LSE Department of
Health Policy

Industry funding of patient organisations in the UK: A retrospective study of commercial determinants, funding concentration and disease prevalence

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WHO Collaborating Centre
for Governance, Accountability and Transparency
in the Pharmaceutical Sector

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the global coalition against corruption

Today’s seminar

- Background and theoretical framework
- Methodology
- Results
- Limitations
- Conclusions

About us

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- *Research focus: Rare diseases, Patient organisations, Pharmaceutical innovation*

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What are POs and why are they important?

- POs are defined as "***not-for-profit organisations****, mainly composed of patients and/or caregivers, that* ***represent and/or support the needs of patients and/or caregivers***" (EFPIA, 2022; Ozieranski et al., 2019)

- POs play a critical role in advocating for patients and supporting drug development, regulatory review, and adoption of new drugs (Fabbri et al., 2020; Geissler et al., 2017)

- They represent patient views, support research design and planning, and provide information and support to patients and clinicians

- POs are involved in pharmaceutical decision-making, and they routinely interact with other key stakeholders such as **pharmaceutical companies**

Drugs development and commercialisation timeline

POs involvement

Research priorities	**Research design and planning**	**Research & operations**	**Regulatory approval**	**Marketing authorisation**	**Post-authorisation**
Setting research priorities	Protocol design	Clinical trials	Regulatory affairs	HTA	Communication and advocacy
• Help defining patient-relevant added value and patient-relevant outcomes • Advocate for the need of innovative/curative technologies • Match patients' unmet medical needs with intended research outcomes	• Help identifying relevant endpoints and patient-reported outcome / quality of life measures	• Facilitate patients' recruitment and participations in clinical trials • Provide administrative support throughout enrollment • Provide grants to support research projects • Provide first-hand medical knowledge and understanding of disease pathophysiology to trial investigators	• Share patients' views on the benefits and risks of medical products and the overall development programme • Active participation in regulatory process (EU: COMP, CHMP; UK: MHRA)	• Provide inputs in HTA appraisals to help policymakers understanding technologies' value, therapeutic need and relevance of health outcomes • Contribute with key information about the ways in which diseases affects patient and their families and how the technology would improve their life with the condition	• Contribute to publications and dissemination of research results to patient community and clinicians • Offer patients with continuous support and information on therapies available • Advocate for policy and legislative actions to ensure that existing medicines are reimbursed and available to patients
Policymakers Manufacturers	Manufacturers	Manufacturers	Regulators	Payers	Patients

