

Patient Preference Study Conducted in Early Drug Development in COPD patients

shortened version

Joint Meeting of the Basel Epidemiology Seminar & Basel Biometric Section; November 22, 2018

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Disclaimer

The views and opinions expressed in this presentation are those of the presenter and do not necessarily reflect the official policy or position of Novartis or any of its officers

Disclosure

I am a permanent employee of Novartis Pharma AG

Agenda

- **The challenge of “going beyond standard endpoints”**

- A structured process, beginning with robust qualitative insights from patients
- Methodological considerations when designing Patient Preference Studies

What is Chronic Obstructive Pulmonary Disease (COPD)?

- Chronic
- Obstructive
- Pulmonary
- Disease

Common,
preventable and
treatable
disease

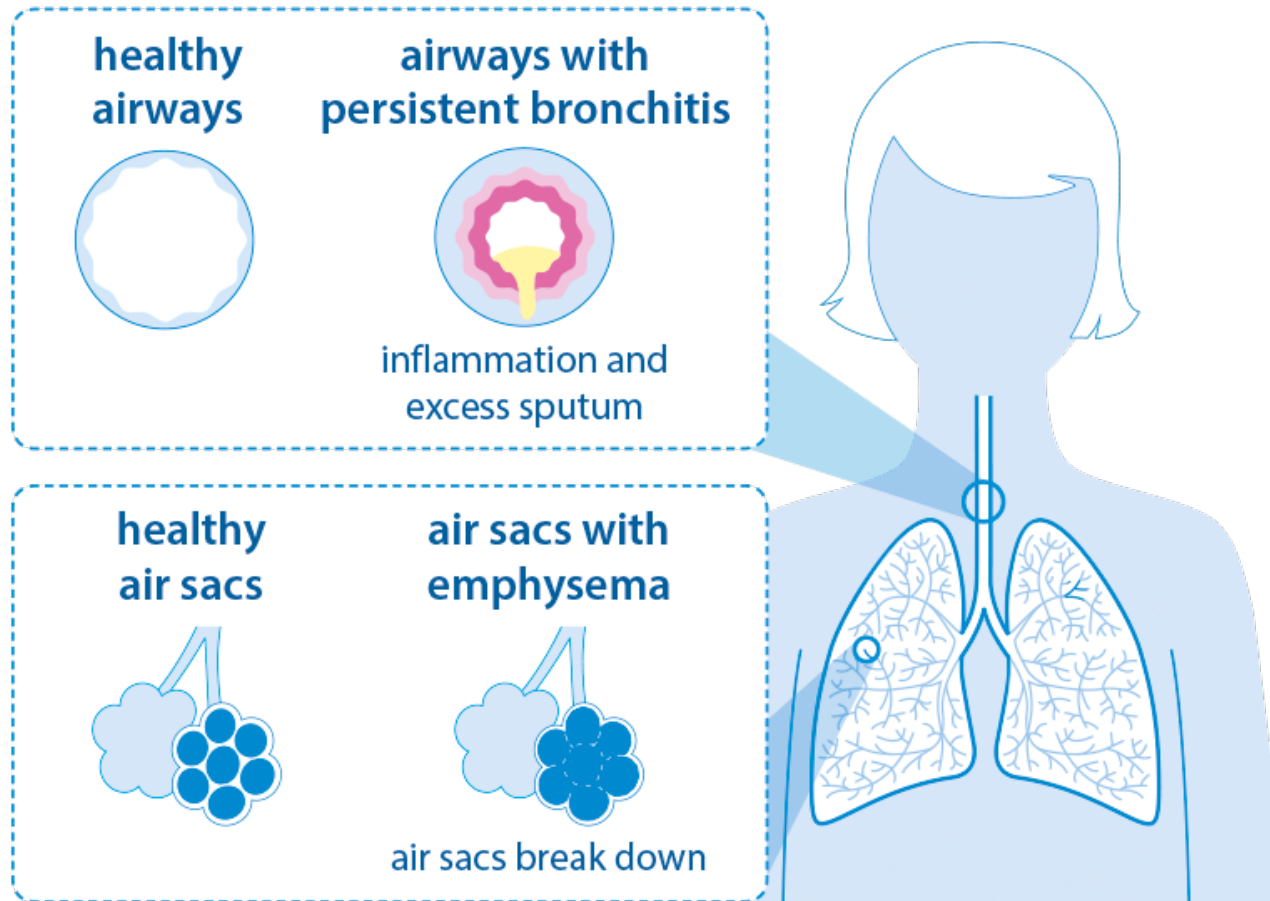
Persistent
respiratory
symptoms

Airflow limitation

Airway and/or
alveolar
abnormalities

Usually caused by
significant exposure
to noxious
particles/gases

COPD is characterised by persistent bronchitis and emphysema



Key symptoms

Shortness of breath,
especially during
physical activities
(and at rest in very
severe stages)

