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Conditional Marketing Authorization:
Becoming Increasingly More Common but Increasingly Less Relevant?

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Richard Macaulay, Senior Vice President,

Global Pricing & Market Access, PRECISIONadvisors

Richard is responsible for the growth and development of consultative, analytic, and data services supporting life science products' commercial performance. Our team excels at understanding the ever-changing and complex healthcare market and helping clients solve issues including access strategy, stakeholder mapping, value communication, strategic pricing, and product positioning.

Conditional Marketing Authorization – Becoming Increasingly More Common but Increasingly Less Relevant?

Agenda

1	What is **Conditional Marketing Authorization** (CMA)?
2	Why is there an **increasing trend** of CMAs?
3	How do CMAs **translate to outcomes** at HTA?
4	What are the **key take-aways**?

The EMA introduced the CMA process in 2006 to provide fast-track approval for medicines addressing diseases with a high unmet need

Introduction to Conditional Marketing Authorisations (CMAs)

CMAs provide **fast-track approval** of a drug that fulfils an unmet medical need based on less comprehensive clinical data than normally required

Products must meet all the following criteria

The **benefit-risk balance** of the medicine is positive

The medicine fulfils an **unmet medical need** in treating, preventing or diagnosing **seriously debilitating or life-threatening diseases**

It is likely that the applicant will be able to provide **comprehensive data post-authorisation**

The benefit of the medicine's availability to patients is greater than the **risk inherent in the fact that additional data is still required**

CMAs are conditional on marketing authorization holders (MAHs) fulfilling specific data collection obligations within defined timelines

Next steps for products granted CMA

Approval

EMA's CHMP **grants conditional marketing authorisation** for a medicine, and publishes the conditions in the EPAR

Data Collection

The MAH must **fulfil specific obligations (SOs) within defined timelines**, for example, completing ongoing/new clinical studies or collecting additional data

Renewal

The CMA is **valid for one year** and can be renewed automatically

Conversion

The CMA can be **converted into a standard MA** once the MAH fulfils the obligations and the data confirms the benefit-risk balance remains positive

On average a conditional MA is converted into a standard MA **within 4 years***

*Source: 2006-2016 CMA 10-year review

Research question

How common are EMA CMAs - and does the expedited regulatory approval they provide, translate into favorable reimbursement?

This research examined what proportion of EMA marketing authorizations were conditional, and how these translated to NICE outcomes

