MINISTERIO
DE SANIDAD, SERVICIOS SOCIALES
E IGUALDAD

agencia española de
medicamentos y
productos sanitarios



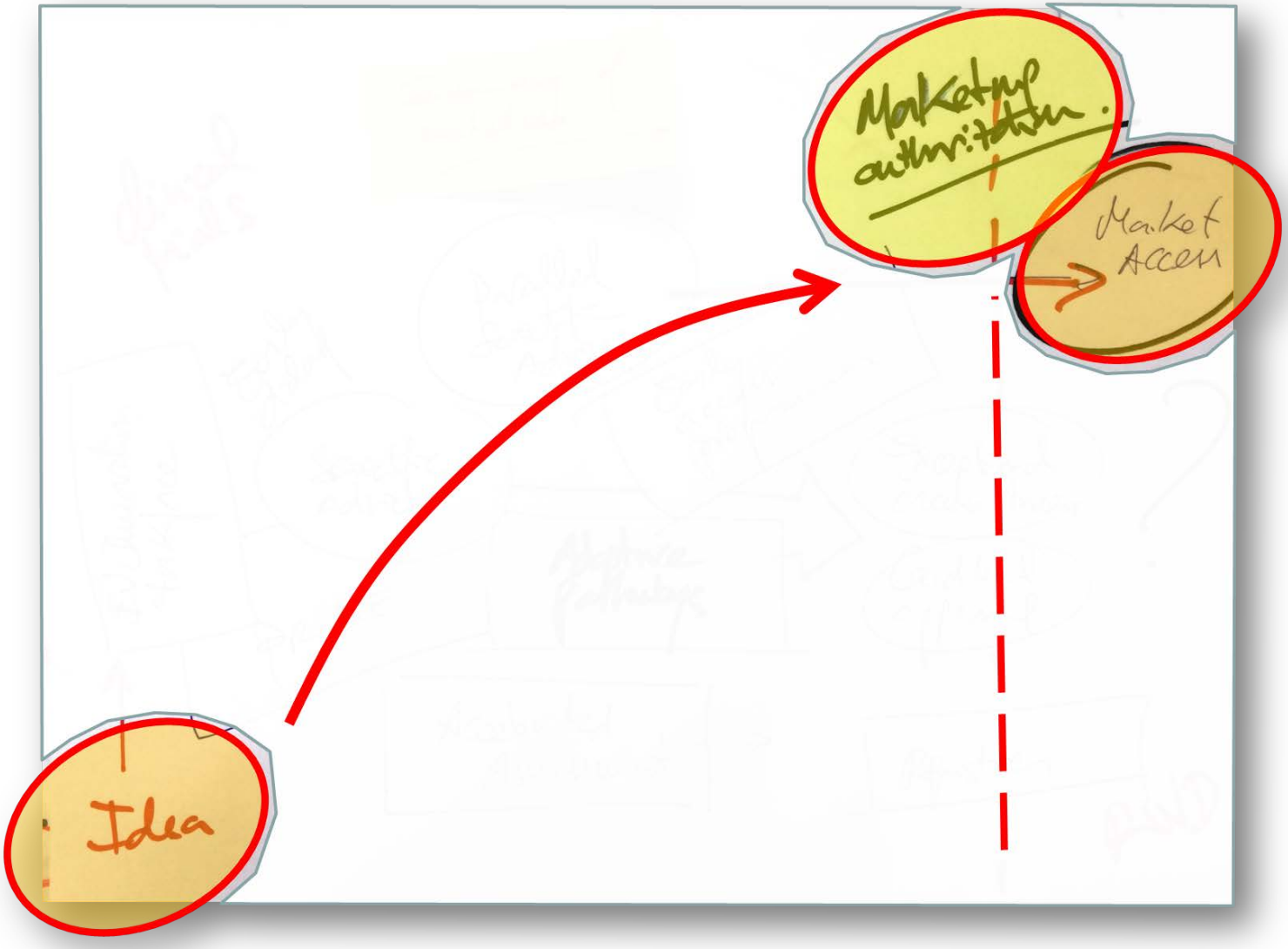
The involvement of patient organisations in the Regulatory Agencies across Europe

