## NICE-Flatiron Health Research Collaboration: Aim 1 Results

# Can Early U.S. Adoption of Cancer Drugs Inform HTA Decision-Making?

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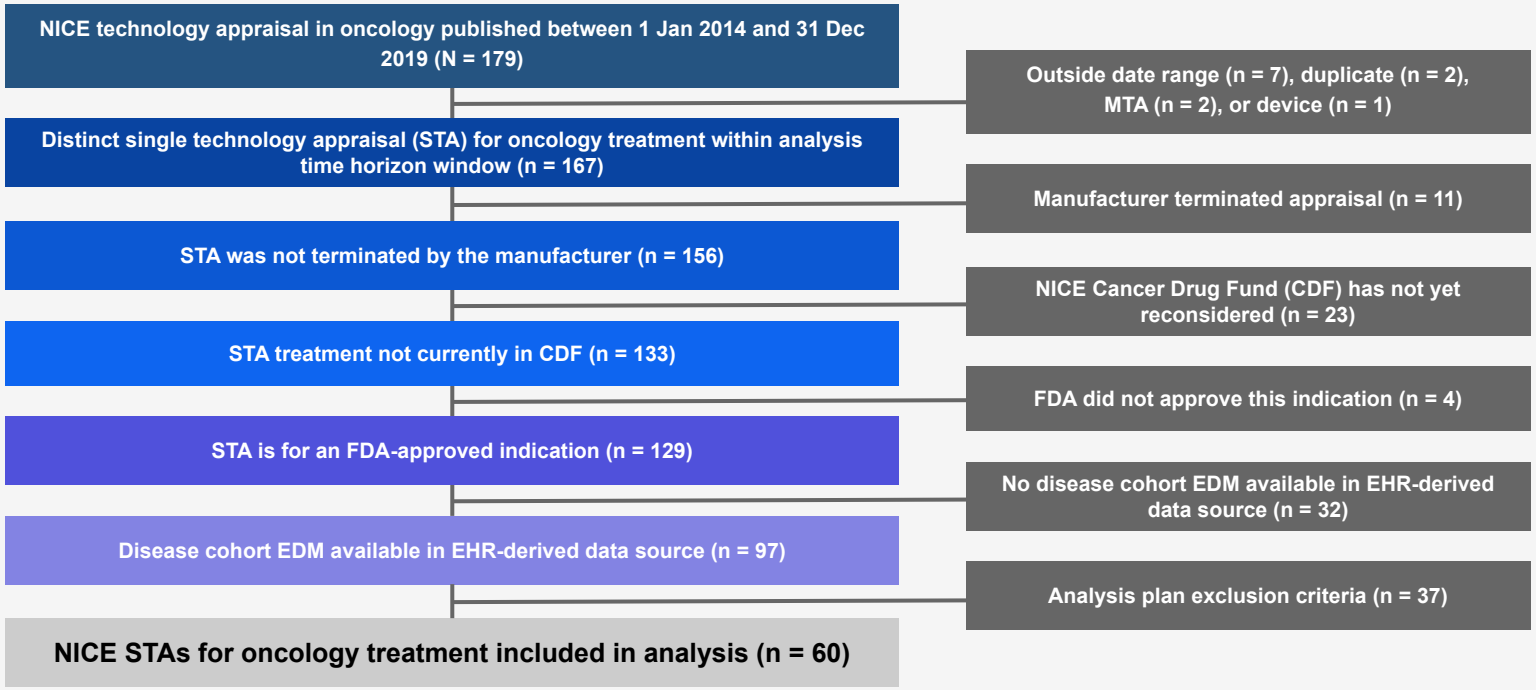
# Methods

- **Study Design:** For each NICE TA in a selected set, we calculated the **time between FDA approval and EMA market authorization, NICE TA submission, and NICE TA publication.** The distributions of time were described using mean, median, range, and stratified by therapy class, cancer type, biomarker-driven indication, submission period (≤2016 or >2017), first-in- class, and NICE decision.

- **Data Sources:** This retrospective study used the Flatiron Health electronic health record (EHR)-derived de-identified data **representing approximately 280 cancer clinics in the United States (~800 sites of care)**. We used research-ready electronic data mart cohorts. We also used **publicly available publications of NICE single technology appraisals.**

- **Outcomes:** The primary outcome was **the mean total number of patients exposed to a new product over time that received both FDA and EMA initial approval or label extension.** We counted the number of patients in the EHR-derived RWD cohort who had started the drug before each milestone date (EMA market authorization, NICE TA submission, and NICE TA publication). We correspondingly calculated the possible follow-up time of each patient that would have been available at each milestone.

