EFCCA CONFERENCE OCTOBER 2024

Optimising the involvement of EFCCA patients in medicines-related decision making

Findings from experts' committees

Néboa Zozaya
Manager of Health Affairs & Policy Research - WEBER

Fundación Weber

Saturday, October 12, 2024

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European Federation of Crohn's & Ulcerative Colitis Associations

A CHANGING REGULATORY FRAMEWORKIN EUROPE

EU PHARMACEUTICAL STRATEGY

EU HEALTH TECHNOLOGY ASSESSMENT (HTA) REGULATION

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WHAT CHANGES WITH THE NEW HTA REGULATION?

Directive 2011/24/EU

Cooperation & exchange of scientific information between the Member States within a ***voluntary*** network made up of national authorities

Non-comparative assessments

2021/2282 HTA Regulation

Joint Clinical Assessments (JCA)

Unique, joint and ***comparative mandatory clinical*** evaluations at the European level

Efficient use of resources

Reduce duplication of scientific evaluations

Transparent and more inclusive framework

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WHAT IS HTA?

Procedure for assessing the added value, effectiveness, costs and broader impact of health care interventions

CLINICAL DOMAINS	• Health problems and currently used health technologies (e.g. medicines, medical devices, surgical procedures). • Description of Health Technology under assessment • Relative clinical effectiveness. • Relative safety.
NON-CLINICAL DOMAINS	• Economic evaluation. • Ethical aspects. • Organisational aspects. • Social aspects. • Legal aspects.

HTA Regulation

National evaluation competences

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THE NEW EU HTA REGULATION

KEY PRINCIPLES OF THE HTA REGULATION

- Only on clinical domains of the assessment: No economic assessment or any conclusion on pricing and reimbursement.
- Driven by EU HTA bodies who remain responsible for drawing conclusions on added value for their health systems.
- High quality, timeliness and transparency.
- Use of joint work in national HTA processes.
- Input from independent experts.
- Stakeholder engagement and inclusiveness.
- Progressive implementation.

FRAMEWORK FOR JOINT HTA COOPERATION

- Joint clinical assessments (JCAs).
- Joint scientific consultations (JSCs).
- Identification of emerging health technologies.
- Common procedures and methodologies across the EU.

Coordination Group

Stakeholder network

- Patient associations
- Consumer organisations
- NGOs in health
- Developers
- Health professionals

Public consultation period is open

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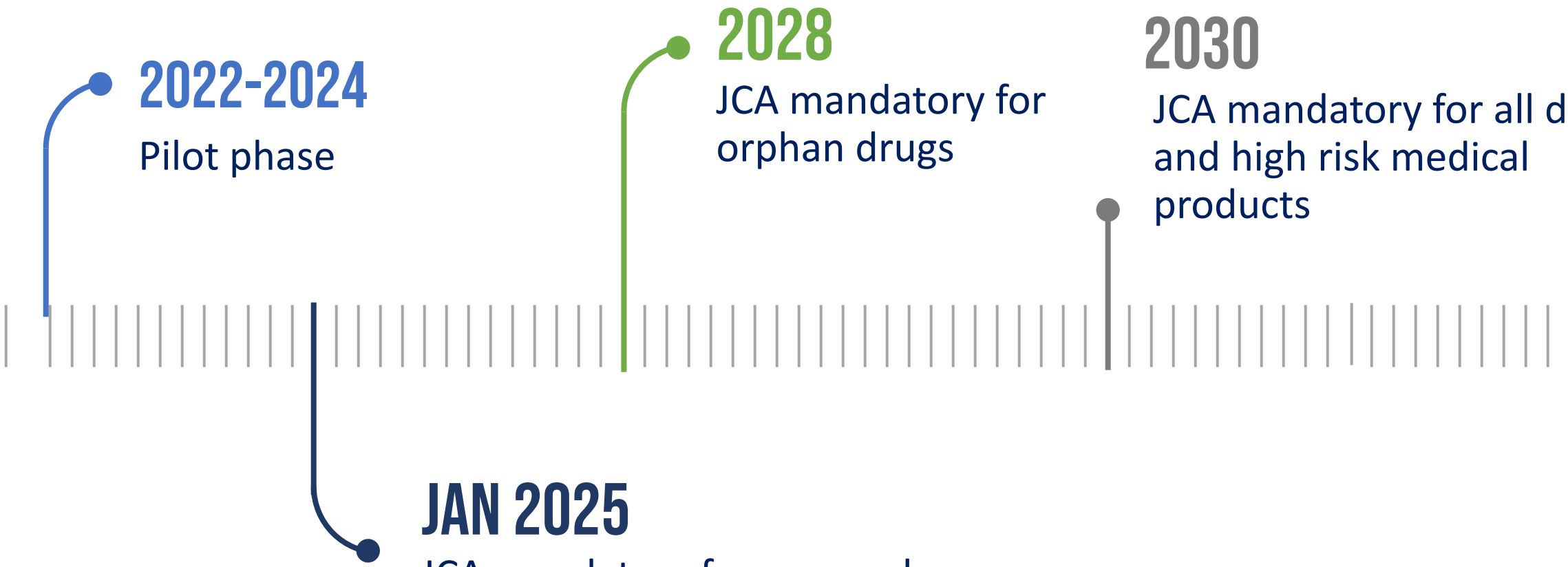
TIMELINE FOR THE IMPLEMENTATION OF JCA



