Patient-Centered Endpoint Strategy for Biologic Trials in Severe Asthma: Integrating Regulatory, Payer, and Patient-Prioritized Endpoints

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OBJECTIVE & LANDSCAPE REVIEW

Objective

Recommend a stakeholder-aligned, fit-for-purpose PROM strategy for phase III severe asthma biologic trials that supports regulatory claims, payer evidence needs, and patient-prioritized endpoints for symptom control, quality of life, and treatment experience.

Targeted landscape assessment

- Phase III severe asthma biologic trials reviewed through FDA sources and ClinicalTrials.gov
- HTA evaluations examined expectations for comparative effectiveness and utility-generating endpoints
- Guidelines, expert consensus statements, and core outcome initiatives assessed for endpoint alignment
- PubMed, Embase, and PsycINFO literature (2015-2025) synthesized to compare PROM properties and feasibility
- Selection balanced validity, respondent burden, interpretability, utility mapping, and operational feasibility

Design Principles:

- Landscape assessment of validated and clinically meaningful measures; pre-specified and multiplicity-aware endpoint strategy.
- Capture early signal detection, sustained benefit, and operational feasibility in global trials.
- Pair disease-specific HRQoL with utility measures when HTA mapping is required.

RECOMMENDED CORE BATTERY

Qualified symptom diaries remain foundational; ACQ, AQLQ, and EQ-5D form the preferred core battery for regulatory, HTA, and patient-centered evidence needs

Daily symptom diary

✓ Asthma daytime/nighttime symptom diaries provide high-granularity symptom burden and reflect FDA-qualified symptom assessment in severe asthma trials.

ACQ-6

✓ Preferred core control measure: guideline-endorsed, MCID established, aligned with exacerbation endpoints, and widely used in biologic trials of severe asthma.

AQLQ-S+12

✓ Preferred disease-specific HRQoL measure for demonstrating patient-perceived benefit beyond symptom reduction and enabling cross-trial comparison.

EQ-5D-5L

- Complementary generic utility measure for HTA submissions.
- Align timing of administration with AQLQ when mapping to utilities is planned.

Why SGRQ is not core

- Too long
- Overlaps with AQLQ
- Multiplicity concerns
- Limited HTA utility (mapping is less consistent across asthma biologic programs)

Global use is feasible, but local adaptation may still be needed where EQ-5D value sets differ across countries.

SUPPLEMENTARY / SUBGROUP MEASURES

Measures to differentiate treatment value

- **WPAI and CIQ** capture absenteeism, presenteeism, and broader disease burden relevant to HTA contextual support and market access.
- **TSQM-9** quantifies convenience, ease of use, perceived effectiveness, and overall satisfaction—useful when dosing schedule or place of administration is a differentiator.
- **PGI-C and PGI-S** can anchor interpretation of symptom diary and ACQ changes but remain supportive rather than validated core endpoints.

Measures to define subgroups and add context

- **SNOT-22** adds meaningful patient-centered evidence in participants with comorbid CRSwNP; align timing to dosing frequency.
- Baseline **HADS** screening may clarify anxiety/depression burden and support subgroup analyses and treatment response heterogeneity.
- Promising measures such as **SAQ** and **AIRQ** are not prioritized for core pivotal battery because regulatory and HTA roles remain less established.

Broad linguistic availability supports global phase III implementation for most recommended instruments.

PROM RECOMMENDATION MATRIX

Instrument		Regulatory critical	HTA relevant	Guideline aligned	Differentiation / value story	Patient context
Daily symptom diary	Core	☑	☑	–	–	–
ACQ-6	Core	☑	☑	☑	–	–
AQLQ-S+12	Core	☑	☑	–	☑	☑
EQ-5D-5L	Core	–	☑	–	–	–
SNOT-22 (CRSwNP)	Supplement	–	–	–	☑	–
WPAI and CIQ	Supplement	–	☑	–	☑	☑
HADS	Supplement	–	☑	–	☑	☑
TSQM-9	Supplement	–	☑	–	☑	☑
SGRQ	Not Core	–	–	–	☑	–

PGI-C and PGI-S may be useful as supportive anchors for responder narratives, but are not validated core endpoints. SGRQ appears in some clinical programs, yet the recommended strategy does not prioritize it for the core set.

Checks indicate where each instrument most directly strengthens the evidence package.

ILLUSTRATIVE ASSESSMENT CADENCE

Exact visit timing should follow protocol dosing cadence and statistical hierarchy. The pattern below reflects the shared design logic across the reviewed sources.

Measure group	Baseline	Early 2-4 wk	Mid-Trial ~24 wk	Late / EOS
Daily symptom diary	●			●
ACQ-6	●	●	●	●
AQLQ-S+12	●	●	●	●
EQ-5D-5L	●	●	●	●
SNOT-22 (CRSwNP)	●		●	●
WPAI / TSQM / HADS	●		●	●

Design principle: dense early control assessment, milestone HRQoL/utility visits, and targeted subgroup/context modules without unnecessary redundancy.

CONCLUSIONS

- A fit-for-purpose severe asthma PROM strategy should pair qualified symptom diaries with ACQ, AQLQ, and EQ-5D as the **foundational evidence battery**.
- **Supplementary modules** should be added only when they answer a distinct stakeholder question: SNOT-22 for CRSwNP, WPAI and CIQ for productivity, TSQM for treatment experience, and HADS for subgroup context and to capture variations in treatment response.
- **Operational success** depends on synchronized timepoints, ePRO readiness, and avoiding redundant measures that add burden without informing decisions regarding the product value.

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Abbreviations

ACQ, Asthma Control Questionnaire; **AIRQ**, Asthma Impairment and Risk Questionnaire; **AQLQ**, Asthma Quality of Life Questionnaire; **CIQ**, classroom impairment questionnaire; **CRSwNP**, chronic rhinosinusitis with nasal polyps; **EQ-5D-5L**, EuroQol-5 Dimensions; **HADS**, Hospital Anxiety and Depression Scale; **HRQoL**, Health-related Quality of Life; **HTA**, health technology assessment; **PGI-C and PGI-S**, Patient Global Impression of Change and Patient Global Impression of Severity; **PRO**: patient-reported outcome; **PROM**, Patient-reported outcome measure; **SAQ**, Severe Asthma Questionnaire; **SGRQ**, St George's Respiratory Questionnaire; **SNOT-22,** Sino-Nasal Outcome Test-22; **TSQM**, Treatment Satisfaction Questionnaire for Medication; **WPAI,** Work Productivity and Activity Impairment.

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