Adapted from Geissler et al., 2017 and Aymé, Kole and Groft, 2008

POs for rare and non-rare conditions

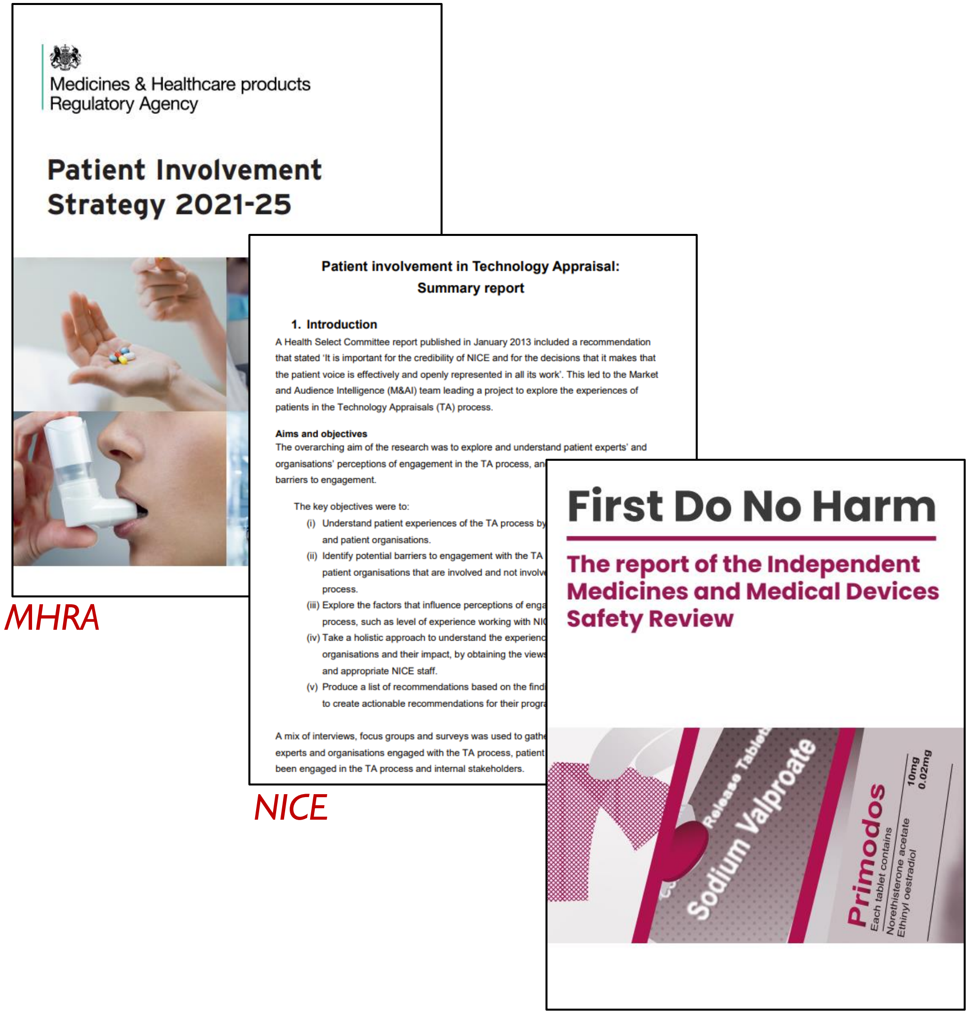
- POs are active across a number of conditions, their contributions are particularly relevant in the context of **rare diseases** (Polich, 2012; Mavris and Le Cam, 2012)

Cardiovascular diseases	**Diabetes**	**Rare diseases**
7 million	**4 million**	**3 million**

- Due to the differences between rare and non-rare conditions, POs targeting them serve **different purposes** (Aymé et al., 2008):
  - Fill in missing or inaccessible **medical knowledge**;
  - Improve understanding of **disease natural history**;
  - Support with **trial recruitment**;
  - Advocate for **legislative/policy** attention

The role of POs in the UK

- POs in the UK have an established platform for **formal engagement** in both in the regulatory and appraisal processes (MHRA, 2020; NICE, 2014)
- In 2020, **Julia Cumberlege** presented to the UK government an independent review that exposed how the UK has neglected patient wellbeing in terms of drug safety and efficacy (Cumberlege, 2020, Haskell, 2020)



Medicines & Healthcare products Regulatory Agency

Patient Involvement Strategy 2021-25

MHRA

Patient involvement in Technology Appraisal: Summary report

1. Introduction

A Health Select Committee report published in January 2013 included a recommendation that stated 'It is important for the credibility of NICE and for the decisions that it makes that the patient voice is effectively and openly represented in all its work'. This led to the Market and Audience Intelligence (M&AI) team leading a project to explore the experiences of patients in the Technology Appraisals (TA) process.

Aims and objectives

The overarching aim of the research was to explore and understand patient experts' and organisations' perceptions of engagement in the TA process, an
barriers to engagement.

The key objectives were to:

- (i) Understand patient experiences of the TA process by
and patient organisations.
- (ii) Identify potential barriers to engagement with the TA
patient organisations that are involved and not involv
process.
- (iii) Explore the factors that influence perceptions of enga
process, such as level of experience working with NI
- (iv) Take a holistic approach to understand the experienc
organisations and their impact, by obtaining the views
and appropriate NICE staff.
- (v) Produce a list of recommendations based on the find
to create actionable recommendations for their progra

A mix of interviews, focus groups and surveys was used to gath
experts and organisations engaged with the TA process, patient
been engaged in the TA process and internal stakeholders.