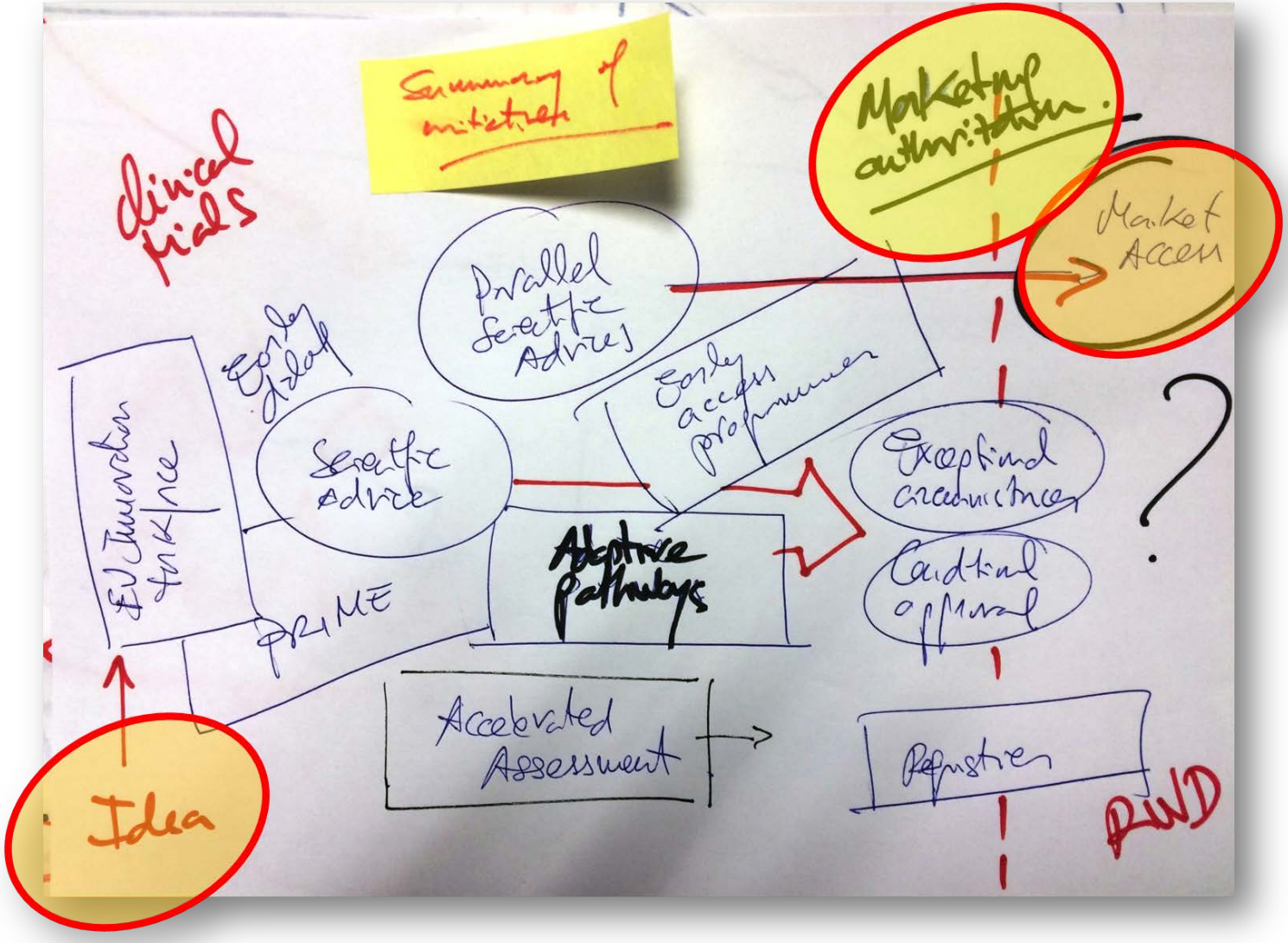
The Spanish case for medicines

Our view...

An integrated view of life cycle of medicines

- Integration of procedures along development of medicines up to access
- Integration into a single, cooperative regulatory network
- Integration with any relevant stakeholder, notably patients, in any pace of this journey


