

# Comparing non-oncological rare condition submissions to the National Institute for Health and Care Excellence (NICE) versus Scottish Medicines Consortium (SMC) from 2001 to 2021



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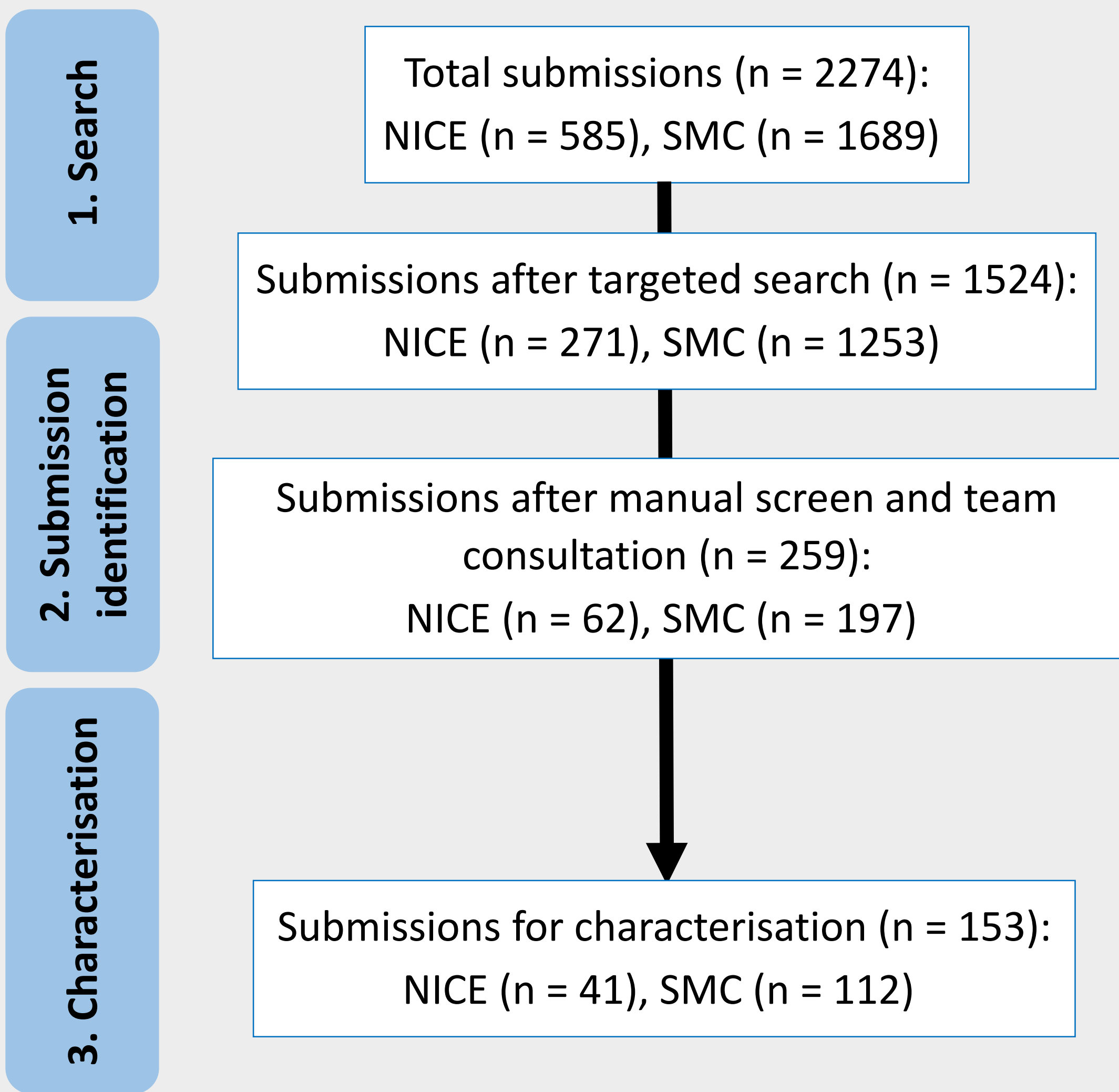
## Aims and objectives

- This project aims to build an understanding of UK reimbursement challenges in rare diseases. There is concern that sufficient concessions are not made for rarity when evaluating evidence.
- The initial stage of this project is to identify, characterise, and compare the frequency and types of submissions for treating non-oncological rare diseases (rare defined as <5 in 10,000; and ultra-rare <1 in 50,000) made to NICE and SMC from inception until November 2021.