NICE

First Do No Harm

The report of the Independent Medicines and Medical Devices Safety Review

Cumberlege review

Literature on POs and existing gaps

- The existing literature on POs has focused on:
  - Examining the **large number and high value** of payments from industry to POs (Ozieranski, Rickard and Mulinari, 2019; Rose et al., 2017; Fabbri et al., 2020; Mulinari et al., 2020)
  - The **uneven distribution** between and within therapeutic areas (Ozieranski, Rickard and Mulinari, 2019; Mulinari et al., 2020)
  - The **concentration** of payments coming from a small number of pharmaceutical firms (Ozieranski, Rickard and Mulinari, 2019; Ozieranski et al., 2019; Ozieranski et al., 2022; Rose et al., 2017; Fabbri et al., 2020; Mulinari et al., 2020)
  - **Concordance** between companies marketed drugs and contribution to POs (Mulinari et al., 2020)
- Limitations and gaps of current body of literature:
  - Not **UK** focused
  - No focus on the **pipeline** (only launched drugs)
  - No comparative analysis on industry relationship with **rare** and **non-rare POs**

Research question(s)

- **Main research question**: What is the concordance between the commercial interests of pharmaceutical companies and POs' activities?
- **Sub-research questions**:
  1. What are the general dynamics, such as the number, frequency and value of payments, that exist between pharmaceutical companies and POs?
     - Who are the top funders?
     - Which are the most funded therapeutic areas?
  2. What is the concentration of industry funding (i.e., how many companies funded each POs and the extent to which organisations might have been reliant on funding from a single company)
- **For all RQs above, we investigated whether differences existed between rare and non-rare diseases**

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Methodology

1. Data on industry payments
2. Data on POs
3. Commercial interests
4. Industry funding concentration

Data on industry payments

- Data on payments from pharmaceutical companies to POs from **2018** to **2020** were retrieved in February 2022 from the websites of companies abiding by the ABPI Code of Practice
- Disclosing payments to POs is a requirement of **Clause 29** of ABPI Code of Practice
- Companies signed up to abide by the **ABPI Code**, accepting the jurisdiction of the PMCPA (Code regulator) extends beyond those who are ABPI members and is expected to include **most pharmaceutical companies operative in the UK**
- All payments were first adjusted for inflation using the ONS Consumer Price Index and then converted to British Pounds, using the ONS historical yearly conversion rates
- All payments are in 2020 GBP

Abbreviations: ABPI: The Association of the British Pharmaceutical Industry; PMCPA: The Prescription Medicines Code of Practice Authority; ONS: Office for National Statistics

Data on POs

- POs' websites were screened to understand the condition(s) they focused on
- The condition(s) targeted by POs were translated into **ICD-11 codes** using the online ICD-11 database (WHO, 2021)

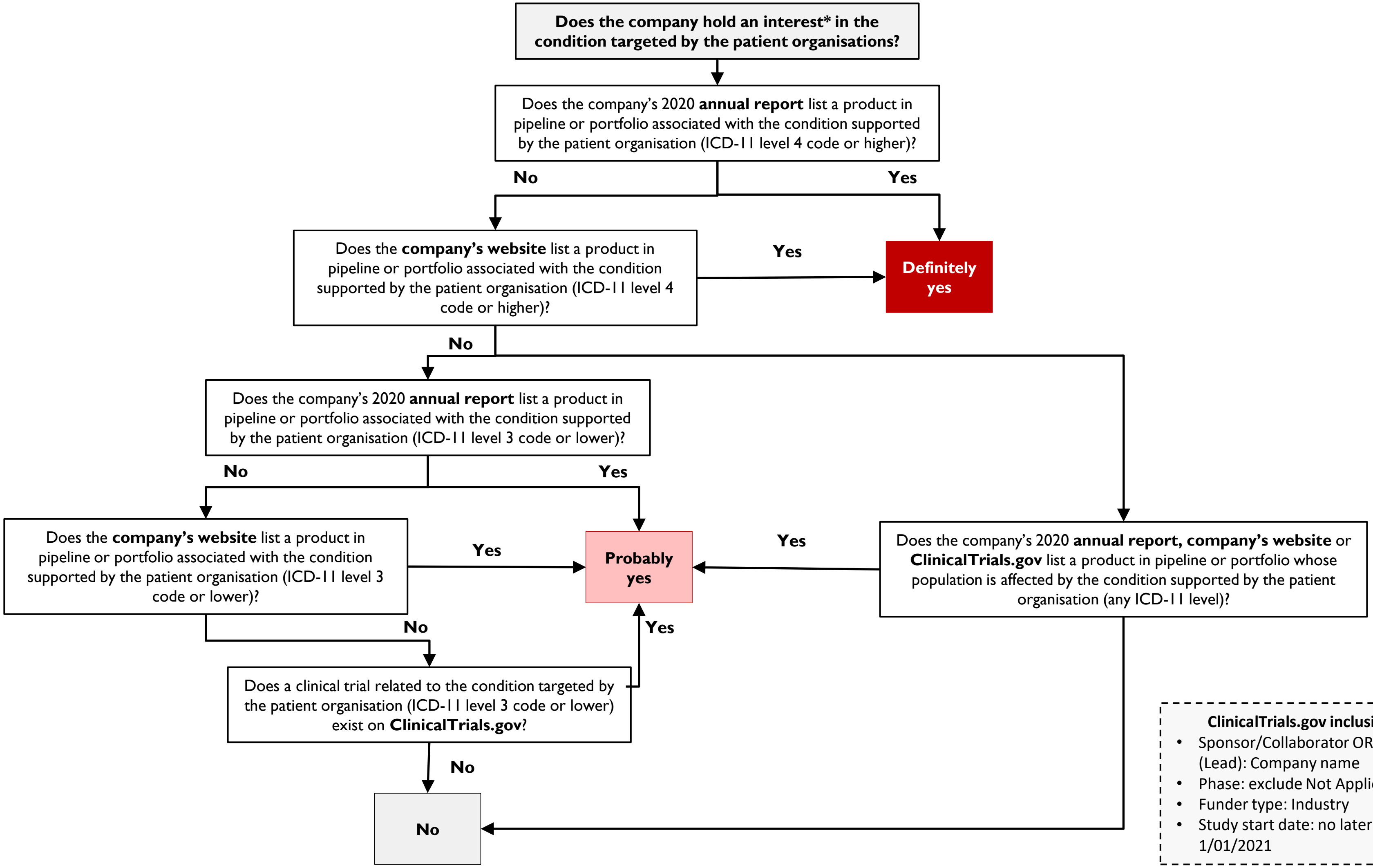
PO name	Condition	ICD-11 code
Blood Cancer UK	Blood cancer	2A, Neoplasms of haematopoietic tissues