# Selection of NICE Oncology Appraisals for Analysis




Includes one reappraisal. Analysis plan excluded disease cohort EDM if there was less than two years since the EDM was initiated or insufficient experience with disease specific data models.
*Abbreviations: CDF, Cancer Drug Fund; FDA, US Food and Drug Administration; EDM, electronic data mart; MTA, multiple technology appraisal; STA, single technology appraisal.*

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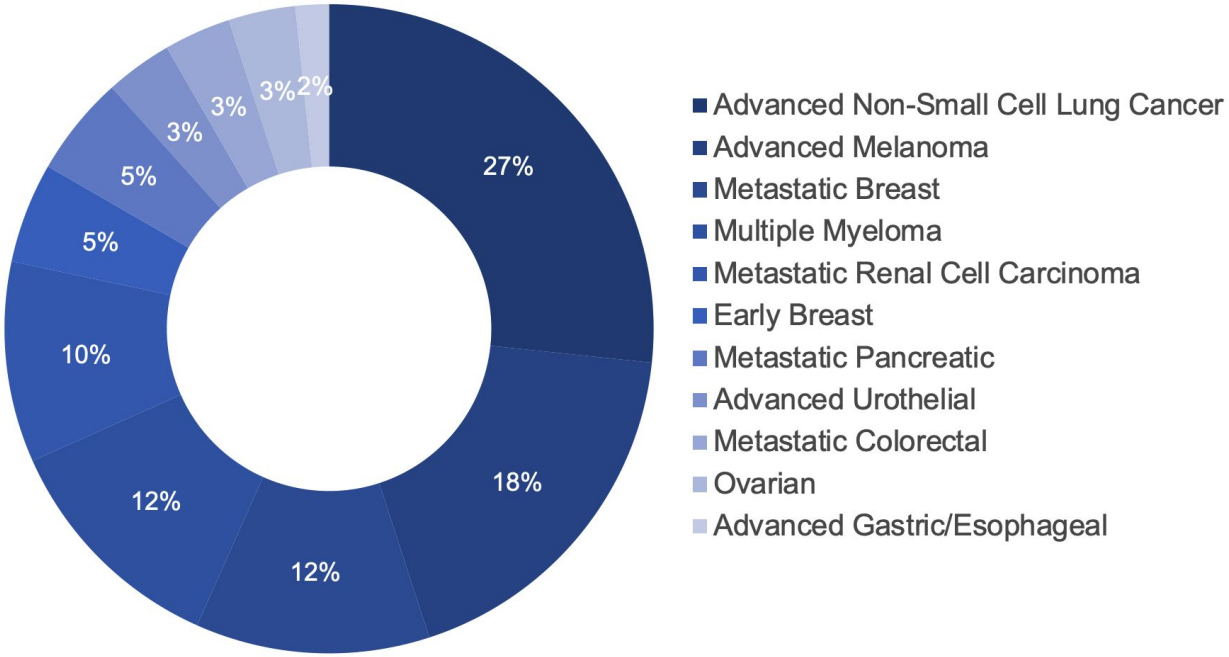
# NICE Technology Appraisals (N = 60)