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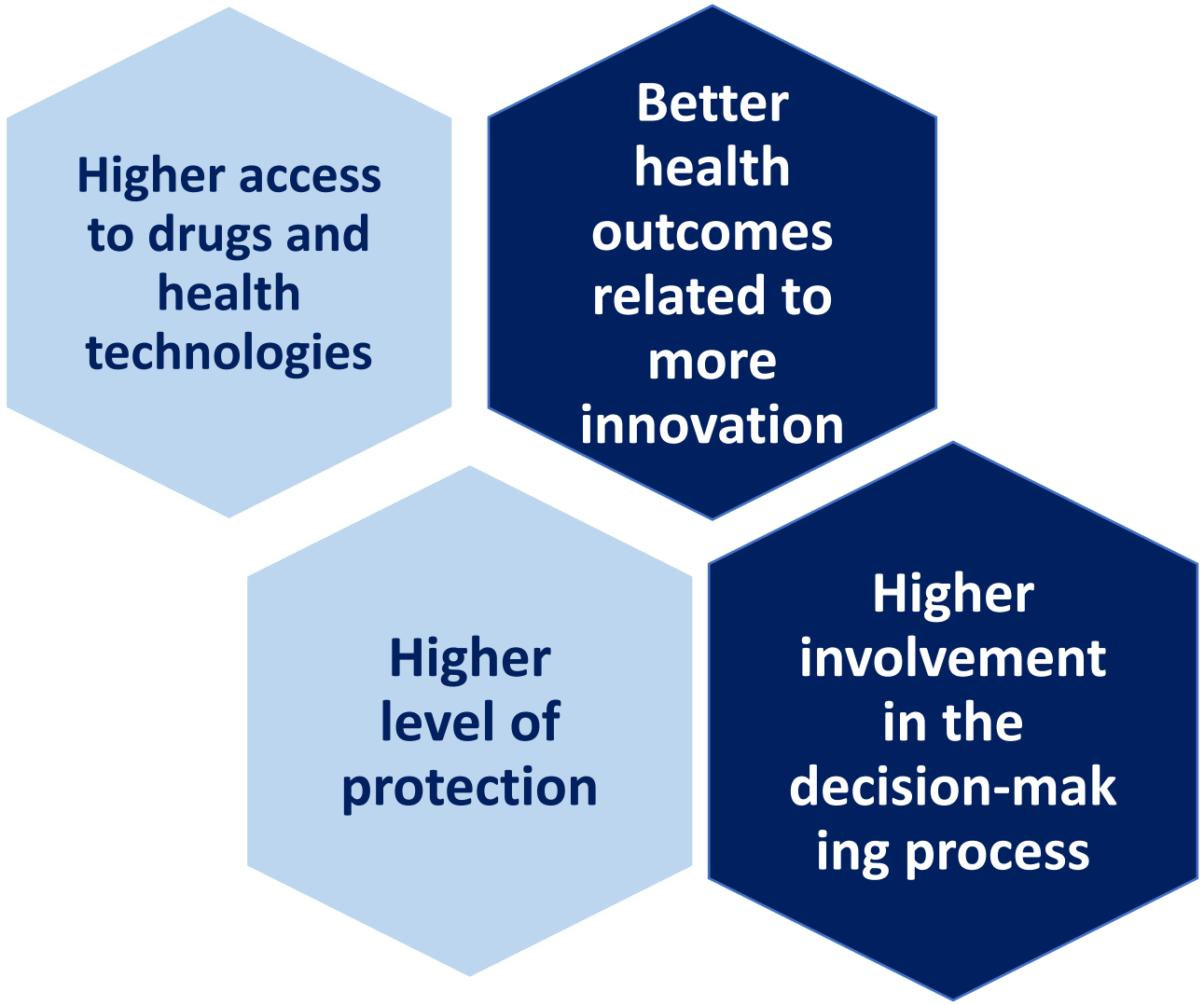
BENEFITS FOR PATIENTS OF THE NEW REGULATION



Patient involvement is crucial for building trust in medication decision-making processes

Democratic	**Scientific**	**Instrumental**
you have the right to participate	enrich available information	improve the quality of the process