- Conditions were further classified into **rare** and **non-rare**
  - Conditions were considered rare if they appeared in the Orphanet database of rare diseases regardless of their classification
  - When condition sub-types appeared in the Orphanet database, the PO's website was screened to check whether its focus was on rare conditions
  - Should a PO focus on a broader condition such as blood cancer with no sole focus on rare conditions, the organisation would be conservatively considered non-rare
- A third category (**unclear**) was created for non-disease-specific POs

Determining commercial interests

- An interest is when there is, or could be perceived to be, an **opportunity for a pharmaceutical company to benefit in the disease area where the PO operates** (NICE, 2018)
  - The pharmaceutical company has a drug developed or in development for a condition targeted by the PO;
  - A drug in the company's portfolio or pipeline is restricted to a specific population affected by the disease supported by the PO
- We searched companies' **annual reports**, **websites** and the **ClinicalTrials.gov** registry to determine whether each company had an interest in the condition targeted by the PO receiving the payment

Determining commercial interests



Does the company hold an interest* in the condition targeted by the patient organisations?

Does the company's 2020 **annual report** list a product in pipeline or portfolio associated with the condition supported by the patient organisation (ICD-11 level 4 code or higher)?

Does the **company's website** list a product in pipeline or portfolio associated with the condition supported by the patient organisation (ICD-11 level 4 code or higher)?

Definitely yes

Does the company's 2020 **annual report** list a product in pipeline or portfolio associated with the condition supported by the patient organisation (ICD-11 level 3 code or lower)?

Does the **company's website** list a product in pipeline or portfolio associated with the condition supported by the patient organisation (ICD-11 level 3 code or lower)?

Probably yes

Does the company's 2020 **annual report, company's website** or **ClinicalTrials.gov** list a product in pipeline or portfolio whose population is affected by the condition supported by the patient organisation (any ICD-11 level)?

Does a clinical trial related to the condition targeted by the patient organisation (ICD-11 level 3 code or lower) exist on **ClinicalTrials.gov**?

