



## Expanded Access: Access to Unlicensed Drugs – A Potential for Evidence Generation?

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May 2023



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# mytomorrows

Enabling earlier and better access to all  
possible pre-approval options.



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# Today's agenda

- 1 What if a patient is not eligible for a trial?
- 2 What is expanded access?
- 3 Can we get Real-World insights from expanded access?
- 4 A practical example
- 5 The value of evidence generation

**What if a patient is not  
eligible for a trial?**

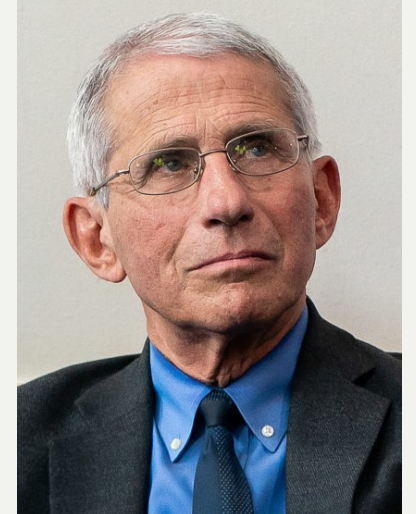






# Primary anger directed at the director of NIAID.

National Institute of Allergy and Infectious Diseases – A young dr. Anthony Fauci



In 1987, the FDA **formalized** expanded access  
amidst the AIDS epidemic  
to provide access to unlicensed drugs for  
**terminally ill** patients outside trials.

# What is expanded access?



# Expanded Access

A legal, regulatory pathway to access unlicensed drugs outside trials

- For a specific subset of patients.
  1. Patient who do not have registered treatment options
  2. Patient who cannot enter trials
  3. Patient who are terminally ill / seriously debilitated
- Approval by various stakeholders required.
  1. Pharmaceutical company
  2. Regulatory and health authority bodies
  3. The patient and physician

# Confusing Terminology

## Expanded Access

## Early Access

## Compassionate Use

## Managed Access

## Special Access

## Single-Patient (Access/IND)

## Pre-Approval Access



# The Dutch system

Two different pathways to deliver ‘geneesmiddelen zonder handelsvergunning’

## Leveren op artsverklaring (NPP)

- Physician-driven
  - Single patient
- Both pre- and post-approval



Inspectie Gezondheidszorg en Jeugd  
Ministerie van Volksgezondheid,  
Welzijn en Sport

