

## for rare diseases in Europe

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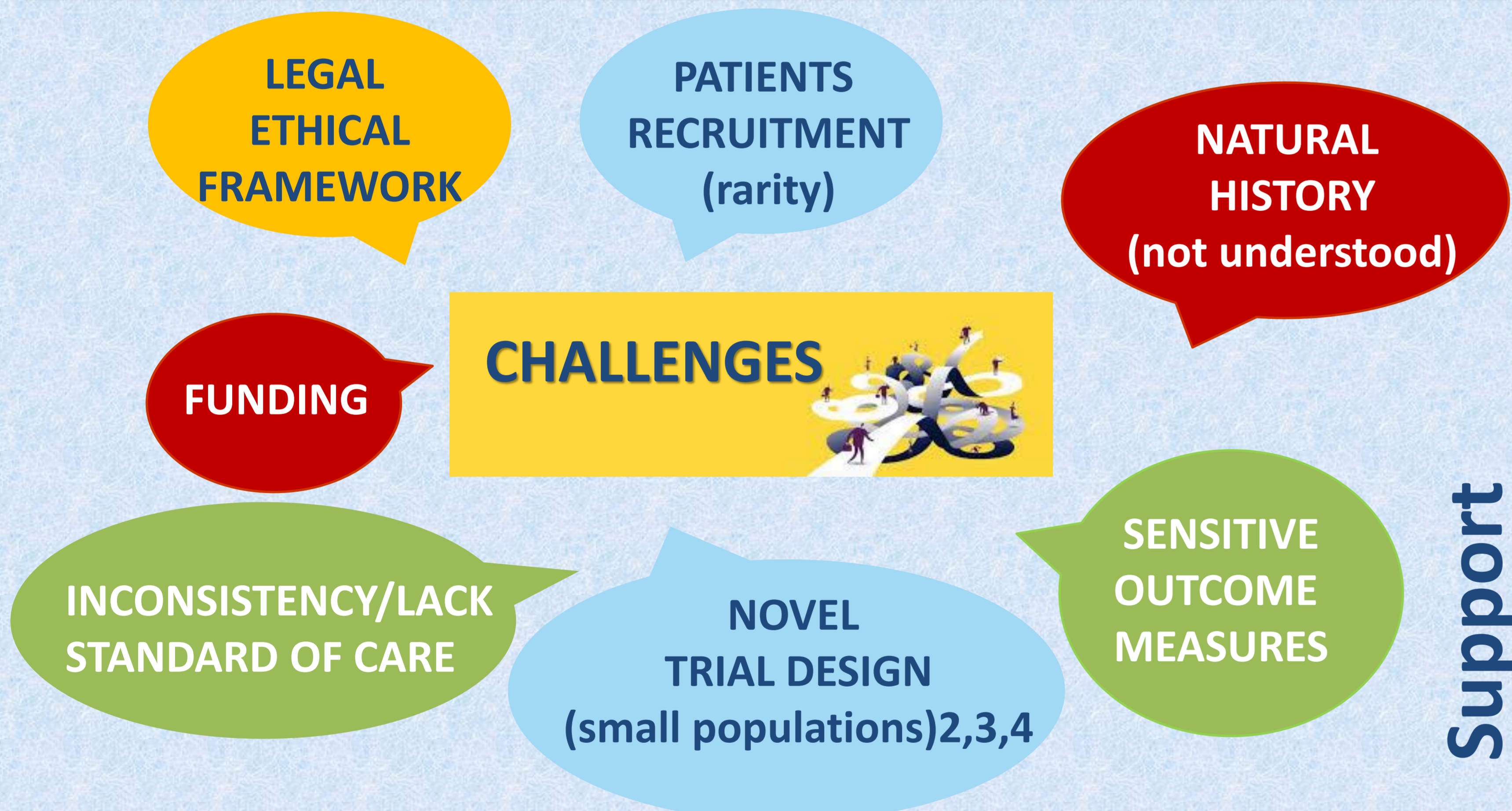
### Background

Investigator-initiated trials are conducted mainly as single-centre or multiple-centre setting in one country. This fact might bias studies' outcomes or limit trial initiation within one country. This last constraint is especially remarkable in the case of rare diseases (RD), considering the limited number of patients per country. The main hurdle for academic investigators is due to the fragmented health and legal systems within Europe. ECRIN's unique pan-European organisation enables to successfully work across borders, coordinating Clinical Trial Units and other stakeholders from multiple countries.

ECRIN can help to circumvent the challenges associated to RD clinical research by supporting European Reference Networks (ERN) investigators in design, planning and performing clinical studies:

- (a). Providing support to plan multinational clinical trials, through a Clinical Trials Helpdesk for Rare Diseases aiming to facilitate access to RD specific expertise, including design aspects, in the framework of the EJP RD project.
- (b). Providing support to perform multinational clinical trials (operational coordination), as part of ECRIN services.

### 1. Clinical Trials for Rare Diseases



Main barriers to the conduct of randomised clinical trials on rare diseases<sup>1</sup>



Some EU initiatives to overcome challenges<sup>1</sup>

1. Rath *et al.* *Trials*. 2017, 18:556
2. ASTERIX Project: Advances in Small Trials Design for Regulatory Innovation and excellence
3. IDEAL Project: Integrated Design and Analysis of small Population group trials
4. INSPIRE Project: Innovative Methodology for Small Population Research
5. List of research infrastructures useful in Europe. Orphanet Report Series

Request

### 2. ECRIN-Supporting RD multinational clinical trials



#### (a) Clinical trial PLANNING



CLINICAL TRIALS SUPPORT OFFICE

- Innovative statistical design (2, 3, 4)
- Methodology tailored to small populations
- RD experts mentoring
- Country selection
- Cost evaluation



Clinical Trials Helpdesk (Task 20.2 Work Package 20 of EJP-RD)<sup>6</sup>

#### (b) Clinical trial EXECUTION



### OPERATIONAL COORDINATION

- Study management and coordination
- Regulatory and ethical submission
- Selection and provision of qualified resources
- Monitoring
- Vigilance
- Data management

ECRIN support services<sup>7</sup>. ECRIN provides advice, consultancy and operations management for multinational clinical trials.

6. <http://www.ejprarediseases.org/>

7. <http://www.ecrin.org/>

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