

Characterizing Patient Experience Publications in Rare Diseases, Neuroscience, and Oncology

Simon R. Stones^{a,b}, Jacqui Oliver^a, Oleks Gorbenko^c, Catherine Elliott^a, Sarah Thomas^c
^aEnvision Pharma Group, Wilmslow, Cheshire, United Kingdom; ^bPatient/caregiver author; ^cIpsen, London, United Kingdom



POSTER #45

For further information, please send your question(s) to: Simon Stones, simon.stones@envisionpharma.com

Copies of this eposter obtained through the QR Code are for personal use only and may not be reproduced without written permission from the authors.

Background

- Patient experience data (PED) are defined as “information that captures patients’ experiences, perspectives, needs, and priorities related to (but not limited to):”¹
 - The symptoms of patients’ disease and its natural history
 - The impact of the disease on patients’ functioning and quality of life
 - Patients’ experience with treatments
 - Input on which outcomes are important to patients
 - Patient preferences for treatment
 - The relative importance of any issue as defined by patients
- PED are collected using different methodologies, and include quantitative (eg, patient-reported outcomes) and qualitative (eg, interviews) data sources.²
- PED are increasingly recognized as drivers for improving health services, research, and patient outcomes.³
- It is also increasingly acknowledged that patients should be involved in the planning, conducting, and subsequent publication of PED to enhance the relevance and credibility of data.⁴
- To date, there has been limited characterization of the nature and extent of PED publications, and how accessible these publications are.
 - It is also unclear as to whether PED publications include authors with patient or caregiver experience.

“PED at all stages of medicine development and regulatory decision-making ensures that new medicines address the outcomes and preferences that matter to patients.”

