

# Patient Reported Outcomes in Randomized Controlled Trials of Nervous System and Mental Health Disorders: Current Status in India

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## Background

- Patient-reported outcomes (PROs) are "A measurement based on a report that comes directly from the patient (i.e., study subject) about the status of a patient's health condition without amendment or interpretation of the patient's response by a clinician or anyone else"<sup>1</sup>
- PROs include Quality of life (QoL), symptom measures, and functional outcomes of disease<sup>2</sup>
- The use of PROs in randomized controlled trials (RCTs) has increased over time, in addition to the efficacy and safety measures and endpoints<sup>2</sup>
  - 2004 to 2007: 14% of RCTs included PROs
  - 2007 to 2013: 27% of RCTs included PROs
- Regulators also have actively started looking for changes in QoL after an intervention, in addition to the routinely collected disease-related measures
- We were interested to measure the extent to which RCTs in India conducted on the nervous system and mental health disorders collect and report PRO information

## Objective

- To evaluate the current status of patient-reported outcomes (PROs) captured in randomized controlled trials (RCTs) performed on any nervous system and mental health disorders in India

## Methodology

### Eligibility Criteria

Facet	Inclusion	Exclusion/ Not of interest (NOI)
Population	<ul style="list-style-type: none"> <li>• Humans suffering from any nervous system and mental health disorder</li> <li>• Any age</li> <li>• Residing only in India</li> </ul>	<ul style="list-style-type: none"> <li>• No human subjects</li> <li>• Healthy human volunteers</li> <li>• Subjects residing in any country out of India</li> <li>• Multi-country studies</li> </ul>
Intervention	<ul style="list-style-type: none"> <li>• Any intervention</li> </ul>	<ul style="list-style-type: none"> <li>• No restriction</li> </ul>
Comparator	<ul style="list-style-type: none"> <li>• Any comparator</li> </ul>	<ul style="list-style-type: none"> <li>• No restriction</li> </ul>
Outcome	<ul style="list-style-type: none"> <li>• PROs: quality of life, patient satisfaction, all other PROs</li> </ul>	<ul style="list-style-type: none"> <li>• All other outcomes</li> </ul>
Study design	<ul style="list-style-type: none"> <li>• Randomized controlled trials</li> </ul>	<ul style="list-style-type: none"> <li>• All other types of papers</li> </ul>
Databases	<ul style="list-style-type: none"> <li>• PubMed, Embase, Google Scholar, WoS</li> </ul>	<ul style="list-style-type: none"> <li>• -----</li> </ul>