Wheezing

Chest tightness

Having to clear your
throat first thing in
the morning, due to
excess mucus in
lungs

A chronic cough that
may produce mucus
(sputum) that may be
clear, white, yellow
or greenish

Recurrent respiratory
infections

<https://www.mayoclinic.org/diseases-conditions/copd/symptoms-causes/syc-20353679>

Definition of a COPD Exacerbation

“an acute worsening of symptoms that results in additional therapy”

GOLD Statement Updated 2018

COPD endpoints: standard vs novel

Primary Endpoints for current standard treatments¹:

Lung function

Exacerbations

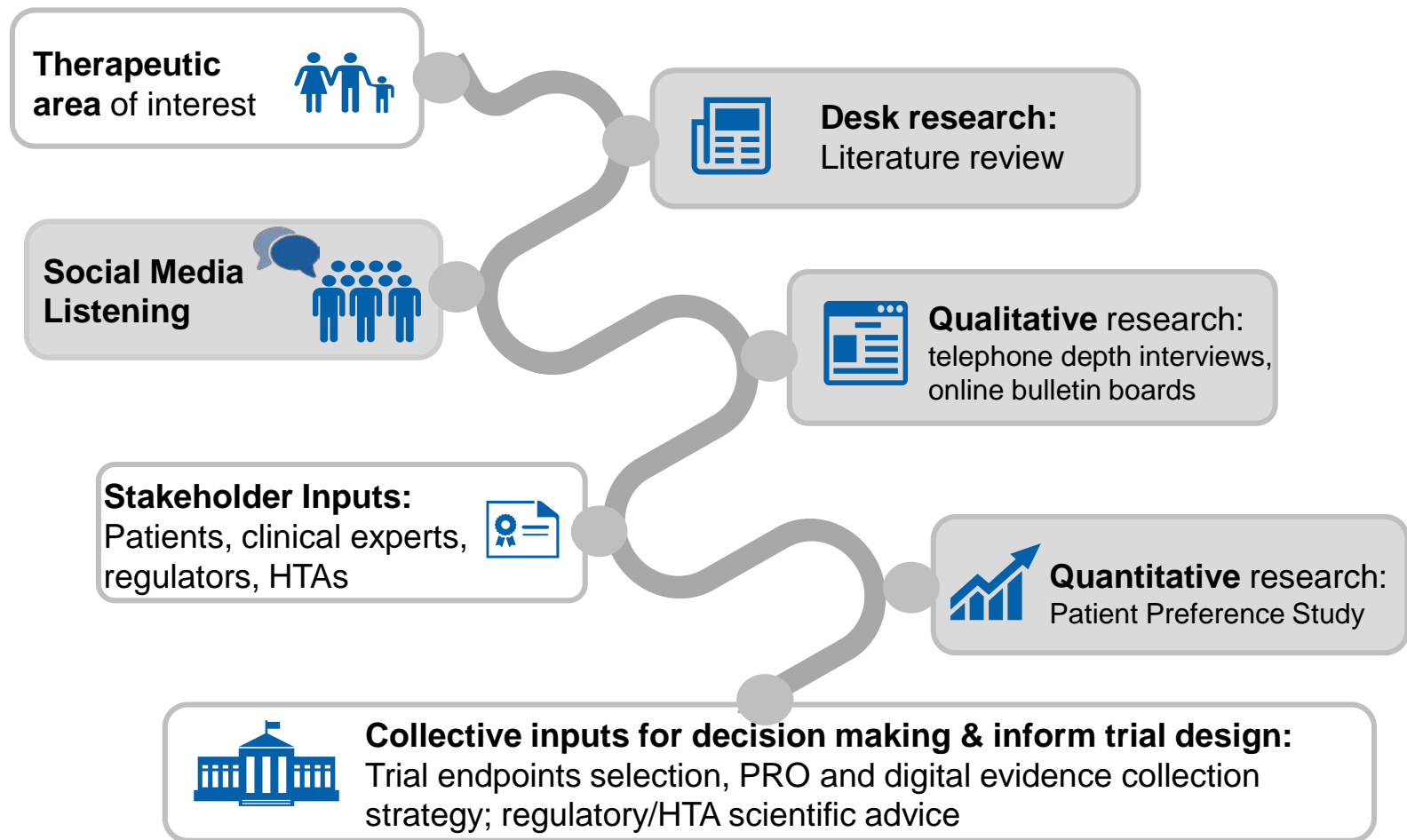
- What if focus for a new drug would be on endpoints like **cough & sputum**?
 - Need to show to stakeholders, why endpoints are relevant for patients
 - Need to create awareness for relevance of endpoints