— European Patients Forum, 2024⁵

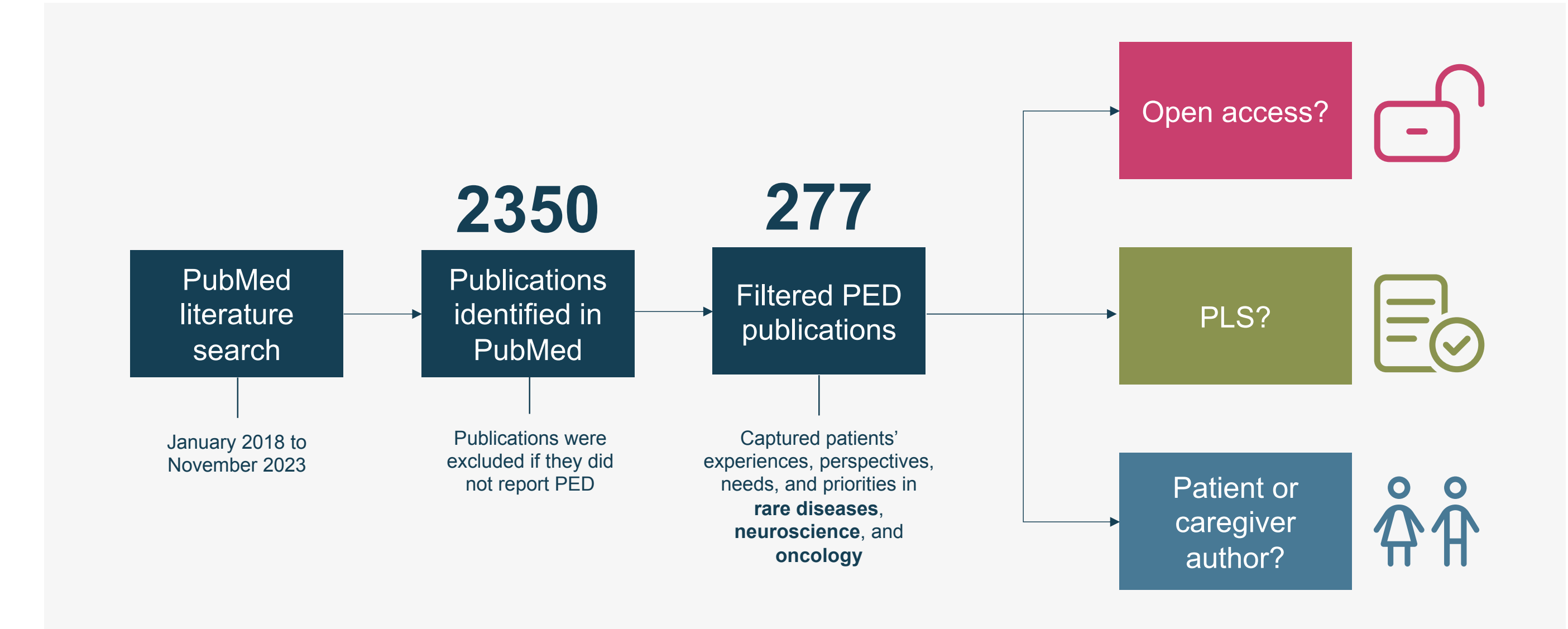
Objectives

- The objectives of this study were to better understand PED publications by quantifying:
1. The types of publications in which PED have been published
 2. PED publications available in open access
 3. PED publications with plain language summaries (PLS)
 4. PED publications with patient or caregiver authors

We wanted to explore this in 3 disease areas in which active patient engagement and involvement in research has taken place⁶: rare diseases, neuroscience, and oncology.

Methods

- We searched PubMed to identify a dataset of PED publications (January 2018 to November 2023).
- PED publications in rare diseases, neuroscience, and oncology were identified by searching the title/abstract search for key terms, and limited to English language. Publications were filtered to include only PED.
- PubMed index terms were used to identify PLS and patient or caregiver authors.
 - An additional manual search of original publications was also performed to identify PLS published as supplementary material or as standalone PLS of publications.
 - In the absence of a clear patient or caregiver author affiliation, an additional manual search using a search engine was performed to identify authors with patient or caregiver experience.

