CO52

Comparative Analysis of Patient-Reported Outcomes (PRO) in Asthma Biologic Late Phase Studies and Approved Labels: Implications for Entry and Product Differentiation

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ISPOR EU 2025 GLASGOW, SCOTLAND, OCTOBER 9-12

Objective

To assess the landscape of patient-reported outcome (PRO) label claims among approved and late phase asthma biologics to support understanding of entry endpoints and illustrate areas for product differentiation.

METHODS

Identify comparator Asthma Biologics (May 2025)

- In late phase (phase 3) clinical trials
- Excluding biosimilars
- Approved for Asthma by United States FDA and/or EMA

Collect available information

- Trial designs (protocols, clinicaltrial.gov, publications)
- FDA labels
- EMA labels
- Regulatory feedback from Health Authority assessment reports

Evaluate PRO endpoints

- Inclusion in trials
- Presence and characteristics in product labelling
- Clinical endpoints and placement in statistical hierarchies
- Health Authority insight on acceptability and interpretability

RESULTS

Presence and characteristics of PROs in product labelling

- Asthma symptom control, health-related quality of life (HRQoL) and symptoms were outcomes accepted on labels.
- Most consistently accepted outcomes and measures were:
  - Asthma symptom control as measured by ACQ.
  - HRQoL as measured by AQLQ.
- There was a lack of consistency in how symptoms were assessed.
- Assessment of symptoms is less consistently accepted on labels compared to other PROs.
- Endpoints assessing early and sustained improvements in PROs were presented in EMA labelling to further describe treatment benefit.

Placement in statistical hierarchies

- PRO labelling claims were most often associated with endpoints that had been included in the statistical hierarchy.

Regulatory feedback

- Emphasised that PRO endpoints should be pre-specified, multiplicity-controlled, and clinically meaningful to be accepted into labelling.

Table 1. ***PRO measures assessing asthma symptom control, health-related quality of life, and symptoms in pivotal trials and label acceptance***

Biologic Name *Brand Name*	Asthma approval year *(FDA/EMA)*	Measures				
		Asthma Symptom Control	Health-Related Quality of Life	Daily Symptoms		Symptoms
				Day	Night	
In late phase (phase 3) clinical trials						
Depemokimab	-	ACQ-5	SGRQ	ADSD	ANSD	-
Dexpramipexole	-	ACQ-6	AQLQ(S)	ASD	ASD	-
Approved for Asthma						
Tezepelumab *Tezpire*	*2021/2022*	ACQ-6	AQLQ(S) SGRQ	ASD	ASD	-
Dupilumab *Dupixent*	*2018/2019*	ACQ-5	AQLQ(S)	PM SS	AM SS	-
Benralizumab *Fasenra*	*2017/2018*	ACQ-6 ACT	AQLQ(S)	AS Scale	AS Scale	-
Reslizumab *Teva*	2016/2016	ACQ-7	AQLQ	ACD	ACD	PSIA
Mepolizumab *Nucala*	*2015/2015*	ACQ-5	SGRQ	-	-	ASUI
Omalizumab *Xolair*	*2003/2005*	ACQ ACT	AQLQ	DA Score	NA Score	-

ACD = Asthma Control Diary; ACT = Asthma Control Test; ACQ = Asthma Control Questionnaire –5 symptoms only, –6 + rescue use, –7 + lung function; AM/PM SS = AM symptom score/PM symptom score; ASD = Asthma Symptom Diary; ADSD/ANSD = Asthma Daytime/Nighttime Symptom Diary; AS Scale = Asthma Symptom Scale; ASUI = Asthma Symptom Utility Index; AQLQ = Asthma Quality of Life Questionnaire; AQLQ(S) = Asthma Quality of Life Questionnaire with Standardized Activities; DA/NA Score = Daytime/Nocturnal Asthma Score; PSIA = Patient Specific Impact Assessment/Predominant Symptom and Impairment Assessment; SGRQ = Saint George's Respiratory Questionnaire

Measure colour legend
Black: In trial
Blue: EMA label only
Orange: FDA and EMA label
Green: FDA label only

Accepted PRO analysis on labels

- EMA labels more commonly reported change from baseline analysis, often with associated p-values.
- FDA labels more commonly reported responder analyses, without p-values.
- The responder definition was consistent across products for ACQ and AQLQ at ≥0.5 for both measures.

Conclusions

- PRO ACQ-5 and AQLQ responder (≥0.5) and change from baseline endpoints are established entry criteria for asthma biologics, with regulatory precedent supporting their inclusion.
- Symptom diary endpoints and other novel endpoints addressing patient-prioritised outcomes offer opportunities for differentiation.

DISCLOSURES: JRW and RR are Sanofi employees and may hold shares and/or stock options in the company.
FUNDING: Study funded by Sanofi.