No

ClinicalTrials.gov inclusion criteria

- Sponsor/Collaborator OR Sponsor (Lead): Company name
- Phase: exclude Not Applicable
- Funder type: Industry
- Study start date: no later than 1/01/2021

Industry funding concentration

- The following dimensions of industry funding concentration were explored:
  1. Number of companies funding each POs;
  2. Share of overall industry funding coming from each contributing company;
  3. Share of industry funding of each organisation comprised by the single highest payment

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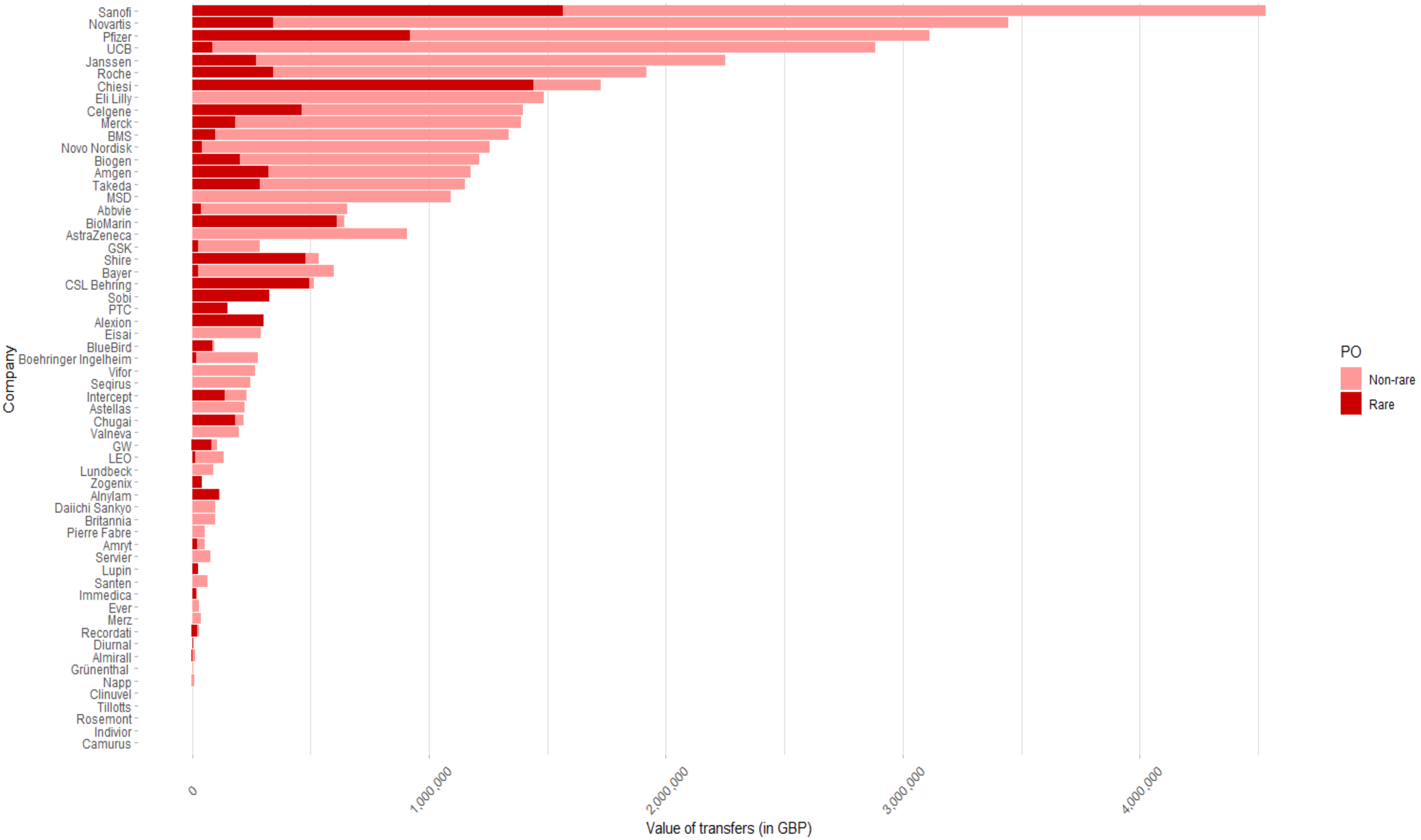
General dynamics – Value and volume of payments

