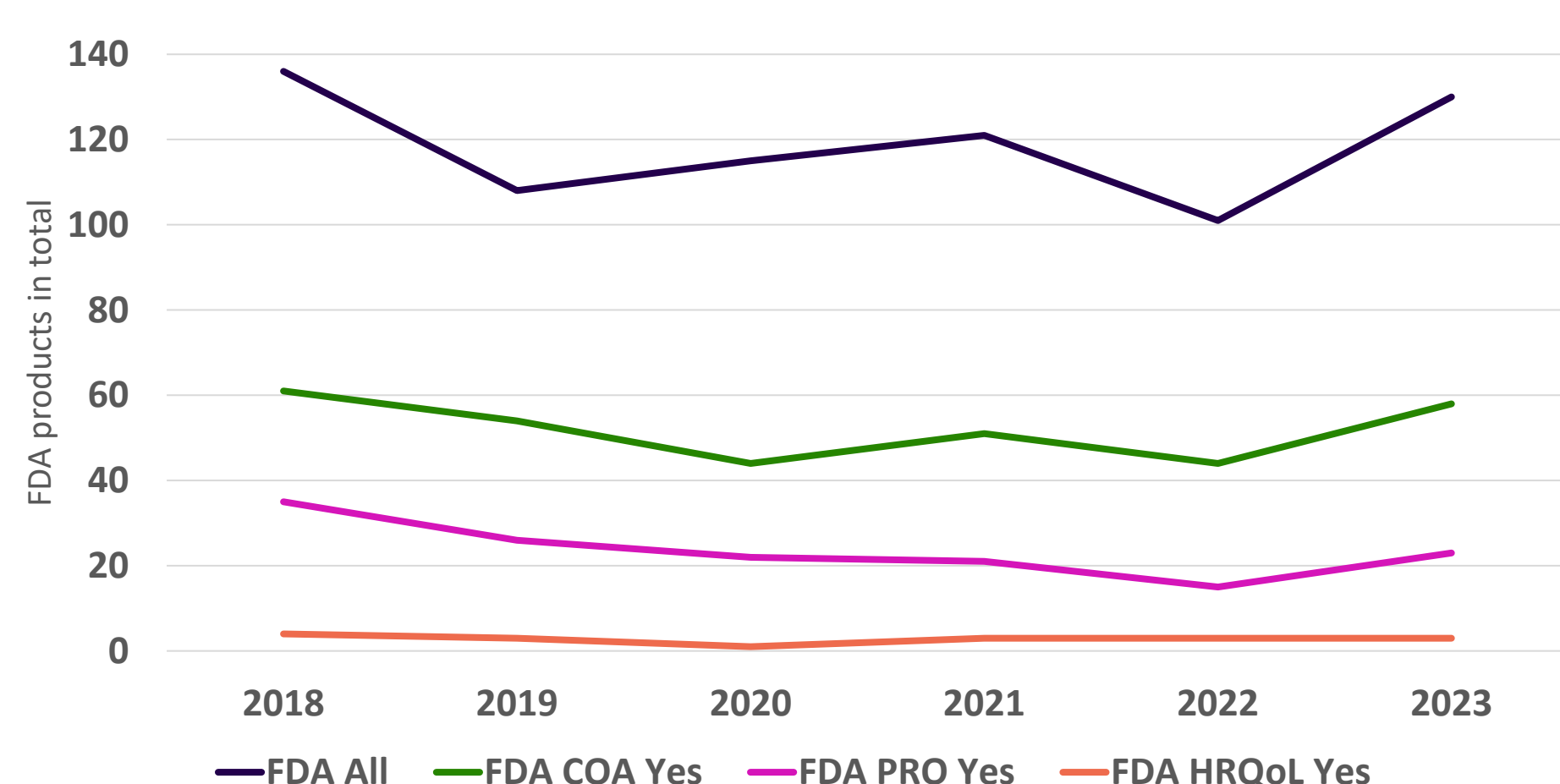


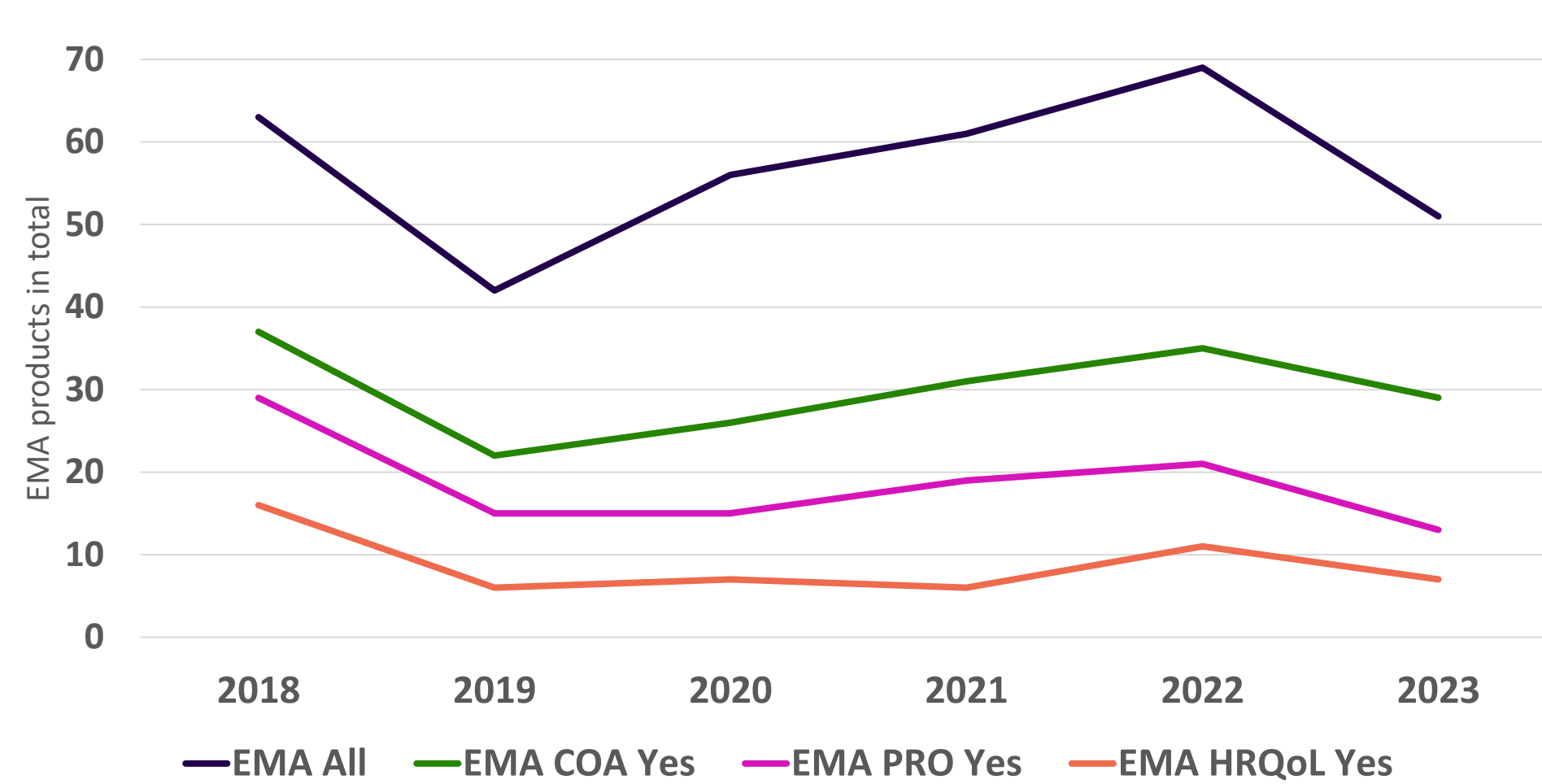
Key learnings

- Among newly approved products between 2018 and 2023, Clinical Outcome Assessments (COAs)-labeled drugs represented half of EMA approvals (53%) and fewer than half of FDA approvals (44 %).
- The number of newly approved products incorporating COAs, including Patient-Reported Outcomes (PROs) and Health-Related Quality of Life (HRQoL) assessments, remained consistently steady from 2018 to 2023.
- Among COAs, Patient-Reported Outcomes (PROs) are the most common type for both FDA and EMA new product labels.
- PROs included on labels were most frequently validated measures, and secondarily study-specific scales.