¹example: Wedzicha JA, et al. N Engl J Med. 2016;374(23):2222–34

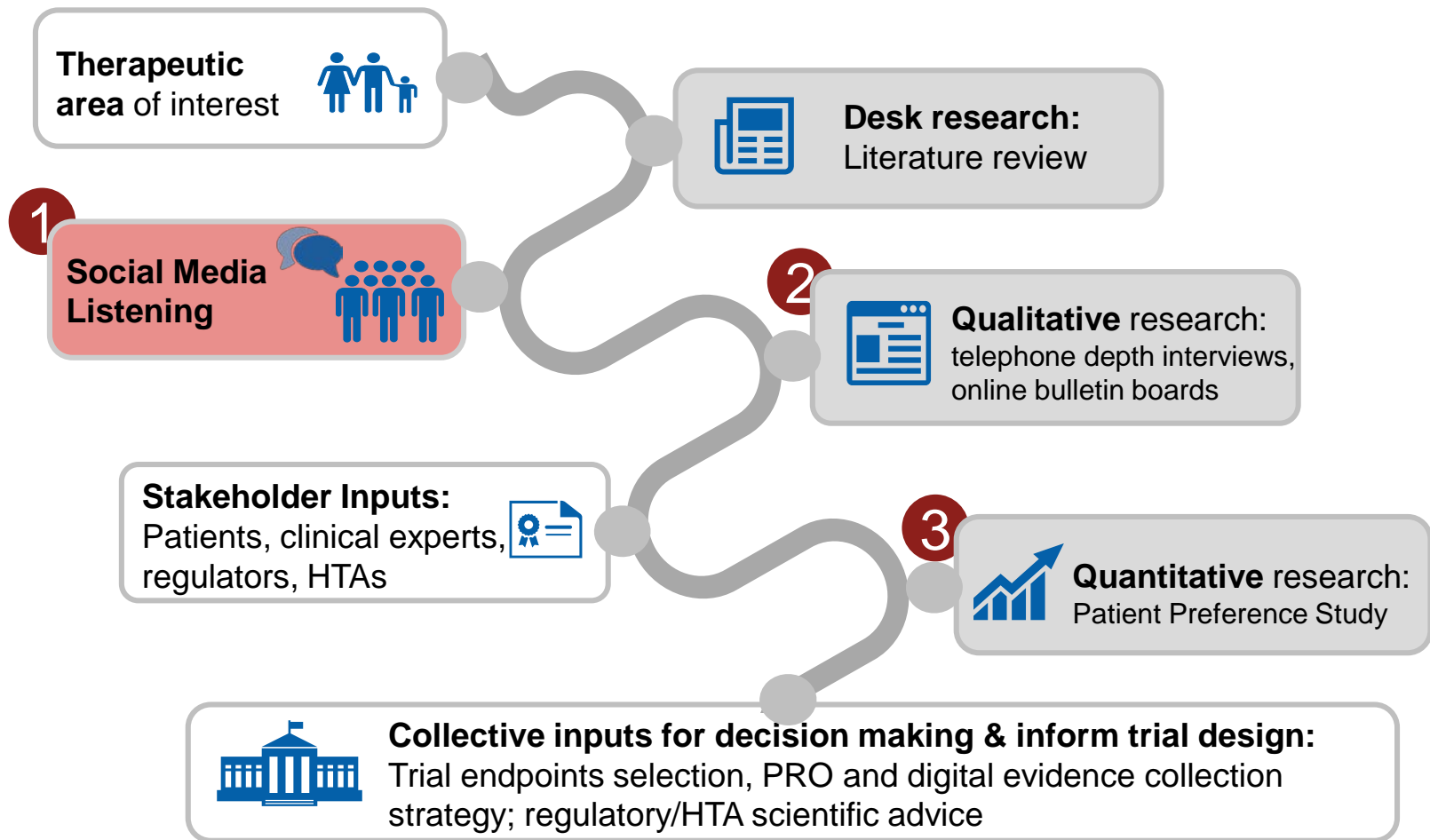
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