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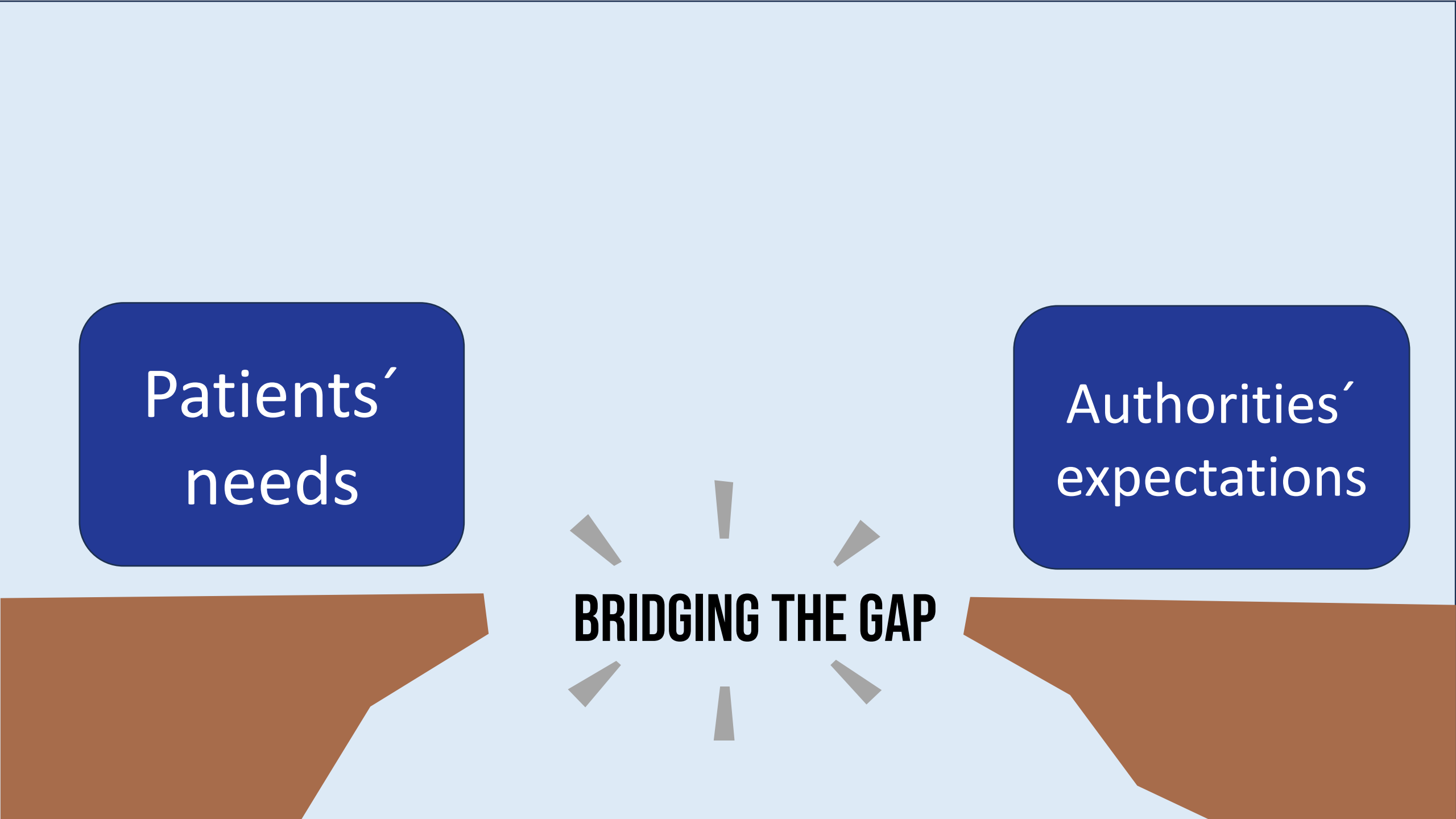
Patients´ needs
Authorities´ expectations
BRIDGING THE GAP

RATIONALE FOR EFFCA´s INITIATIVE

1

Establish a more structured and informed approach to EFCCA's contributions

2

The patient association as the one who organizes an Advisory Committee with experts