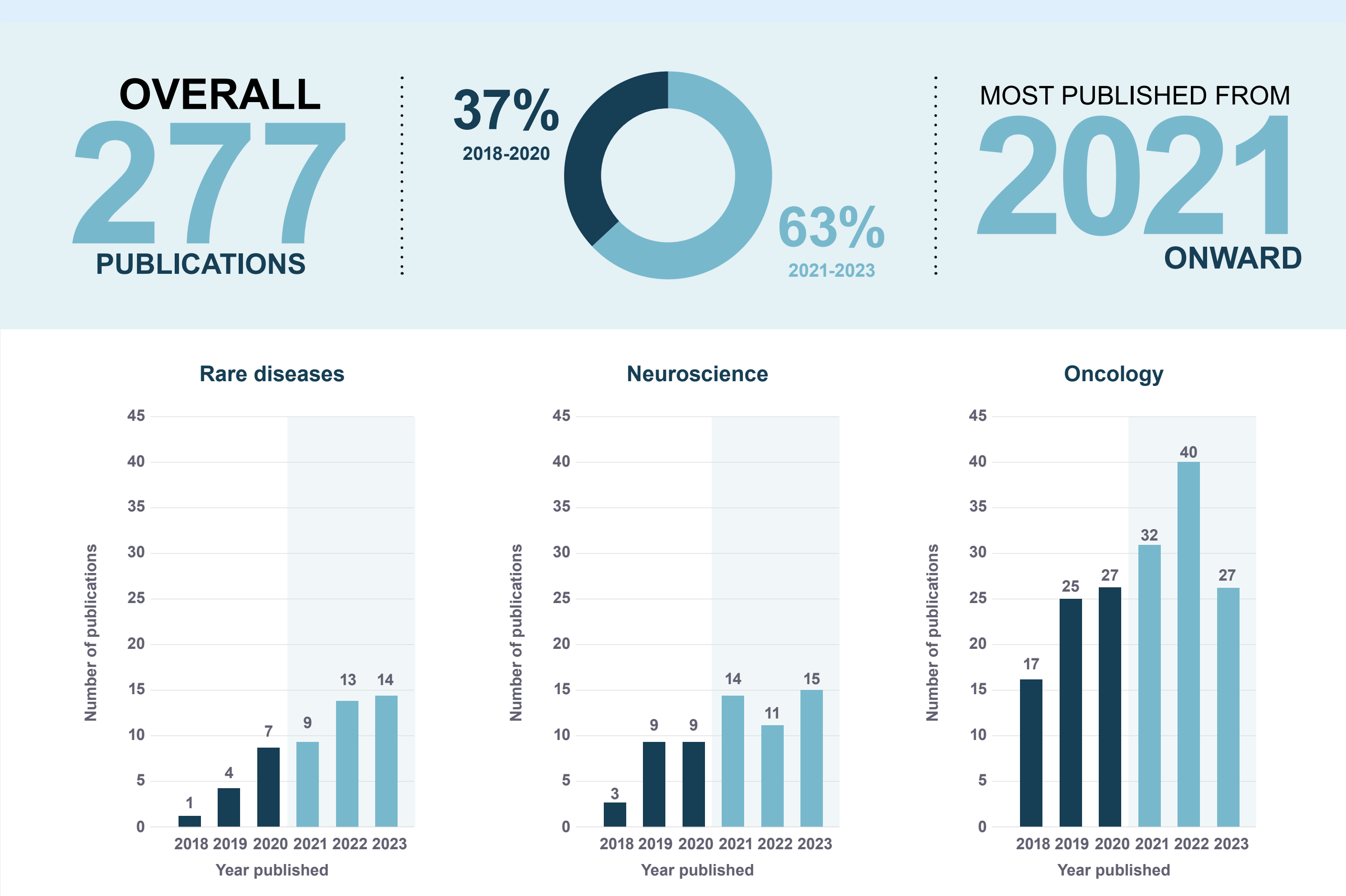


Limitations

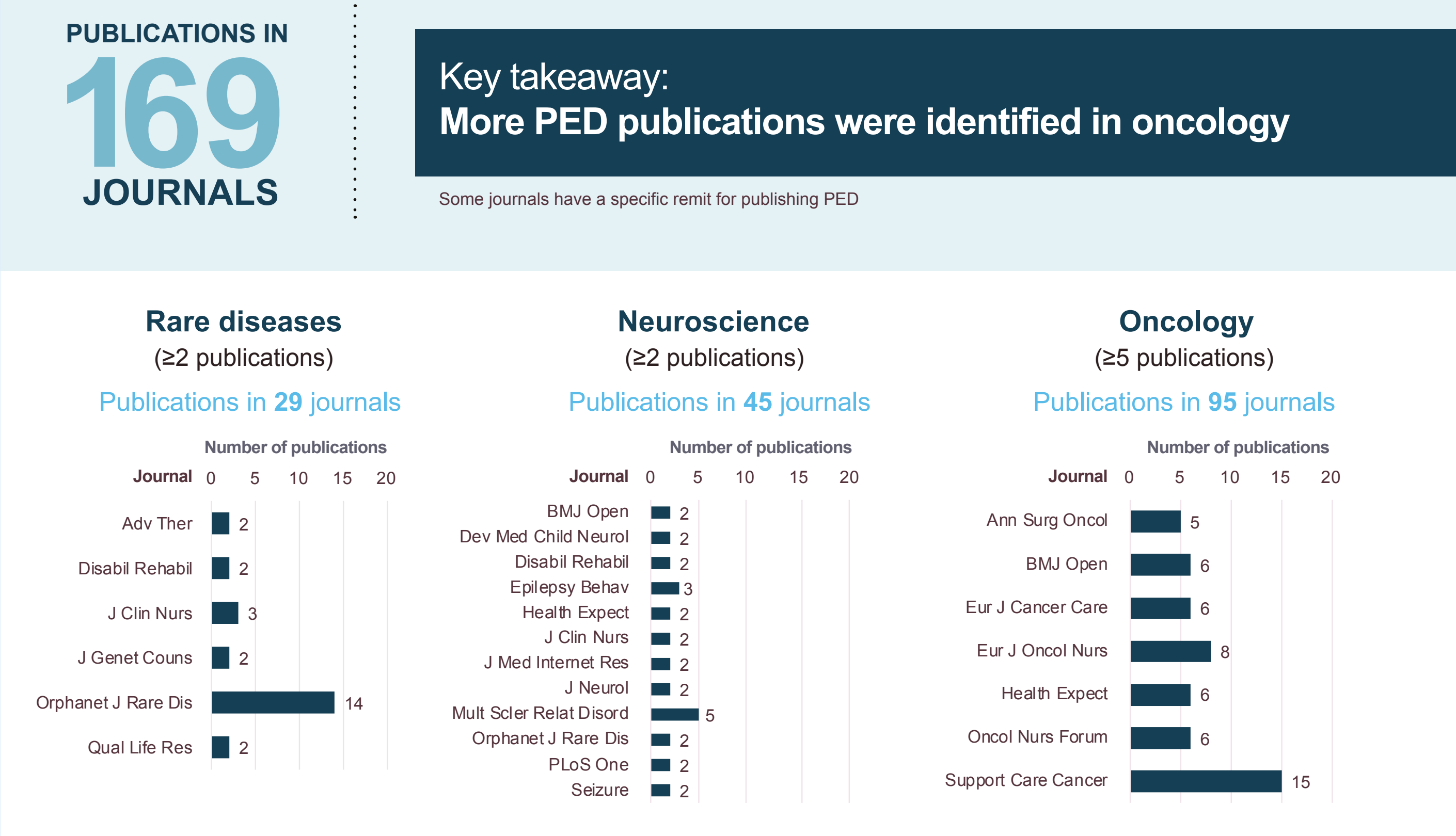
- Restricted search of PubMed (eg, specific disease search terms).
- Pragmatic review methodology (ie, non-systematic review search strategy and process).
- Author affiliation searches that were limited to specific terms, potentially excluding other relevant affiliations (eg, patient organizations); however, additional manual searches are likely to have addressed this limitation.