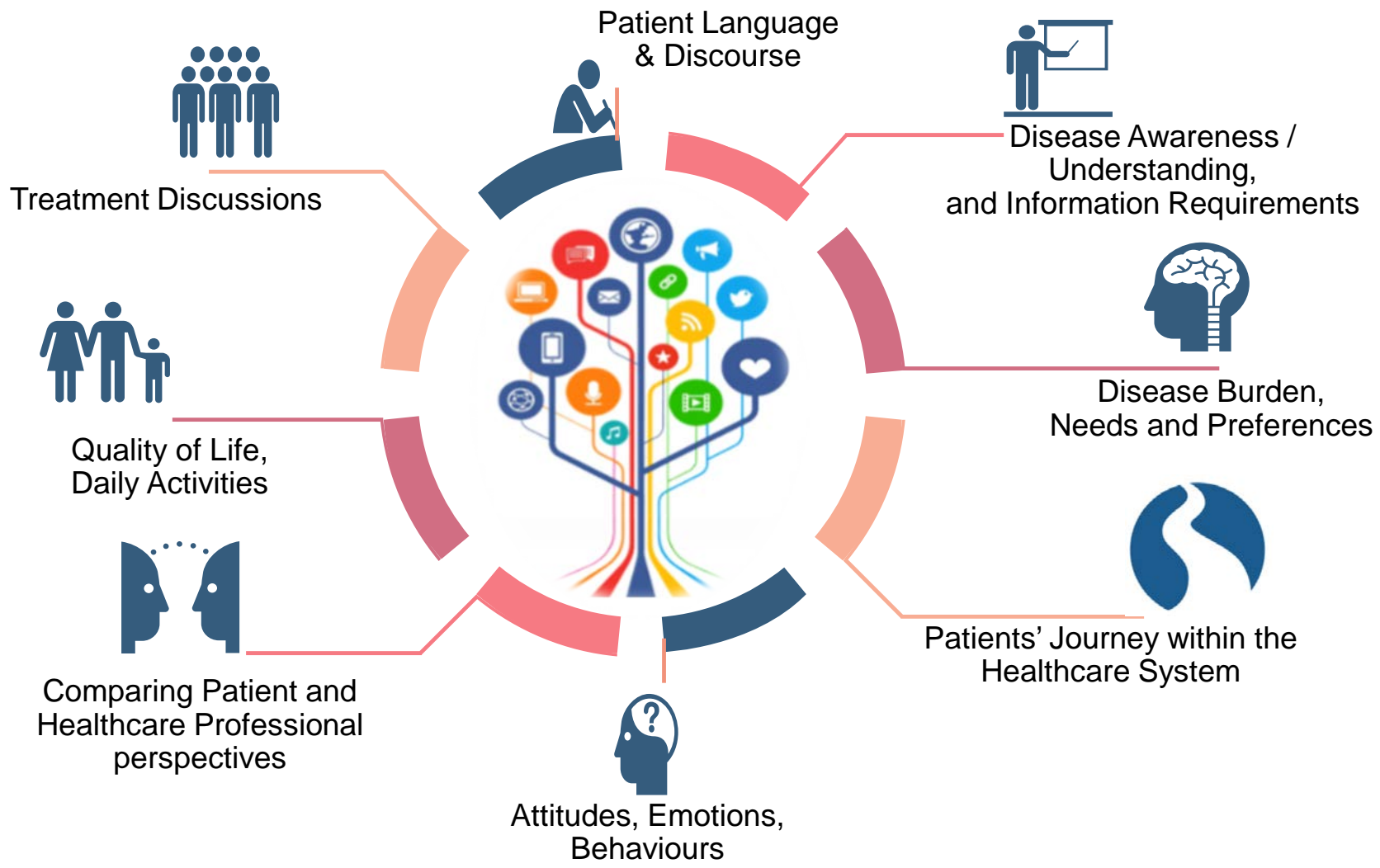
Designing a Structured Process for Patient Insight Gathering in Early Drug Development



