Analysis to Support Go/No Go Clinical Investment Decision

Assignment

- A large multinational pharma company needed to define the target product profile, clinical trial design requirements and market opportunity for several COPD product candidates to support a Phase 2a investment decision

Key Tasks

- Define the nature and scope of the opportunity associated with each asset by estimating the likely role each pipeline compound would have in the future treatment algorithm
- Create and validate target product profiles for the highest priority assets
- Provide guidance on clinical trial design including comparators, primary endpoints, and inclusion and exclusion criteria
- Provide prescribing estimates and sensitivities through in-depth interviews with high prescribing pulmonologists

Methodology

- Extensive secondary research; in-depth interviews accompanied by a web-based quantitative exercise with KOLs, pulmonologists and payers in the US, UK, Germany, France, and Italy

Deliverables

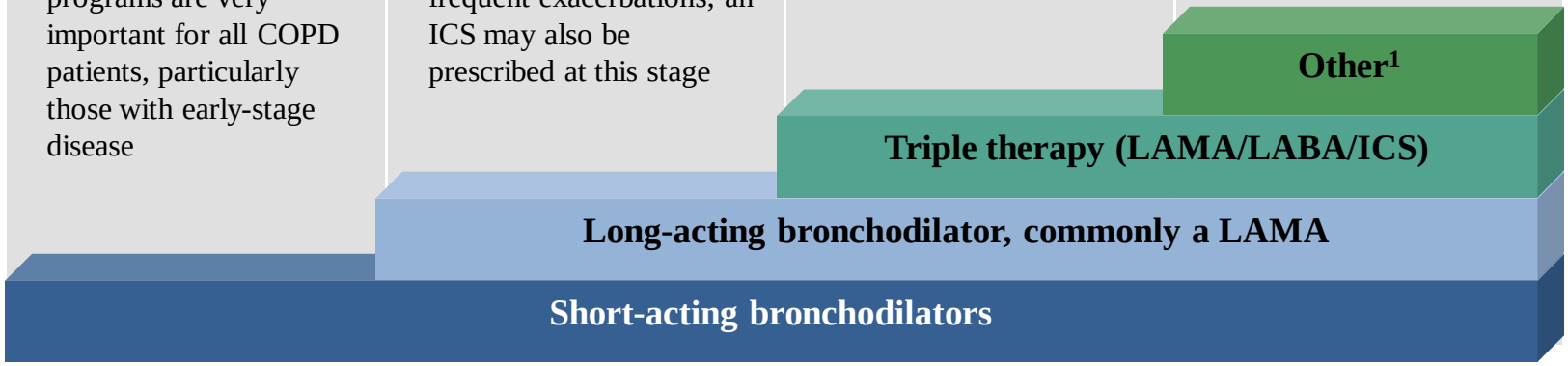
- An expert consensus on the product characteristics that would be required, at a minimum, to justify proceeding to a Phase 2a trial. Attributes identified included primary endpoints, minimum attribute values, and biomarker requirements
- The competitive positioning strategies required to maximize success given evolving standards of care and emerging competitors
- The data and insight necessary to forecast demand, including pulmonologist prescribing estimates and payer predictions about the barriers and opportunities for successful reimbursement

Treatment Hierarchy

New therapies are prescribed on top of (not instead of) existing therapies. Standard of care (SOC) for GOLD 3 and 4 patients is triple therapy, i.e. two long-acting bronchodilators and an inhaled steroid