Idea


Clinical trials
Summary of activities
Marketing authorisation
Market Access
Parallel Scientific Advice
Early dialogue
Scientific advice
Early access programmes
EU Innovation Network
PRIME
Adaptive Pathways
Conditional approval
Accelerated Assessment
Registries
Idea
R&D
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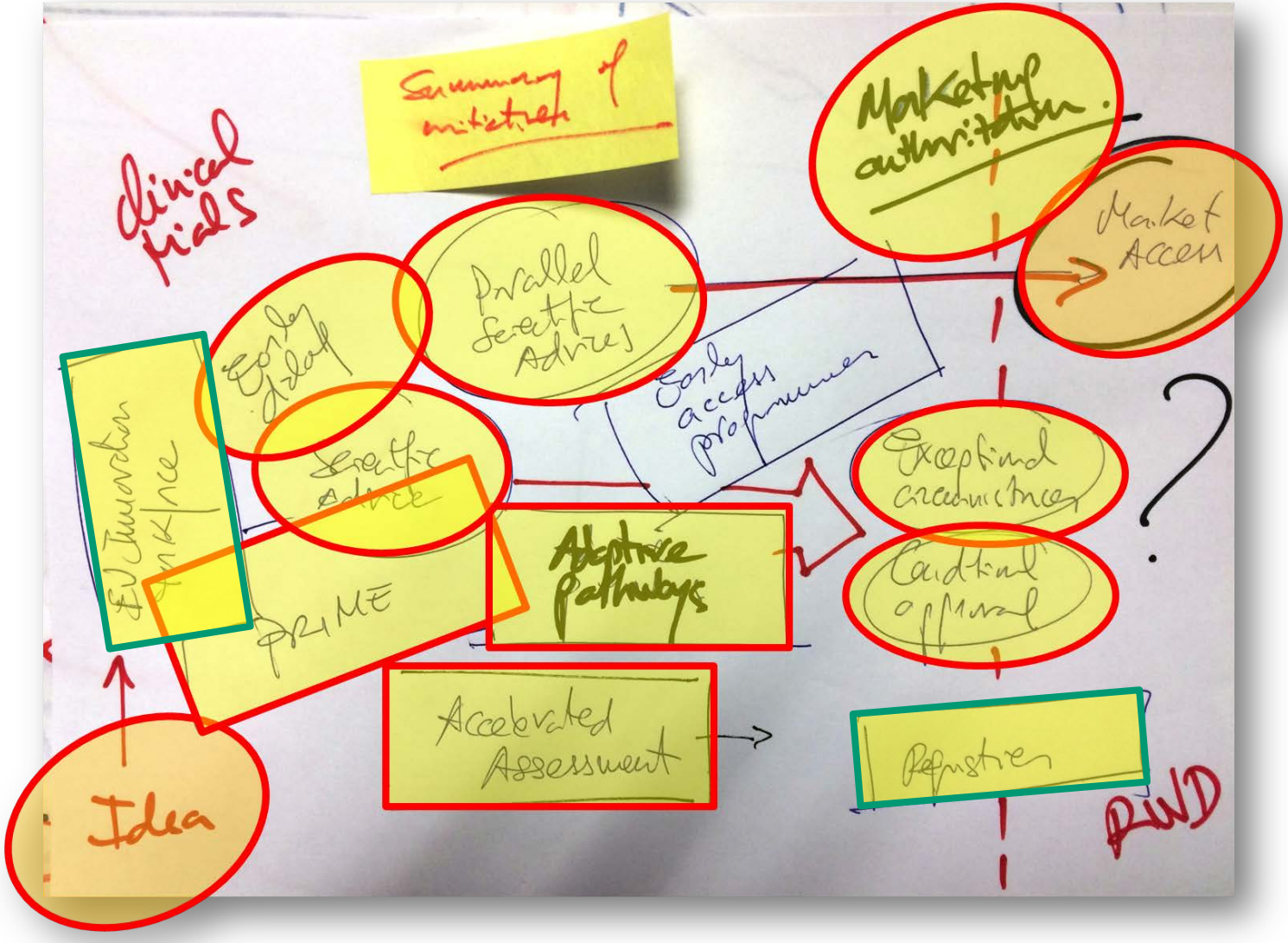
Summary of
Marketing
authorisation
clinical
trials
Market
Acces
Parallel
Scientific
Advice
Early
Dialog
Early
access
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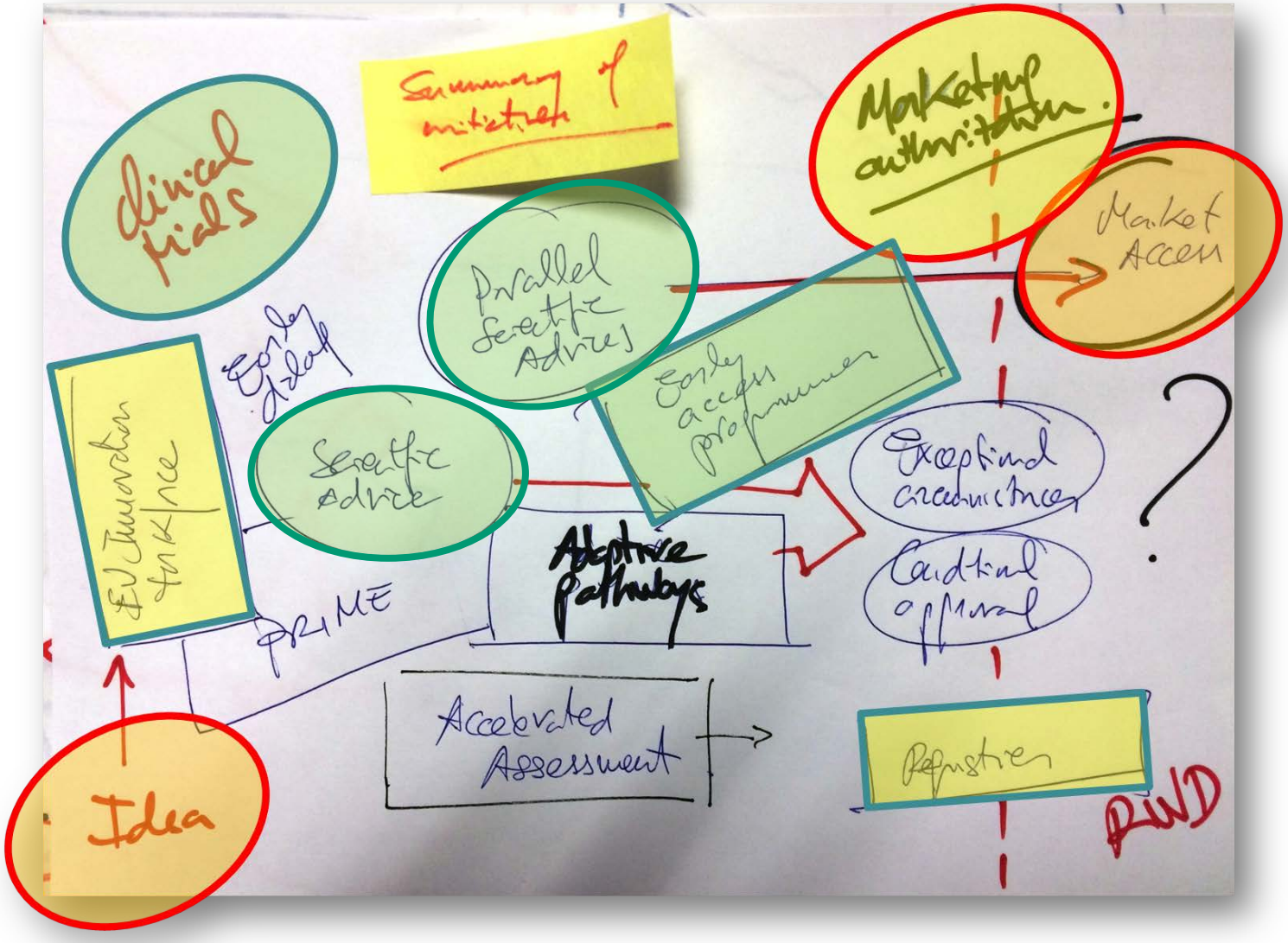
