

# **INNOVATIVE FUNDING PARTNERSHIPS: CHALLENGES & OPPORTUNITIES**

**DARIA JULKOWSKA**

9th European Congress on Rare Diseases  
and Orphan drugs  
10-12 of May 2018  
Vienna, AUSTRIA

# INTRODUCTION

- Current opportunities and existing partnerships at EU scale level
- Encountered challenges
- Next steps: European Joint Programme on Rare Diseases

# 100 MILLION €

- invested in rare diseases research every year through competitive funding\*
- the majority provided by 5 EU countries (DE, ES, FR, IT, NL)
- National RD Plan ≠ dedicated research funding
- public money = obligation to inform

\* *Analysis by E-Rare, covers 15 EU countries*

# WHY PARTNERSHIPS ARE NEEDED?

- 100 M€ but 7000 rare diseases
- To facilitate high quality research with real benefit for patients
- To foster the opportunities for "confidential" collaboration between patients and research groups

# EXISTING TYPES OF PARTNERSHIPS

- Large Patient Organizations work with public funders to co-finance or finance research projects
- Represented by "well-trained" persons sitting on evaluation panels and familiar with public funders world
- E-Rare pilot action



# WHY TO WORK WITH PUBLIC FUNDERS?

*WHAT PUBLIC FUNDERS CAN OFFER?*

- Management of the call for projects process
- Quality and impartiality of evaluation process
- Management of financial process and monitoring
  
- Access to large portfolio of projects and research teams
- Important investment (co-investment) in research

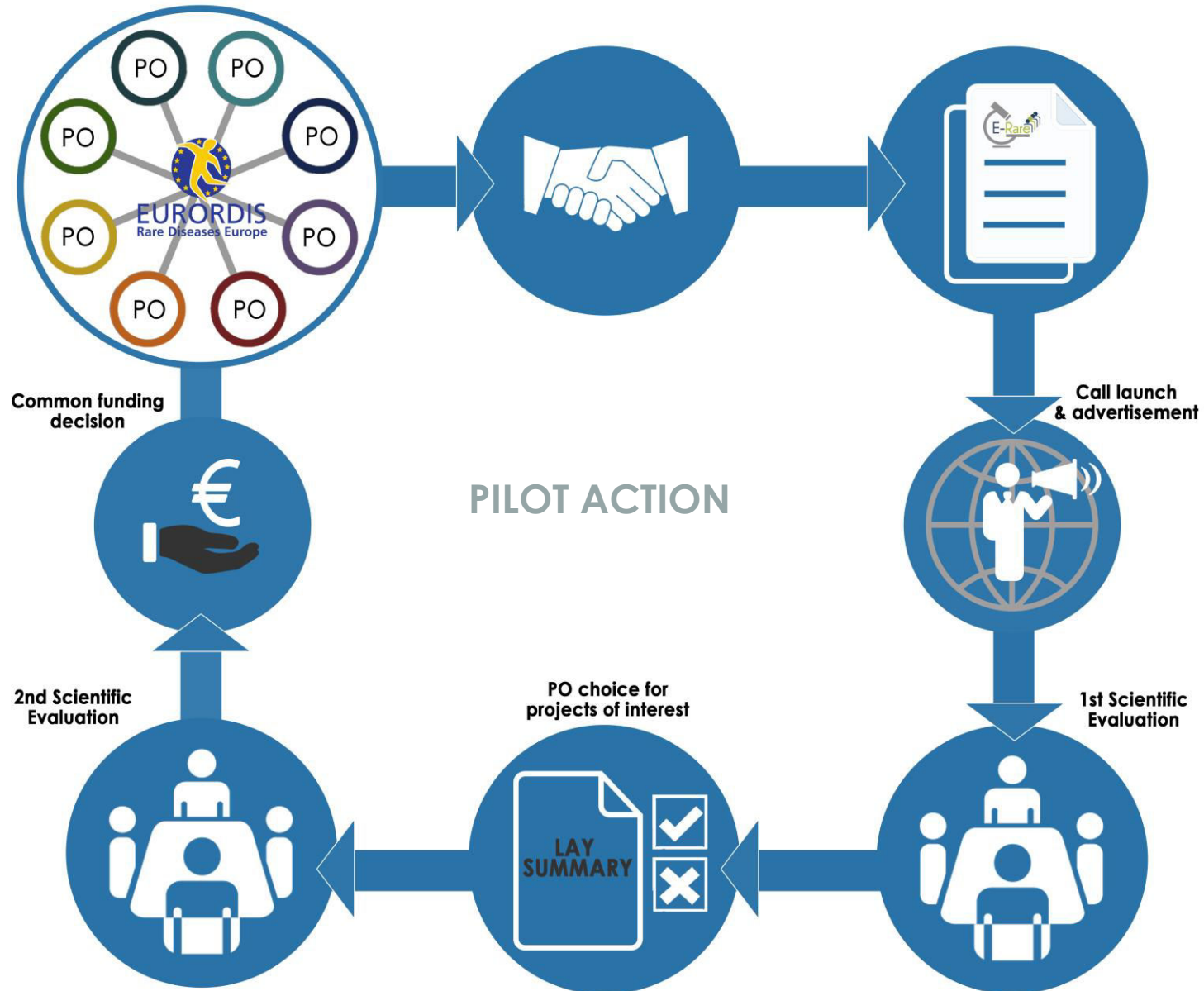
**E-RARE** PILOT ACTION ON NEW MODELS OF  
ENGAGEMENT OF PATIENT ORGANISATIONS  
IN FUNDING OF RESEARCH ON RARE DISEASES