## Methods

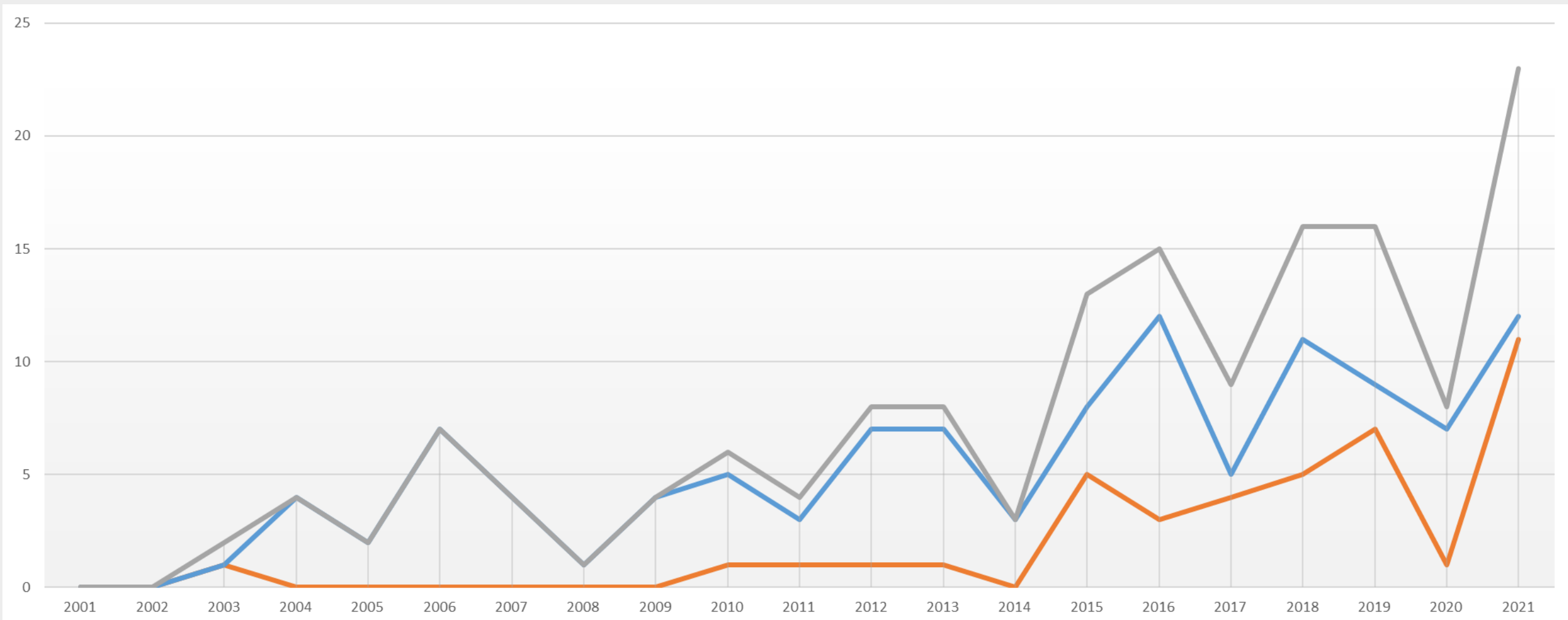
- Three approaches were applied to identify NICE<sup>1</sup> and SMC<sup>2</sup> submissions: i) targeted keyword searching ii) manual identification iii) review team consultations.
- Characterisation and comparison (Figures 1-4) of included submissions: i) frequency and proportion by type of NICE and SMC submission ii) frequency by year and cumulative growth between 2001-2021 iii) frequency of submission by disease.