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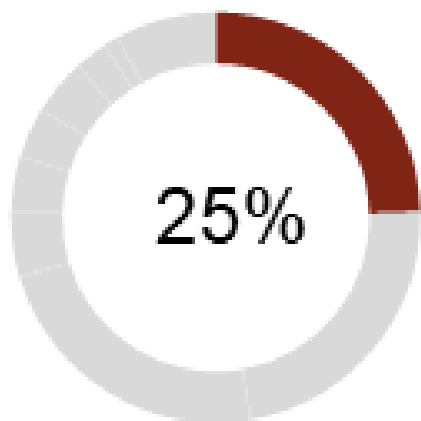


Social Media Listening to deepen our understanding of patient perspectives, needs and attitudes

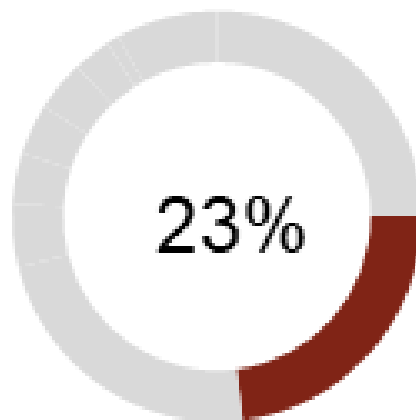


Social Media Listening

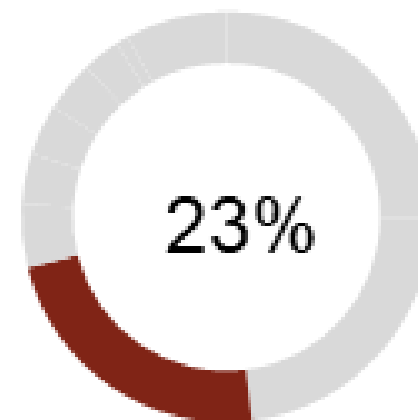
- Impact of symptoms
 - and wording: sputum vs mucus



Cough



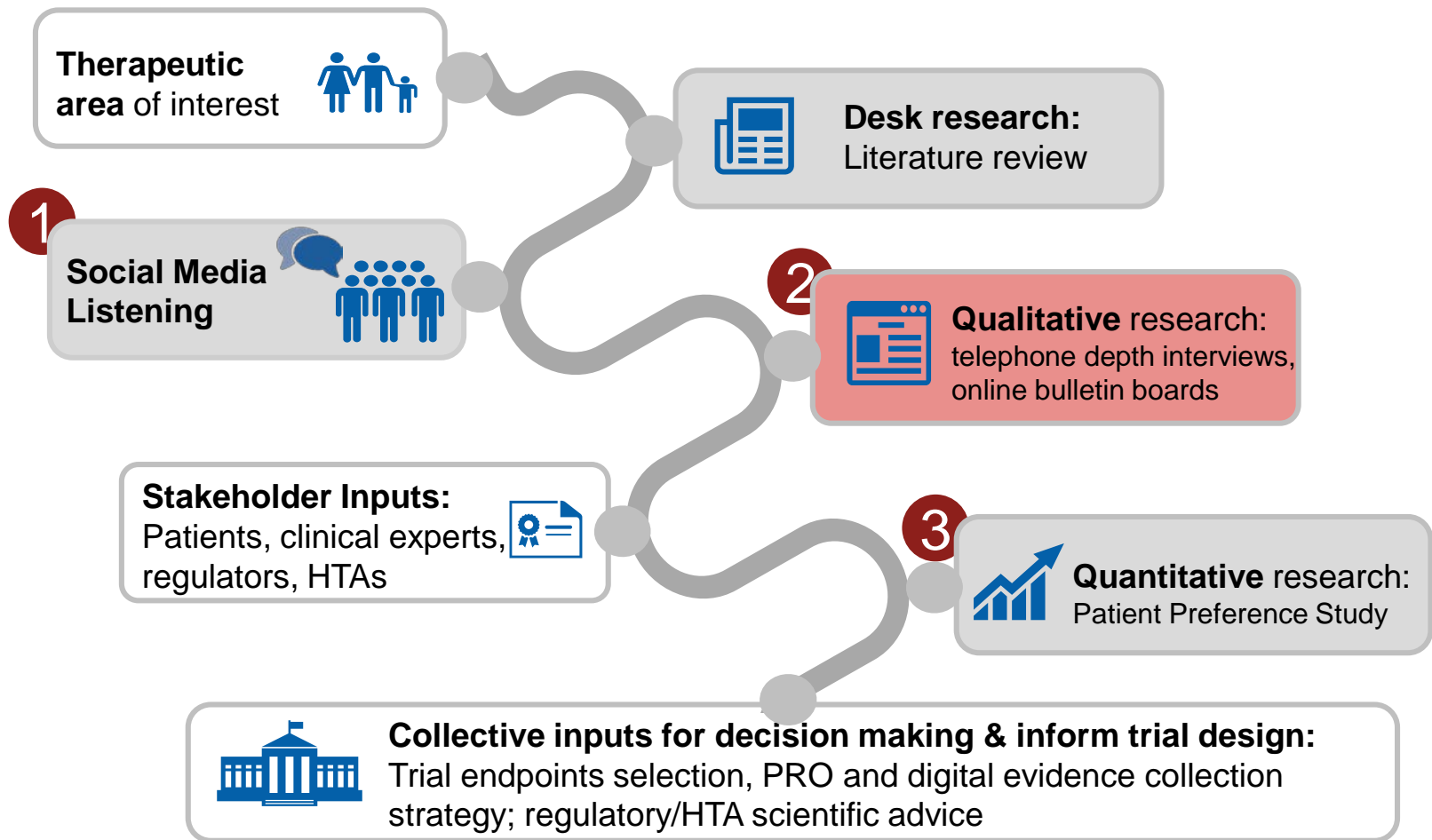
Mucus



Shortness of Breath

Gutzwiller FS, et al. Poster presented at the European Respiratory Society International Congress, 2018, Paris