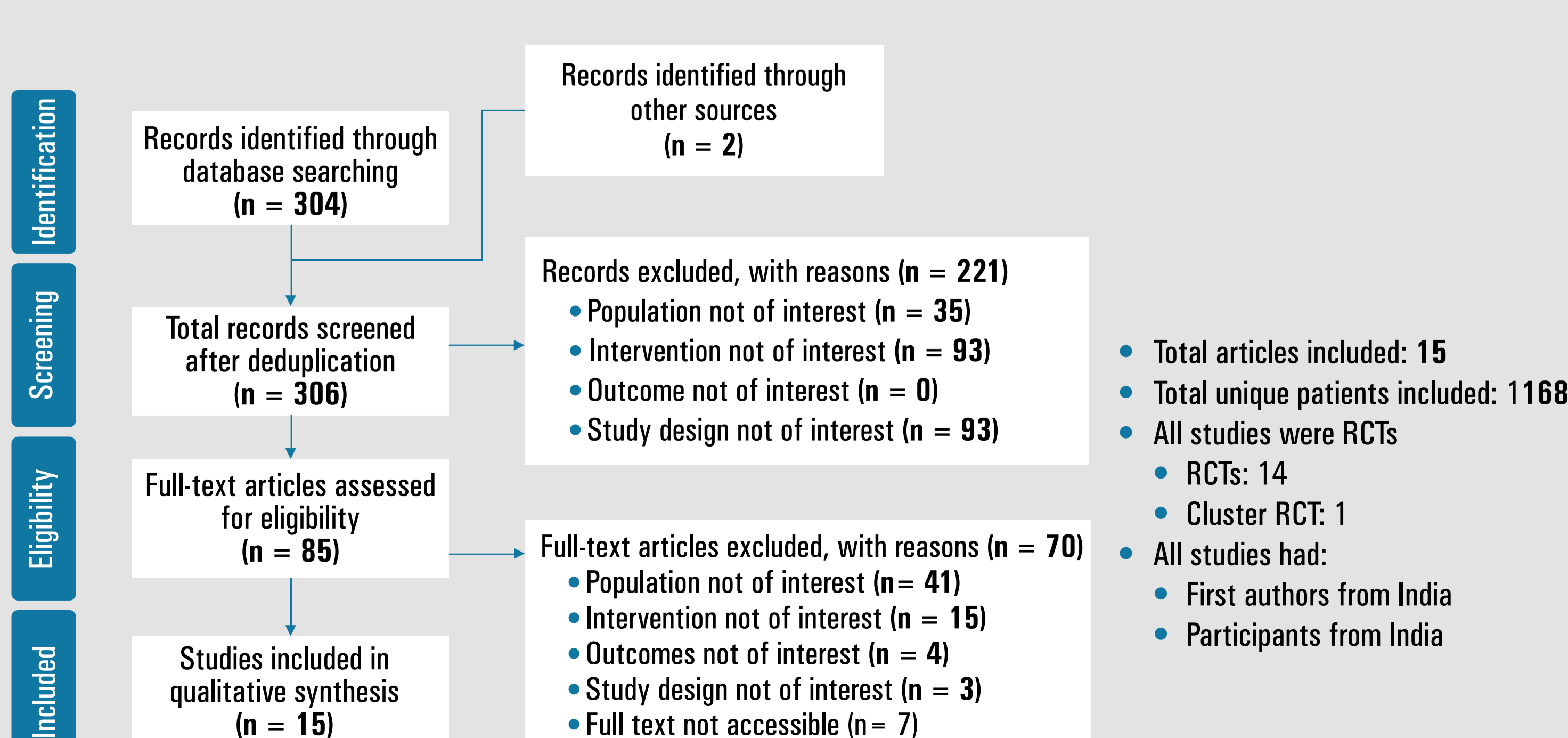
## PubMed Search Strategy

From inception till 31<sup>st</sup> March 2022

No.	Terms	Hits	Facet
#1	"quality of life"[MeSH Terms] OR "quality of life"[All Fields] OR "Life Quality"[All Fields] OR "QOL"[All Fields] OR "health related quality of life"[All Fields] OR "health related quality of life"[All Fields] OR "HRQOL"[All Fields]	4,06,170	All QoL studies
#2	"Patient Reported Outcome Measures"[MeSH Terms] OR "Patient Reported Outcome Measure"[All Fields] OR "patient reported outcome"[All Fields] OR "patient reported outcome"[All Fields] OR "PROs"[All Fields] OR "PROM"[All Fields] OR "Satisfaction"[All Fields] OR "Experience"[All Fields] OR "Questionnaire"[All Fields]	14,14,321	All PRO studies
#3	#1 OR #2	16,93,284	All studies about QoL/ other PROs
#4	"Randomized Controlled Trials as Topic"[MeSH Terms] OR "Randomized Controlled Trial"[Publication Type] OR "Randomized Controlled Trial"[All Fields] OR "RCT"[All Fields] OR "randomized clinical trial"[All Fields] OR "randomized controlled clinical trial"[All Fields]	7,74,704	All RCTs
#5	"Nervous System Diseases"[Mesh] OR "Mental Disorders"[Mesh]	36,48,420	All studies about nervous system diseases or mental disorders
#6	#3 AND #4 AND #5	24,323	All RCTs about nervous system diseases/ mental health disorders containing QoL/ other PROs
#7	India	7,00,489	All studies containing the word 'India'
#8	#6 AND #7	304	All RCTs about nervous system diseases/ mental health disorders from India containing QoL/ other PROs

Note: Restrictions were not imposed on the search strategy; ineligible articles were manually screened out

