

# SOS for Breath!

***Confronting the challenge of advancing new therapies using lung imaging outcome measures:***

***The urgent need for regulatory acceptance of innovative validated Drug Development Tools across Chronic Lung Diseases***

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# Chronic Lung Disease Biomarker & Clinical Outcome Assessment Qualification Consortium (CBQC)



A collaborative public-private partnership, aiming to undertake regulatory qualification of biomarkers and clinical outcomes assessments (COAs) to facilitate the development and approval of novel treatments for CLDs

## ❖ **FDA Patient Listening Session October 8<sup>th</sup>, 2021**

Shared 8 patient stories focusing on unmet medical needs

Proposed “Concept of Interest” framework for Chronic Lung Disease

Discussed opportunities to efficiently advance important biomarkers and COAs

**Followed by an advocacy event with legislators on October 12<sup>th</sup>**

# Letter of Intent for CT Lung Density Submitted in Feb 2021

**Biomarker name:** Volume-adjusted lung density at the 15<sup>th</sup> percentile of the CT histogram

(VALD<sub>Perc 15</sub>).

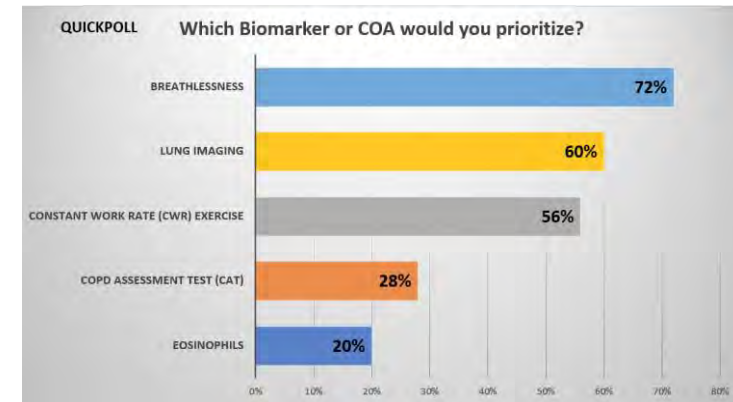
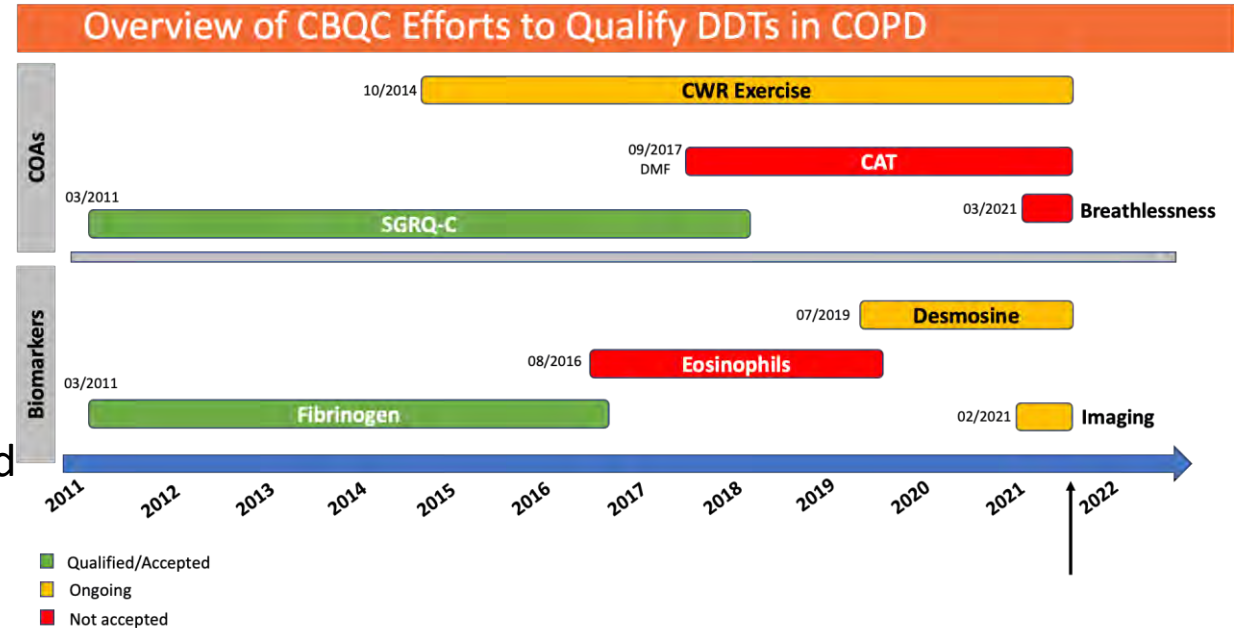
**Biomarker Type:** Radiologic

## Context of Use Statement

We proposed the use of quantitative CT scanning, as measured by VALDPerc15, as a monitoring biomarker of emphysema severity for use in clinical trials.

Specifically, the difference from comparator in VALDPerc15 on serial assessment will be used as an outcome to objectively measure disease progression in clinical trials aiming to assess the effect of active treatment in comparison to placebo (or Standard of Care) at a population level.