Research methodology

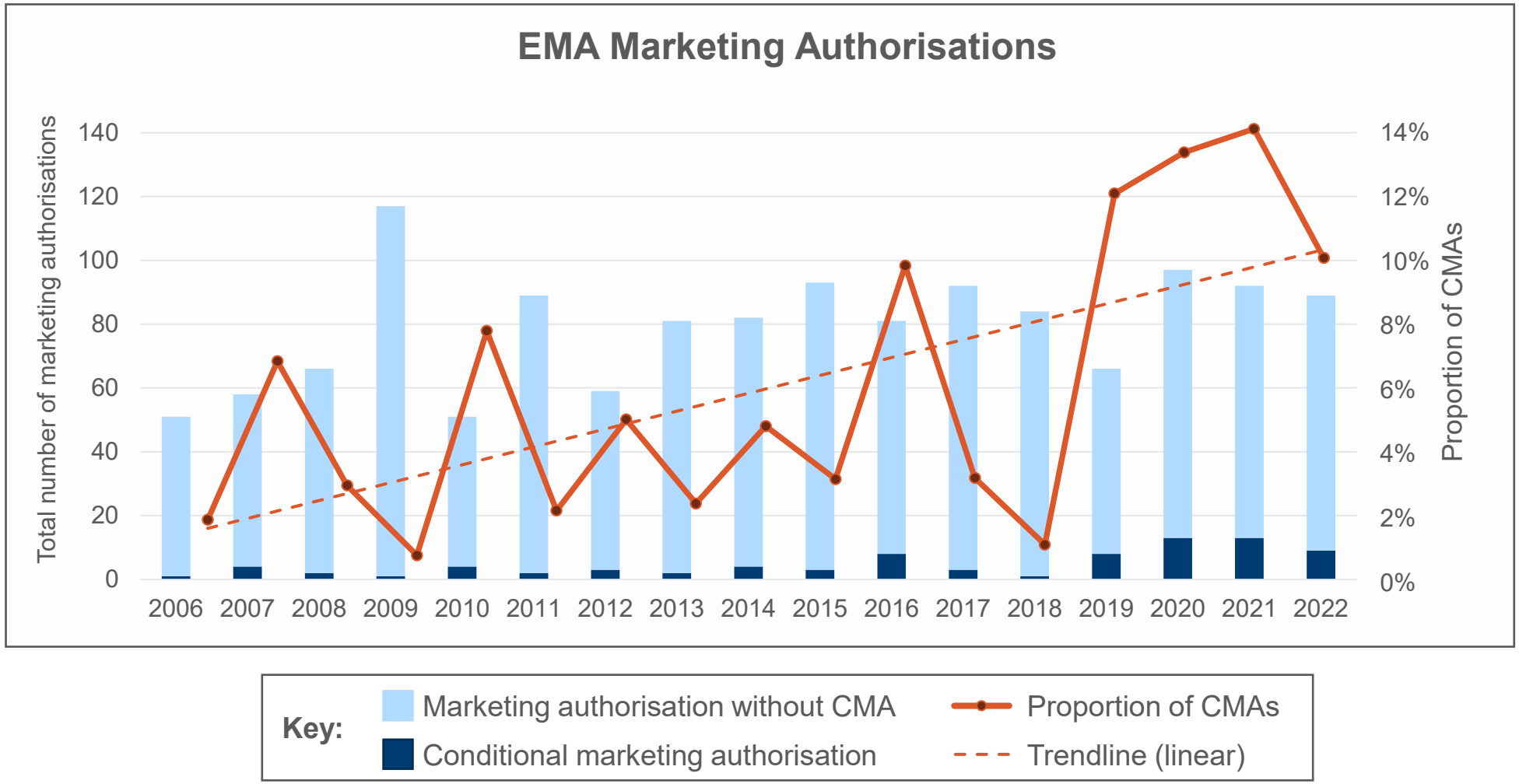
Identify publicly available CMAs (01-Jan-2006 to 31-Dec-2022)
(from the EMA annual reports)

Cross-reference with NICE **HTA Outcomes** (01-Jan-2018 to 29-Jun-2023)
(from the NICE website)

Key information extracted and analysed

EMA CMAs are becoming an increasingly common route to market for new medicines, representing over 10% of new EMA approvals each year since 2019

EMA conditional marketing authorisations



This represents a trend of therapies increasingly being approved by regulators at early stages of their clinical development, supported by immature trial data

2022 CMA medicines breakdown

In 2022, **9/89 (10%)** of EMA-approved therapies were approved under the **Conditional Marketing Authorisation** pathway, and the majority of these did not have comparative Ph. III data

Drug	Indication	Oncology?	Comparative Phase 3 data?
Carvykti	Multiple Myeloma	Y	N
Hemgenix	Haemophilia B	N	N
Kinpeygo	Glomerulonephritis	N	Y
Lunsumio	Follicular Lymphoma	Y	N
Paxlovid	COVID-19	N	Y
Roctavian	Haemophilia A	N	N
Spevigo	GPP	N	N
Tecvayli	Multiple Myeloma	Y	N
Zynlonta	DLBCL	Y	N

4/9 are oncology assets
(others include gene therapies & a COVID-19 treatment)

7/9 had no comparative phase 3 data

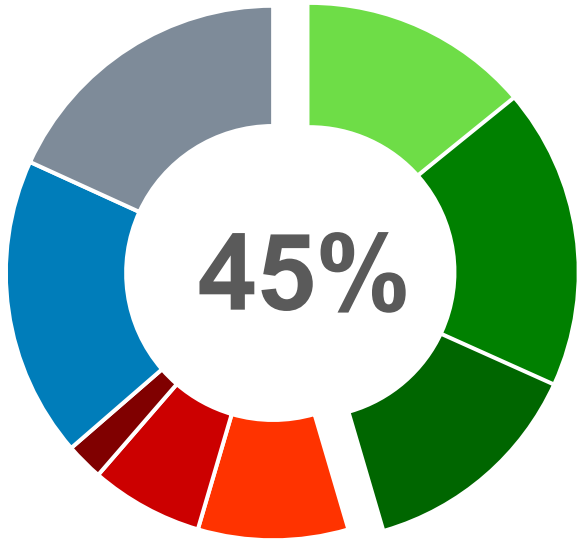
How has the increased regulatory acceptability of these products translated to HTA?