# E-RARE COLLABORATION WITH PATIENT ORGANIZATIONS

- EURORDIS accompanied E-Rare from the start but was mainly involved in the External Advisory Board
- In 2014 (E-Rare-3) EURORDIS joins the consortium as full partner and contributes to the establishment of pilot for patient organisations in transnational calls
- The pilot scheme is based on the Canadian model where POs work hand in hand with the funding agency and jointly finance projects
- The preliminary work of EURORDIS indicated that (in principle) there are at least 60 POs interested by collaboration with E-Rare



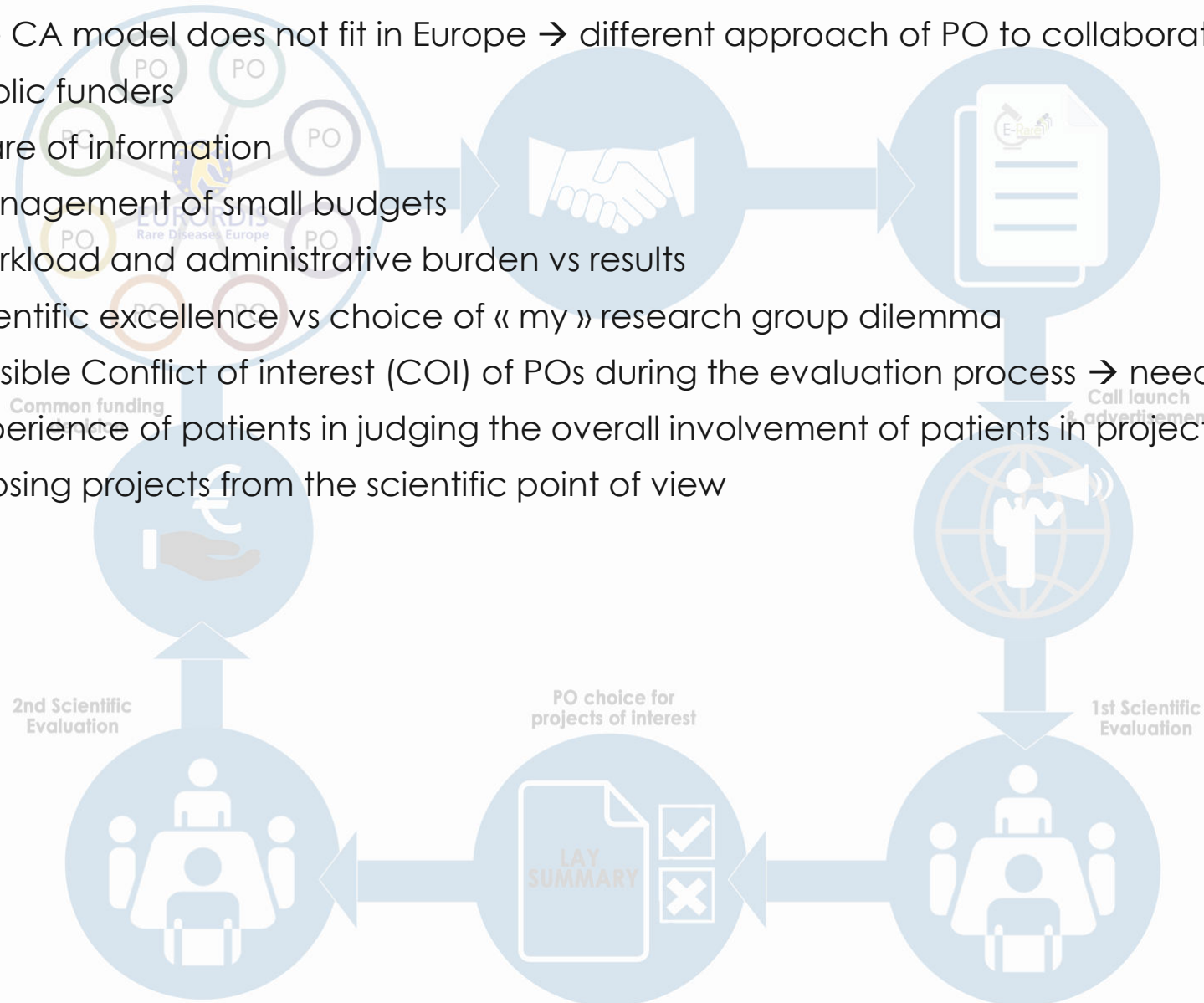
