Disclosures

I and my spouse/partner have no relevant relationships with commercial interests to disclose.

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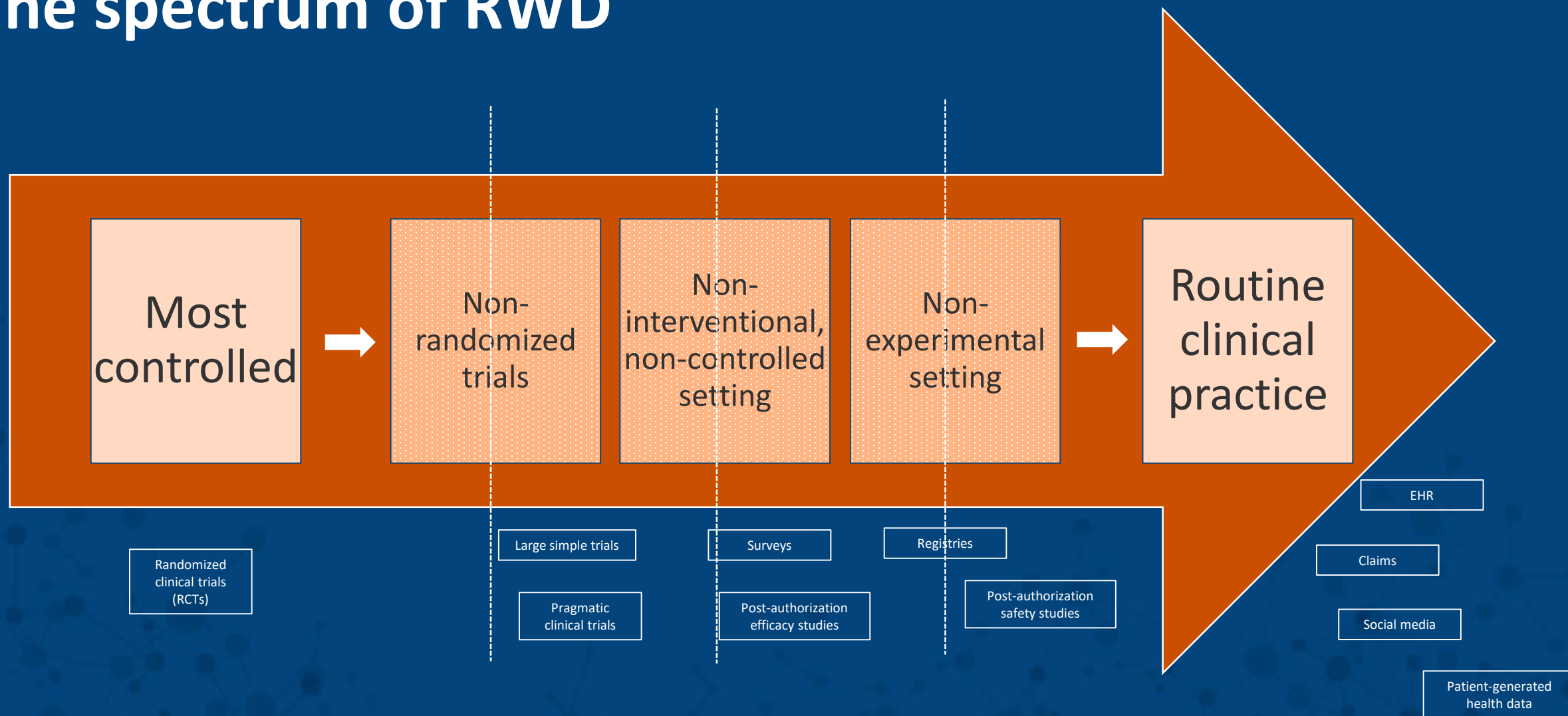# What is real-world data/evidence (RWD/RWE)?