NICE Outcomes of CMA products since 2018

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

?

NICE National Institute for Health and Care Excellence



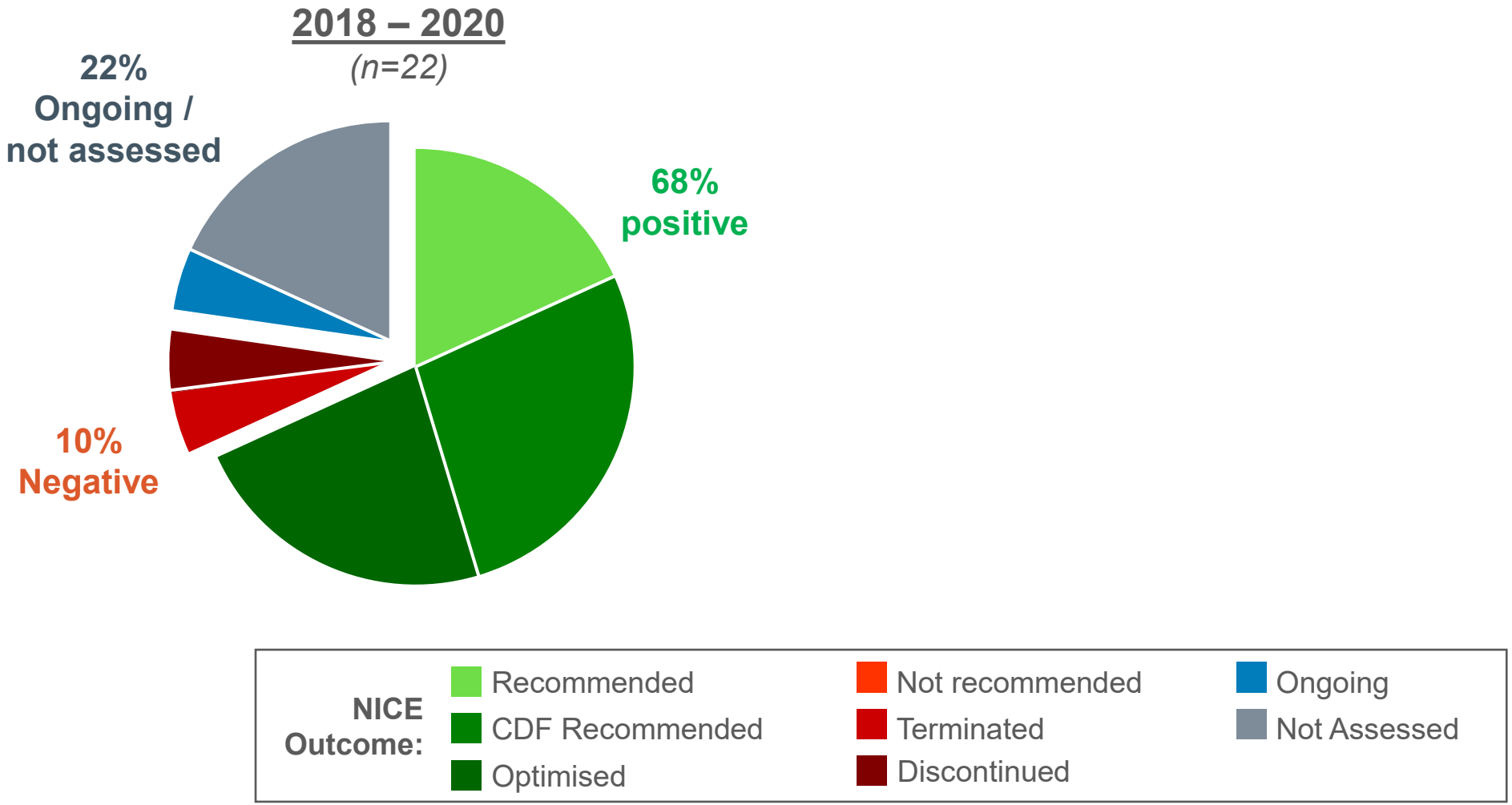
of CMAs since 2018 have translated to positive NICE outcomes

NICE Outcome:

- Recommended
- CDF Recommended
- Optimised
- Not recommended
- Terminated
- Discontinued
- Ongoing
- Not Assessed

