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Themes in the Design of Pharmaceutical Value-Based Contracts in the United States and Europe

Authors | Sruthi Adimadhyam[2], Andrew Weckstein[1], Amanda Patrick[1], Pattra Mattox[1], Ashley Jaksa[1]
Presenter | Andrew Weckstein
[1] Aetion, [2] Harvard Pilgrim Healthcare Institute

BREAKOUT SESSION 2
P3: REIMBURSEMENT & ACCESS POLICY RESEARCH
Monday, November 4, 2019
14:15 - 15:15

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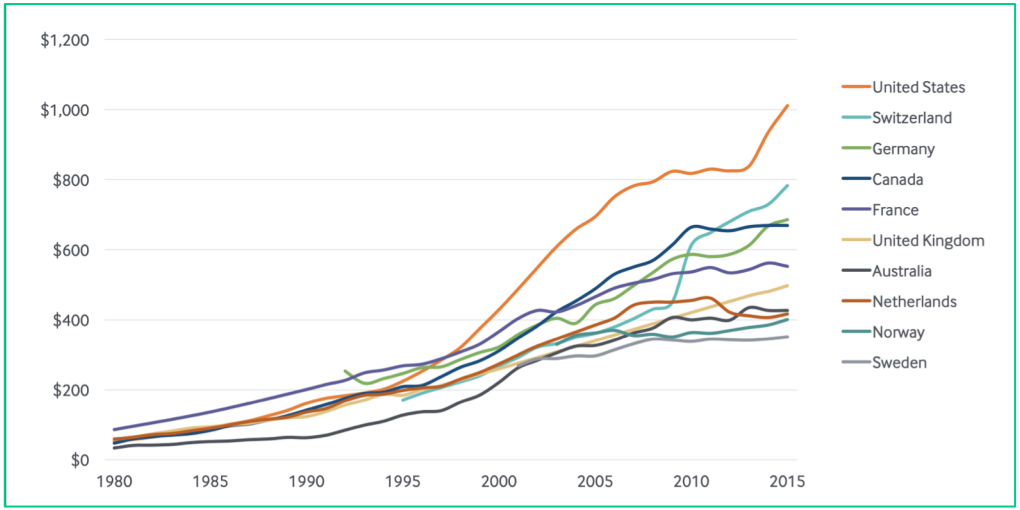
Disclosures

Andrew Weckstein, Pattra Mattox, Amanda Patrick, and Ashley Jaksa are employees of Aetion and have stock options in the company. Sruthi Adimadhyam was employed at Aetion during development of this research and is currently an employee at Harvard Pilgrim Healthcare Institute.

Escalating health care costs and drug prices: Are we approaching a tipping point for value-based care?

National Trends in Per Capita Pharmaceutical Spending, 1980–2015



Source: Commonwealth Fund, October 2017.

FDA approves Novartis' $2.1 million gene therapy — making it the world's most expensive drug

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By Michelle Cortez

January 3, 2018, 5:30 AM PST *Updated on January 3, 2018, 6:34 AM PST*

Outcomes-Based Contracts are an emerging cost containment strategy

- **Background:** Outcomes-Based Contracts (OBCs) link reimbursement and coverage decisions to "real-world" performance.
- **Advantages:** OBCs spread the risk of uncertainty *(Table 1)* to both payers and manufacturers, which preserves patient access to innovative treatments.
- **Known barrier:** Measurement and assessment of outcomes is one of the key components and challenges to OBC implementation.