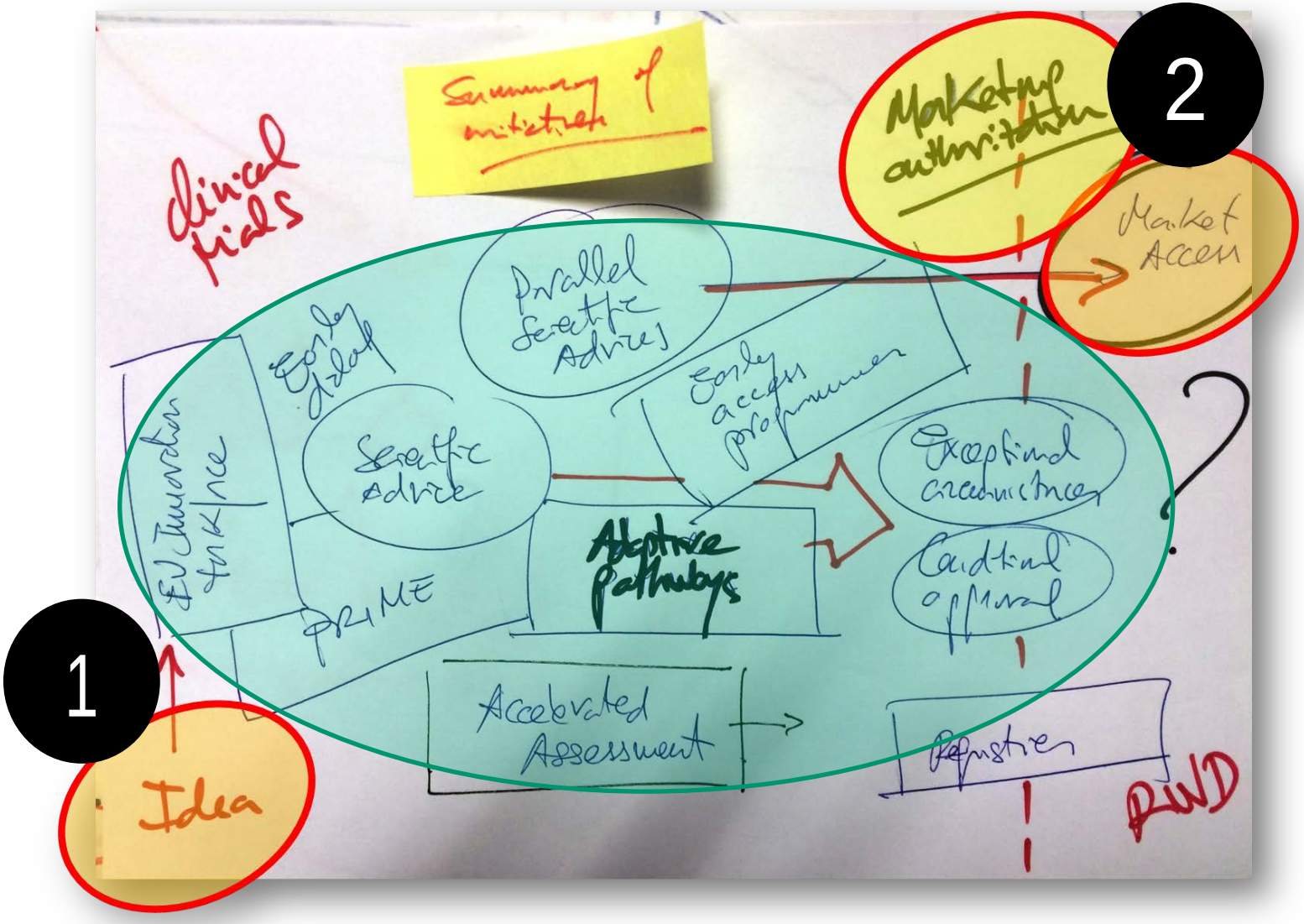
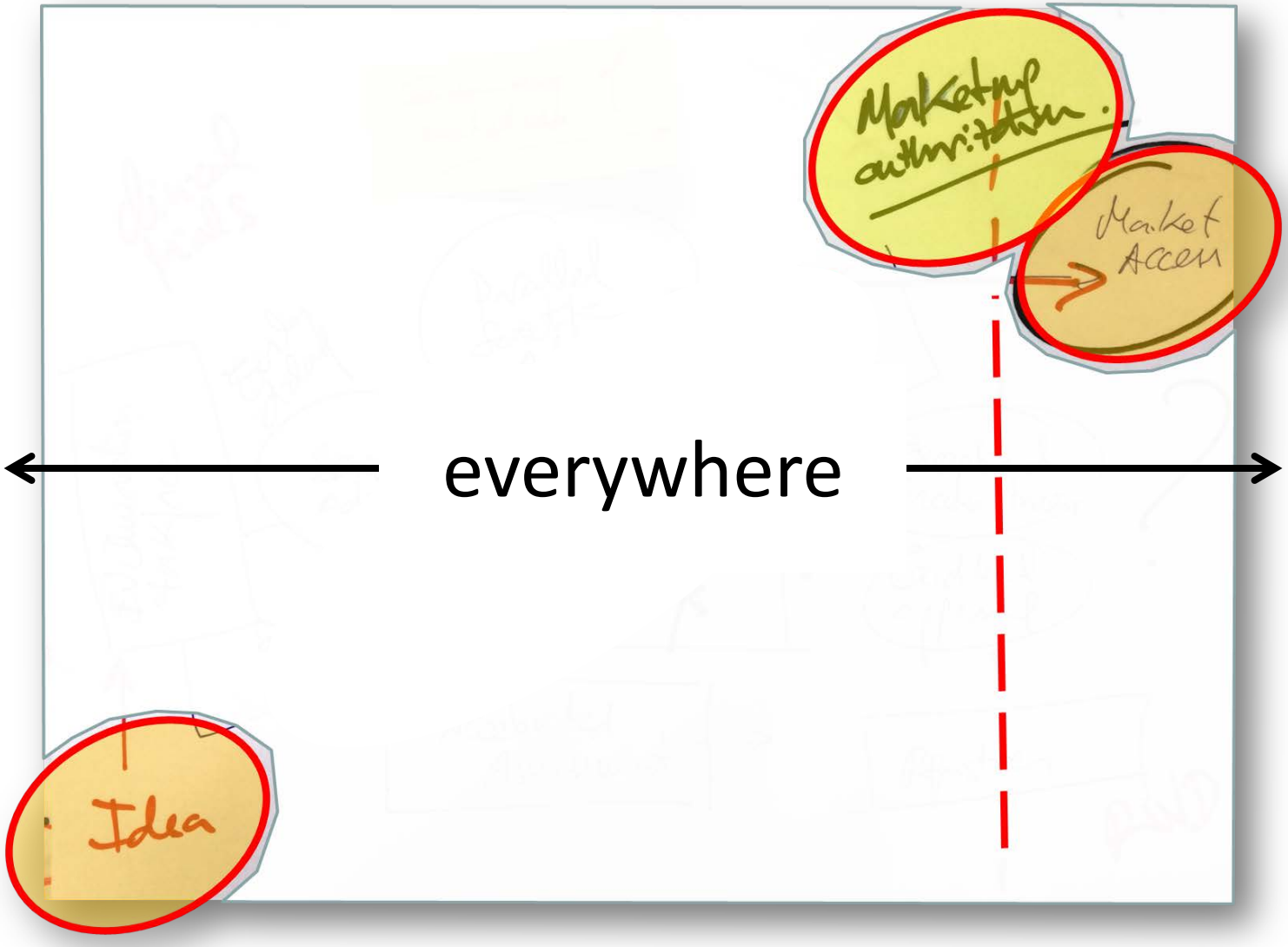
EU Innovation
Scientific
advice
Exceptional
circumstances
Adaptive
pathways
PRIME
Conditional
approval
Accelerated
Assessment
Registration
Idea
R&D