	2018	2019	2020	All years (2018-2020)
Number of payments	924	1,063	1,168	3,155
Median payment (IQR; overall)	£5,136 (£678 - £12,756)	£5,085 (£636 - £12,680)	£9,000 (£1,894 - £15,205)	£5,400 (£921 - £15,000)
Median payment (IQR; rare)	£7,190 (£1,249 - £15,408)	£5,085 (£1,236 - £12,204)	£8,500 (£2,500 - £15,000)	£7,000 (£1,777 - £15,000)
Median payment (IQR; non-rare)	£3,082 (£616 - £11,468)	£4,800 (£508 - £12,712)	£9,120 (£1,540 - £16,175)	£5,085 (£740 - £14,880)
Value of payments (£; overall)	£10,933,715	£13,046,079	£18,015,722	£41,995,516
Value of payments (£; rare)	£2,329,017	£3,281,001	£4,180,892	£9,790,909
Value of payments (£; non-rare)	£7,991,072	£9,109,462	£12,570,027	£29,670,563
Number of pharmaceutical companies	37	50	60	60
Number of patient organisations	221	268	294	429

General dynamics – Value and volume of payments



Commercial interests

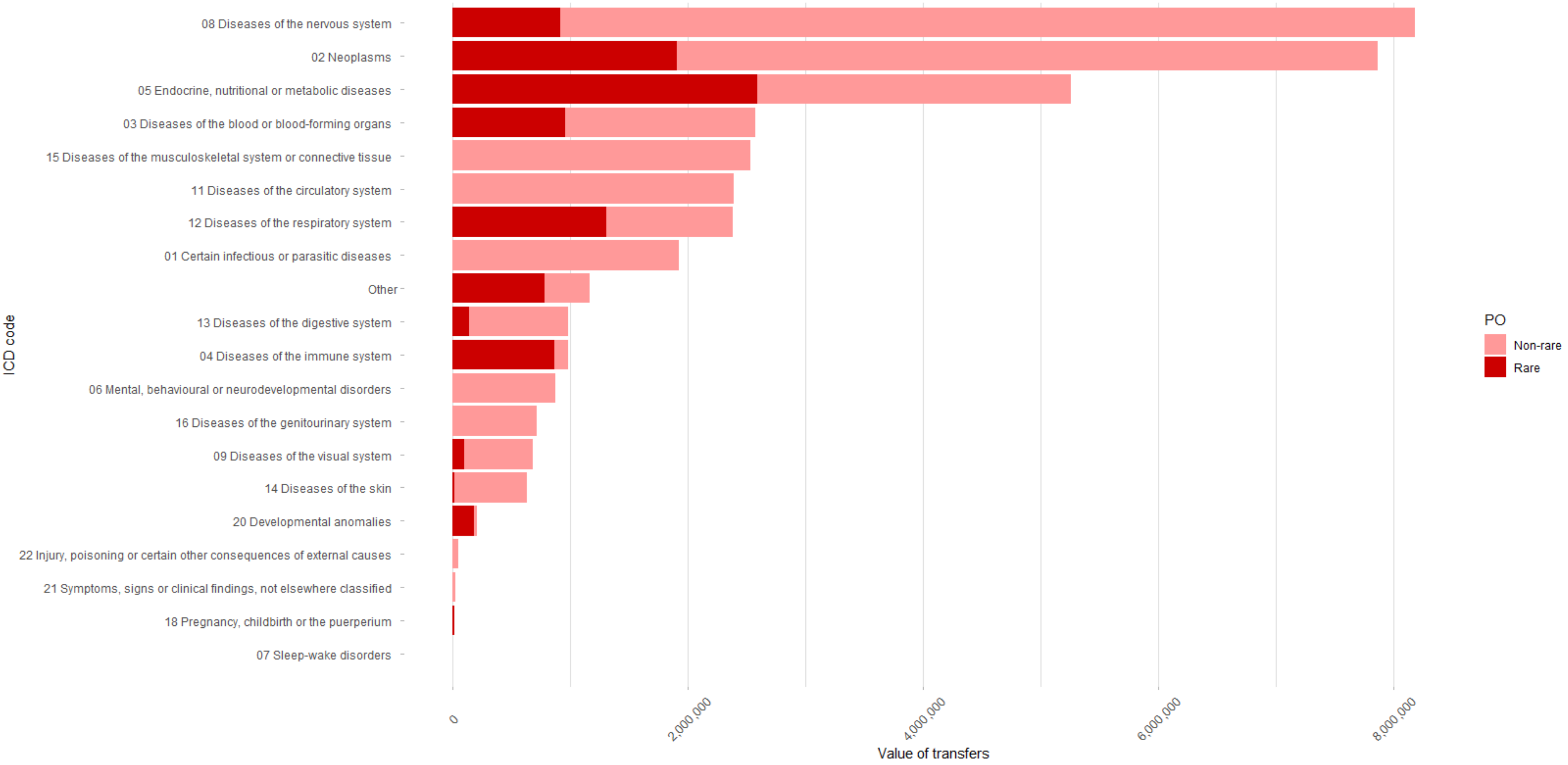
- **92%** of the payments were directed to POs that were judged to be aligned with their portfolio and pipeline
- Payments to POs targeting a disease for which the company has a product developed or in development (*definitely yes*) made up around **52%** regardless of the rarity of the condition targeted
- **No significant difference** was found between rare and non-rare POs