# Timeline with median months between FDA approval and HTA milestones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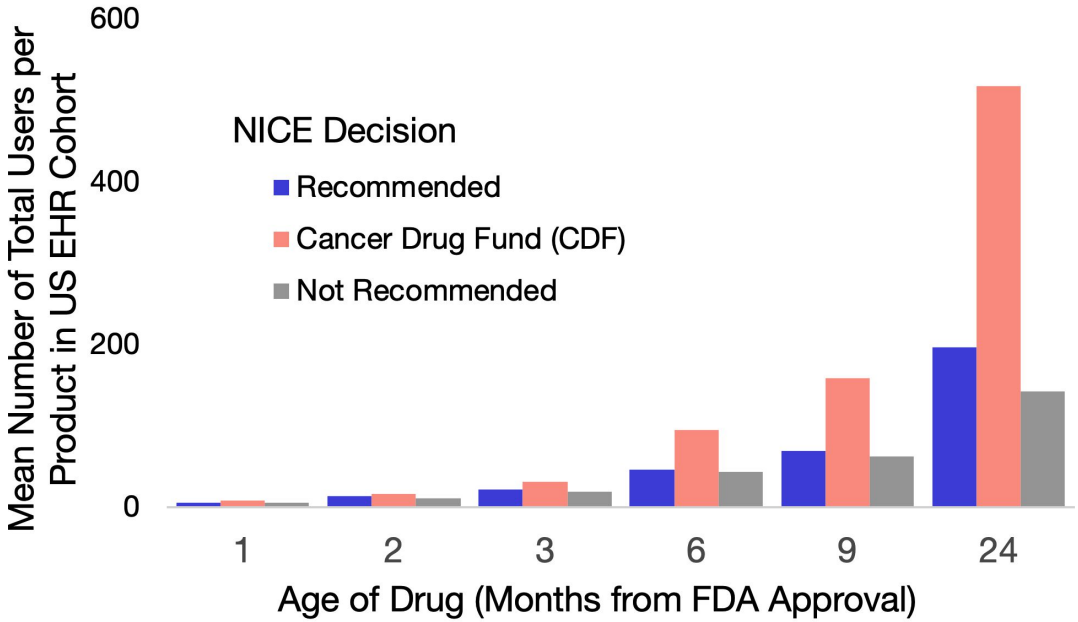
# Uptake of new drugs after FDA approval