- Often defined in the negative ("data that is not part of a clinical trial")
- FDA definitions[1]:

  <u>RWD</u> = *data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources*

  <u>RWE</u> = *the clinical evidence about the usage and potential benefits or risks of a medical product derived from analysis of RWD*

[1]US Food and Drug Administration, Framework for FDA's Real-World Evidence Program (2018). https://www.fda.gov/media/120060/download

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The spectrum of RWD

| Most controlled | Non-randomized trials | Non-interventional, non-controlled setting | Non-experimental setting | Routine clinical practice |

Randomized clinical trials (RCTs)

Large simple trials

Pragmatic clinical trials

Surveys

Post-authorization efficacy studies

Registries

Post-authorization safety studies

EHR

Claims

Social media

Patient-generated health data

Adapted from Figure 2 in Makady A, de Boer A, et al. What Is real-world data? A review of definitions based on literature and stakeholder interviews. Value Health 2017; 20:858-865.

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Real world data – complementary to RCTs?

Advantages

- Strong external validity
- Captures outcomes of patients in usual practice setting
- Captures uncommon events
- Responsive to changes in practice
- Reflects "what does work" (effectiveness)

Disadvantages

- Data missingness (may be not at-random)
- Data elements often not standardized
- Heterogeneous population may mask treatment effect
- No blinding, control groups
- Outcome assessment subject to multiple sources of bias

Attrition bias

Detection bias

Performance bias

Selection bias

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Limitations of EHR data in studying the older adult population

- Limited to what is available in the source systems
  - Geriatric assessments (GA), when performed, often not captured in structured data
- Elements of GA domains may be sparsely represented:
  - Functional status (ADL/IADL) ✗
  - Falls ✗
  - Cognitive function ✗
  - Comorbidities 🟥 *as ICD9/10 codes – missingness issue*
  - Nutritional status (> BMI, wt Δ) ✗
  - Psychological state ✗
  - Social support ✓ *marital status alone*
- Outcomes of importance to older adults (e.g., impact of Rx on function or cognition) can only be obtained through curation or PROs

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CancerLinQ® database description

By Anatomic Site[1]

Categories (left to right): Breast, Digestive, Melanoma/Skin, Respiratory, Male Genital, Female Genital, Lymphoma, Urinary, Leukemia, Unspecified, Head and Neck, Ill-Defined, Thyroid/Endocrine, Multiple Myeloma, Bone and Connective…, Brain/CNS, Neuroendocrine, Other Specified Type, Ocular, Mesothelial/Soft Tissue
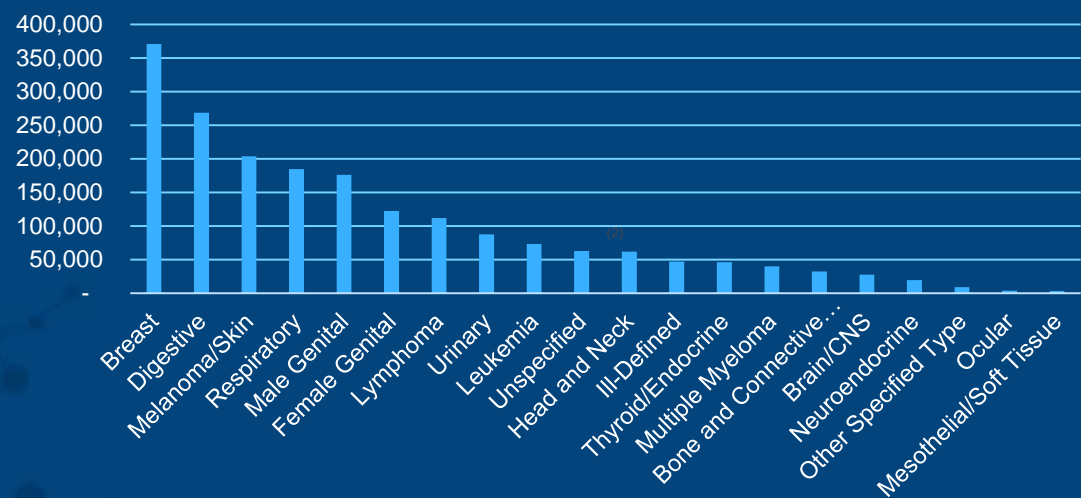
By Age at Initial Cancer Diagnosis[2]