Clinical trials
Summary of evidence
Marketing authorisation
Market Access
Parallel Scientific Advice
Early access programmes
Early dialogue
Scientific advice
EU Innovation Network
Exceptional circumstances
Adaptive Pathways
Conditional approval
PRIME
Accelerated Assessment
Registration
Idea
R&D
?



Take off and landing

Where can patients be integrated?


everywhere
Idea

Clinical trials...

- As experts for the sponsors (both commercial and non-commercial)
- As sponsors of non-commercial CT
  - *The agency gives full support to this initiatives through the Office for Supporting Clinical Investigation*
- As members of Ethics Committees (EC)
  - *Since January 2016 all EC should incorporate patients representatives*
- As participants in CT

In the evaluation of the results…

- As members of the Committees
  - *The agency had already representatives of consumers in its Committees*
  - *Since 2016 also incorporate patients representatives in the Safety Committee and the Committee of Medicines for Human Use*
- *Patients' associations are regularly consulted in the Therapeutic Positioning Reports that coordinates the Agency as HTA for medicines*

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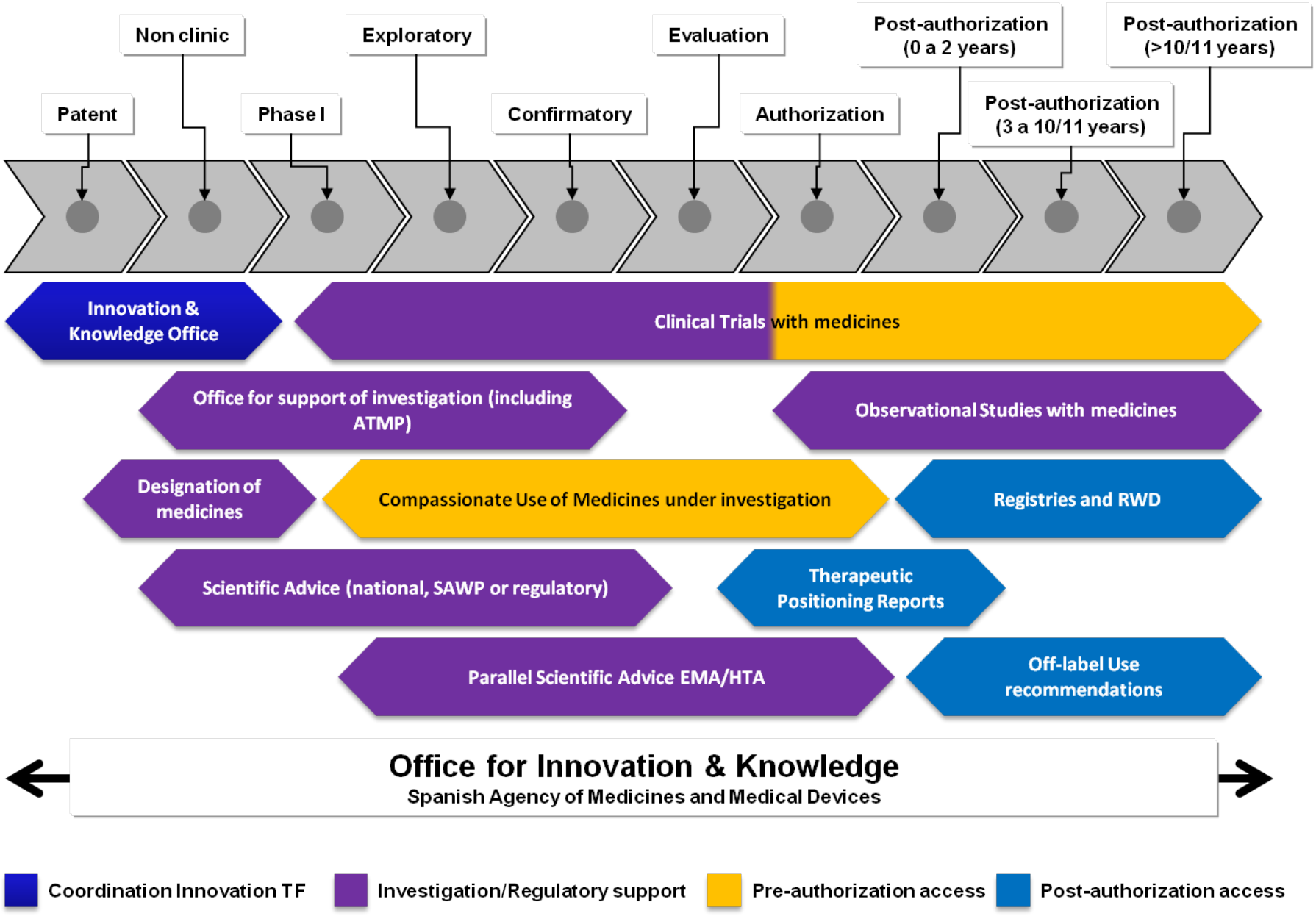
Services provided by the office for **support of innovation** and knowledge of medicinal products

01. Coordination with the European Innovation Offices Network

02. Support in Investigation

03. Access to medicinal products prior to their authorisation

04. Access to medicinal products after their authorisation


Patent
Non clinic
Phase I
Exploratory
Confirmatory
Evaluation
Authorization
Post-authorization (0 a 2 years)
Post-authorization (3 a 10/11 years)
Post-authorization (>10/11 years)
Innovation & Knowledge Office
Clinical Trials with medicines
Office for support of investigation (including ATMP)
Observational Studies with medicines
Designation of medicines
Compassionate Use of Medicines under investigation
Registries and RWD
Scientific Advice (national, SAWP or regulatory)
Therapeutic Positioning Reports
Parallel Scientific Advice EMA/HTA
Off-label Use recommendations
Office for Innovation & Knowledge
Spanish Agency of Medicines and Medical Devices
Coordination Innovation TF
Investigation/Regulatory support
Pre-authorization access
Post-authorization access

3.2. Compassionate use of investigational medicinal products

Along with the clinical trials, compassionate use of the investigational medicinal products is another access route prior to authorisation which is regulated by Royal Decree 1015/2009 on the use of medicinal products in special situations.