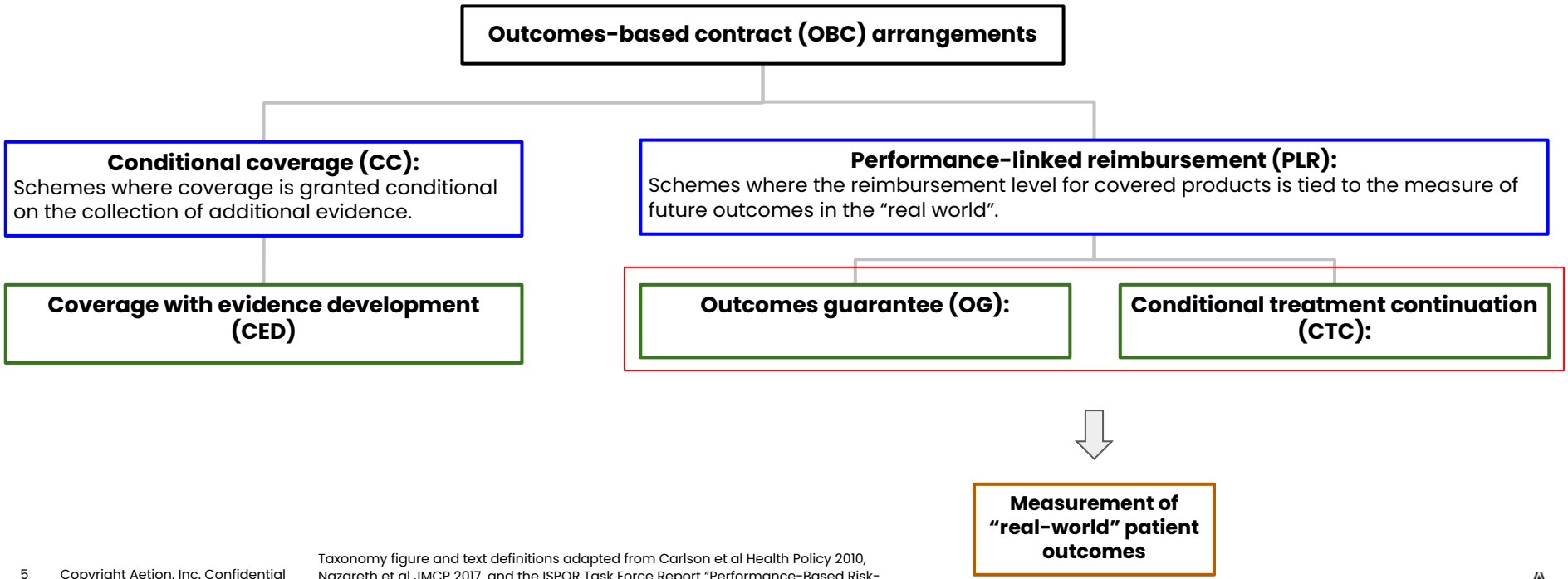
Objective: To evaluate themes in outcomes and contract structures in OBCs across the US and Europe.

Table 1. Areas of uncertainty addressed with an OBC
Will this product work in my patient population?
How does this treatment compare to the current standard of care in the "real-world"?
How does this treatment impact clinical or economic outcomes not studied in clinical trials or pre-licensing studies?
How will reimbursement of this product impact my budget?

Table 2. Known barriers to implementing OBCs
Estimating contract risk due to uncertainty of real-world impact
Sufficient data infrastructure to execute and track outcomes
Transparency and alignment in defining and measuring outcomes
Establishing causality between the biopharmaceutical intervention and target outcome(s)

Step 1: Extracted outcomes-based contracts and categorized by contract type

*Outcomes-based contracts in the United States and select European nations (Italy, UK, Netherlands, and Spain) were extracted from the **University of Washington PBRSA** database. Each agreement was categorized by **contract type** using a published taxonomy of risk sharing agreements.*