## Results



## Population: Demographics

### Sample size

- Overall: 1168
- Range: 10-260

### Age group

- Adult population only: 8 studies
- Pediatric population only: 5 studies

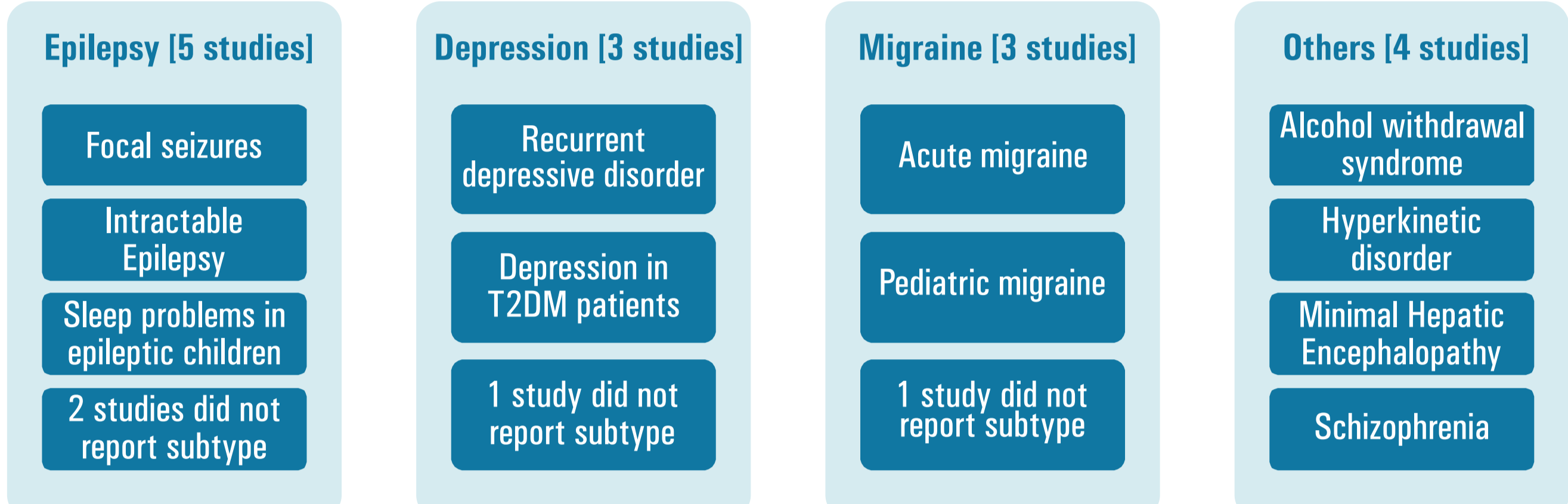
### Age

- Mean  $\pm$  SD: 6.6  $\pm$  3.9 to 50.0  $\pm$  1.73 years (9 studies)
- Mean (95% CI): 51.7 (49.0-54.3) to 54.3 (51.6-57.1) years (1 study)
- Median (range): 23 (13-49) to 24 (12-50) years (1 study)
- 4 studies did not report