Results

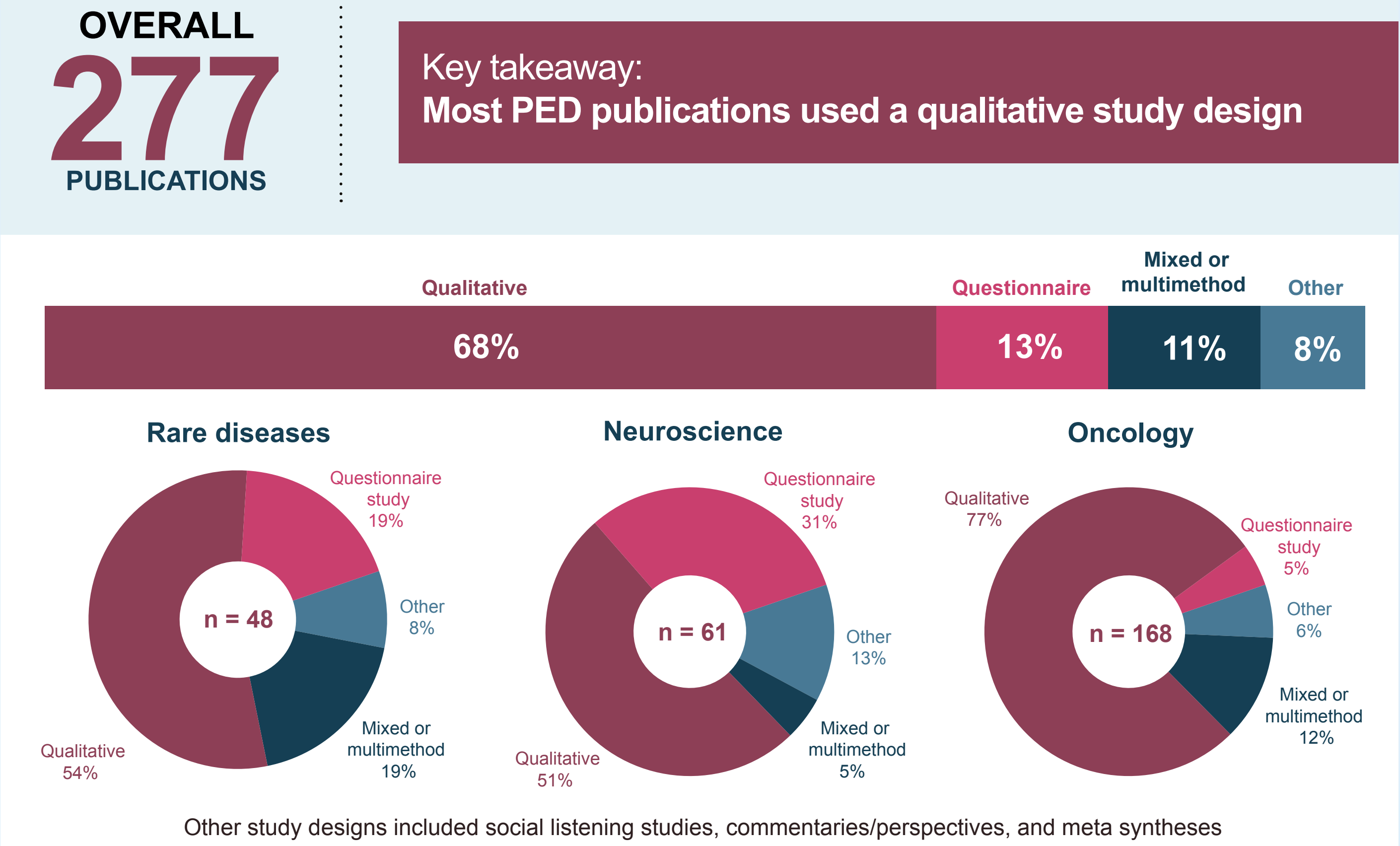
1. The types of publications in which PED have been published



