

Understanding pharmaceutical company and patient involvement in rare disease case reports

Charles Pollitt,^a Corin Wing,^b Angelyca Jackson,^{c*} Tamzin Gristwood,^d David Gothard^d

^aIpsen, Wrexham, UK; ^bIpsen, Slough, Berkshire, UK; ^cIpsen, Cambridge, MA, USA;

^dOxford PharmaGenesis, London, UK

*At the time the study was performed



For further information, please send your question(s) to Charles Pollitt (charles.pollitt@ipsen.com).
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Objective

- Rare disease case reports are considered an important source of educational data for healthcare professionals, patients, and caregivers.
- To better understand the publication landscape around rare disease case reports, we explored both reporting quality and pharmaceutical company (pharma) and patient involvement in their development (**Panel 1**).

Research design and methods

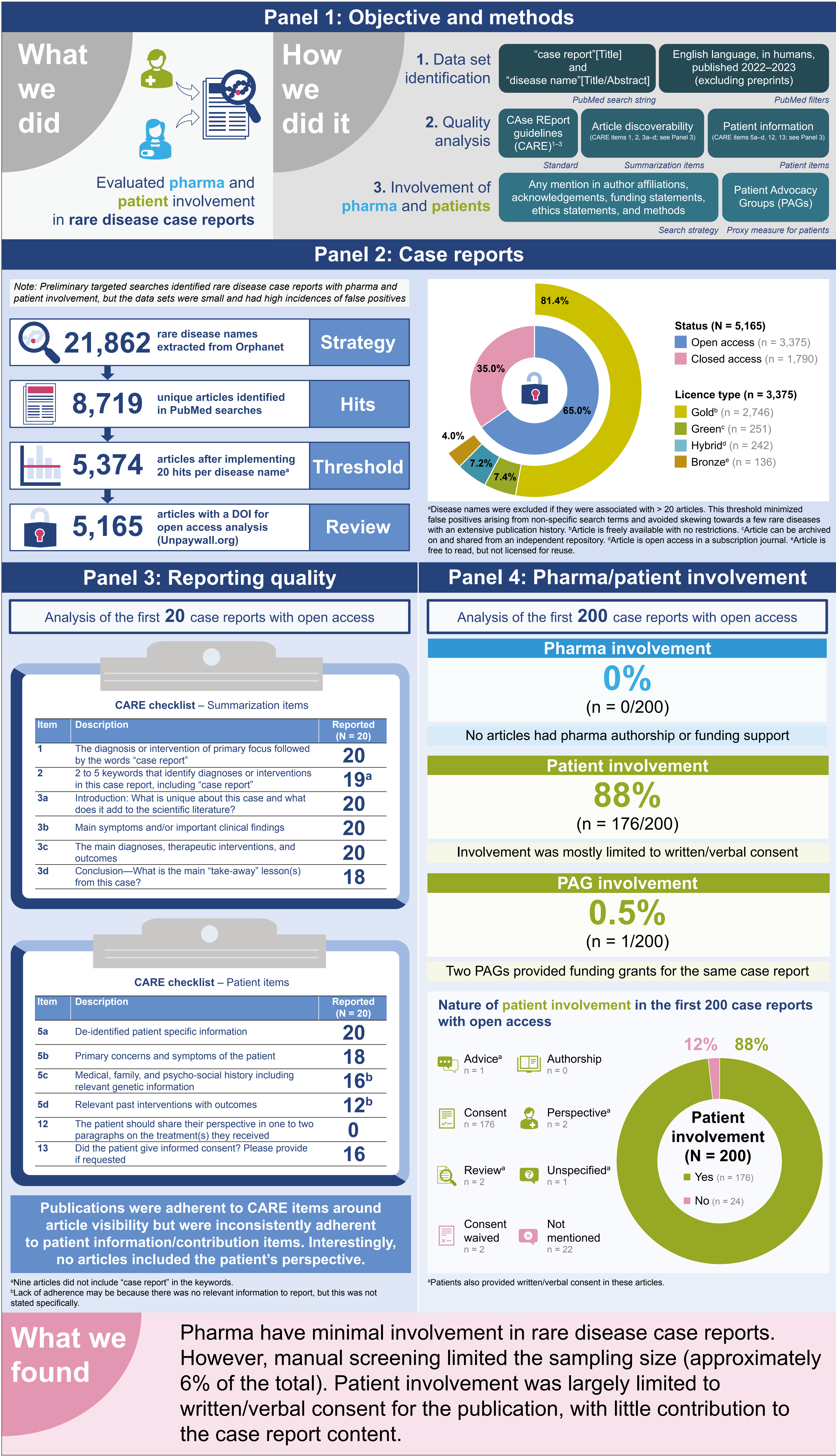
- After initial scoping searches, we conducted semi-automated literature searches in PubMed on 11–12 September 2023 to identify a robust data set of rare disease case reports (**Panel 1**).
- Using 21,862 names of rare diseases (affecting ≤ 1/2,000 people in Europe) extracted from Orphanet, we searched “case report”[Title] and “disease name”[Title/Abstract], and filtered for English-language articles in humans (excluding preprints), published from 2022 to the present (**Panels 1 and 2**).
 - Disease names were excluded if they were associated with > 20 articles. This threshold minimized false positives arising from non-specific search terms and avoided skewing towards a few rare diseases with an extensive publication history.
- Reporting quality (CARE items related to discoverability and patient representation) was assessed in the first 20 most recent open-access articles, and pharma/patient involvement was assessed in the first 200 articles (~ 6%) (**Panel 1**).

Results

- Our data set comprised 5,165 rare disease case reports; 3,375 (65%) were open access and 81.4% had a gold open-access licence (**Panel 2**).
- Screened articles were generally adherent to CARE summarization items, but inconsistently adherent to patient items (**Panel 3**).
- No screened article had pharma involvement, despite some pharma involvement having been identified during scoping searches (**Panel 4**).
- Beyond written/verbal consent in 176 screened articles (88%), patient involvement included providing advice (one article), reviewing (two articles), or providing patient perspectives (two articles); one article had an unspecified patient contribution, and one had patient advocacy group funding (**Panel 4**).

Conclusions

- Our data suggest that pharma involvement in rare disease case reports is uncommon. However, manual screening and search criteria limitations impeded the identification of relevant articles.
- Patients contributed little beyond consent and were represented inconsistently.
- Adherence to CARE summarization items supported article discoverability.
- Further adherence to CARE items could improve the quality of industry-sponsored case reports and ensure greater patient involvement.



Abbreviations CARE, CARE REport guidelines; DOI, digital object identifier; PAG, patient advocacy group.

References 1. Gagnier JJ et al. *Glob Adv Health Med* 2013;2:38–43; 2. Riley DS et al. *J Clin Epidemiol* 2017;89:218–35; 3. 2013 CARE Checklist. Available from: <https://www.care-statement.org/checklist> (Accessed 14 November 2023).

Author contributions All authors provided substantial contributions to study conception/design or acquisition/analysis/interpretation of data; drafting of the publication or reviewing it critically for important intellectual content; and gave their final approval of the publication.

Disclosures CP, CW: employees of Ipsen and may hold stock or stock options. AJ: employee of Ipsen at the time the study was performed and currently an employee of Takeda Pharmaceutical Company, Ltd. TG, DG: employees of Oxford PharmaGenesis.

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