PO type	Company's interest	Volume; n (%) All years (2018-2020)	Value: £ (%) All years (2018-2020)
Overall	Definitely yes	1,627 (52%)	£26,002,527 (62%)
	Probably yes	1,265 (40%)	£12,724,965 (30%)
	No	263 (8%)	£3,262,205 (8%)
Rare	Definitely yes	339 (54%)	£6,725,300 (69%)
	Probably yes	262 (41%)	£2,713,531 (28%)
	No	34 (5%)	£352,078 (4%)
Non-rare	Definitely yes	1,276 (55%)	£19,121,806 (62%)
	Probably yes	977 (42%)	£9,827,287 (35%)
	No	71 (3%)	£721,468 (3%)

Rare vs non-rare-focused POs

- **23%** of the value of payments to POs were directed to rare disease-focused vs **71%** to non-rare-focused ones
- From 2018 to 2020, payments to POs targeting rare diseases **increased more** compared to those focusing on more prevalent conditions (80% vs 57%)
- Median payments received by POs were **significantly different** ($p<0.001$) depending on the rarity of the disease they focused on, with rare POs receiving **higher payments**
- Irrespective of the rarity of the disease(s) targeted, the top three most funded disease areas represented **more than half of overall funding**

General dynamics – Therapeutic areas



Industry funding concentration

- On average, each PO received payments from approximately **two companies**, with no significant differences between rare and non-rare POs
- The median company contribution to rare-focused POs comprised **42%** (IQR: 14.5%-100%) of their overall industry funding versus **31%** (IQR: 11.6%-99.7%) for non-rare POs (χ^2= 7.141, p-value = 0.008)
- The single highest payment to POs amounted to an average of **73%** (SD: 0.29) of overall payments, ranging from a minimum of 10% to a maximum of 100%

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Main study limitations

- Data availability
  - Lack of mandatory reporting of payments to patient organizations by companies that **do not comply** with the ABPI Code (Ozieranski et al., 2021);
  - **Underreporting** of payments to patient organization (Ozieranski et al., 2020);
  - **Removal** of disclosure reports from the public domain (ABPI Code of Practice)

- Our analysis focused on a **recent though short** time period (2018-2020)
  - It is unclear whether these trends hold over time and their generalisability to other periods

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Conclusions (1/2)

- Almost all industry payments during our study period – in terms of both volume (92%) and value (92%) – were to POs aligned with pharmaceutical companies' portfolios and pipelines
- Despite rare diseases affecting only **5%** of the UK population, almost a **quarter** of reported industry payments to POs from 2018 to 2020 is directed towards rare-focused organisations (£9.8 million out of £42 million)

	UK population	**Payments to UK POs**
Share represented by rare diseases	**5%**	**25%**

- This is likely to reflect the **commercial attractiveness** of such conditions and the important role POs play in the rare disease patient community

Conclusions (2/2)

- The rare conditions that attracted more funding were **highly prevalent diseases** (e.g., cystic fibrosis, multiple myeloma) for which multiple therapeutic alternatives have been developed and are in commerce
  - This poses the risk of widening already existing health inequities

- Particular attention should be paid to payments immediately before or after endorsements of products by POs to maintain their integrity

- POs focusing on rare diseases are funded by very few companies, relying on a single payment for over **80%** of their industry-reported income
  - Government support needs to be secured to avoid overreliance on industry funding

Thank you!

For any further question, please feel free to reach out at
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