Designing a Structured Process for Patient Insight Gathering in Early Drug Development



Online Bulletin Board

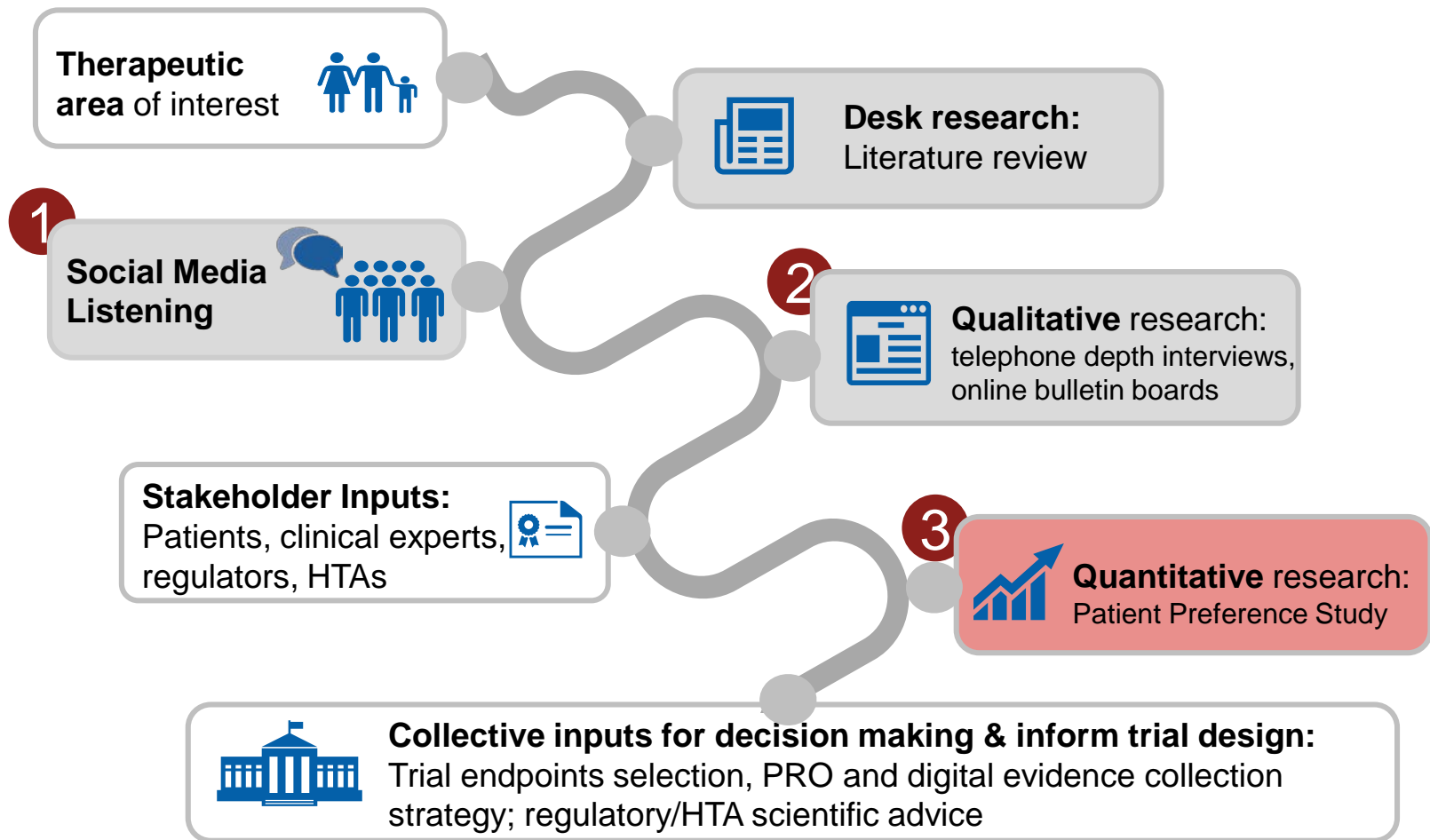
Qualitative patient/caregiver research approach offering advantages over traditional methods like phone interviews or focus groups

- Different question types (directed to all or just one person)
- Can include pictures, graphs, exercises
- **Can adjust questions on a daily basis** (e.g. based on previous posts)
- **Anonymous** – good to explore emotional and more sensitive issues (hygiene, sex life..)