By Geography

© GeoNames, Microsoft, Navinfo, TomTom, Wikipedia
Powered by Bing
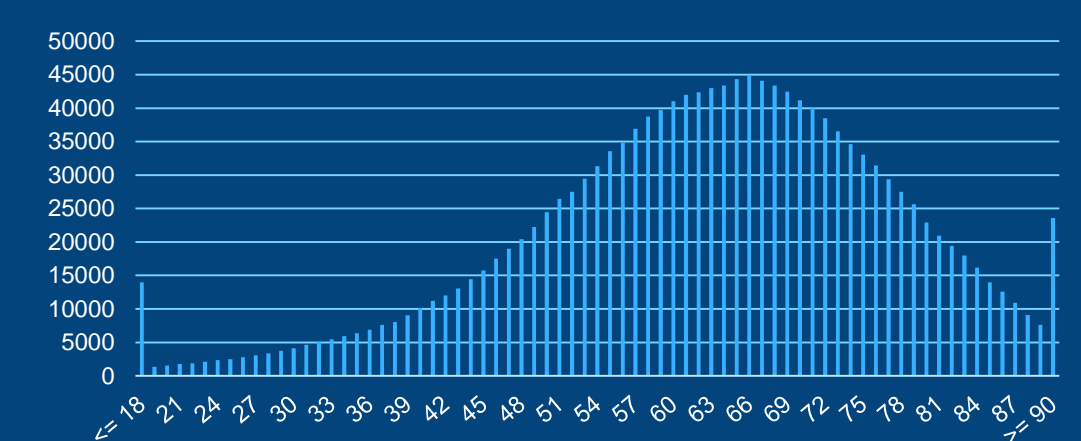
By Data Element

Data Category	Data Available per Patient	
	Median	Mean
Lab tests	312	745
Physical exam results	154	344
Drug orders	42	148
Drug administrations	90	416
Encounters	28	284

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Note: As of June 22, 2020
(1) Reflects cancer diagnosis data
(2) Age 0-18 and 90 and above aggregated

Case study: Analysis of PARAMOUNT trial results using CancerLinQ Discovery® data

PARAMOUNT: Final Overall Survival Results of the Phase III Study of Maintenance Pemetrexed Versus Placebo Immediately After Induction Treatment With Pemetrexed Plus Cisplatin for Advanced Nonsquamous Non–Small-Cell Lung Cancer

Luis G. Paz-Ares, Filippo de Marinis, Mircea Dediu, Michael Thomas, Jean-Louis Pujol, Paolo Bidoli, Olivier Molinier, Tarini Prasad Sahoo, Eckart Laack, Martin Reck, Jesús Corral, Symantha Melemed, William John, Nadia Chouaki, Annamaria H. Zimmermann, Carla Visseren-Grul, and Cesare Gridelli