## Compassionate Use Programma's (CUP)

- Sponsor-driven
  - Cohort of patients
- Marketing authorization close by

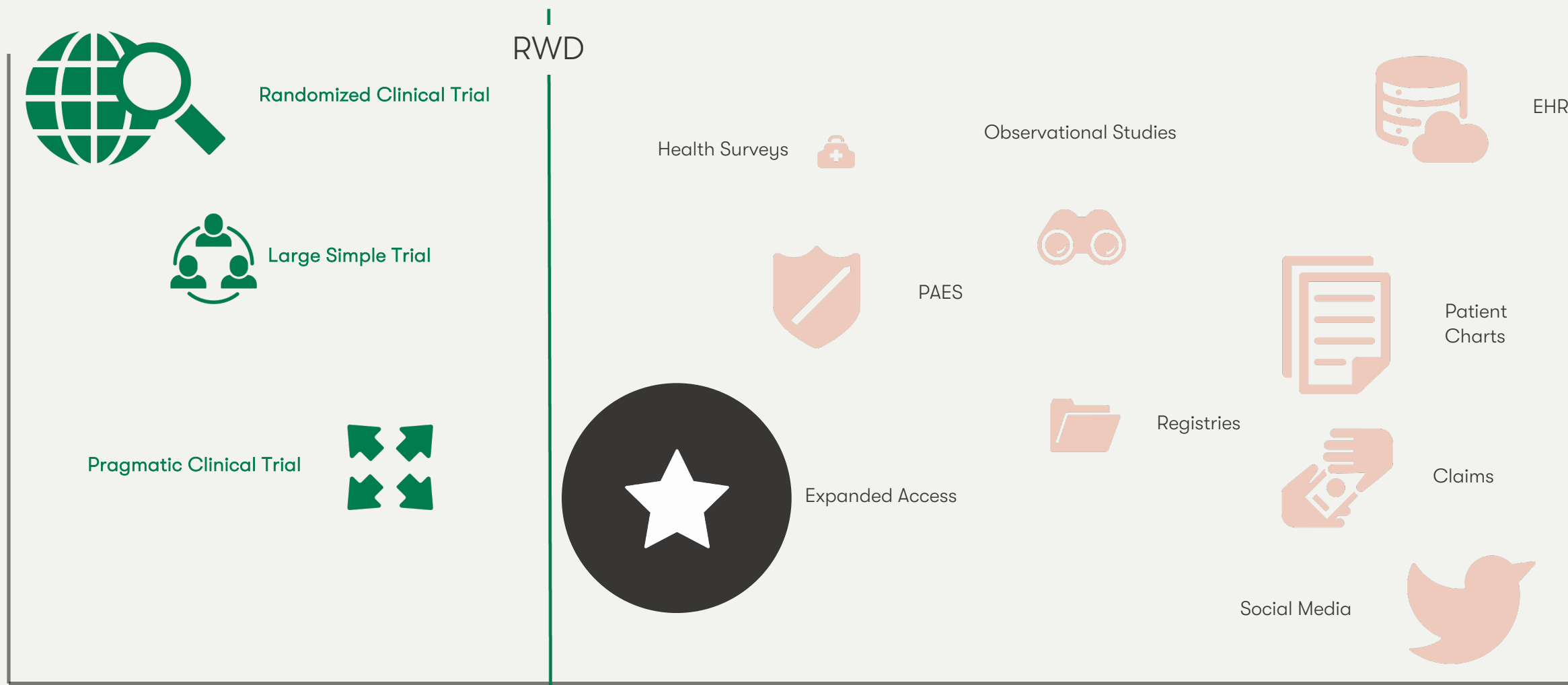


Can we get Real-World  
insights from expanded  
access programs?



# The place of EAP in the RWD landscape

Expanded Access is one small slice of the entire RWD pie.



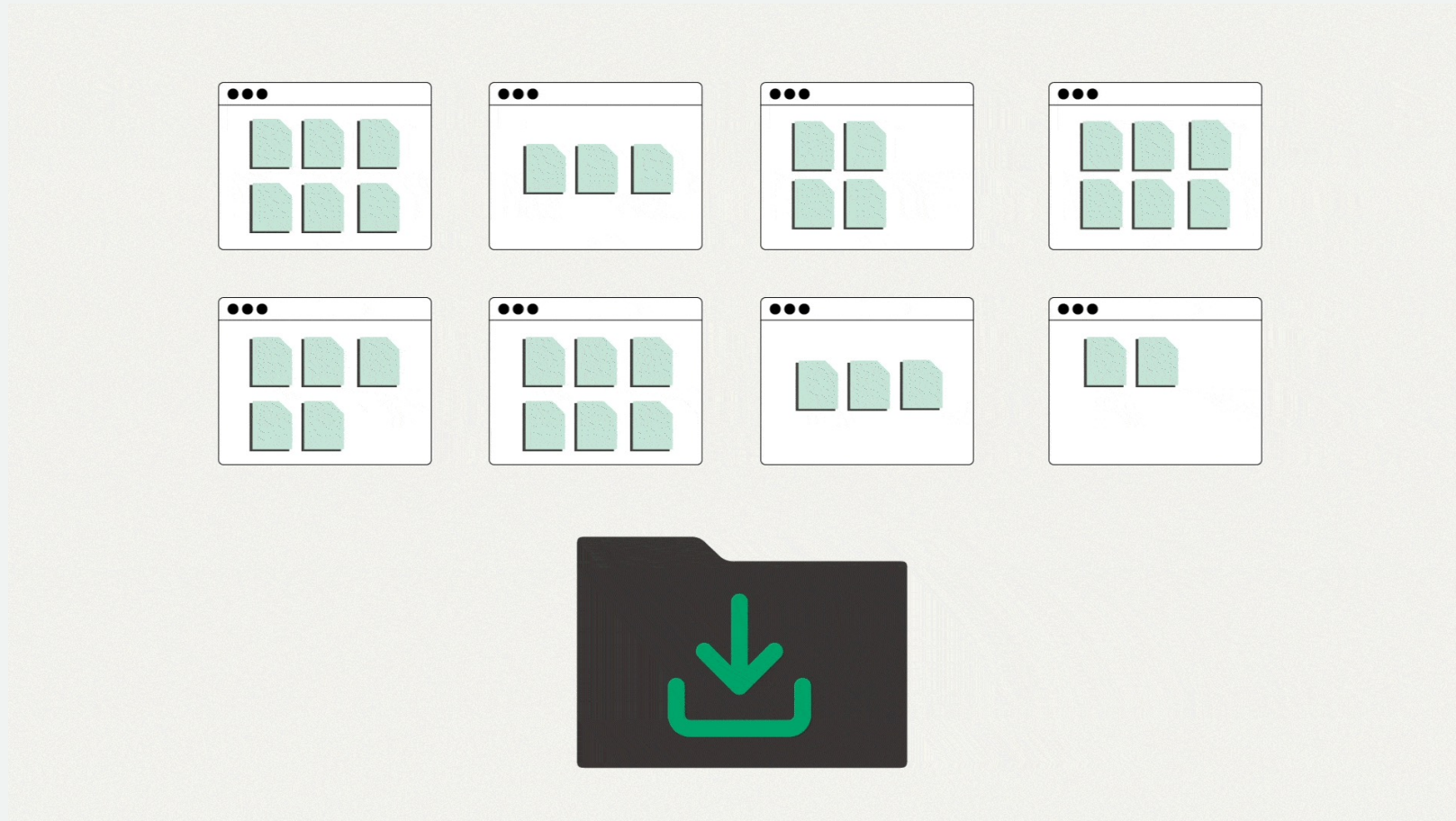
We investigated whether **authorities** used Real World Data from expanded access programs