Agenda

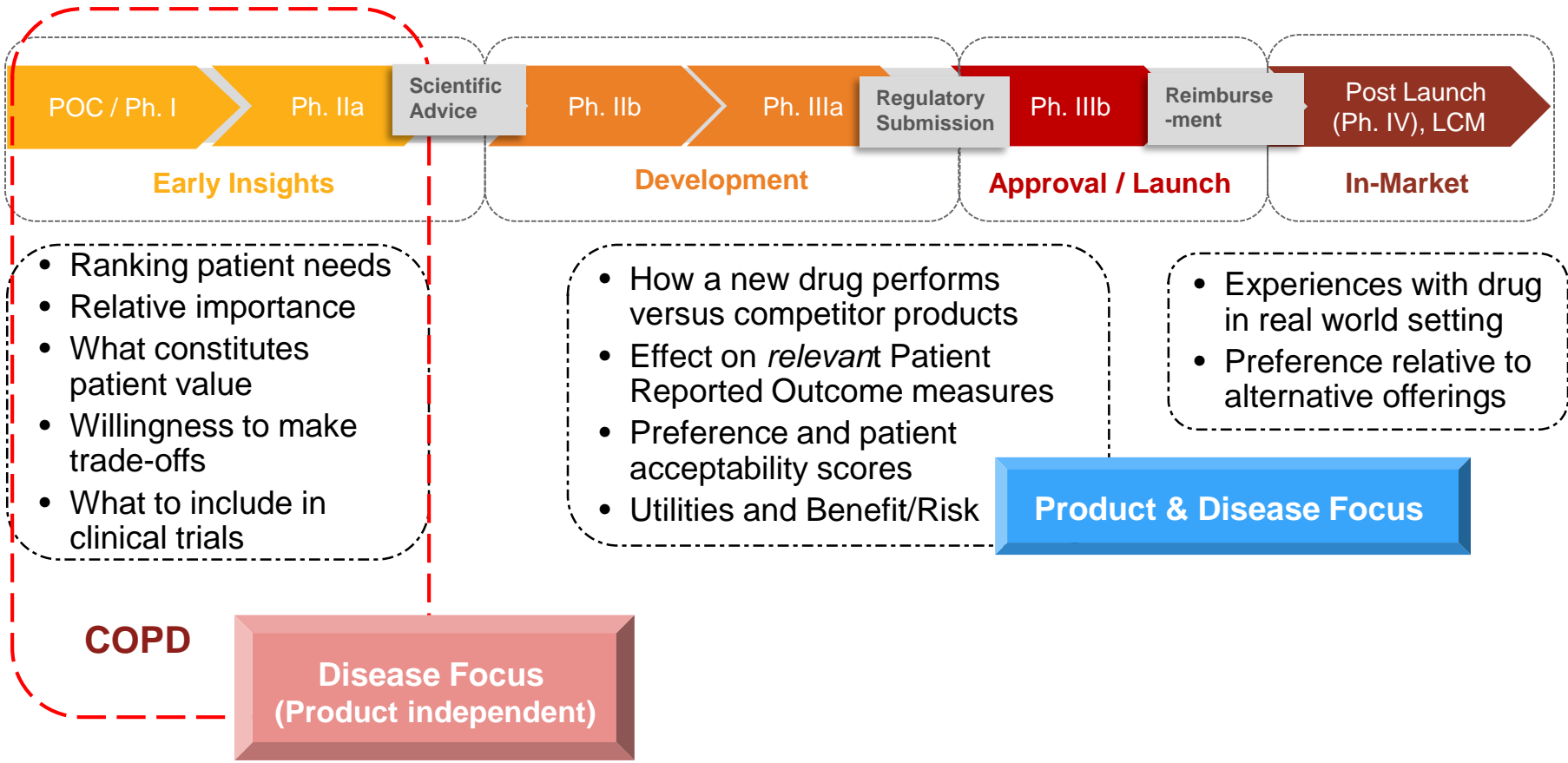
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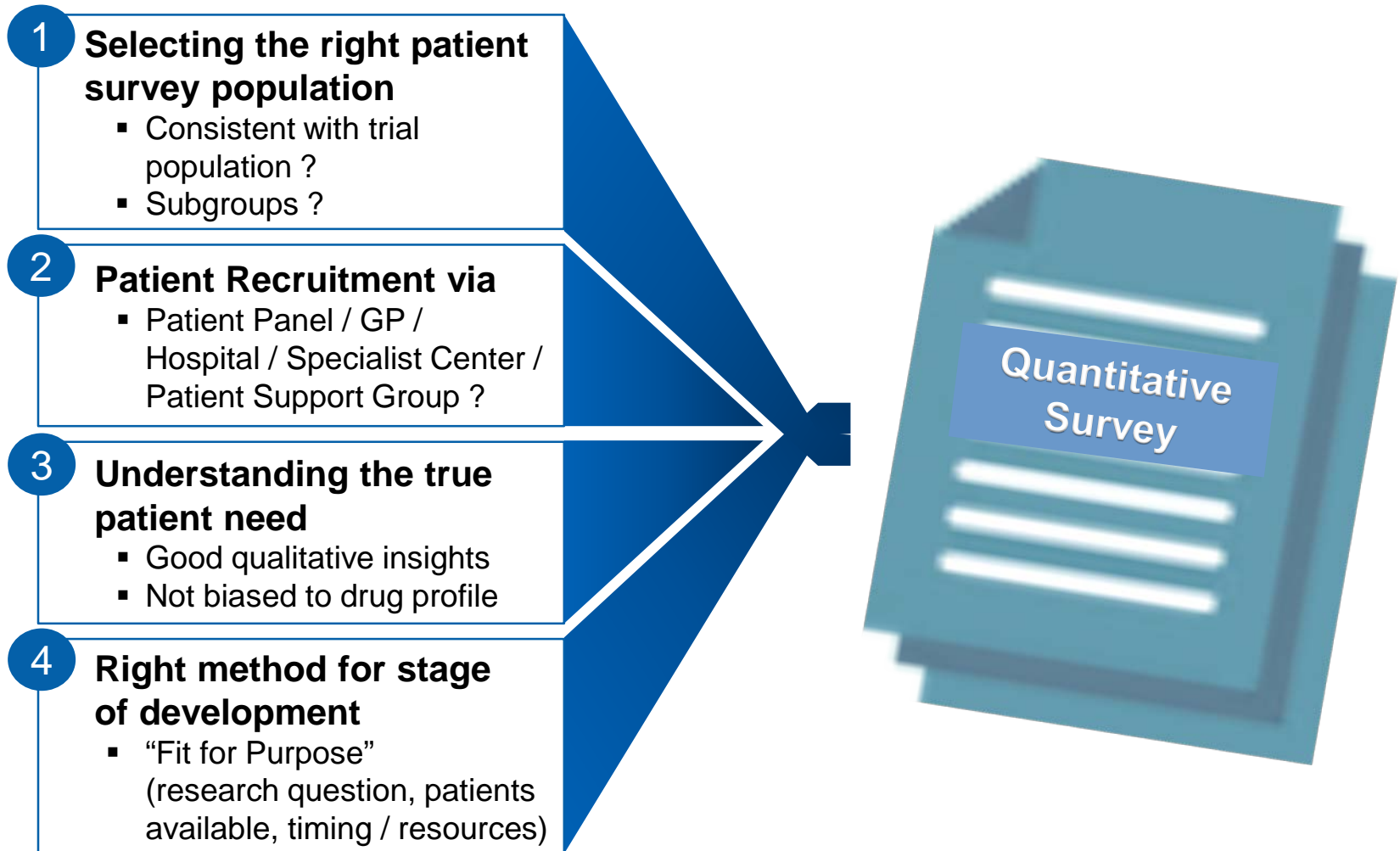