Journals



Study design

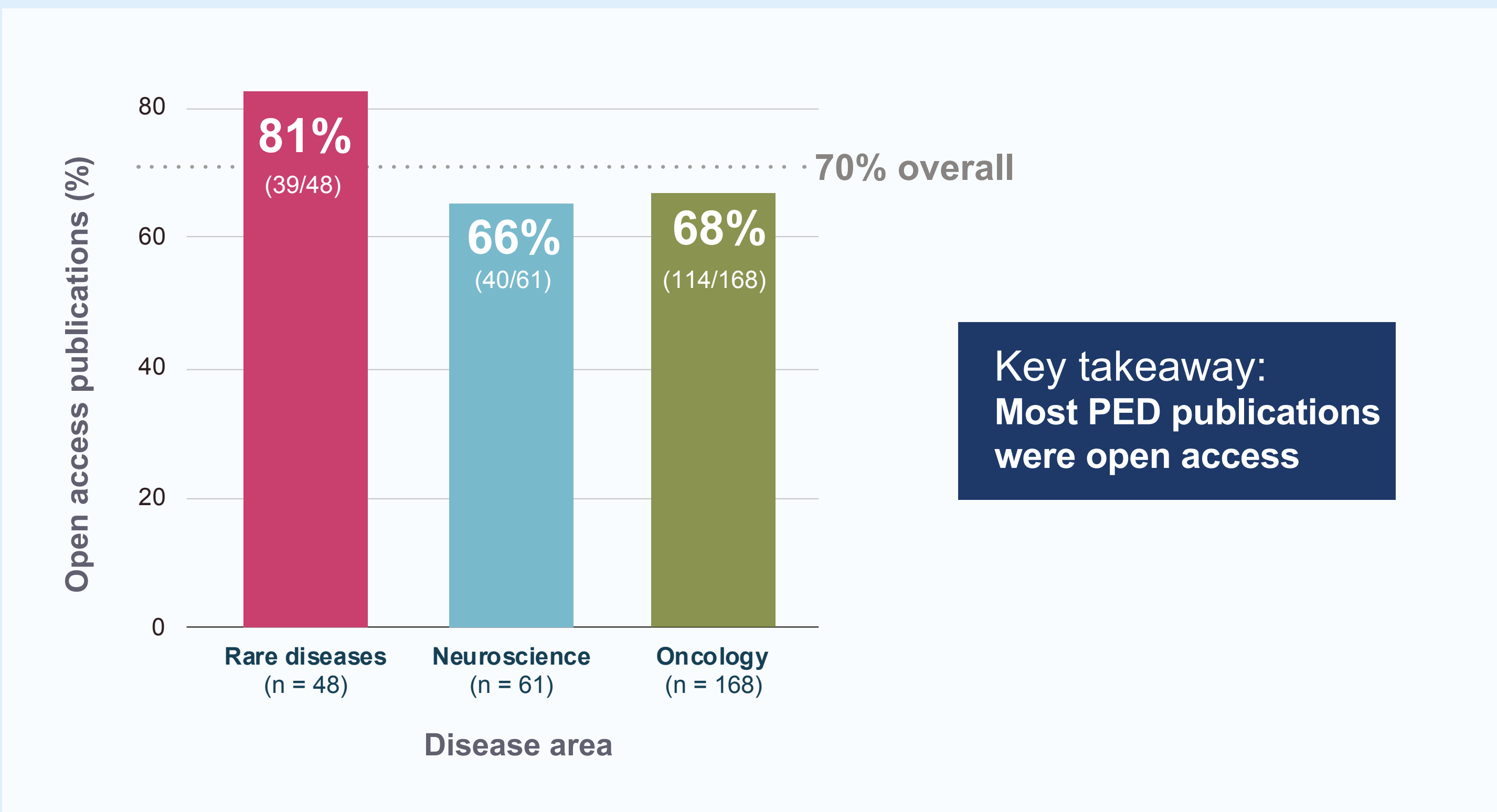


Conclusions

- Since 2018, the number of PED publications has increased in rare diseases, neuroscience, and oncology.
- Most existing PED publications were in