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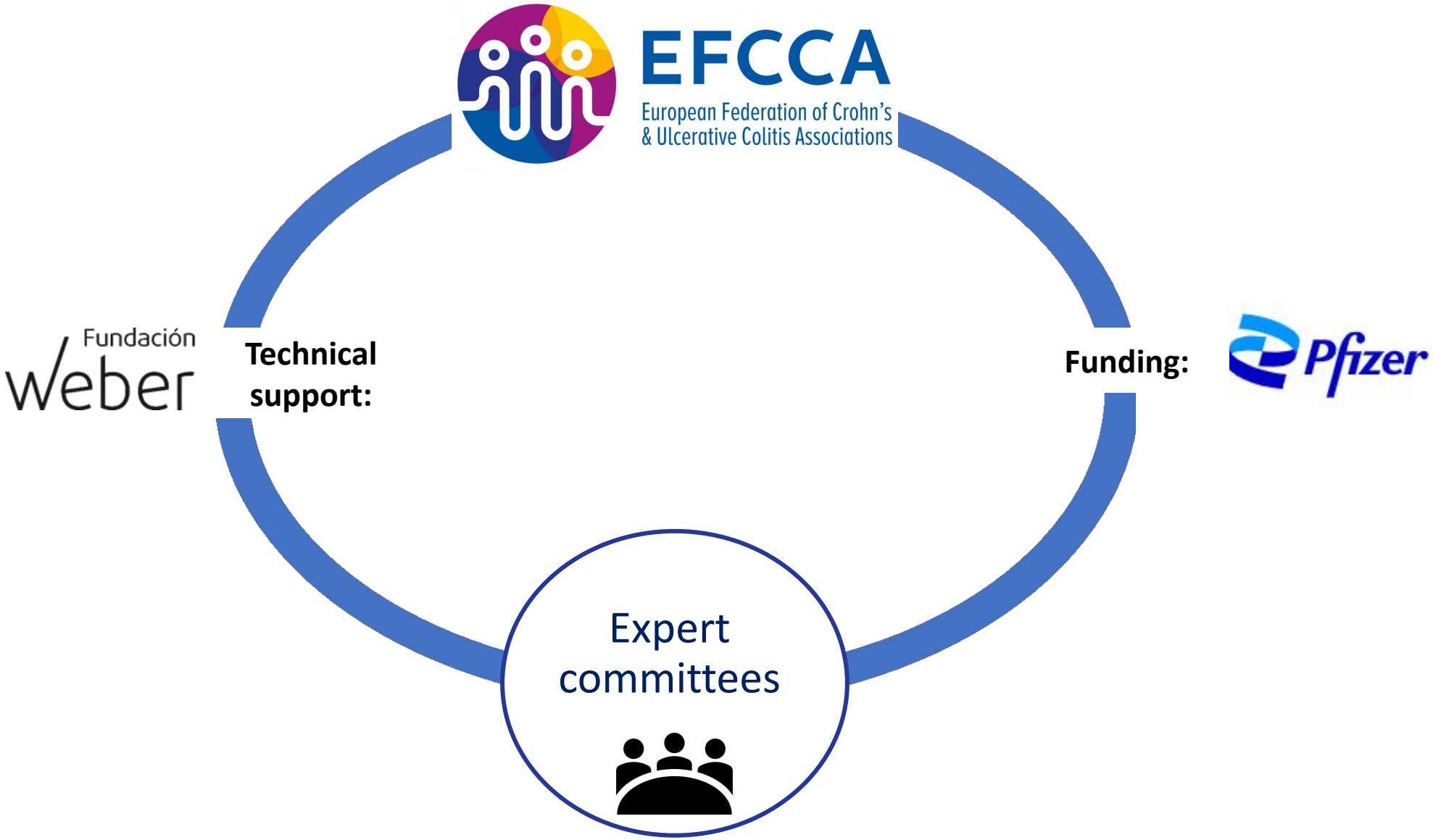
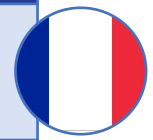
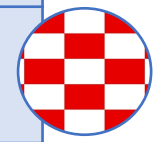
INITIATIVE PARTICIPANTS



EXPERTS PARTICIPATING IN THIS INITIATIVE

	Name	Position, country
Meeting No1 (June 2024)	**Chantal Belorgey**	Ex French National Authority for Health (HAS), France
	Meindert Boysen	Ex Head of International Affairs at the National Institute for Health and Care Excellence (NICE), England
	Luka Voncina	Expert in HTA processes, Croatia
Meeting No2 (September 2024)	**Claudio Jommi**	Professor of Health Care Management at the Department of Pharmaceutical Sciences, Università del Piemonte Orientale, Novara. Advisor to the Italian Medicines Agency (AIFA)
	Jorge Mestre	Expert in pharmaceutical policy, Spain
	Alessando Armuzzi	Inflammatory Bowel Diasease Member of the Center. IRCCS Humanitas Research Hospital. Humanitas University. Milan, Italy. European Chron´s and Colitis Organisation (ECCO)





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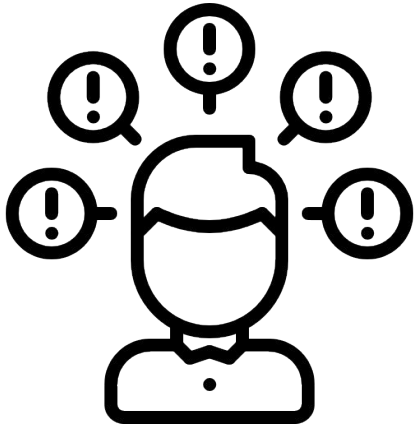
METHODOLOGY



10 main areas

- **Analyse the situation**
- **Share experiences**
- **Give tips, clues, advise**
- **Propose recommendations**

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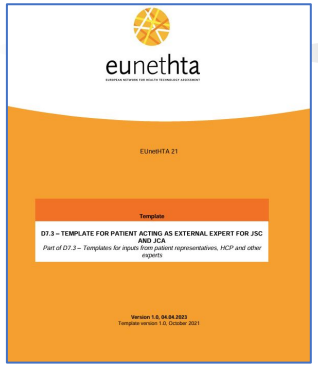
QUESTIONS THAT EFCCA REPRESENTATIVES HAVE HAD TO ANSWER IN RECENT YEARS