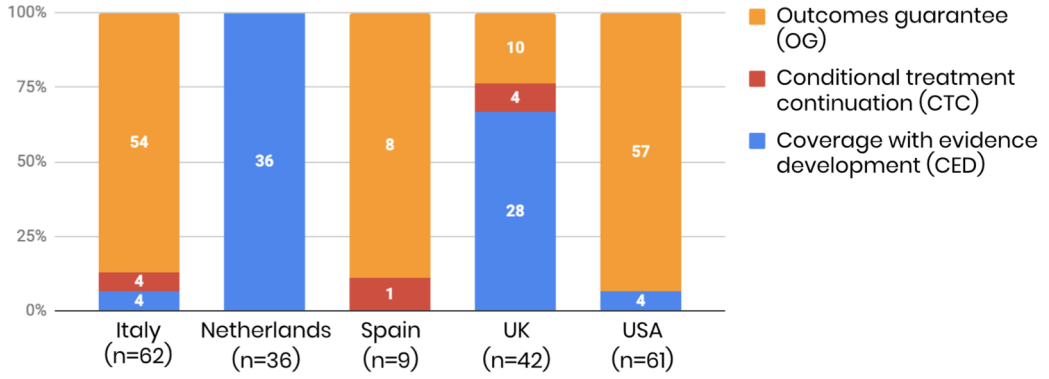


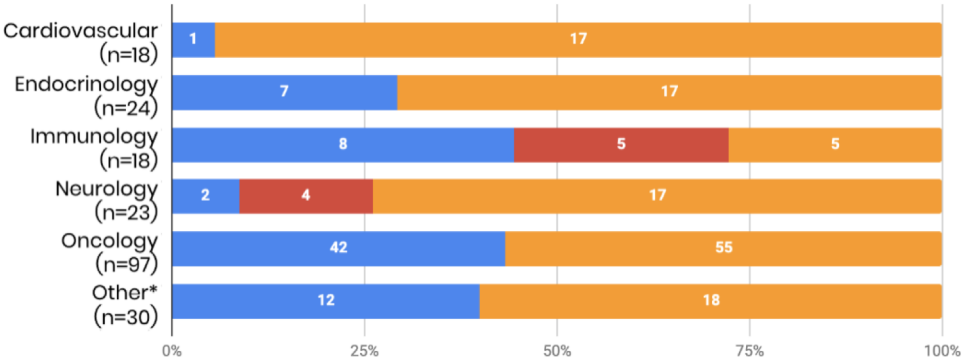
Taxonomy figure and text definitions adapted from Carlson et al Health Policy 2010, Nazareth et al JMCP 2017, and the ISPOR Task Force Report "Performance-Based Risk-Sharing Arrangements - Good Practices for Design, Implementation and Evaluation."

Step 2: Next, we categorized performance-linked reimbursement contracts on dimensions related to measurement of "real-world" outcomes.

01	What type of outcome?	• Clinical: surrogate or direct endpoint • Financial • Adherence/persistence • Mixed (multiple types)
02	Was reimbursement determined at the patient-level or aggregated at the population-level?	• Patient-level • Population-level (aggregated)
03	Was performance assessed on a comparative or absolute threshold-based scale?	• Comparative • Absolute-threshold based
04	Was the assessment restricted to a compliant population?	• Yes • No
05	What was the form of reimbursement?	• Manufacturer covers cost of adverse events • Other (rebate / refund / price adjustment)