Summary of PARAMOUNT:
- 939 pts w/ Stage IIIB-IV non-squamous NSCLC; 700 pts w/ CR/PR/SD post induction Rx
- 539 pts w/ 2:1 randomization to maintenance pemetrexed vs placebo
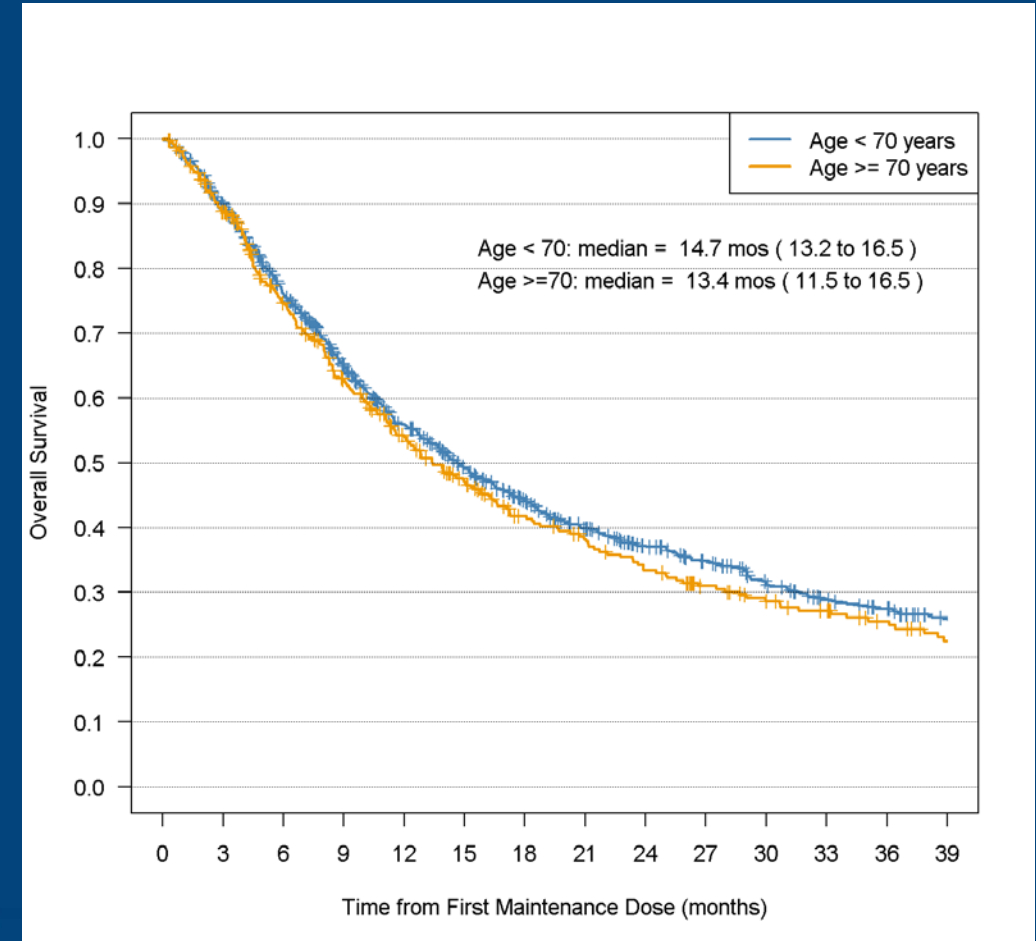- Median OS 13.9 mos PEM vs. 11.0 mos PLAC, HR=0.78 (p=0.0195)

Paz-Ares LG, de Marinis F, et al. PARAMOUNT: Final overall survival results of the phase III study of maintenance pemetrexed versus placebo immediately after induction treatment with pemetrexed plus cisplatin for advanced nonsquamous non-small-cell lung cancer. J Clin Oncol 2013; 31:2895-902.

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CancerLinQ® PARAMOUNT case study: Results

	PARAMOUNT (N=359)	CancerLinQ (N=1,285)
Gender		
Male	201 (56%)	657 (51%)
Female	158 (44%)	626 (49%)
Unknown		2 (<1%)
Age Group		
<65 years	238 (66%)	680 (53%)
≥65 years	121 (34%)	605 (47%)
Race		
Asian	16 (4%)	21 (2%)
Black	4 (1%)	162 (13%)
White	339 (94%)	952 (74%)
Other/Unknown Race		150 (12%)
Immunotherapy		
≥1 cycle		265 (21%)
0 cycles		1,020 (79%)

Age < 70 years
Age >= 70 years

Age < 70: median = 14.7 mos (13.2 to 16.5)
Age >=70: median = 13.4 mos (11.5 to 16.5)

Overall Survival vs. Time from First Maintenance Dose (months)

Using RWD to benefit the treatment of older adults with cancer: Strategies

1. **Goal = improving data quality**

   - Improved adoption of existing EHR data standards (e.g., mCODE®)

   - Documentation of relevant data elements for SDOH & geriatric assessment

   - Use of (PRO-) CTCAE in the EHR to document adverse events

2. **Goal = use existing RWD sources (CancerLinQ, registries, etc.), where they are well-suited, to measure:**

   - Metrics of disease burden in populations

   - Guideline adherence

   - Access to care and timeliness of care

   - Utilization of new agents in older adults

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