- Symptoms duration, intensity, location
- Differences between subgroups of patients
- Impact of the disease in patients' lives
- Impact of treatments on sympoms and lives
- Frequency of hospitalizations
- Risk-benefit willing to accept to avoid symptoms
- Financial considerations

Main topics

Aligned with the EUnetHTA 21 template



- Patient convenience
- New PROs
- Impact on productivity
- Avoided hospitalizations

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MAIN CHALLENGES FOR PATIENTS

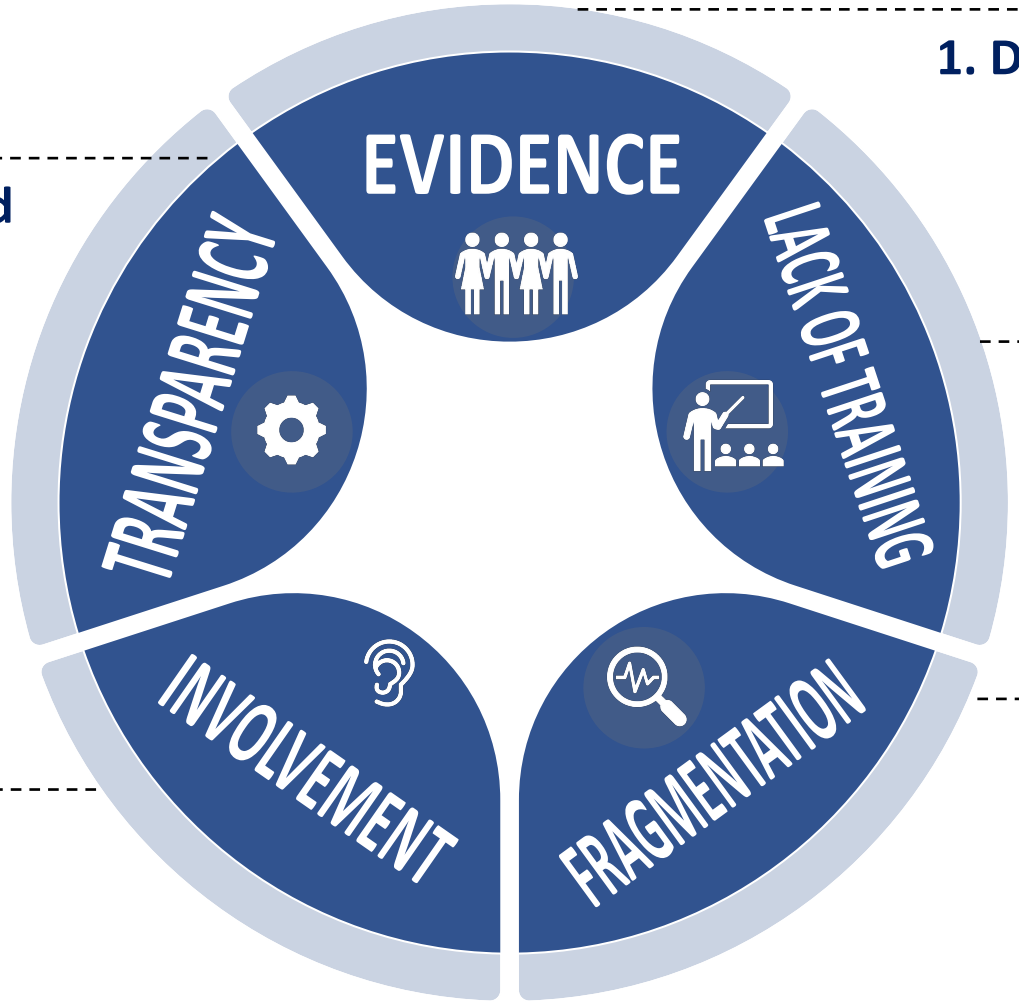
1. Data to justify your answers

2. Training to generate scientific evidence

3. Fragmented framework and engagement

4. Wrong time, wrong reasons for involvement

5. Lack of transparency and conflict of interests



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RECOMMENDATIONS (I)

1. Data to justify your answers

SCIENTIFIC DATA + **TESTIMONIAL/ ANECDOTICAL DATA**

Strong preference for scientific evidence

Valid when scientific evidence is not feasible

Definition of PICOT:

- Population
- Intervention
- Comparator
- Outcomes
- Timeframe

Measure the impact of the disease/treatment on daily life

1. **Patient surveys**
2. **Focus groups**
3. **Interviews**

- ***How representative are your finding?***
- ***Do you have information from patients that are seldom heard?***

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RECOMMENDATIONS (I)

1. Data to justify your answers

Recommendations

- Promote the collection of evidence of data
- Provide clear facts, information and summaries
- Give a **concise**, accurate and balanced overview
- Use key messages and **prioritize** impactful contributions
- Be **practical**: use existing patient submission templates
- Be **transparent**: clearly state information sources

- Find a clear and objective measure of QoL
- Point out differences among individuals regarding gender, age or area
- Collaborate with companies **earlier** in the trial planning stages to define PROs
- Help the industry to **identify and prioritize** PRO that truly reflect patients' needs
- Focus not only on providing evidence but also on the implementation of JCA across countries
- **Collaborate with the academia**!