## Results: Submission identification



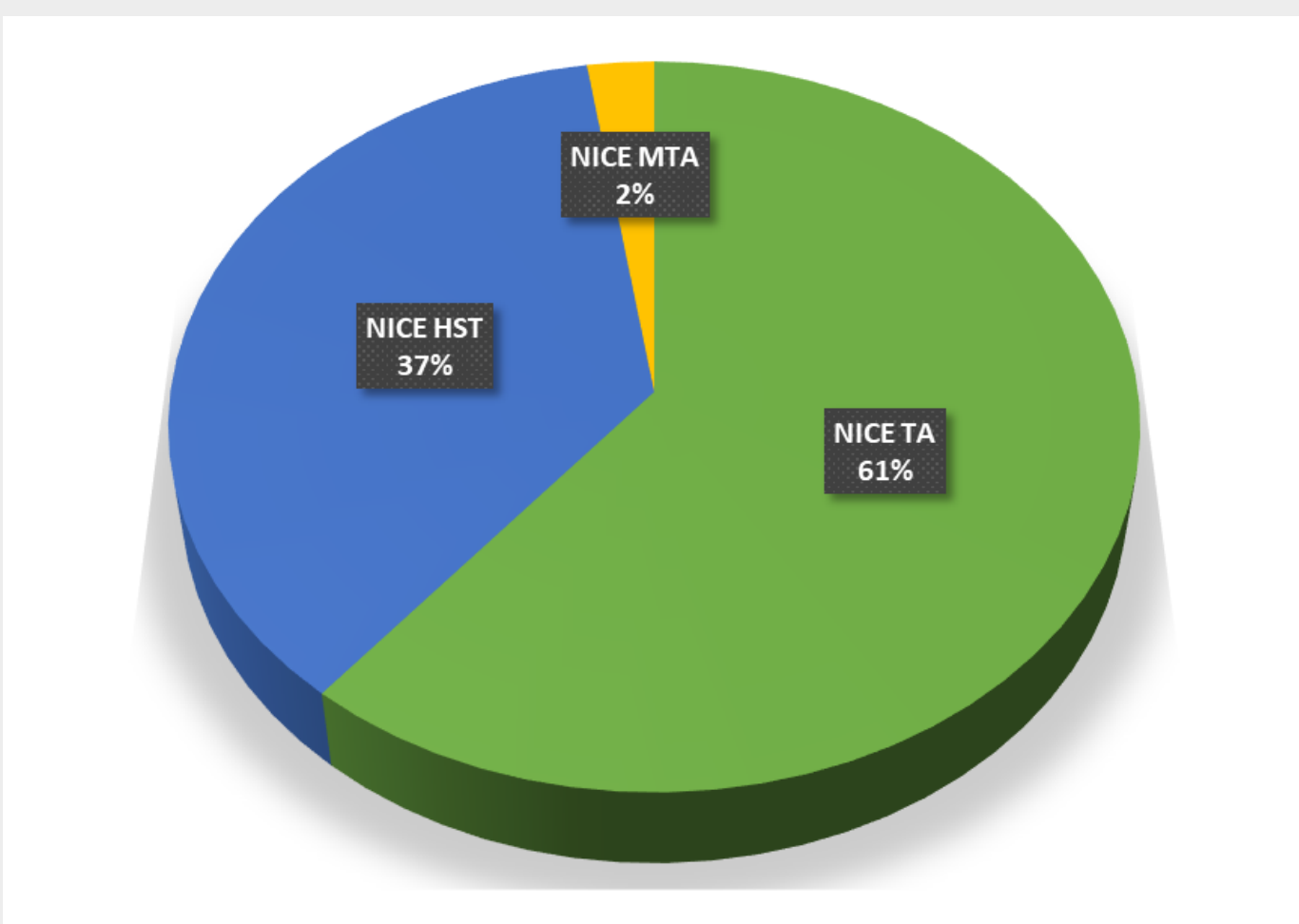
## Results: Characterisation and comparison