Overall, contract type varied by country & therapeutic area





Identified 210 outcomes-based contracts from the UW database

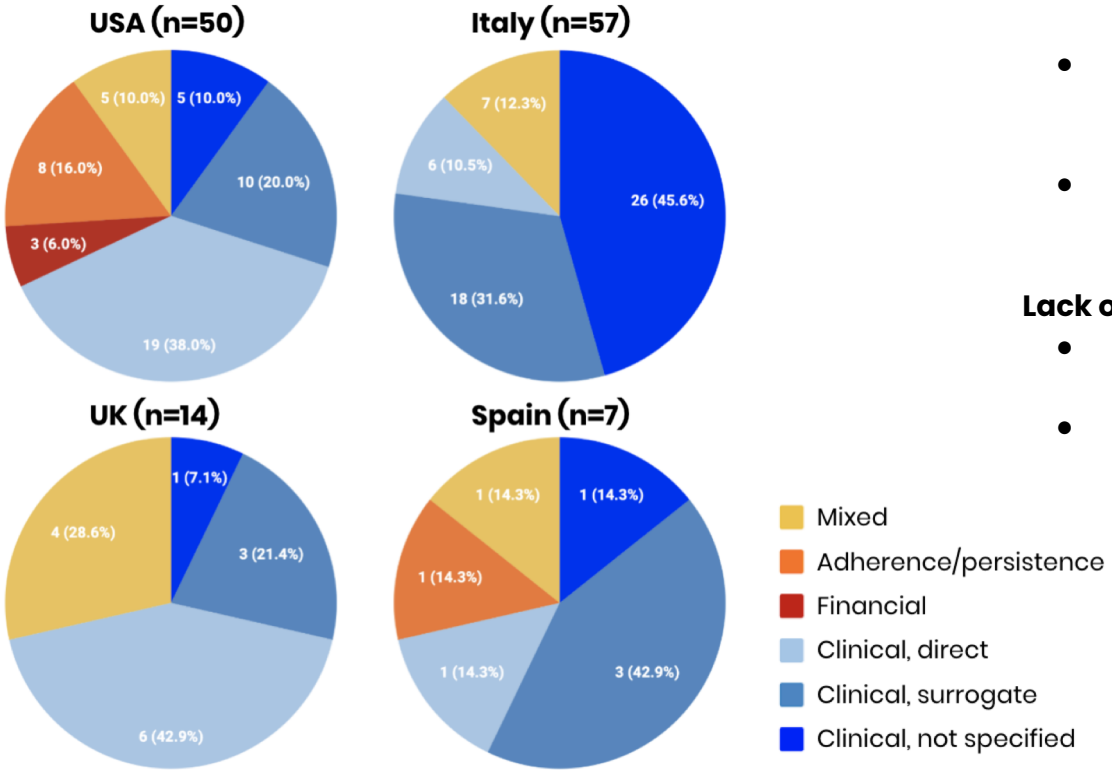
- OG: 129 (61%)
- CED: 72 (34%)
- CTC: 9 (4%)

Countries: OG schemes most common contract types in Italy, Spain, and the US

Therapeutic area: OG schemes most common contracts for Cardiovascular, Endocrinology, and Neurology products

**Other TAs include: Infectious Diseases, Ophthalmology, Respiratory Diseases, Nephrology/Urology, Musculoskeletal, Mental Health, Hematology, and Gastroenterology.*

Clinical outcomes were the most prevalent outcome types used in performance-linked reimbursement contracts



- Overall, 78% of US contracts and 99% of European contracts included >=1 **clinical outcome** component (including mixed contracts)
- **Financial and Adherence-related outcomes** were more common in the US

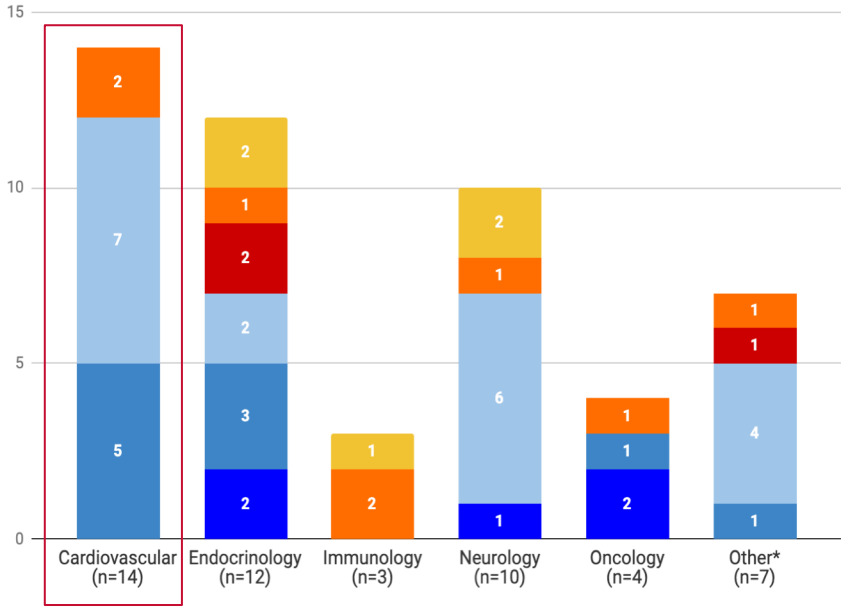
Lack of transparency in outcome definitions

- 20 (14.4%) of contracts were missing data on outcome type
- 33 (24%) mentioned clinical outcomes but could not be classified as surrogate or direct