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RECOMMENDATIONS (II)

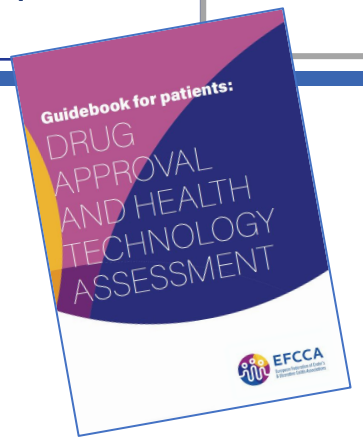
Recommendations

- Learn how to review HTA documentation.
- Learn how to provide input on protocol advice, outcome selection, study design, and comparator choice.
- **Comprehensive training** in HTA methodologies, including courses on statistical methodologies, to understand indirect comparisons.
- Continue working on highly specific strategies tailored to each condition.
- **Plan in advance**!

2. Training to generate scientific evidence

Training for an effective participation in HTA

Significant role of patient organizations in training patients	Use online training materials of **EUnetHTA**	Funding from European sources



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RECOMMENDATIONS (III)

3. Fragmented framework and engagement

- **Uncoordinated nature**
- **Different capabilities**

Recommendations

- Avoid a fragmented representativeness: some issues managed by **umbrella´s associations**.
- Make a **more structured** interaction with other patients.
- Translations for non-English speaker patients.
- Give some framework or guidelines on your collaboration.

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RECOMMENDATIONS (IV)

4. Wrong time, wrong reasons for involvement

- **Involving patients just to inform them, without listening to them**
- **Industry involving patients just if they receive a negative P&R decision**

Recommendations

- Ask for a **more structured interaction** with regulators and industry, and for feedback about your contributions.
- **Ask them to plan in advance** and not to use patients at the end of the process or just as a box-ticking exercise.
- Define in which areas patient associations can be useful, **distinguishing technical from political issues**.
- Prioritize impactful contributions that distinctly influence decision-making processes, ensuring clarity and effectiveness.

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RECOMMENDATIONS (V)

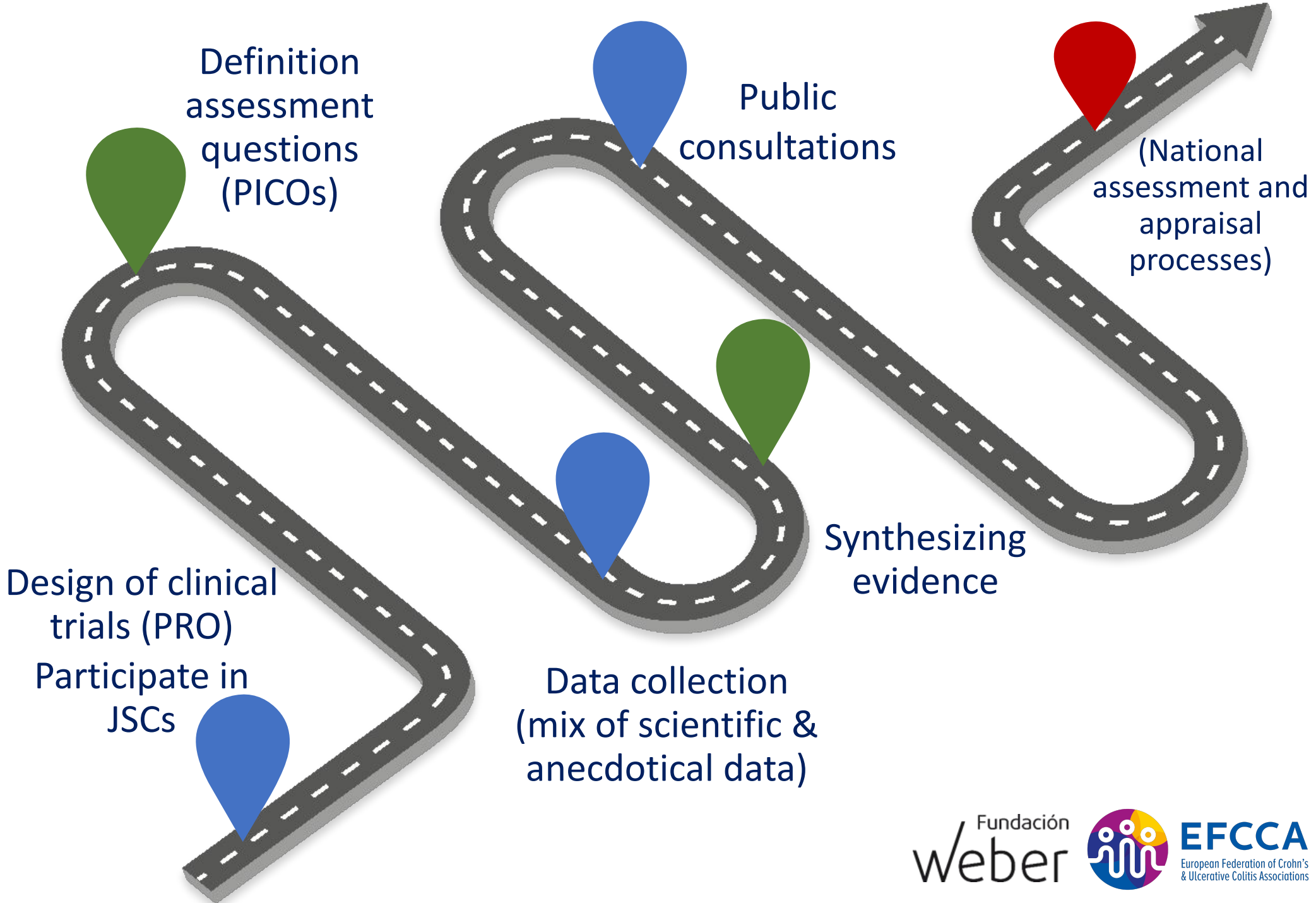
5. Lack of transparency and conflict of interests