Figure 1. Frequency of NICE and SMC submissions by year



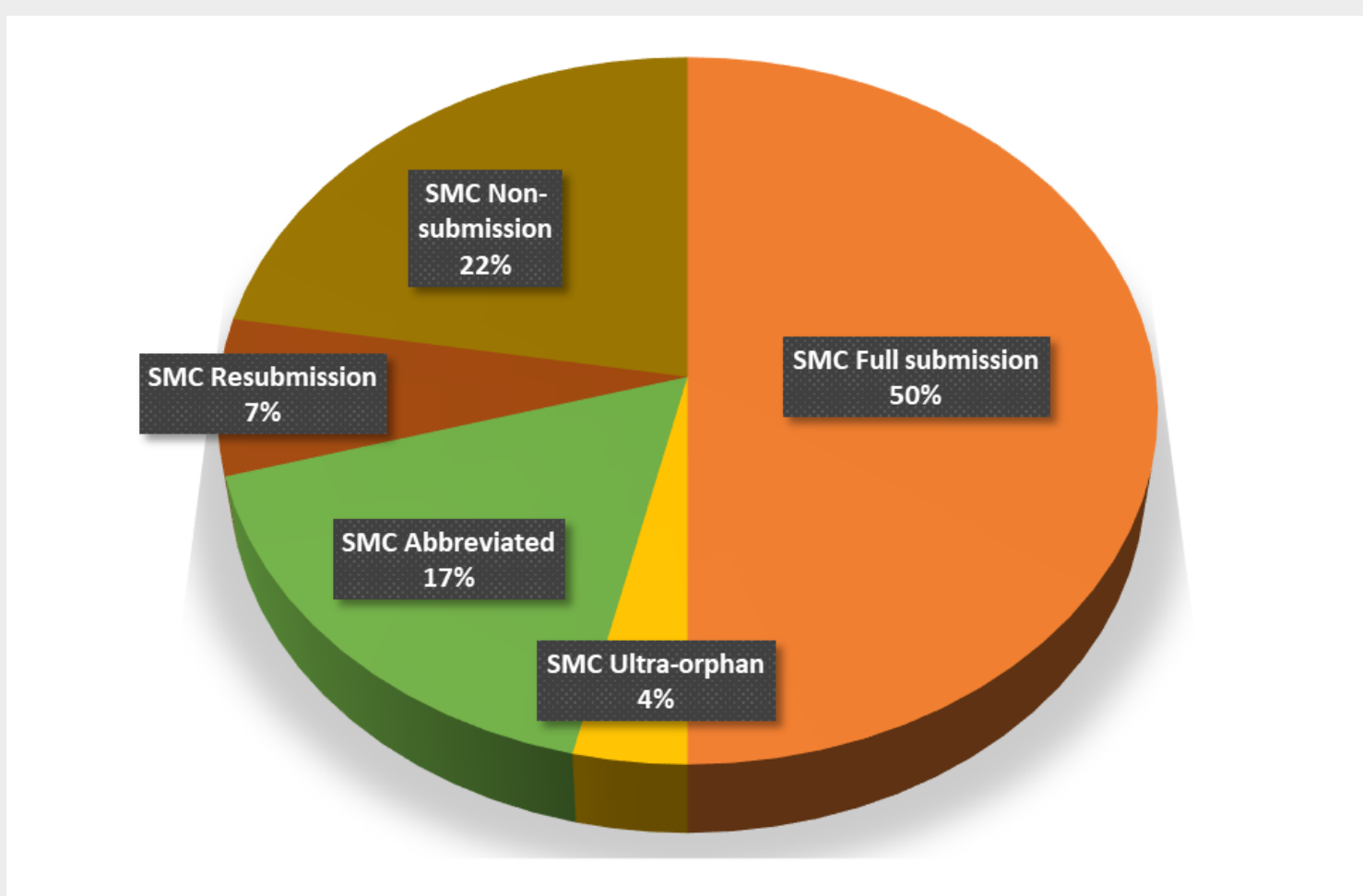
- Data: Total NICE SMC
- Mean total per year = 7 (range 1-23)
- 88% [36/41] NICE vs 57% [64/112] SMC submissions since 2015