oncology; however, this could be influenced by a larger number of overall publications in oncology.
- Most PED publications used a qualitative study design.

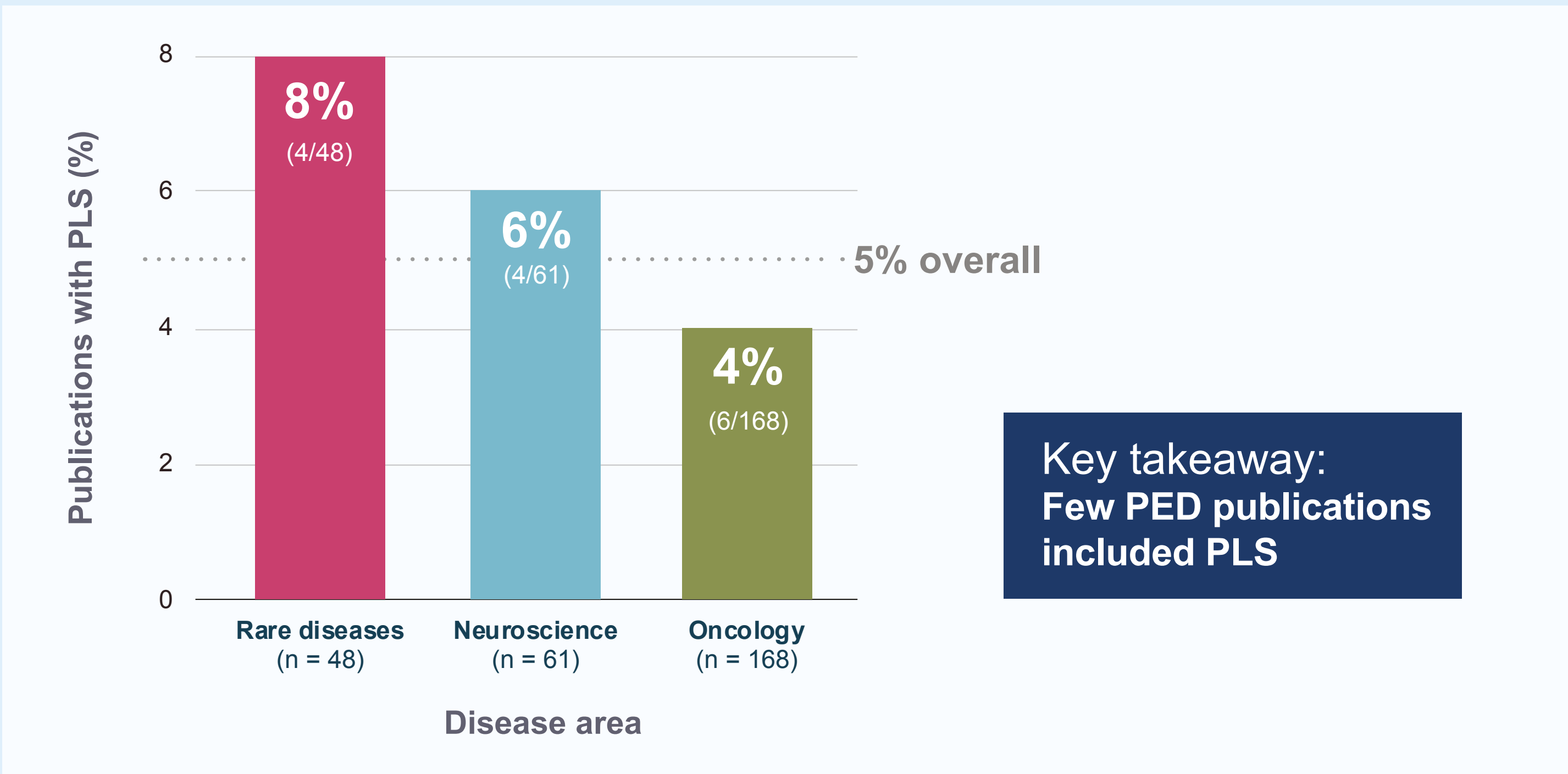
2. PED publications available in open access