Compassionate use is the use of a medicinal product prior to its authorisation in patients suffering from a chronic or seriously debilitating disease or that is considered as life-threatening and is unable to be treated satisfactorily with an authorised medicinal product. Said medicinal product should be subject to an application for marketing authorisation or be undergoing clinical trials.

The application process for compassionate use to the Agency is initiated by a physician when faced with a patient with an unmet medical need.. This application is conducted through the hospital centre, prior approval from the General Directorate of said centre, and should include a clinical report of the responsible physician in which the need of the medicinal product for the patient is justified, the conformity of the stakeholder or sponsor of the clinical trials to provide the medicinal product and the number of containers required. The informed consent of the patient or his/her representative does not form part of the application for authorisation to the Agency but is essential before administration of the medicinal product.

Compassionate use should not substitute clinical trials as the best standard to generate useful knowledge of medicinal products, but may complement this generation of knowledge in certain cases such as, for example, their use under real conditions from the initial stages of use of the medicinal product.

Over and above the general information, individual patients should channel their needs for information through the healthcare professionals who are attending them, given that these are specific situations which require specific clinical care.

Likewise, the AEMPS has a specific mailbox in which the doubts or suggestions of the patient associations to whom questions can be channelled.

The applications of hospital centres are submitted through the application of medicinal products in special situations. As well, the AEMPS has a contact point for healthcare centres and professionals that also serves to channel the doubts of sponsors and stakeholders of medicinal products.

ADDRESSEES
Patients and patient associations, healthcare professionals, healthcare centres

PRODUCTS
Medicinal products not yet authorised in Spain that are under clinical investigation in any of their phases

INTERACTION
Assessment of possible access to medicinal products in the investigational phase

EXPECTED RESULT
Access to treatment with medicinal products in the investigational phase wherever appropriate

MORE GENERAL INFORMATION
https://sede.aemps.gob.es/usoHum/otros/medSituEspe.htm

CONTACT POINT FOR HEALTHCARE CENTRES AND PROFESSIONALS
medicamentosespeciales@aemps.es

4. Access to medicinal products after their authorisation

4.1. Therapeutic Positioning Reports

In collaboration with the health authorities of the Autonomous Communities and the General Directorate of the Basic Services Portfolio of the National Health and Pharmacy System, the AEMPS coordinates the preparation of therapeutic positioning reports (Informes de posicionamiento terapéutico (IPT), in Spanish) aimed at determining the therapeutic value of the new medicinal products when compared to their alternatives in the market.

The Therapeutic Positioning Report is a scientific document that addresses the position which a certain medicinal product occupies in therapy in the light of the existing knowledge at any given time. Healthcare technicians and professionals of the collaborating institutions participate in their preparation, and the document is also subject to consultation by patient associations, scientific societies and the marketing authorisation holder himself.

The Therapeutic Positioning Reports are geared to identifying the therapeutic value of a medicinal product compared to its alternatives so that this may be used in decision-making process of price/reimbursement and, subsequently, the incorporation of the medicinal product in clinical practice.

The network assessment system coordinated from the AEMPS is centred on the so-called Therapeutic Positioning Coordination Group (Grupo de Coordinación del Posicionamiento Terapéutico (GCPT), in Spanish) of Human Use Medicinal Products. The Therapeutic Positioning Coordination Group is composed of the Head of the Department of Human Use Medicinal Products of the AEMPS, a

representative of the General Directorate of the Basic Services Portfolio of the National Health and Pharmacy System, a representative of each Autonomous Community and the secretariat of the group held by the AEMPS.

The Therapeutic Positioning Coordination Group normally meets 11 times a year and, among its functions, establishes the scope of the reports, assigns their execution, ensures compliance with the established standards and time schedules, approves the reports, prioritises their execution and proposes follow-up measures for the reports undertaken.

ADDRESSEES
Health authorities, patient associations, scientific societies, marketing authorisation holders of new medicinal products

PRODUCTS
Medicinal products with new active substances and new indications of medicinal products already authorised