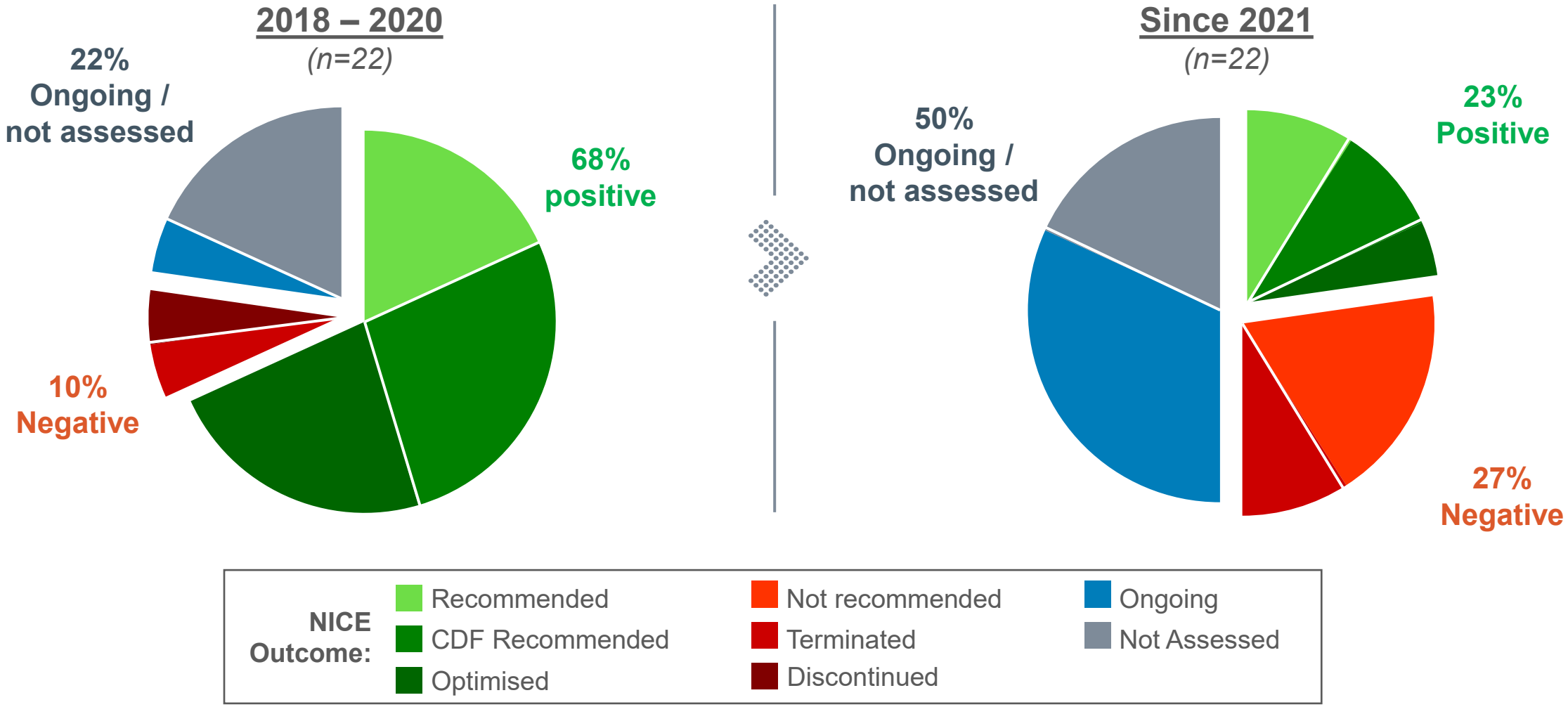
Whereas most medicines approved under an EMA CMA were favorably recommended by NICE prior to 2021…

