Facilitating and Accelerating Patient-Centered Outcomes Measures (PCOMs) Awareness and Use in Clinical Research in Rare Diseases (RD)
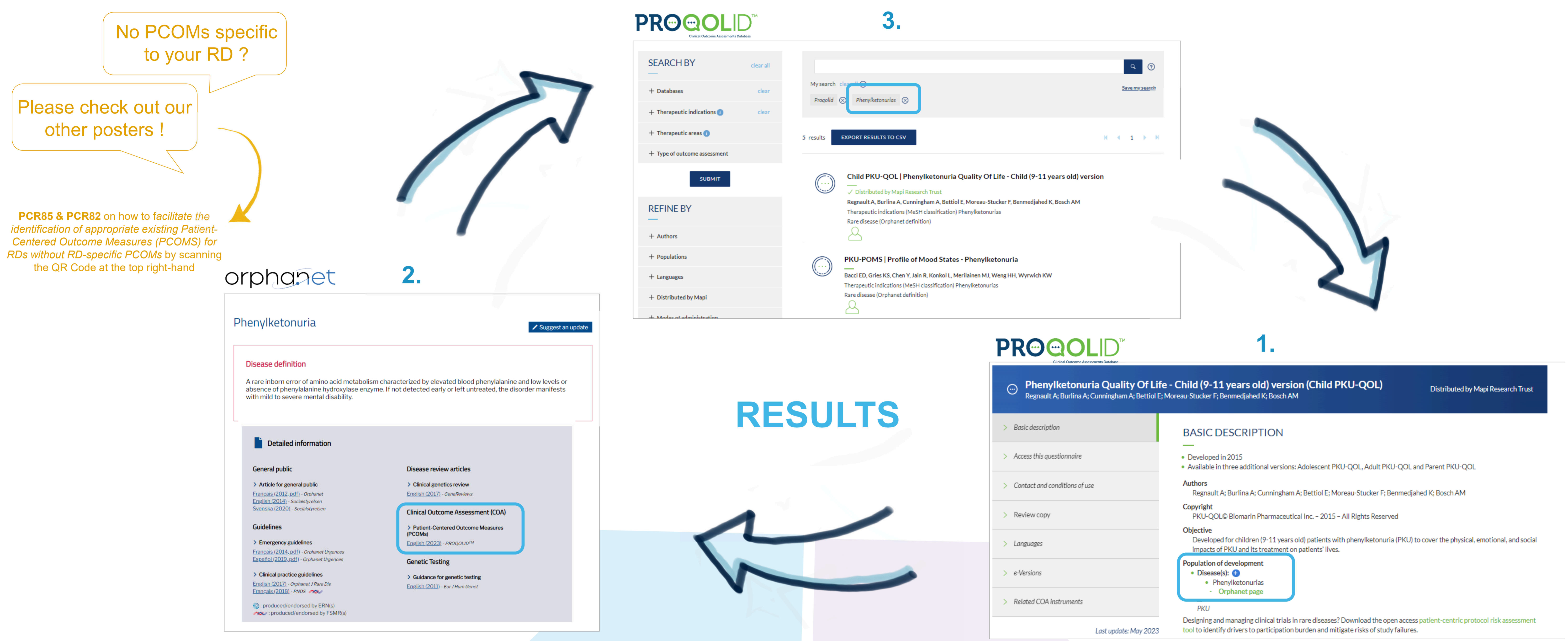
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MAIN OUTCOMES:

- 537 rare disease-specific PCOMs (incl.380 Patient-Reported Outcome Measures) were identified in PROQOLID™ and Orphanet links added
- PCOMs links to PROQOLID™ were included for 680 Orphanet clinical entities
- **Impact**: Connection of the two databases supports the identification and use of PCOMs in RD research

No PCOMs specific to your RD ?

Please check out our other posters !

PCR85 & PCR82 on how to facilitate the identification of appropriate existing Patient-Centered Outcome Measures (PCOMS) for RDs without RD-specific PCOMs by scanning the QR Code at the top right-hand

RESULTS

METHODS

Orphanet identified RDs for which a PCOM was available in PROQOLID™ and and applicable at the Disorder, Subtype and Groups of Disorders levels of the Orphanet Nomenclature

Mesh and OrphaCode classifications were aligned to identify RD-specific PCOMs in PROQOLID™

DATABASES

Content: Information on 6305 Disorders, 1020 Subtypes of Disorders and 2145 Groups of Disorders
Classification: OrphaCode
https://orpha.net/

Content: Information on conditions of use, translations, review copy and validation data for 6.000 PCOMs
Classification: Mesh
https://eprovide.mapi-trust.org/

ORGANISATIONS

OBJECTIVE & CHALLENGES

Increase the visibility of existing PCOMs and optimizing their use in RD clinical research and care

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