Figure 2. NICE submission types



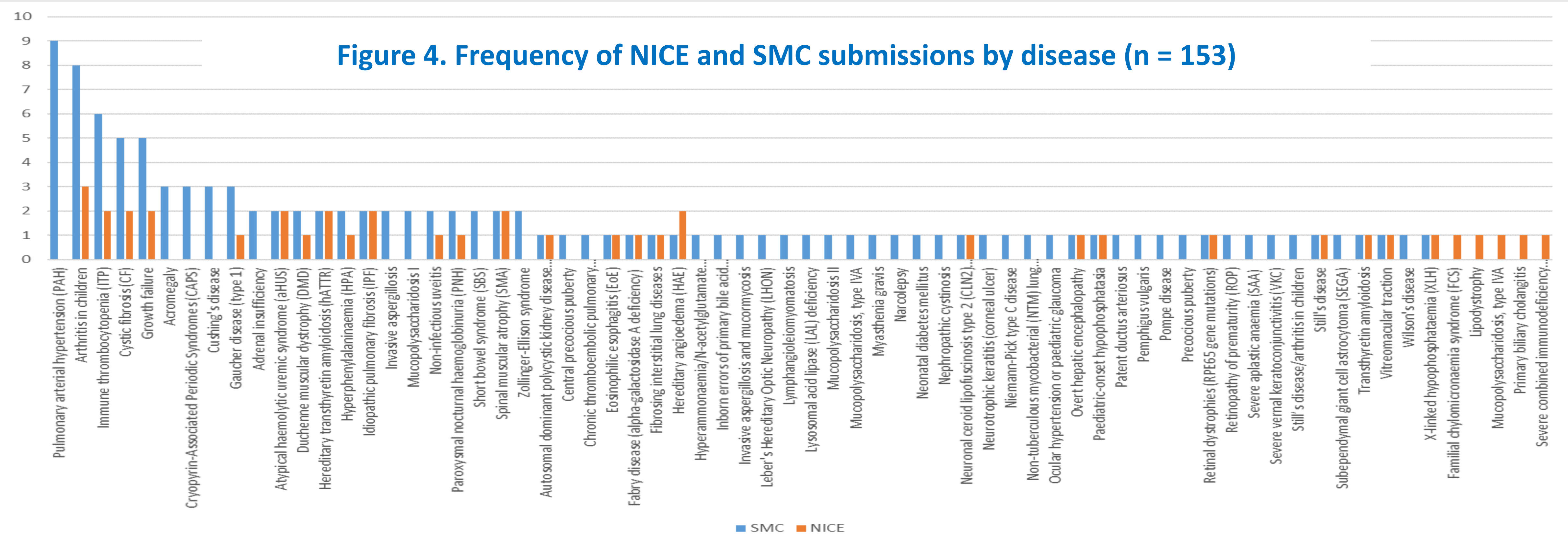
- 41 submissions (25 STA, 15 HST, 1 MTA)

Figure 3. SMC submission types



- 112 submissions (56 full, 19 abbreviated, 8 re-submission, 4 ultra-orphan, 25 non-submission)

Figure 4. Frequency of NICE and SMC submissions by disease (n = 153)



- Submissions for 26 rare diseases were made to both NICE and SMC
- Submissions for 37 rare diseases were made to SMC and not NICE, whereas 5 rare diseases were made to NICE and not SMC

## Conclusions

- The SMC has assessed more technologies for a broader range of non-oncological rare conditions than NICE, possibly because SMC is quicker at issuing appraisal guidance compared to NICE<sup>3</sup> and the orphan and ultra-orphan pathways differ between the agencies.
- The impact of this difference on access and health inequality in England/Wales and Scotland is unclear.
- Future research would include analysis of submissions, to build an understanding of the reimbursement challenges in rare diseases.

## References and funding

- NICE. Technology appraisal guidance static list [Internet]. 2022. Available from: [tinyurl.com/bdfp4vcz](https://tinyurl.com/bdfp4vcz)
- SMC. Medicines advice [Internet]. 2022. Available from: [tinyurl.com/2v9yvdda](https://tinyurl.com/2v9yvdda)
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