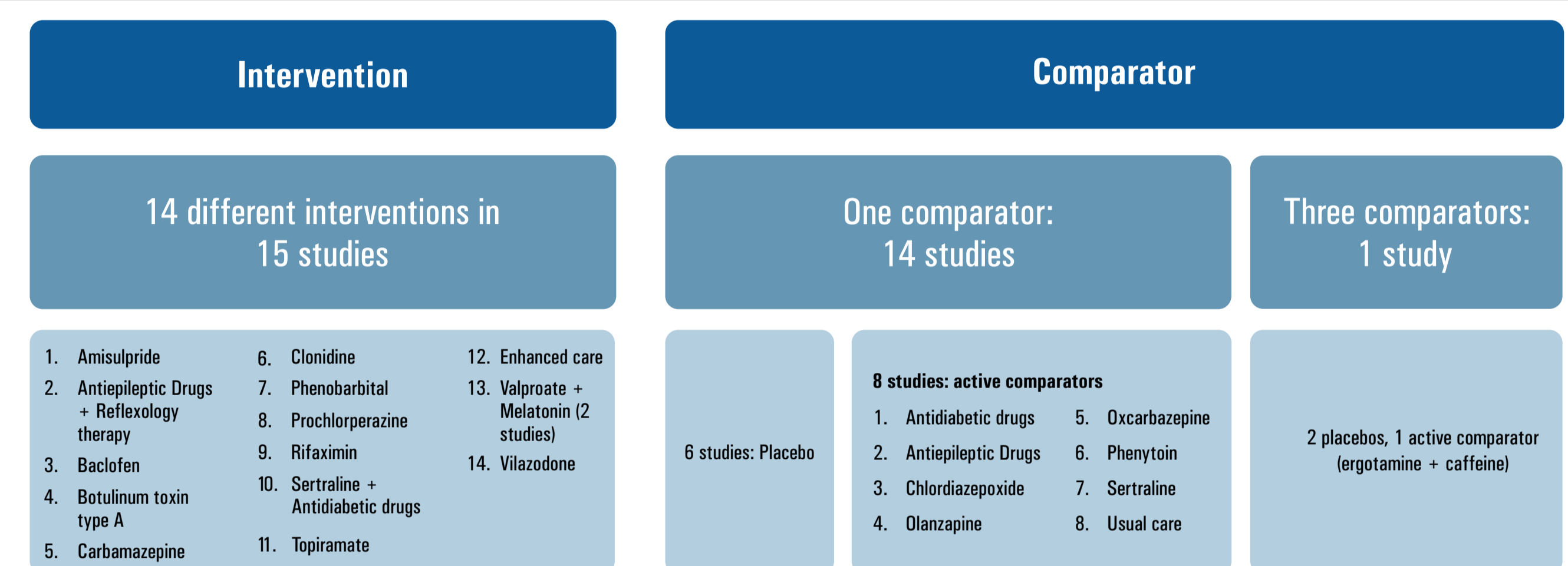
### Sex

- Males: 576 (49.34%)
- Females: 592 (50.66%)

## Population: Nervous System/ Mental Health Disorder



## Intervention/ Comparator Details



## PRO Information

### Number of PROs evaluated per study

- Most studies evaluated 1 or 2 PROs
- Number of PROs evaluated:
  - 1 PRO: 8 studies
  - 2 PROs: 6 studies
  - 3 PROs: 1 study
  - Total evaluations: 23 PROs in 15 studies

### PRO changes over time

- All studies measured PRO changes over time
- Measurement time ranged from 2 hours to 2 years

## PRO Information: Types of PROs

- A total of 7 different PROs were evaluated by the 15 included studies
  - Symptoms: 9 studies
  - QoL: 8 studies
  - Behavior: 2 studies
  - Adherence, Satisfaction, Sexual function, Sleep: 1 study each
  - Total evaluations: 23 PROs in 15 studies
- A total of 27 different PRO scales were used to measure the 7 PROs
  - Symptoms: 12 scales
  - QoL: 8 scales
  - Behavior: 3 scales
  - Adherence, Satisfaction, Sexual function, Sleep: 1 scale each
- 10 scales had subscales
  - 3 scales had 10+ subscales
  - One scale ( QoLCE ) had 16 subscales

## Discussion

- Only 15/306 (4.9%) RCTs on nervous system/ mental health disorders in India reported PROs
- Diverse disorders; lack of uniformity in the definitions used to describe the conditions and subtypes.
- Symptoms (9 studies) and QoL (8 studies) were the most frequently measured PROs
- WHO PRO scales were used for measuring QoL in 2 studies
- Large amount of variation in the measurement of PROs: 27 different scales for measuring 23 PROs in 15 studies
- Many generic scales were used to measure PROs; none were India-specific

## Limitations

- Search was limited to PubMed; databases like Embase were not searched
- Search was restricted to publications in English language only

## Conclusion

- Recording and analyzing PROs in RCTs on nervous system/ mental health disorders in India is inadequate and has a large amount of variation

## References

1. Caldwell, Brittany. Value and Use of Patient-Reported Outcomes (PROs) in Assessing Effects of Medical Devices. 2016; Available from: <https://www.fda.gov/MedicalDevices/ProductsandMedicalProcedures/DeviceApprovalsandClearances/default.htm>
2. Mercieca-Berber R, King MT, Calvert MJ, Stockler MR, Friedlander M. The importance of patient-reported outcomes in clinical trials and strategies for future optimization. Patient Relat Outcome Meas. 2018 Nov;Volume 9:353-67.