Additive Drug Therapies for COPD

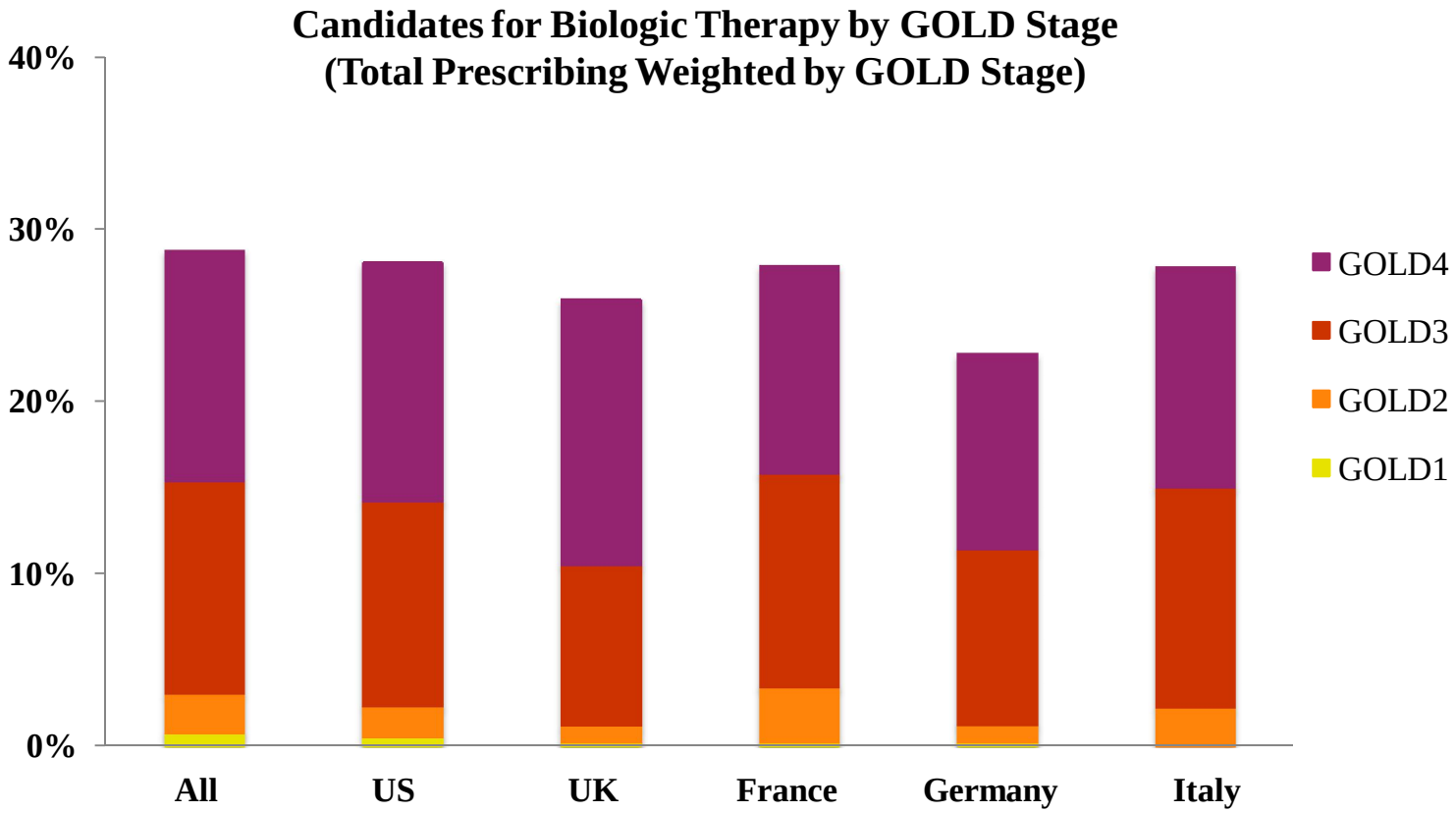
GOLD 1	GOLD 2	GOLD 3	GOLD 4
▪ Short-acting bronchodilator use prn typically controls symptoms in GOLD 1 patients ▪ Smoking cessation programs are very important for all COPD patients, particularly those with early-stage disease	▪ SOC for GOLD 2 patients is a long-acting bronchodilator , i.e. a LAMA, LABA or sometimes both ▪ If the patient experiences frequent exacerbations, an ICS may also be prescribed at this stage	▪ Physicians add therapies as symptoms worsen ▪ By GOLD stage 3, many patients are on triple therapy (LAMA/LABA/ICS)	▪ A variety of other approaches may be tried when symptoms continue despite optimal drug therapy