- Over two-thirds of publications were available in open access (193/277; 70%).
- A small proportion (24/277; 9%) of non-open access publications were available online via third-party platforms (eg, institutional repositories).



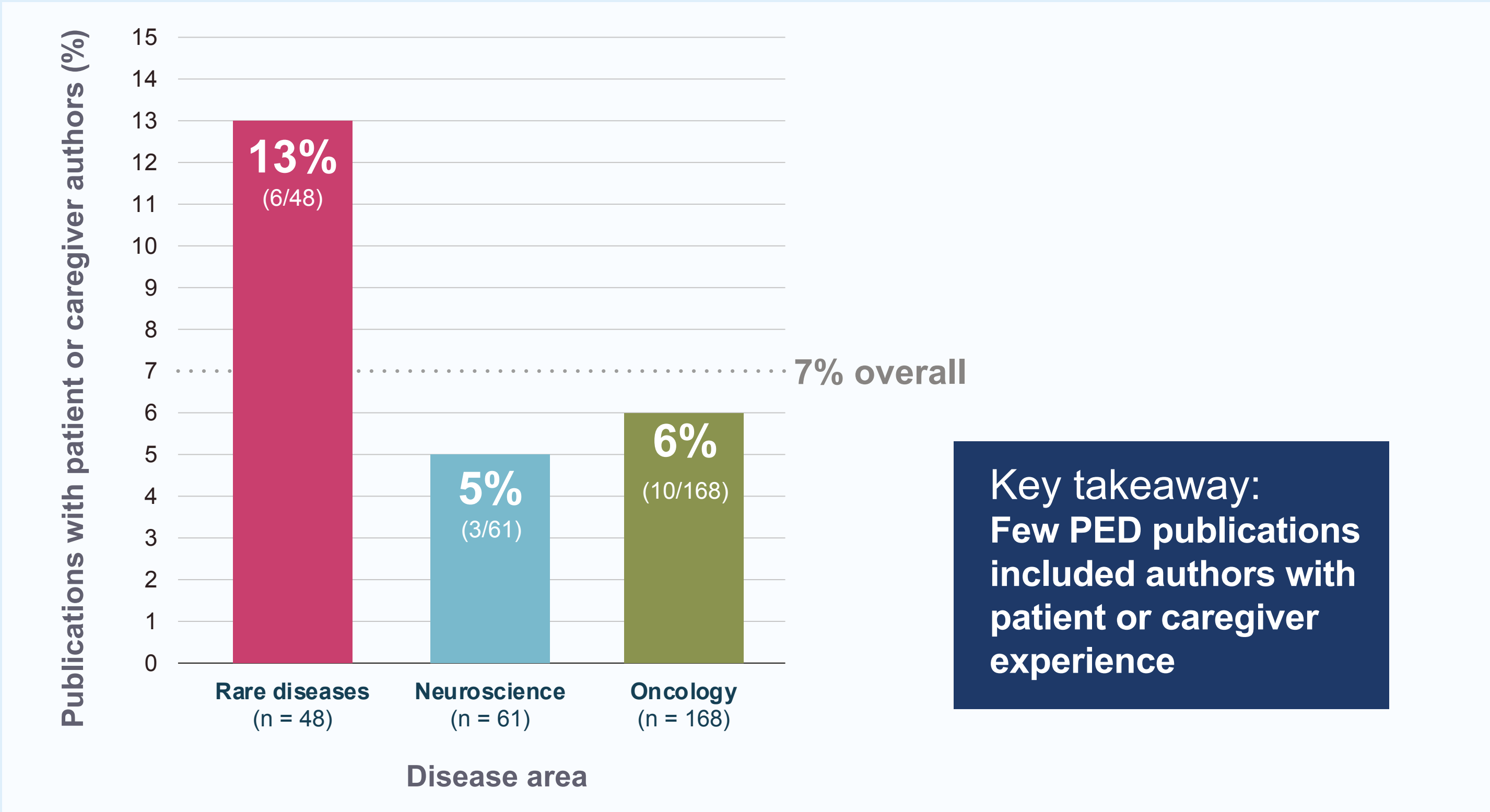
3. PED publications with PLS

- A small number of PED publications included PLS (14/277; 5%), either as part of the publication, as supplementary material, or as a standalone publication.



4. PED publications with patient or caregiver authors

- A small number of PED publications had authors with patient or caregiver experience (19/277; 7%).



References: 1. U.S. Food and Drug Administration. Available from: <https://www.fda.gov/drugs/development-approval-process-drugs/patient-focused-drug-development-glossary>. Accessed March 20, 2024. 2. European Medicines Agency. Available from: https://www.ema.europa.eu/en/documents/other/executive-summary-patient-experience-data-eu-medicines-development-and-regulatory-decision-making-workshop_en.pdf. Accessed March 20, 2024. 3. Schroeder K, Bertelsen N, Scott J, et al. Ther Innov Regul Sci 2022;56:848–58. 4. Bertelsen N, Dewulf L, Ferré S et al. Ther Innov Regul Sci 2024;58:63–78. 5. European Patients Forum. Available from: <https://www.eu-patient.eu/globalassets/20240223-epf-op-ed-on-ped.pdf>. Accessed March 20, 2024. 6. Zvonareva O, Cravej C, Richards DP. Res Involv Engagem 2022;8:29.

Presented at the 20th Annual Meeting of the International Society for Medical Publication Professionals (April 29 to May 1, 2024; Washington DC, USA)

Disclosures and Acknowledgments: This study was sponsored by Ipsen. Editorial and creative support was provided by Miranda Dixon and Greg Flocco, of Envision Pharma Group, and funded by Ipsen. All authors took part in the research, were actively involved in preparing the abstract, and have approved the final poster. SRS, JO, and CE are employees of, and own share options in, Envision Pharma Group, a healthcare communications company that is funded by pharmaceutical companies. OG and ST are employees of Ipsen. OG is a shareholder of Ipsen. SRS has received honoraria from Future Science Group (now part of Taylor & Francis), is a trustee of RAIISE (a charitable incorporated organization registered in England and Wales, Number 1180704), is a member of the ISMPP Patient Engagement Task Force, and is a member of the EULAR PARE Committee. CE is a member of the ISMPP European Meeting Programme Committee 2024. No payments were made for authorship.

Simon R. Stones: ORCID: 0000-0002-5943-1310; Oleks Gorbenko: ORCID: 0000-0002-4771-0128

This study was sponsored by Ipsen