



Grant from:



# Facilitating the Identification of Appropriate Patient-Centered Outcome Measures (PCOMS) in Rare Disease (RD) Clinical Research Using Functional Impacts Matching

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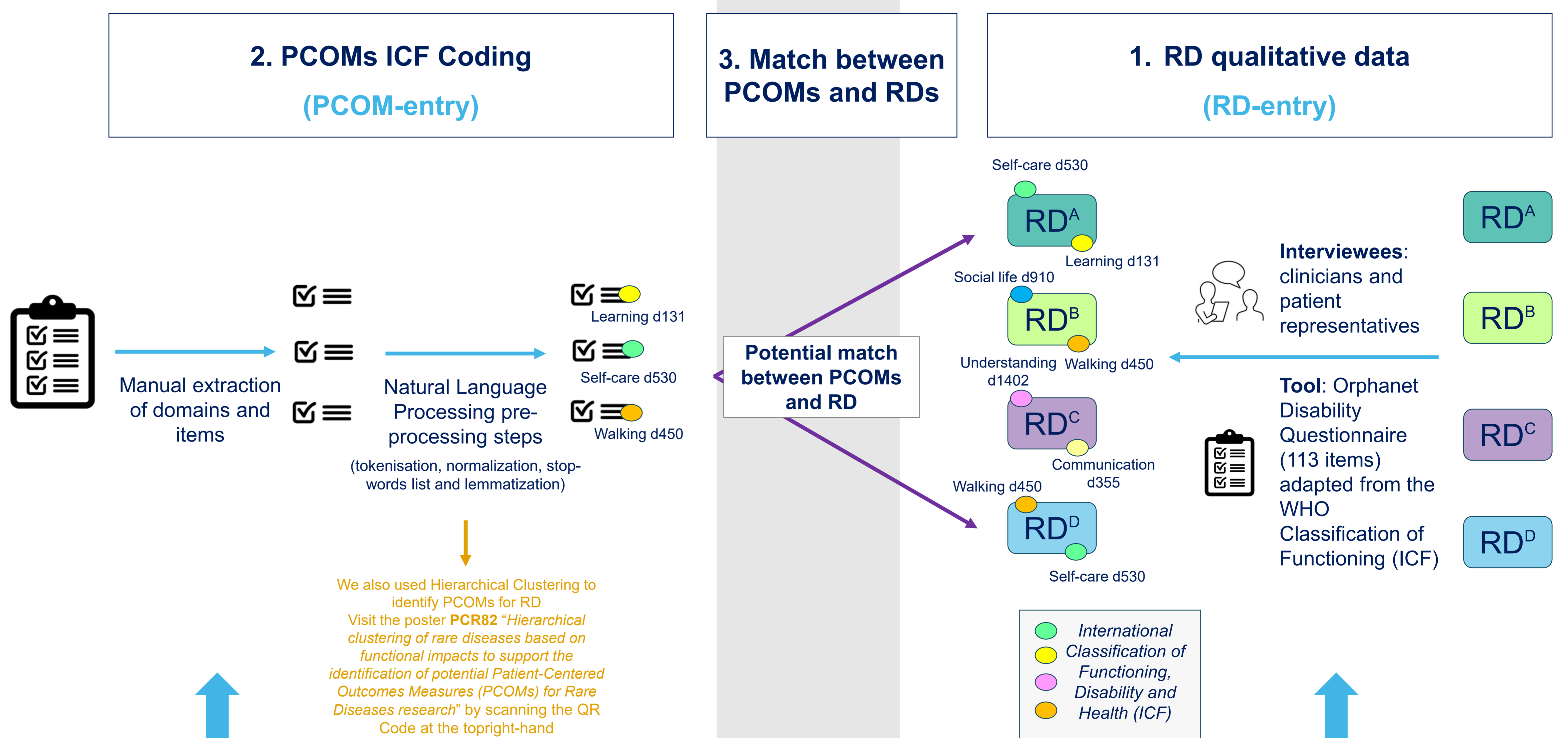
## MAIN OUTCOMES:

- Items and domains of 1132 PCOMS were selected for ICF coding
- Algorithm to automatically ICF-code PCOMS was developed using Natural Language Processing
- **Next steps:** Validation of the algorithm matching PCOM items/domains and ICF codes
- **Impact:** This work is a very promising step for the development of a PCOMS - RD database to support the use and the adaptation of existing PCOMS and turn RD patient experience into meaningful and comparable data

## RESULTS

- **RD-entry:** 551 RD were indexed and ICF-coded by Orphanet
- **PCOMS-entry:**
  - 1132 RD-specific, function-specific and disease area-specific PCOMS were selected for ICF-coding
  - Collection of PCOMS domains and items is on-going
  - NLP pre-processing steps were completed and NLP algorithm finalized
  - **Next steps:** NLP-allocated ICF codes of PCOMS will be quality checked by two reviewers

## METHODS



## RESSOURCES

**PROQOLID™**  
Clinical Outcome Assessments Database  
Mapi ResearchTrust database with data on 6.000 PCOMS  
<https://eprovide.mapi-trust.org/>

We connected these two databases!  
Visit the poster PCR28 "Facilitating and Accelerating Patient-Centered Outcomes Measures Awareness and Use in Clinical Research in Rare Diseases" by scanning the QR Code at the top right-hand

**orphadata**  
Orphanet database of RD functional consequences annotation  
<http://www.orphadata.org>

## OBJECTIVE

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## CHALLENGES

**Identify existing PCOMS suitable for RDs based on functional impacts similarities**

**The lack of specific validated PCOMS in RDs is a strong limitation in patient centric clinical research and development of disease specific PCOMS for more than 7,000 RDs is not a realistic objective**