Stockholm

75008 Paris
France

pro@carenity.com

EVIDENTIQ

Grosse Johannisstr 7
20457 Hamburg

Germany

www.evidentiq.com