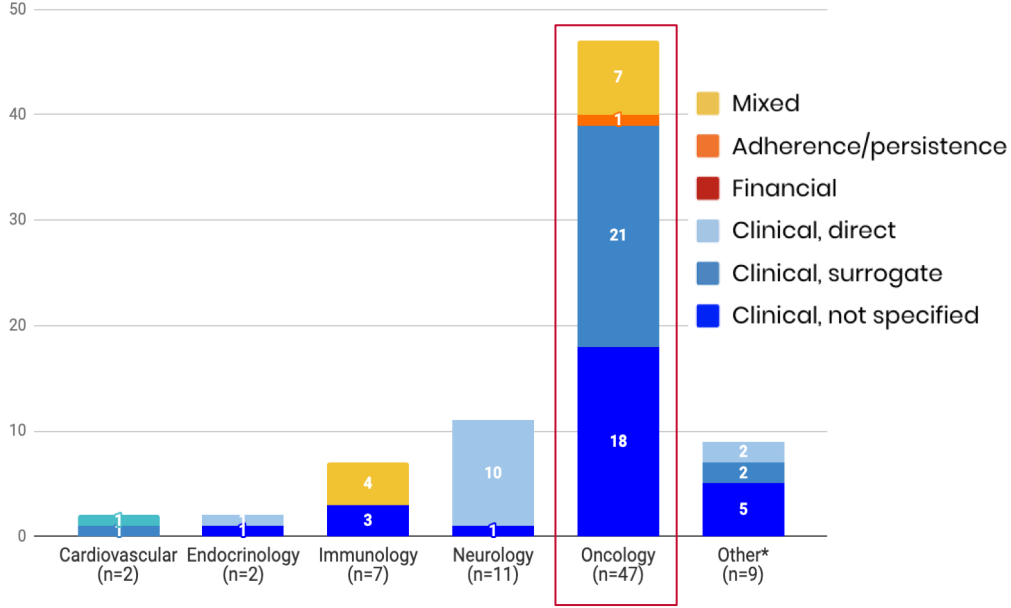
Analysis restricted to performance-linked reimbursement contracts (outcomes guarantee or conditional treatment continuation) with at least one ascertainable outcome type. Contracts with mixed outcome types had a combination of 2 or more of the following outcome types: Adherence/persistence, financial, direct clinical, surrogate clinical. See appendix for further details on outcome type definitions.

Differences in outcome types across regions is driven by therapeutic area

USA: Outcome Types, by Therapeutic Area



EU: Outcome Types, by Therapeutic Area



**Other TAs include: Infectious Diseases, Ophthalmology, Respiratory Diseases, Nephrology/Urology, Musculoskeletal, Mental Health, Hematology, and Gastroenterology.*
EU chart includes contract data from Italy, Spain, and the United Kingdom.