Number of FDA products with COA labeling claims (all COAs, PROs, HRQoL) compared to all FDA products approvals



Number of EMA products with COA labeling claims (all COAs, PROs, HRQoL) compared to all EMA products approvals



Concepts of interest

Concept of interest	Number of products - All COAs (PROs only)			Endpoint positioning – % for All COAs			
	FDA + EMA	FDA	EMA	Primary endpoint	Secondary endpoint	Exploratory endpoint	Not specified
Signs and/or Symptoms	405 (201)	263 (118)	142 (83)	44.6%	32.6%	2.4%	20.3%
Treatment response / remission	101 (17)	64 (10)	37 (7)	36.0%	36.0%	1.2%	26.7%
Functioning	100 (48)	53 (19)	47 (29)	26.9%	47.0%	5.2%	20.9%
HRQoL/ Health status	70 (70)	17 (17)	53 (53)	4.0%	64.0%	1.3%	30.7%
Medication / Rescue medication use	25 (16)	13 (9)	12 (7)	11.1%	70.4%	-	18.5%
Survival	23 (0)	13 (0)	10 (0)	50.0%	26.9%	3.8%	19.2%
Physician global assessment / impression	17 (0)	11 (0)	6 (0)	21.1%	52.6%	-	26.3%
Patient global assessment / impression	13 (13)	6 (6)	7 (7)	14.3%	57.1%	7.1%	21.4%
Patient-Caregiver satisfaction / preference	9 (9)	4 (4)	5 (5)	-	40.0%	10.0%	50.0%
Hospitalization +/-survival	6 (1)	3 (1)	3 (0)	57.1%	42.9%	-	-
Caregiver global assessment / impression	4 (0)	3 (0)	1 (0)	25.0%	50.0%	-	25.0%
Treatment tolerability / toxicity	2 (1)	0 (0)	2 (1)	-	100.0%	-	-
Caregiver wellbeing	1 (1)	0 (0)	1 (1)	-	-	100.0%	-

Therapeutic areas with labeling claims – All COAs versus PROs only

New product approvals with COA labeling claims	Number of products		
	FDA + EMA	FDA	EMA
Nervous System Diseases	109	74	35
Neoplasms	71	36	35
Infections	45	27	18
Skin and Connective Tissue Diseases	43	31	12
Respiratory Tract Diseases	36	17	19
Mental Disorders	30	25	5
Pathological Conditions, Signs and Symptoms	27	24	3
Digestive System Diseases	26	16	10
Hemic and Lymphatic Diseases	26	15	11
Musculoskeletal Diseases	20	12	8
Eye Diseases	20	15	5
Nutritional and Metabolic Diseases	15	5	10
Urogenital Diseases	14	10	4
Cardiovascular Diseases	11	9	2
Immune System Diseases	9	6	3
Reproductive and Urinary Physiological Phenomena	5	3	2
Endocrine System Diseases	5	1	4
Chemically-Induced Disorders	4	2	2
Anesthesia and Analgesia	3	2	1
Integumentary System Physiological Phenomena	3	2	1

New product approvals with PRO labeling claims	Number of products		
	FDA + EMA	FDA	EMA
Nervous System Diseases	67	42	25
Respiratory Tract Diseases	26	9	17
Neoplasms	24	10	14
Skin and Connective Tissue Diseases	23	12	11
Pathological Conditions, Signs and Symptoms	22	20	2
Hemic and Lymphatic Diseases	19	10	9
Musculoskeletal Diseases	14	8	6
Digestive System Diseases	13	7	6
Urogenital Diseases	12	9	3
Nutritional and Metabolic Diseases	9	1	8
Eye Diseases	8	5	3
Immune System Diseases	6	4	2
Cardiovascular Diseases	5	3	2
Infections	5	1	4
Mental Disorders	5	4	1
Endocrine System Diseases	4	0	4
Reproductive and Urinary Physiological Phenomena	4	2	2
Chemically-Induced Disorders	3	2	1
Integumentary System Physiological Phenomena	2	1	1
Anesthesia and Analgesia	1	1	0

Introduction

- COAs in drug labeling play a crucial role in regulatory decisions influencing how therapies are perceived by healthcare providers and patients. COAs help ensure treatments address patient priorities and improve their quality of life.
- A 2013-2017 review of the FDA and the EMA drug approvals showed that around 40% of products have COAs in their label.

Objective

- This study explores the COA trends for newly approved drug products between 2018 and 2023.

Methods

- FDA and EMA websites were systematically reviewed to identify new drug approvals (including new molecular entities, biologics, and vaccines) from 2018 to 2023.
- We collected information on COAs included in drug labeling using the PROLABELSTM database (Mapi Research Trust). All corresponding labels, medical reviews and assessment reports available on the health-authorities' websites were reviewed.
- 711 and 342 new product approvals were granted by the FDA and the EMA, respectively, between 2018 and 2023 from which 44% (n= 312) of FDA drugs and 53% (n=180) of EMA drugs had COAs in their labels.