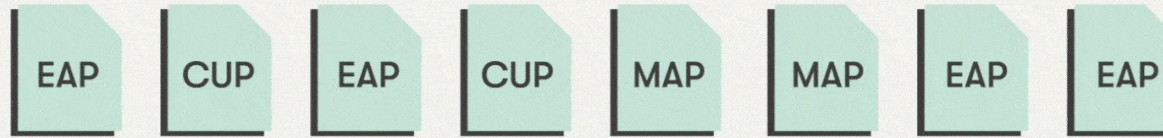
**NICE** National Institute for  
Health and Care Excellence

**ICER**   
INSTITUTE FOR CLINICAL  
AND ECONOMIC REVIEW

We **downloaded** publications, regulatory & reimbursement documents and **searched** for expanded access terms



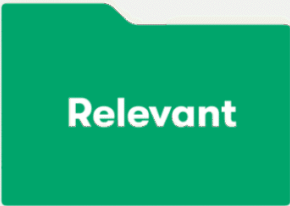
We **categorised** the documents that included an expanded access term





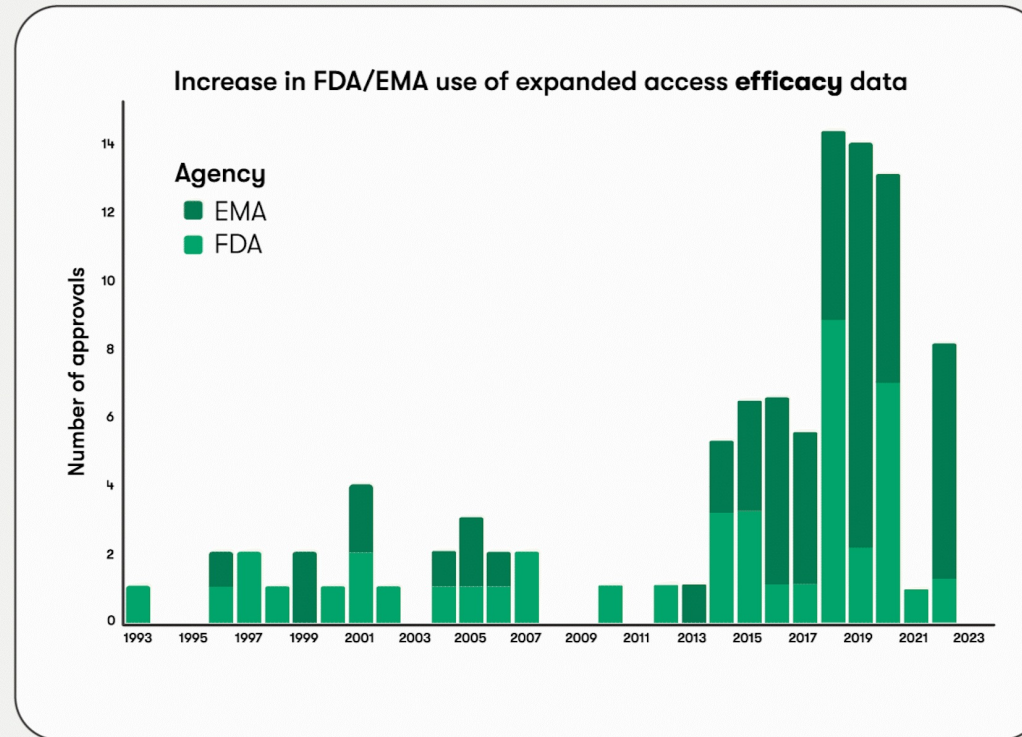
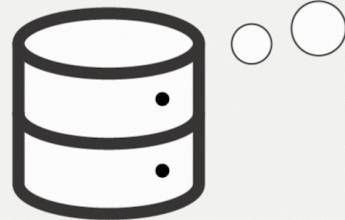
# The results of our research

We **saved** all data in one big database...