Recommendations

- Disseminate the results of surveys, focus groups, etc.
- Ask for more transparency regarding reimbursement decisions.
- The collaboration with pharmaceutical companies is acceptable when done transparently and fairly.
- Maintain transparency to ensure that any collaboration is driven by **genuine concerns** and objectives of the patient community.
- **Important to assert your own voice and priorities while being open about partnerships**.
- Clear governance of the collaboration with the industry to ensure credibility.

Patient participation must be ensured at all stages

The sooner, the better



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MAIN MESSAGE : ”Be prepared, be proactive”

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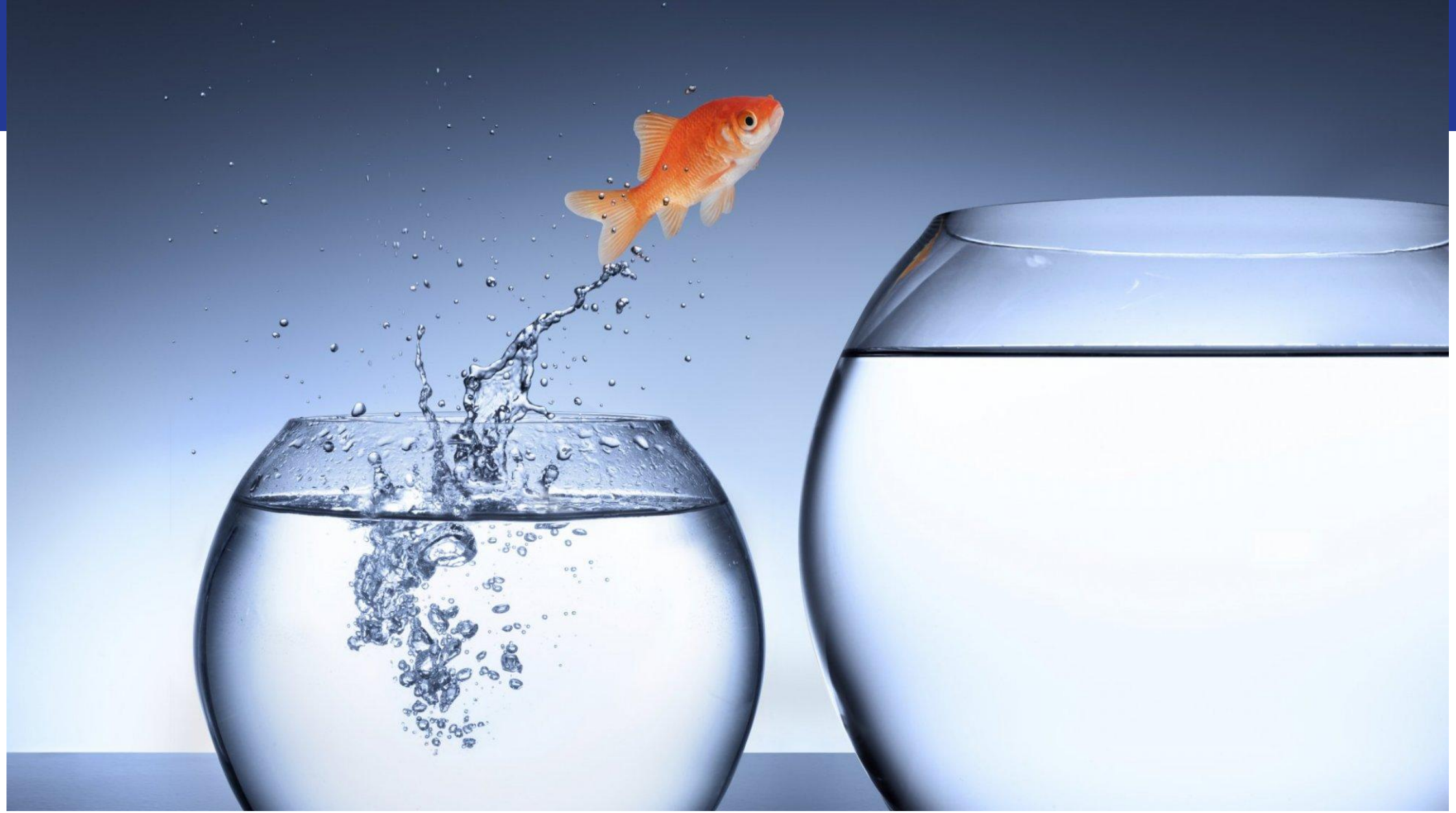