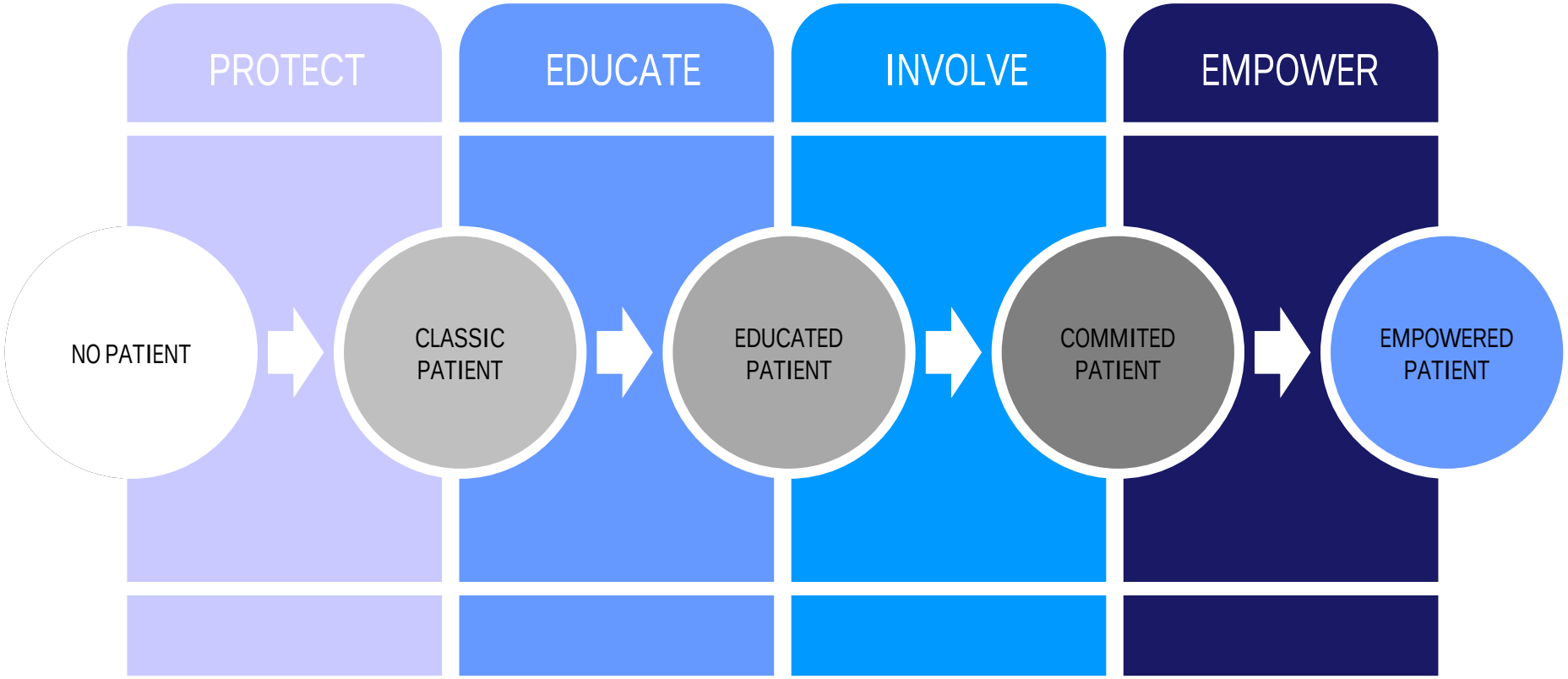
INTERACTION
Participation and consultation in the preparation of therapeutic positioning reports

EXPECTED RESULT
Determination of the therapeutic value of new medicinal products when compared to their alternatives in the market

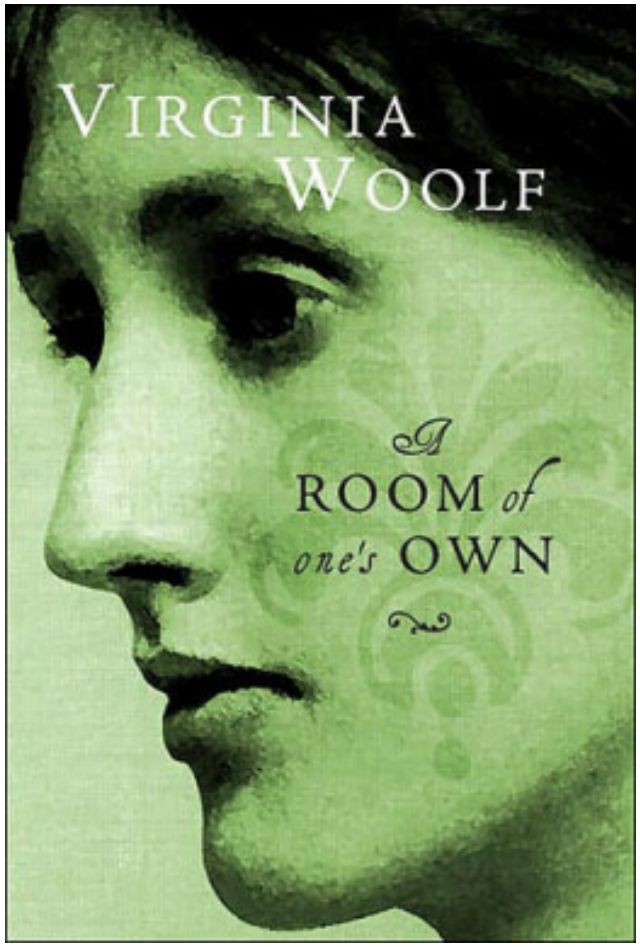
MORE INFORMATION
http://www.aemps.gob.es/medicamentosUsoHumano/informesPublicos/home.htm

CONTACT POINT
gcpt@aemps.es

The journey of patients...



What do the patients need?
How can we help?



It was 1929...

Virginia Woolf gave a conference about the reasons why there had been so few women writers up to then.

According to her views, women lacked their own space (a room of their own), independence (five hundred pounds a year) and tradition (a group of women writers who could be their models)

What do the patients need?
How can we help?

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Collaboration with EUPATI Spain

Initiatives ongoing…

- A close relationship with EUPATI Spain and other patient's initiatives to promote a critical mass of trained patients who can participate in all activities related to the development, authorization and access to medicines
- A kind of ombudsman for patients?
- A Committee just for patients?

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Thank you for your attention

More information

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