Composite measures with PRO instruments used in label

Acronym - Name of composite PRO measure (used in ≥2 products)	Type of composite	PRO component in the composite measure	Number of products		
			FDA + EMA	FDA	EMA
mMS - modified Mayo Score	PRO / ClinRO	Stool frequency, rectal bleeding	10	6	4
ACR20/50/70 - American College of Rheumatology 20/50/70 Response Criteria	PRO / ClinRO / Biomarker	Patient global assessment, HAQ-DI, pain VAS	9	5	4
CDAI - Crohn's Disease Activity Index	PRO / ClinRO	Liquid stools, abdominal pain, general wellbeing	9	6	3
ACQ-7 - Asthma Control Questionnaire-7	PRO / Biomarker	Symptoms	5	1	4
DAS-28 CRP - Disease Activity Score - C-Reactive Protein	PRO / ClinRO / Biomarker	Pain VAS	5	2	3
NYHA Functional Classification - New York Heart Association Functional Classification	PRO / ClinRO	Symptoms	4	2	2
Mayo - Mayo Score	PRO / ClinRO	Stool frequency, rectal bleeding	3	1	2
PCWG2 criteria - Prostate Cancer Clinical Trials Working Group 2 criteria	PRO / ClinRO / Biomarker	Pain, HRQoL	3	3	-
PCWG3 criteria - Prostate Cancer Clinical Trials Working Group 3 criteria	PRO / ClinRO / Biomarker	Pain, HRQoL	3	-	3
ASDAS - Ankylosing Spondylitis Disease Activity Score	PRO / Biomarker	Back pain, duration of morning stiffness, patient global assessment-disease activity, peripheral pain, swelling	2	-	2
BILAG Index - British Isles Lupus Assessment Group Index	PRO / ClinRO / Biomarker	Fatigue, nausea, vomiting, headaches, chest pain	2	2	1
LYRIC - Lymphoma Response to Immunomodulatory Therapy Criteria	PRO / ClinRO / Biomarker	Tiredness, itching, bruising and bleeding, cough, breathlessness, sweats, swallowing problems	2	2	-
paMS - Partial adapted Mayo Score	PRO / ClinRO	Stool frequency , rectal bleeding	2	-	2
PsARC - Psoriatic Arthritis Response Criteria	PRO / ClinRO	Patient global assessment	2	-	2

Type of COA and Endpoint positioning

COA Type	Number of products			Endpoint positioning %FDA + EMA			
	FDA+EMA	FDA only	EMA only	Primary	Secondary	Exploratory	Unspecified
PRO	254	142	112	22.9%	52.9%	2.5%	21.6%
ClinRO	204	130	74	36.6%	40.3%	1.9%	21.2%
Composite*	185	98	87	36.2%	41.4%	0.6%	21.8%
PerfO	66	39	27	34.6%	44.1%	1.6%	19.7%
ObsRO	42	25	17	38.6%	46.6%	2.3%	12.5%

ClinRO: Clinician-Reported Outcome – PerfO: Performance-Reported Outcome – ObsRO: Observer-Reported Outcome
* Composite: any measure including a PRO, ClinRO, PerfO or ObsRO component +/- biomarker or survival components

Validated PRO instruments used in label

Acronym - Name of PRO instrument (used in ≥5 products)	Number of products		
	FDA + EMA	FDA	EMA
PGI-C - Patient Global Impression scale - Change	11	5	6
SF-36® / SF-36v2® - SF-36 Health Survey	11	3	8
FACIT-Fatigue - Functional Assessment of Chronic Illness Therapy - Fatigue Scale	10	3	7
DLQI - Dermatology Life Quality Index	9	-	9
SGRQ - St George's Respiratory Questionnaire	9	4	5
HAQ-DI - Health Assessment Questionnaire-Disability Index	8	4	4
Norfolk QOL-DN - Norfolk Quality of Life Questionnaire - Diabetic Neuropathy	7	4	3
EORTC QLQ-C30 - EORTC Quality of Life Questionnaire - Core Questionnaire	6	-	6
MG-ADL - Myasthenia Gravis Activities of Daily Living Profile	6	3	3
BASDAI - Bath Ankylosing Spondylitis Disease Activity Index	5	2	3
EQ-5D-5L - EuroQoL 5-Dimension 5-Level	5	-	5
ESS - Epworth Sleepiness Scale	5	3	2