[1] *Supportive therapies including oxygen and pulmonary rehabilitation are commonly prescribed for late stage patients; patients may receive nebulized therapies at home; chronic oral steroids are prescribed for a very small subset of patients*

Candidates for a Biologic

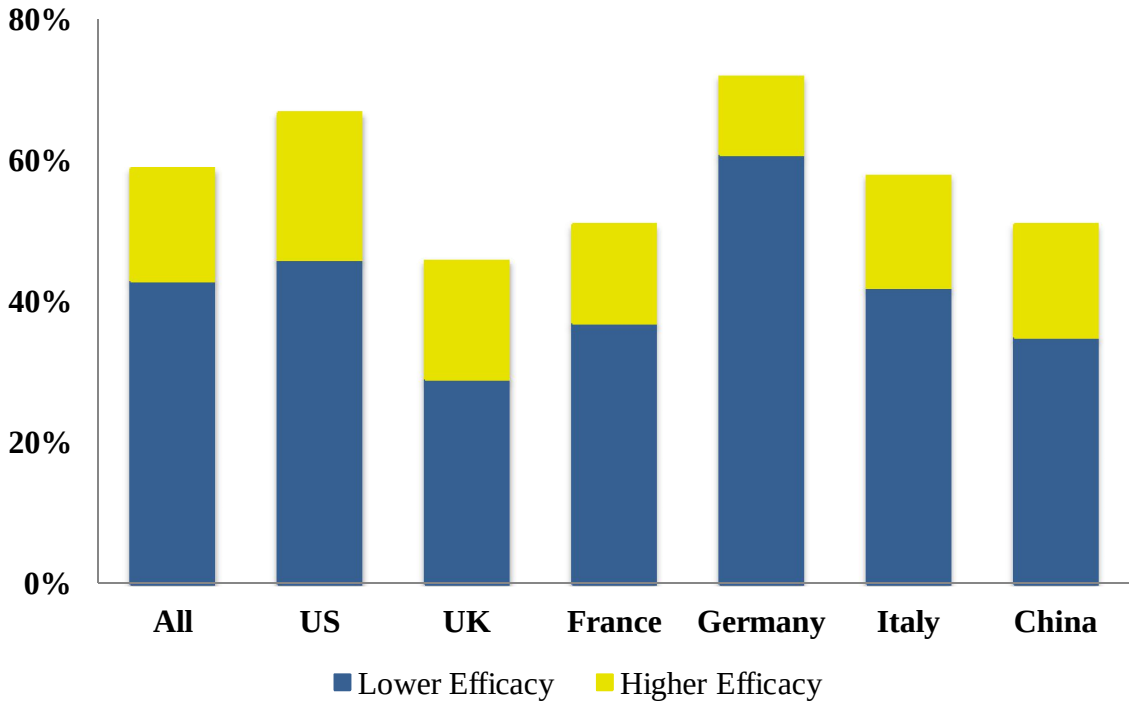
Of all the COPD patients treated by pulmonologists interviewed, almost 30% are candidates for a biologic therapy. Candidates are almost exclusively GOLD 3 and 4



Potential Prescribing

Although pulmonologists acknowledge qualitatively that the higher efficacy values are more desirable, this opinion translates to only slightly higher prescribing estimates due to the very high unmet needs among GOLD 3 and 4 patients

Incremental Difference Between Higher and Lower Efficacy Scenarios



Pulmonologist Comments

- *"We have to cherish the possibility of achieving a minimal improvement for patients at stage four"* (Italian KOL)
- *"I'll put them on the drug and see if it works [for them]. If it doesn't work, I'll take them off"* (German Pulmonologist)