RWD fit-for-purpose: Outcomes reflect available data sources within each region

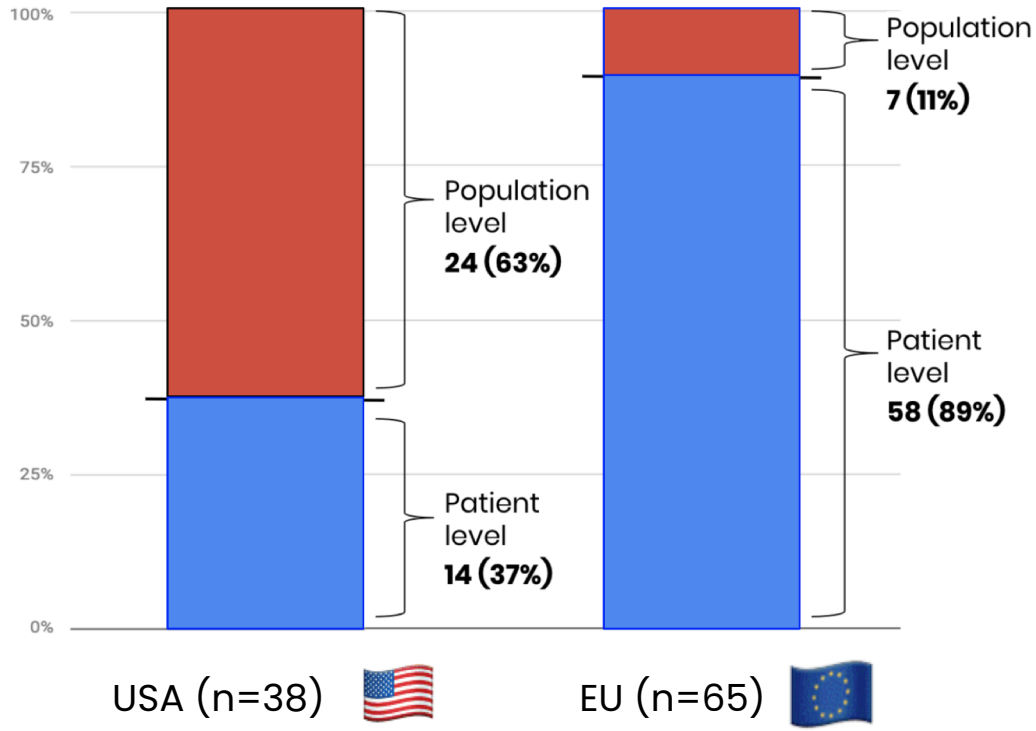
	USA	EU
Neurology	Hospitalization ER visits (relapse) Discontinuation Adherence Medical cost	Mini Mental Status Exam Expanded Disability Status Scale Mobility/motor function tests Numeric rating scale for spasticity (patient reported)
Immunology	Persistence Adherence	ACR (RA response criteria) EULAR (RA response score)
Oncology	Disease progression Discontinuation	Mortality Disease / tumor progression Biomarker lab tests (CD34 counts, Serum M, others) Discontinuation
Endocrinology	Fracture A1c Adherence Persistence Medical cost	Fracture
Cardiovascular disease	CV-related hospitalization Acute CV events (HF, MI) LDL Adherence	LDL Combined efficacy score, including: *Walking distance test, Time to clinical worsening, Dyspnea Borg Index, QoL patient questionnaires, & NT-Pro BNP lab test components.*

Population-level reimbursement contracts more common in the United States

2

Could be driven by:

- Frequent turnover in commercial insurance plans
- Administrative complexity



Population-level reimbursement

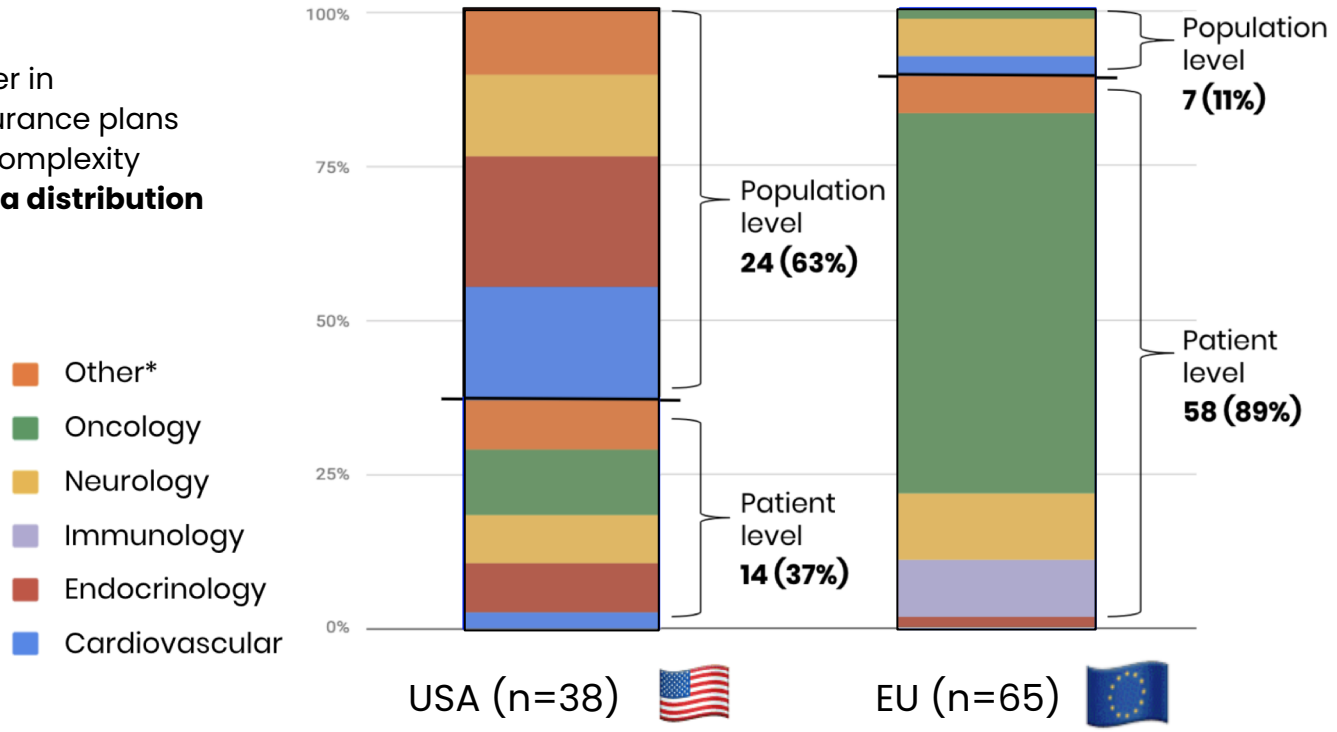
Patient-level reimbursement

*Other TAs include: Rheumatology, Respiratory Diseases, Musculoskeletal, Infectious Diseases, Immunology, Mental Health, Ophthalmology, Hematology. n and percent (%) are out of contracts categorized on this patient vs. population-level reimbursement dimension.

Level of reimbursement is linked to disease-specific factors

Could be driven by:

- Frequent turnover in commercial insurance plans
- Administrative complexity
- **Therapeutic area distribution across regions**



*Other TAs include: Rheumatology, Respiratory Diseases, Musculoskeletal, Infectious Diseases, Immunology, Mental Health, Ophthalmology, Hematology. n and percent (%) are out of contracts categorized on this patient vs. population-level reimbursement dimension.

Sophisticated contract features were more common in the United States

3 Comparative component

4 contracts included a comparative component

- **Countries:** USA (n=4)
- Assessing performance with a comparative design can help establish causality between the intervention and target outcome(s).
- **Requirements:** Advanced analytic capabilities and additional data on confounders for risk adjustment. Also need a comparator population.

4 Compliant population

6 contracts restricted outcome assessment to compliant patients.

- **Countries:** USA (n=4), Italy (n=2)
- Suggests manufacturers and payers are sharing the risk of non-compliance in different ways.
- **Requirement:** Data collection systems for measuring compliance.

5 Reimbursement form

6 contracts required manufacturers to cover cost of adverse outcomes as a form of reimbursement.

- **Countries:** USA (n=5), Italy (n=1)
- Hybrid of cost-sharing and traditional OBC; shifts additional risk to manufacturer.
- **Requirement:** Ability to track all costs associated with target outcome(s) over a specific time period and communicate to manufacturer.

Summary of key takeaways & discussion

- **There is no one-size-fits-all template for contracts:** *Contract type, outcome type, and reimbursement structure varied by country & therapeutic area*
  - Country-specific, system factors - like private vs. public payer mix and data infrastructure - likely influence the outcome types and contract structures we observed
- **Data fit-for-use is key:** *Available data sources were likely driving the types of outcomes and contract structures used by each country*
  - Simple, generalizable outcome measures identifiable in payer data like medical cost, persistence, and hospitalizations can support more scalable contracts across disease areas
- **Lack of transparency:** *Manufacturers and payers continue to keep contracting details confidential*
  - Lack of disclosure despite the need for transparency to support collaborative and scalable contracts
  - Transparency necessary for evaluation, which supports evidence-based scaling of value-based models

Development of new RWE methods and data sources are necessary to support scaling of more sophisticated (and evidence-based) contract structures

Thank you!

Questions?

Contact:
andrew.weckstein@aetion.com

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