# E-RARE COLLABORATION WITH PATIENT ORGANIZATIONS



# DIFFICULTIES ENCOUNTERED

## ON FUNDERS SIDE:

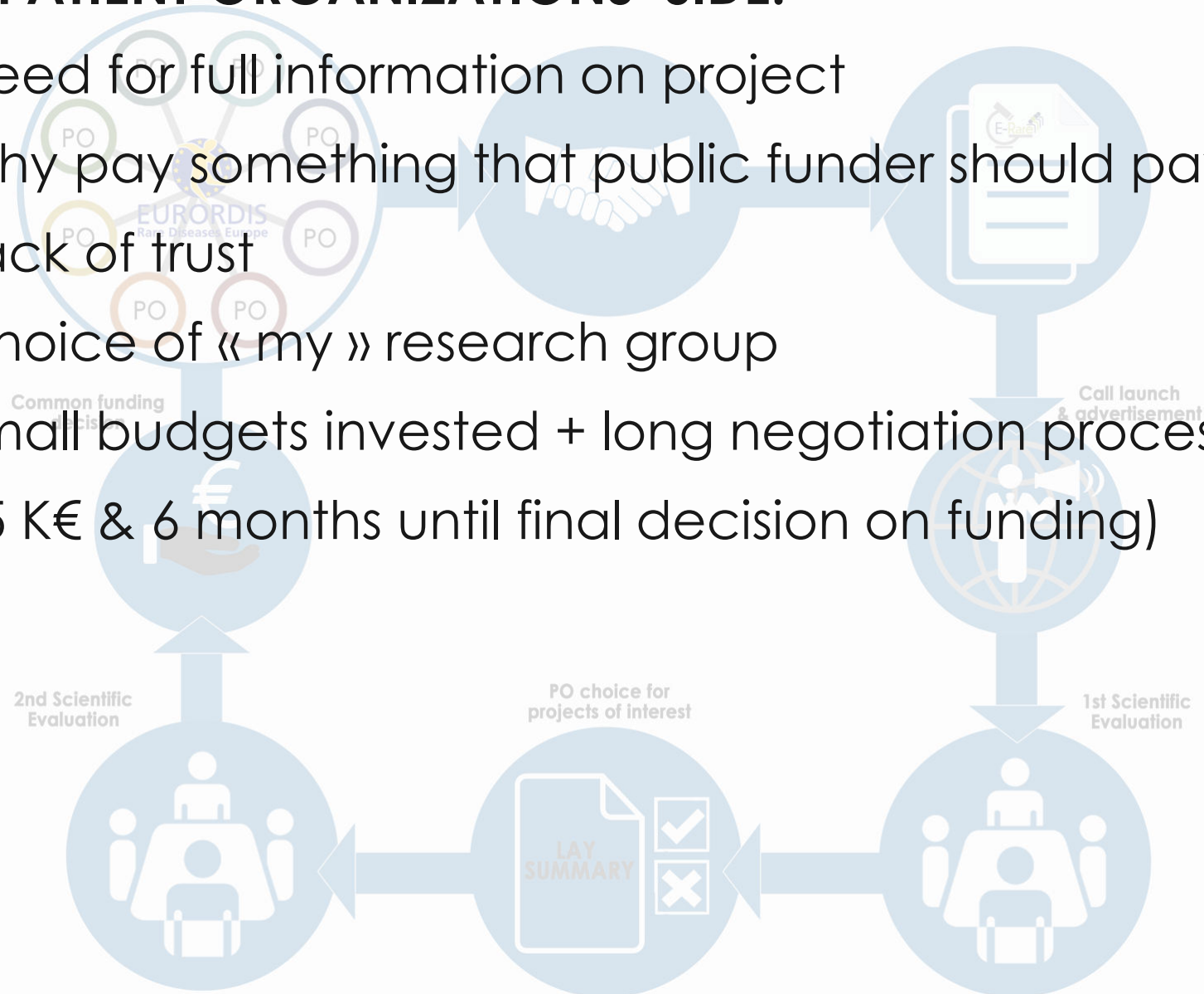
- The CA model does not fit in Europe → different approach of PO to collaboration with public funders
- Share of information
- Management of small budgets
- Workload and administrative burden vs results
- Scientific excellence vs choice of « my » research group dilemma
- Possible Conflict of interest (COI) of POs during the evaluation process → need for experience of patients in judging the overall involvement of patients in projects not in choosing projects from the scientific point of view