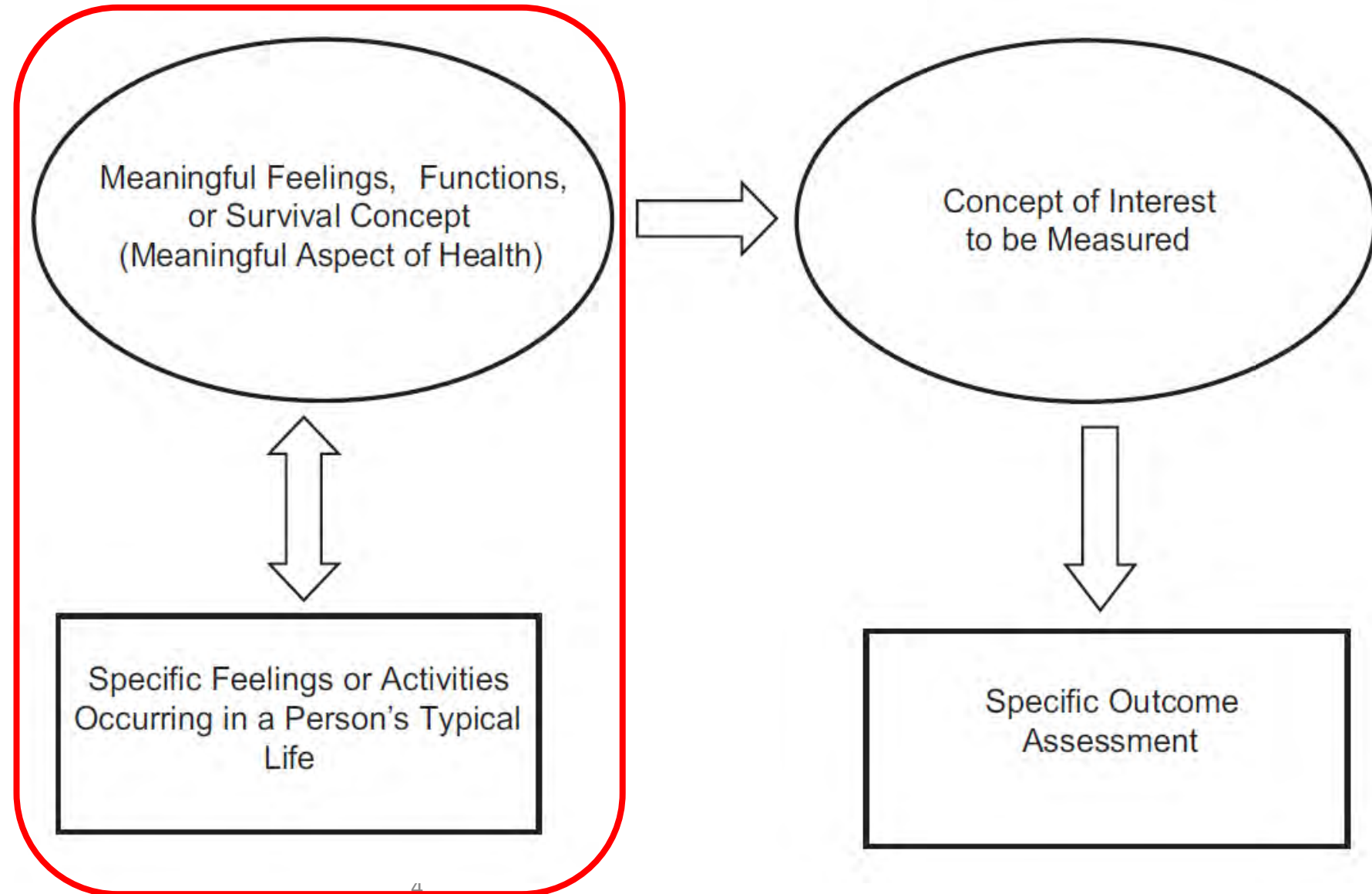
Ash SY, San José Estépar R, Fain SB, Tal-Singer R, Stockley RA, Nordenmark LH, Rennard S, Han MK, Merrill D, Humphries SM, Diaz AA, Mason SE, Rahaghi FN, Pistenmaa CL, Scirba FC, Vegas-Sánchez-Ferrero G, Lynch DA, Washko GR; COPDGene Investigators and the COPD Biomarker Qualification Consortium. Relationship between Emphysema Progression at CT and Mortality in Ever-Smokers: Results from the COPDGene and ECLIPSE Cohorts. *Radiology*. 2021 Apr;299(1):222-231. doi: 10.1148/radiol.2021203531. Epub 2021 Feb 16. PMID: 33591891; PMCID: PMC7997617.



# COA Development Across Chronic Lung Diseases

## CBQC Recommendation

COA development grounded in a unified conceptual framework that identifies ***meaningful aspects of health impacted across chronic lung diseases***



Adapted from Walton et al. *Clinical Outcome Assessments: Conceptual Foundation: Report of the ISPOR Clinical Outcomes Assessment – Emerging Good Practices for Outcomes Research Task Force. Value in Health* 2015;18:741-752