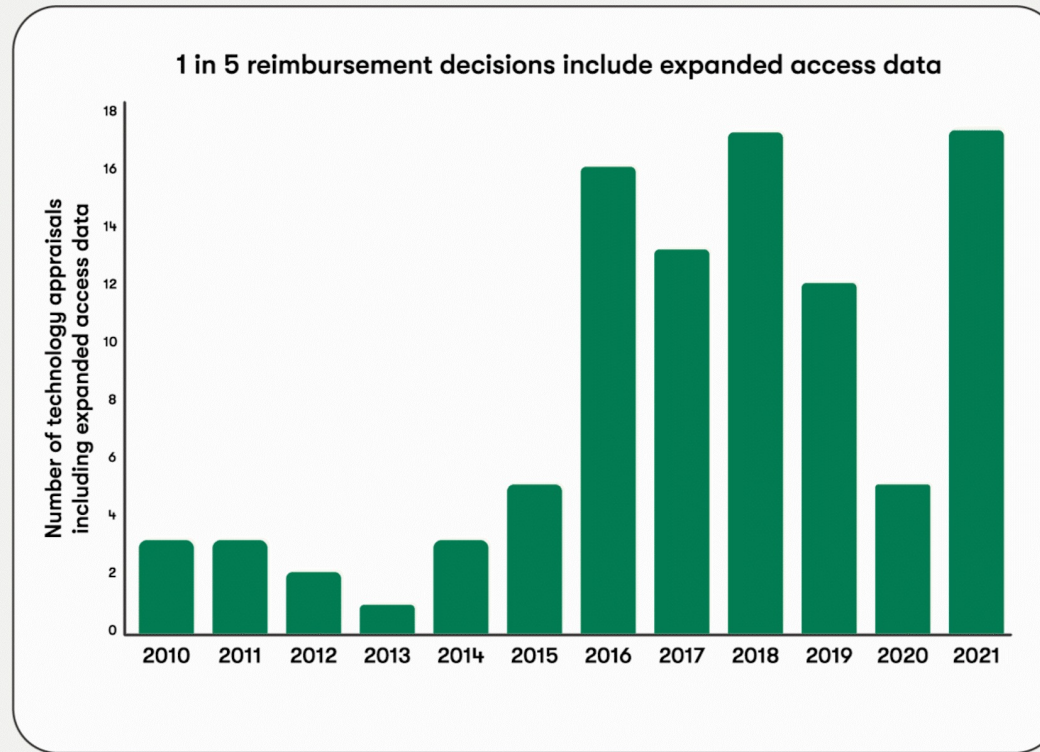


Drug name	Indication	Therapeutic Area	N	Patient Population	Study Design	Study Type	Sponsor	Country

... and saw an **increasing trend** over time in the use of data from expanded access programs

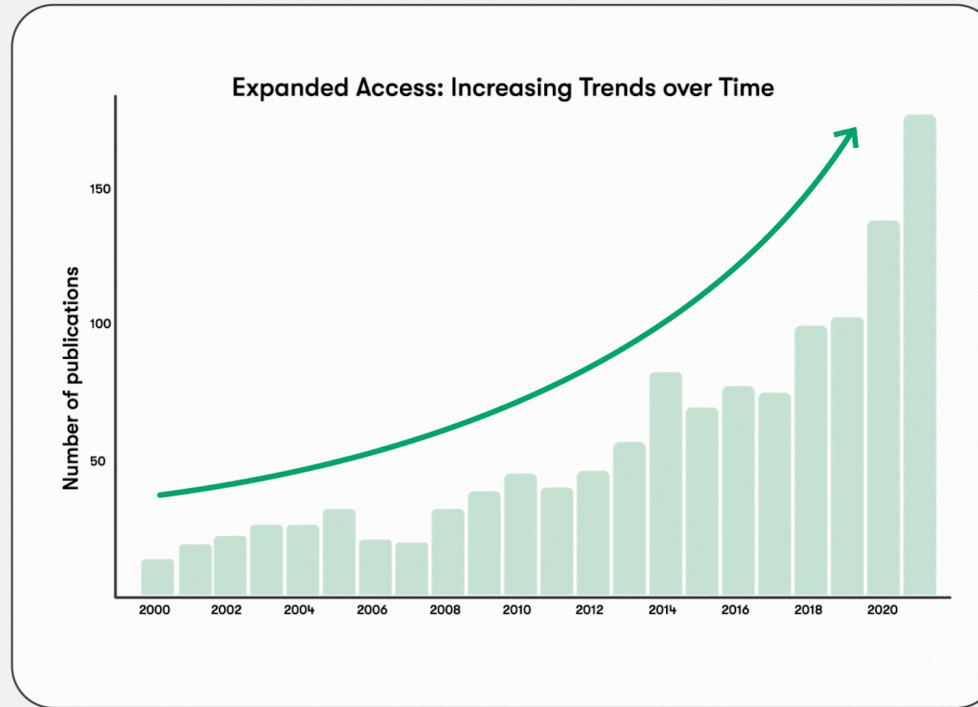
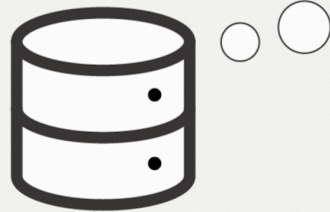