# DIFFICULTIES ENCOUNTERED

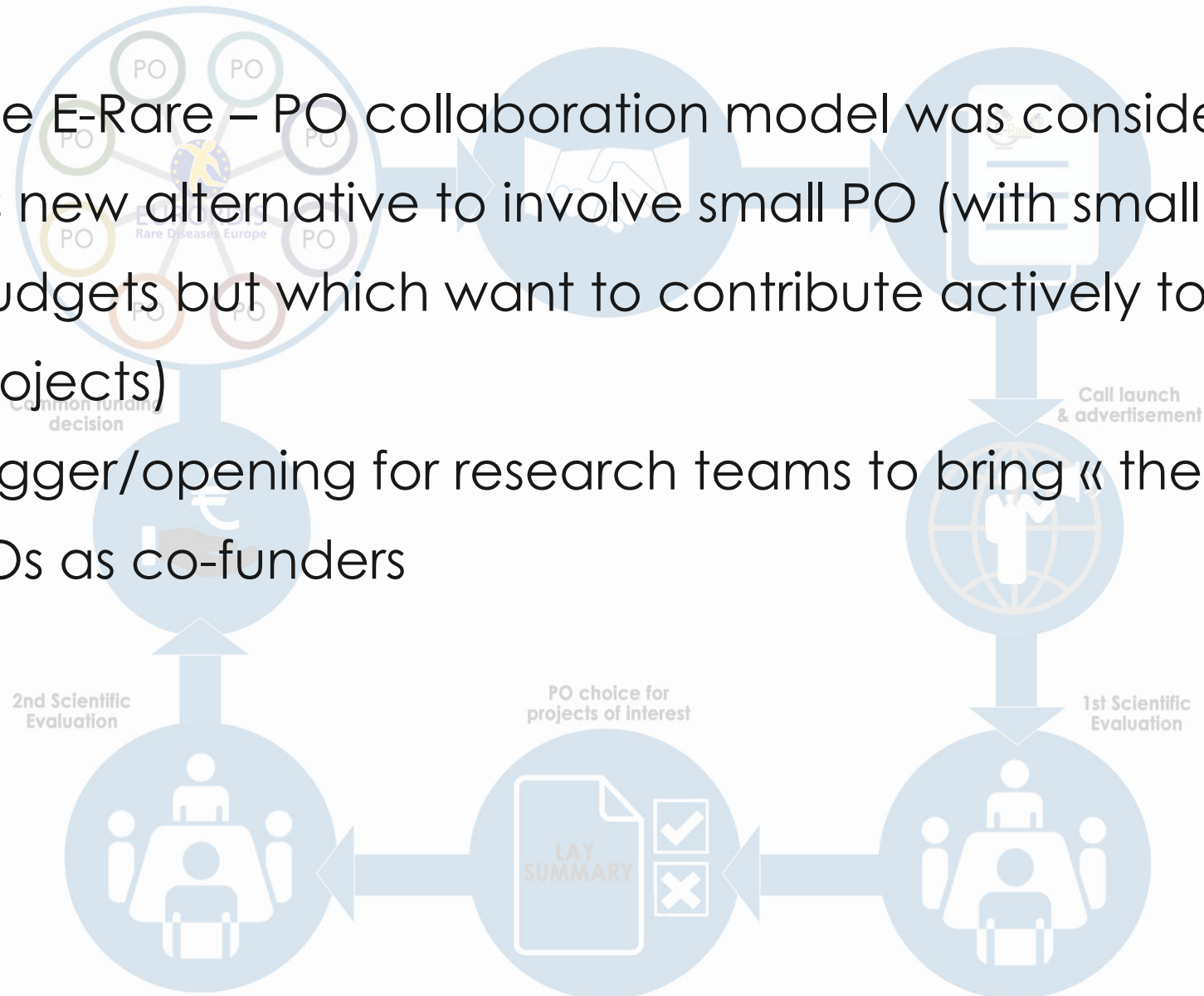
## ON PATIENT ORGANIZATIONS' SIDE:

- Need for full information on project
- Why pay something that public funder should pay?
- Lack of trust
- Choice of « my » research group
- Small budgets invested + long negotiation process (e.g. 15 K€ & 6 months until final decision on funding)



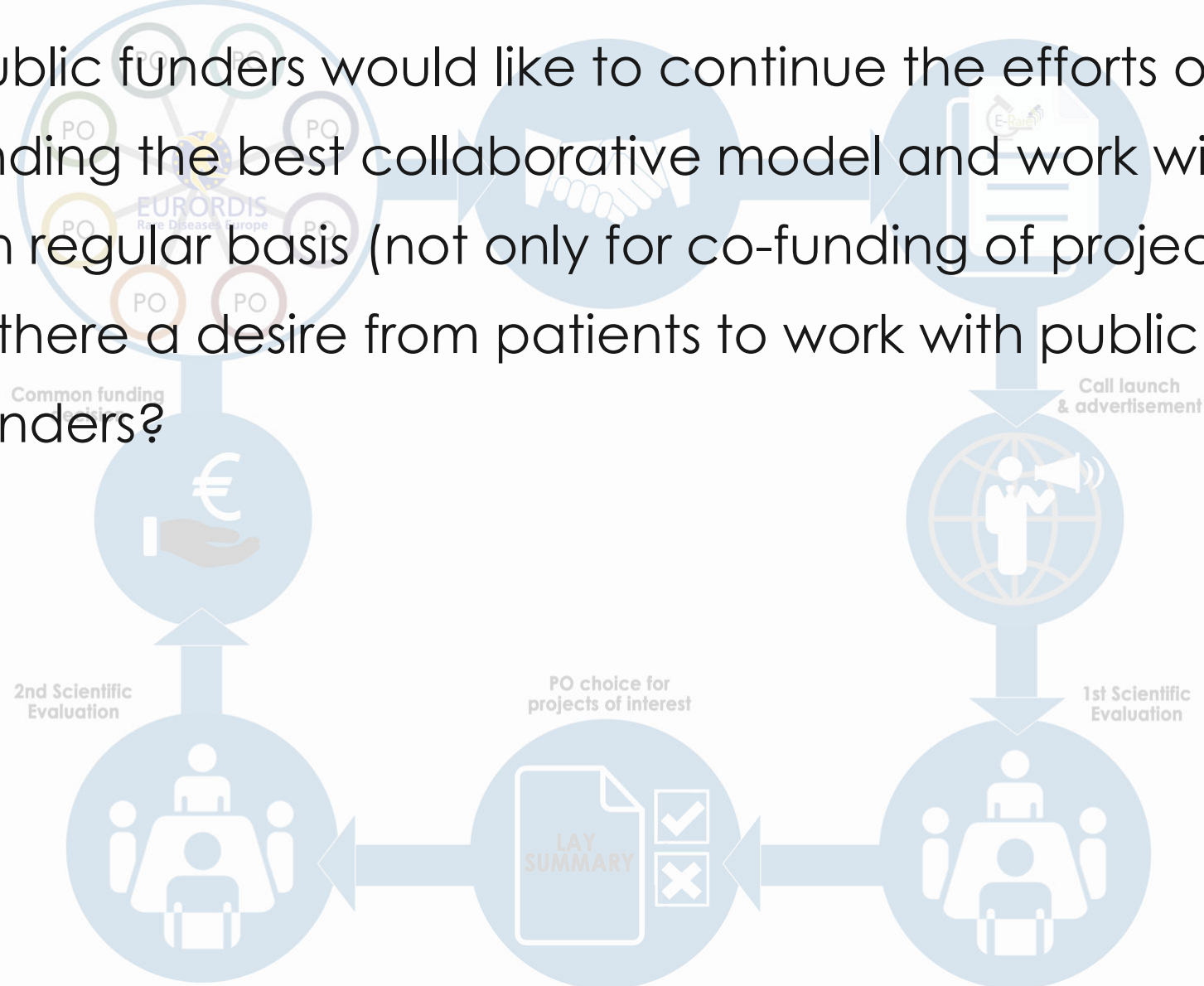
# POSITIVE OUTCOMES

- The E-Rare – PO collaboration model was considered as new alternative to involve small PO (with small budgets but which want to contribute actively to the projects)
- Trigger/opening for research teams to bring « their » POs as co-funders



## SHOULD WE ABANDON?

- Public funders would like to continue the efforts on finding the best collaborative model and work with PO on regular basis (not only for co-funding of project)
- Is there a desire from patients to work with public funders?



**EJP RD –  
THE EUROPEAN JOINT PROGRAMME  
ON RARE DISEASES**



## COORDINATION & TRANSVERSAL ACTIVITIES

**INTEGRATIVE RESEARCH STRATEGY**

**SUSTAINABILITY**

**ETHICAL, LEGAL, REGULATORY & IPR ISSUES**

**COMMUNICATION & DISSEMINATION**

**1**

**RESEARCH  
FUNDING**

**2**

**COORDINATED  
ACCESS TO  
DATA &  
SERVICES**

**3**

**CAPACITY  
BUILDING &  