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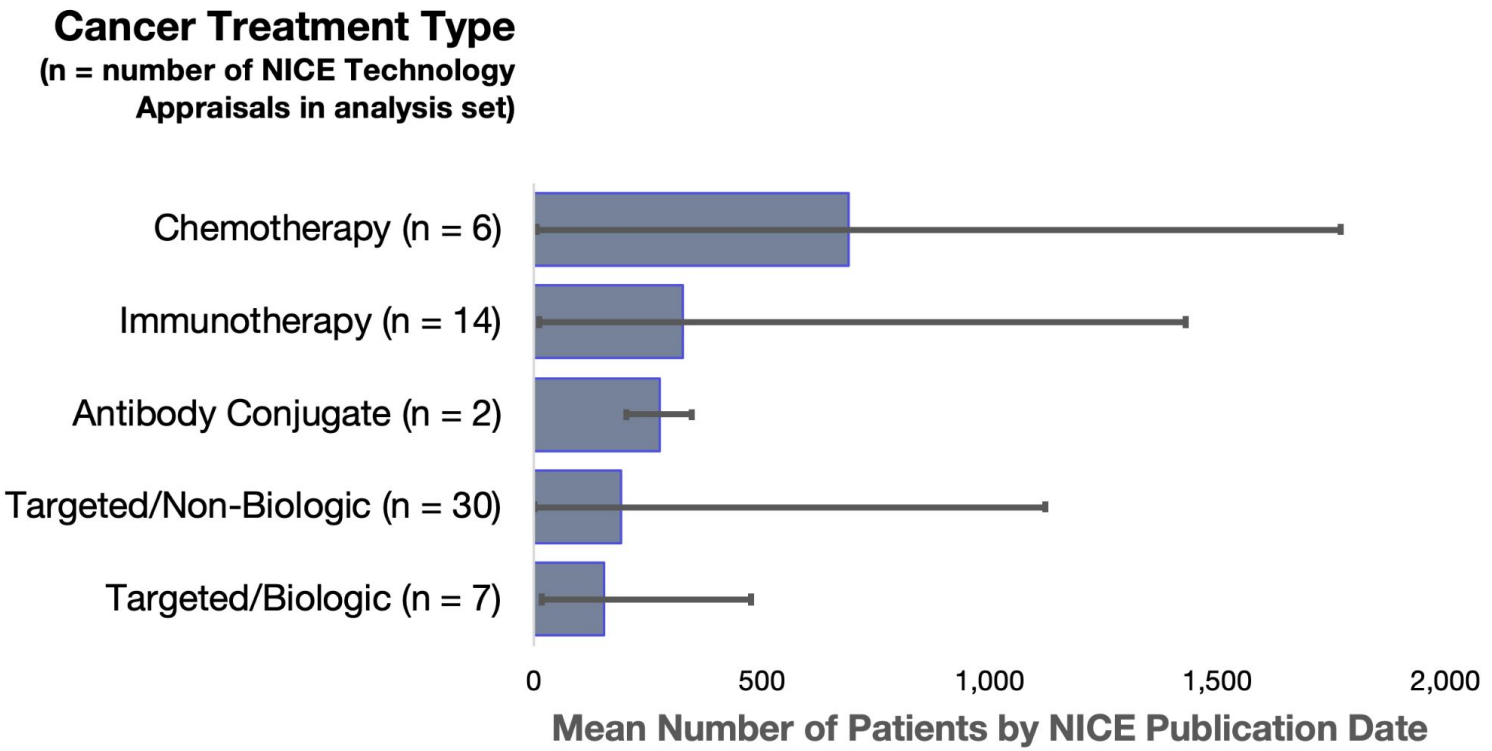
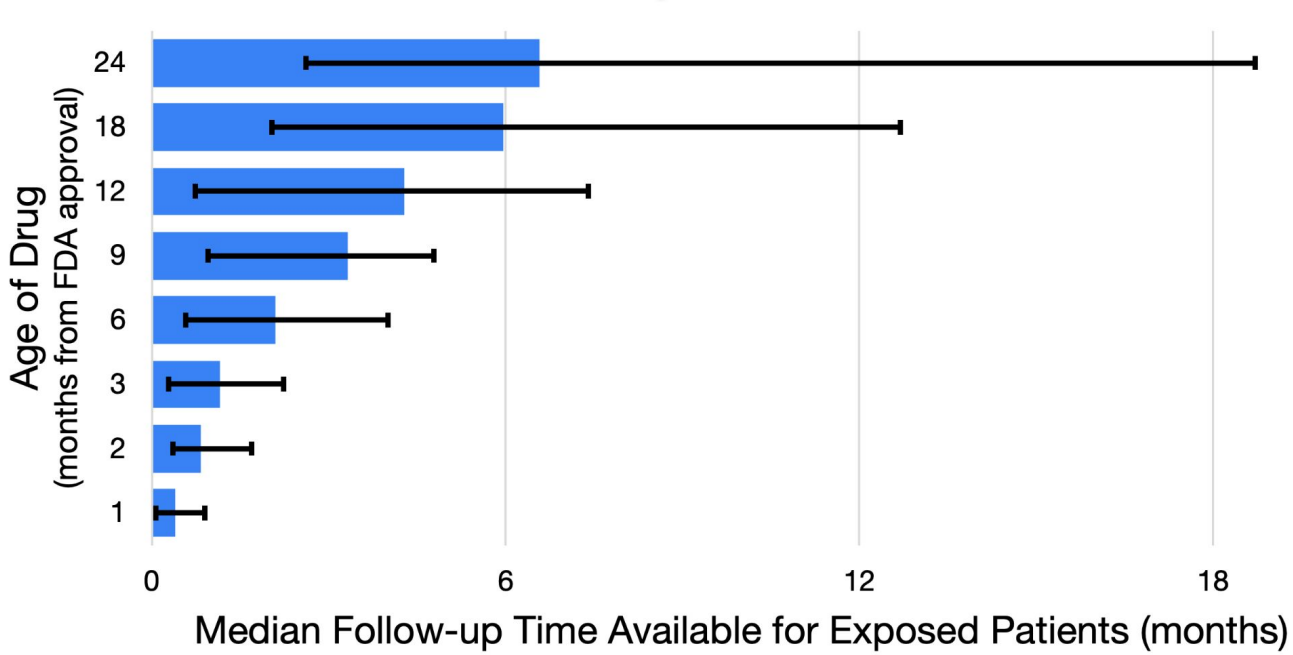


# Patient follow-up time available

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# Discussion

The time from FDA approval to NICE guidance may provide **an opportunity to inform reimbursement decisions with real-world US patients.**

**Time for real world evidence.** Median time from FDA to NICE submission and final guidance publication was **5.6 and 18.5 months**, respectively, for a set of **60 oncology assessments between 2014–2019.**

**Cancer Drug Fund data.** Use of products recommended for the Cancer Drug Fund contributed more US patients per month by the time of NICE appraisal publication than products recommended and not recommended by NICE, especially after the drug has been approved in the US for at least six months. **EHR-derived RWD could provide a meaningful contribution in this area to better understand treatment effectiveness.**

**Transportability of RWE. Opportunities to use international EHR-derived oncology data will vary by cancer type**, the nature of uncertainties identified—most frequently cited for cancer products are longer term measures such as overall survival, progression-free survival, as well as quality of life—and will depend on whether the data is reflective of UK patient characteristics, treatment settings, and clinical pathways.

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# Thank you

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