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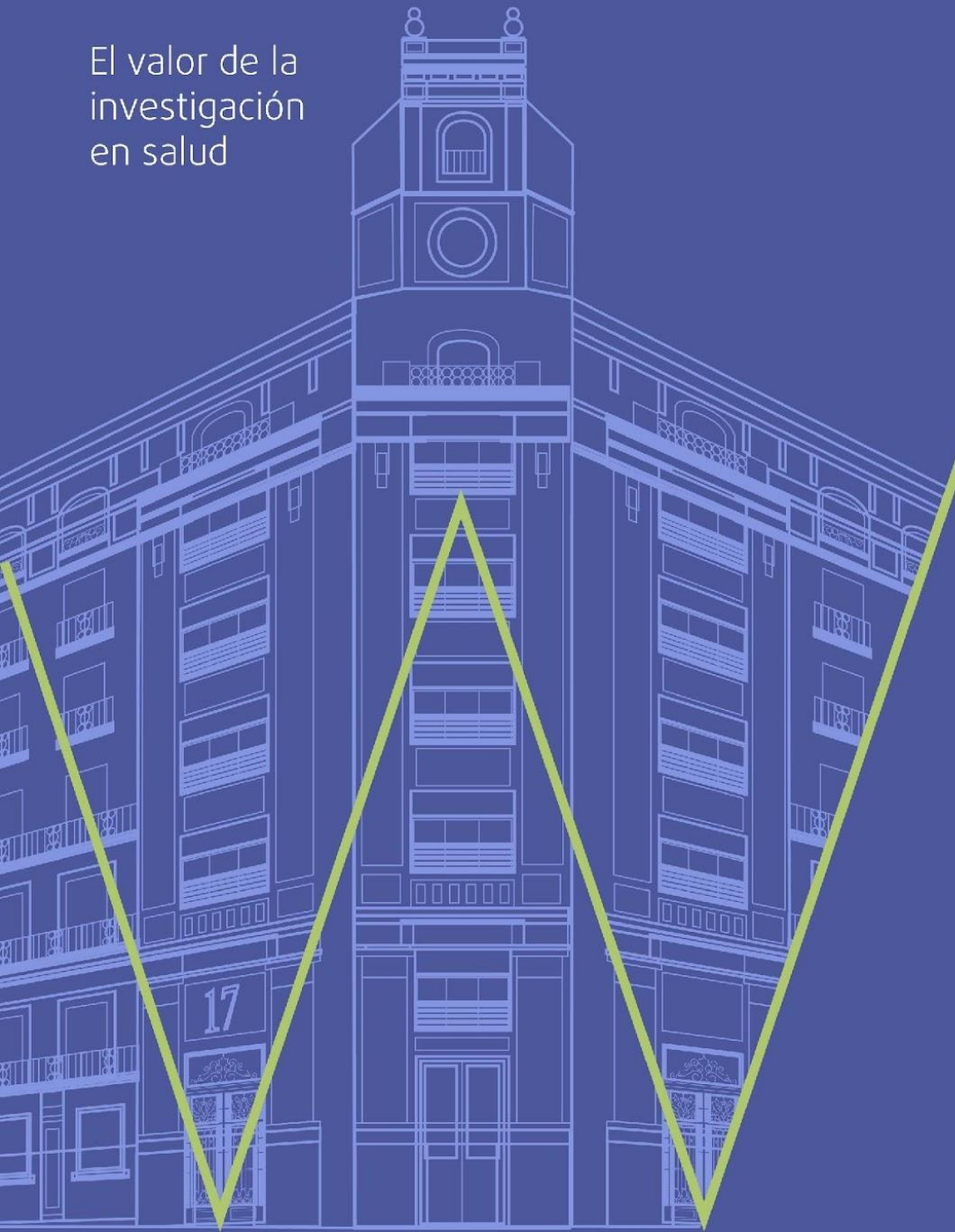
El valor de la investigación en salud



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Thank you!

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