EMPOWERMENT**

**4**

**ACCELERATING  
TRANSLATION**



# WP1 COORDINATION & MANAGEMENT

WP2  
STRATEGY

WP3  
SUSTAINABILITY

WP4  
ETHICS, LEGAL, REGULATORY & IPR

WP5  
COMMUNICATION & DISSEMINATION



WP6  
Joint Transnational Calls

WP7  
Networking scheme

WP8  
RDR Challenges

WP9  
Monitoring of funded projects

WP 10  
User-driven strategic planning for P2

WP 11  
Virtual Platform for data & resources

WP 12  
Enabling sustainable FAIRness

WP 13  
Holistic approaches for rare disease diagnostics and therapeutics

WP 14  
Training on data management & quality

WP 15  
Capacity building and training of patients and researchers

WP 16  
Online Academic education course

WP 17  
ERN RD training and support programme

WP 18  
Development and adaptation of training activities

WP 19  
Facilitating partnerships and accelerating translation

WP 20  
Validation, use and development of innovative methodologies for clinical studies

Coordinated by



# THANK YOU

for any questions contact:

Daria julkowska

[daria.julkowska@inserm.fr](mailto:daria.julkowska@inserm.fr)  
[daria.julkowska@anr.fr](mailto:daria.julkowska@anr.fr)