Conducting patient preference studies throughout the drug development lifecycle

Suitable methodologies for preference studies may differ depending on the stage of the lifecycle and questions to be addressed





Considerations during design of quantitative patient preference studies




Choosing the most appropriate patient preference methodology


DISCRETE CHOICE ANALYSIS

- 
- Regulators familiar
 - Established use in quantitative benefit-risk assessments
 - Statistical robustness

- 
- Needs large sample sizes
 - Cost; limitations in some populations
 - Can handle 6-7 attributes maximally
 - Cognitively challenging for patients

ADAPTIVE CHOICE-BASED CONJOINT, BEST/WORST SCALING, SELF-EXPLICATED CONJOINT

- 
- Simpler designs (less cognitively challenging)
 - Smaller sample sizes
 - Larger number of attributes possible

- 
- Regulators / HTAs / Payers less familiar
 - Suitability for benefit / risk, trade-off's and utility measurements still tbd.

There are still many open questions concerning the conduct and use of preference studies

Why not just rely on patient group input, why do we need preference studies?

You didn't include my country, can the results be transferred?

Why not just stick to Discrete Choice as the definitive and accepted method?



What should be the role of Pharma in conducting preference studies?

From market research to science: what next?

Will preference studies contributing to better decision-making, or just complicate the picture?

Questions?