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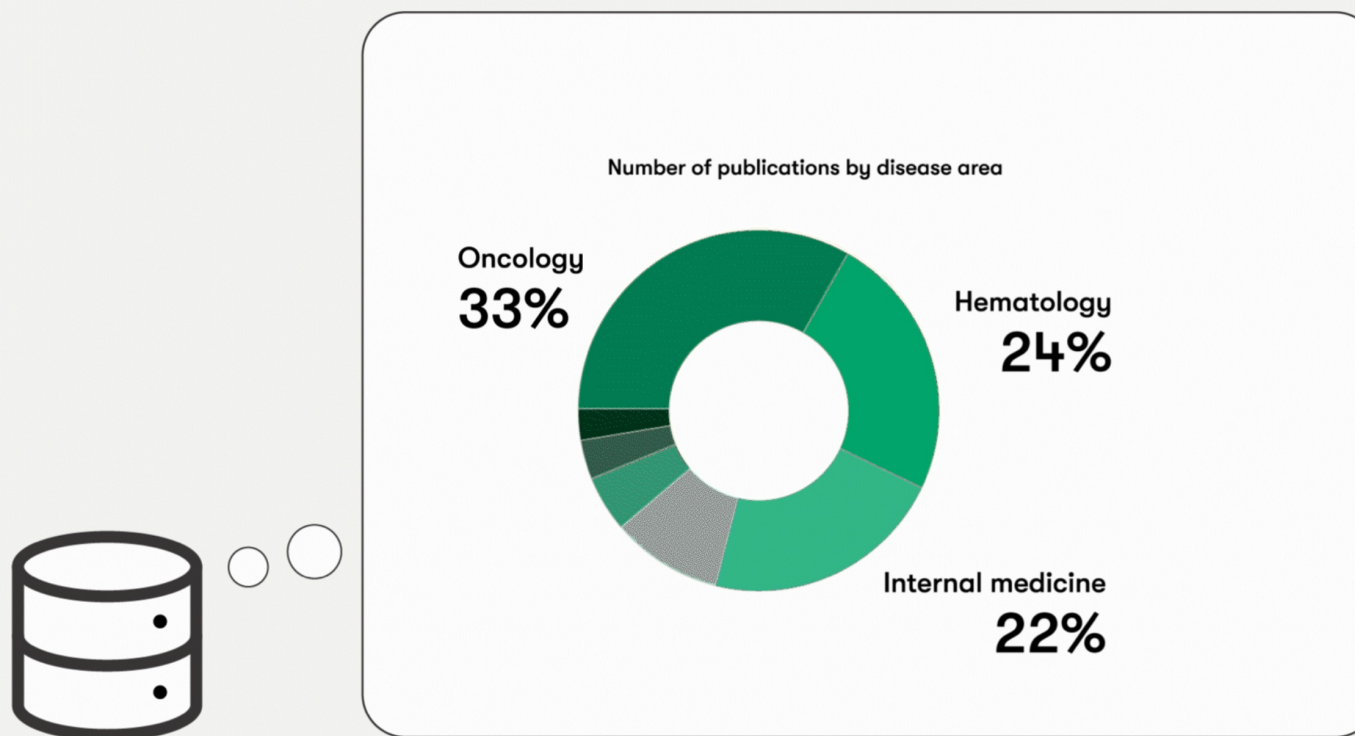




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# Expanded Access publications across disease area's and geography



# The value of evidence generation

# The value of collecting RWD within Expanded Access

RWD within Expanded access:

- may be used as **addition** to the treatment group of clinical trials.
- may provide **support** in the **approval** and **commercial** launch process of investigational drugs
- may potentially contribute to a quicker and more efficient **development** of drugs





# Questions and discussion

# Thank you!

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