NICE Outcomes of products approved under CMA pre-2021



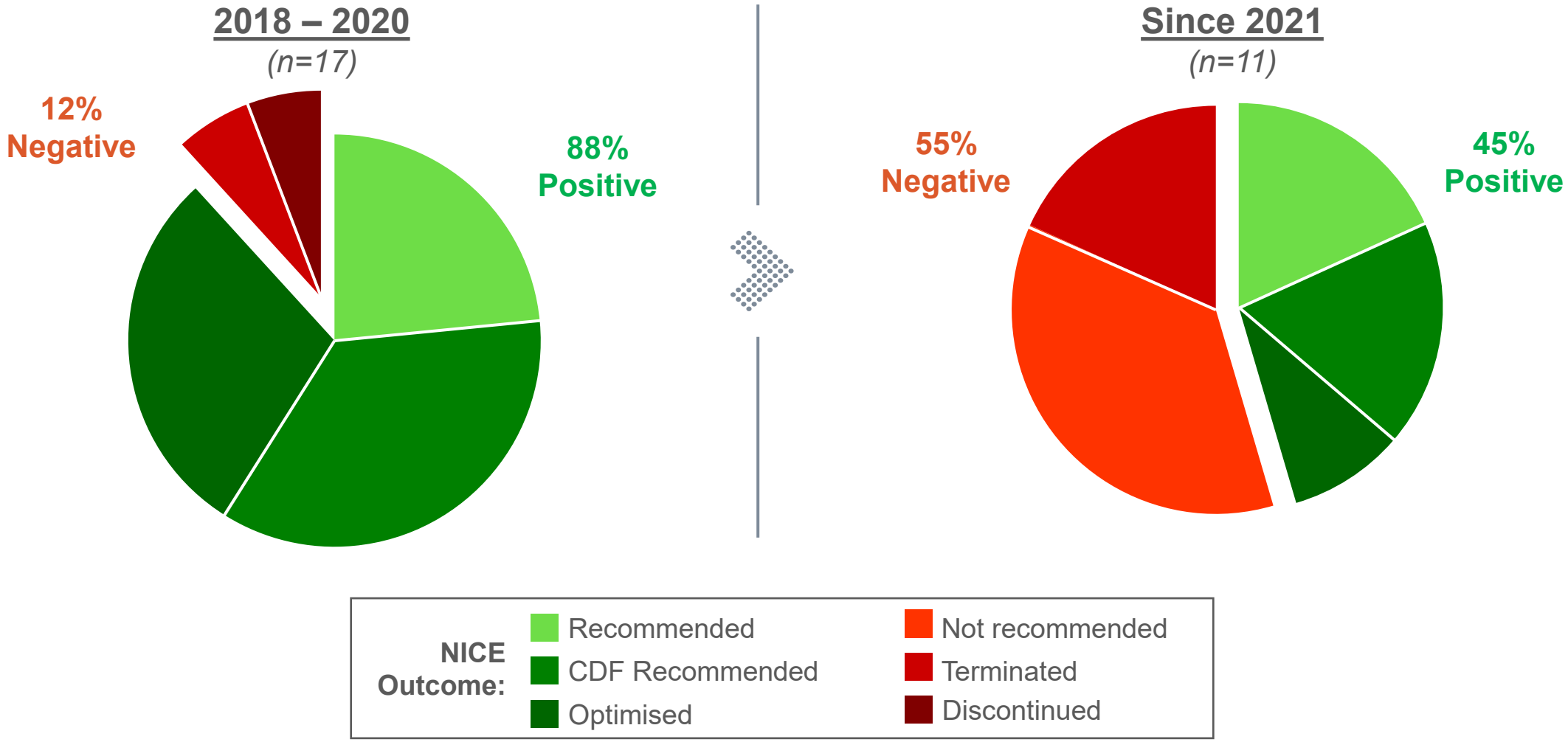
… since 2021, only a minority of medicines approved under an EMA CMA have received a favorable NICE recommendation

NICE Outcomes of products approved under CMA pre-2021 vs. post-2021



This trend is maintained if you remove 'ongoing assessments' or 'not assessed' from the analysis

NICE Outcomes of products approved under CMA pre-2021 vs. post-2021 (completed appraisals only)



CMAs allow for regulatory approval at earlier stages, but HTA evidence demands remain high, generating widening evidentiary divergence between the two bodies

Summary and conclusions

CMAs are becoming increasingly common, with regulatory approval possible at early stages of their clinical development, supported by immature data often without comparative phase III trials

However, these CMAs are **not necessarily translating to positive reimbursement outcomes** in the UK, with an **increasingly negative trend over time**

This is despite the UK having **dynamic reimbursement pathways** that allow temporary reimbursement whilst additional data is collected (Cancer Drugs Fund & Innovative Medicines Fund)

Further research is needed to elucidate if this trend is mirrored across other major European markets

THANK YOU!

we look forward to staying in touch

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