

Putting patient priorities at the center of clinical endpoints



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When FEV₁ (lung function) doesn't tell the full story

Clinical Data

FEV₁ improvement:
<5% in 5 years

FEV₁ **<40% predicted**

Often **ineligible for trials**

May appear **minimal treatment response**

My Reality since modulator therapy

Hospitalizations: **every few months → once in 5 years**

Less daily mucus burden

Breathing is easier

Dramatically improved
quality of life

FIRST OPINION

One number keeps me from taking part in cystic fibrosis trials. That's wrong

By ELLA BALASA / NOVEMBER 5, 2018



Author Ella Balasa using a home spirometer to measure her lung function.

Why multiple endpoints matter to patients

For patients with more advanced lung disease, FEV₁ may not be a sensitive marker of therapeutic benefit because structural lung damage limits how much lung function can recover.

For me, real treatment benefit can be captured through:

- Quality of life improvements
- Reduced exacerbations & lower infection burden
- Functional capacity like SpO₂ & exercise tolerance
- Reduced cough

Numerous outcomes in CF trials



Lung clearance index

BMI

Infections

FEV₁

Oxygen Saturation (SpO₂)

CT lung damage

Pulmonary exacerbation rate

Antibiotic usage

Overall quality of life

Fatigue

Weight change

Treatment burden

Exercise tolerance

Bacterial load

Hospitalizations due to
infections /respiratory causes

Coughing

Work attendance

Respiratory symptoms

Inflammatory biomarkers

Sweat chloride concentration

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Including multiple prioritized outcomes into a single treatment assessment: a beginner's guide

Experimental



Control

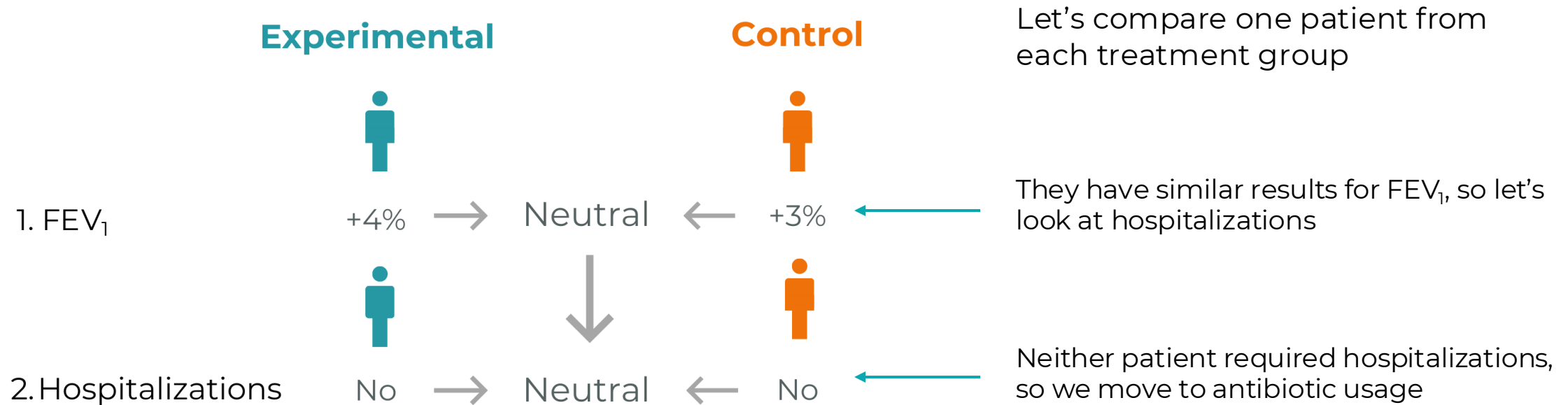


Let's compare one patient from
each treatment group

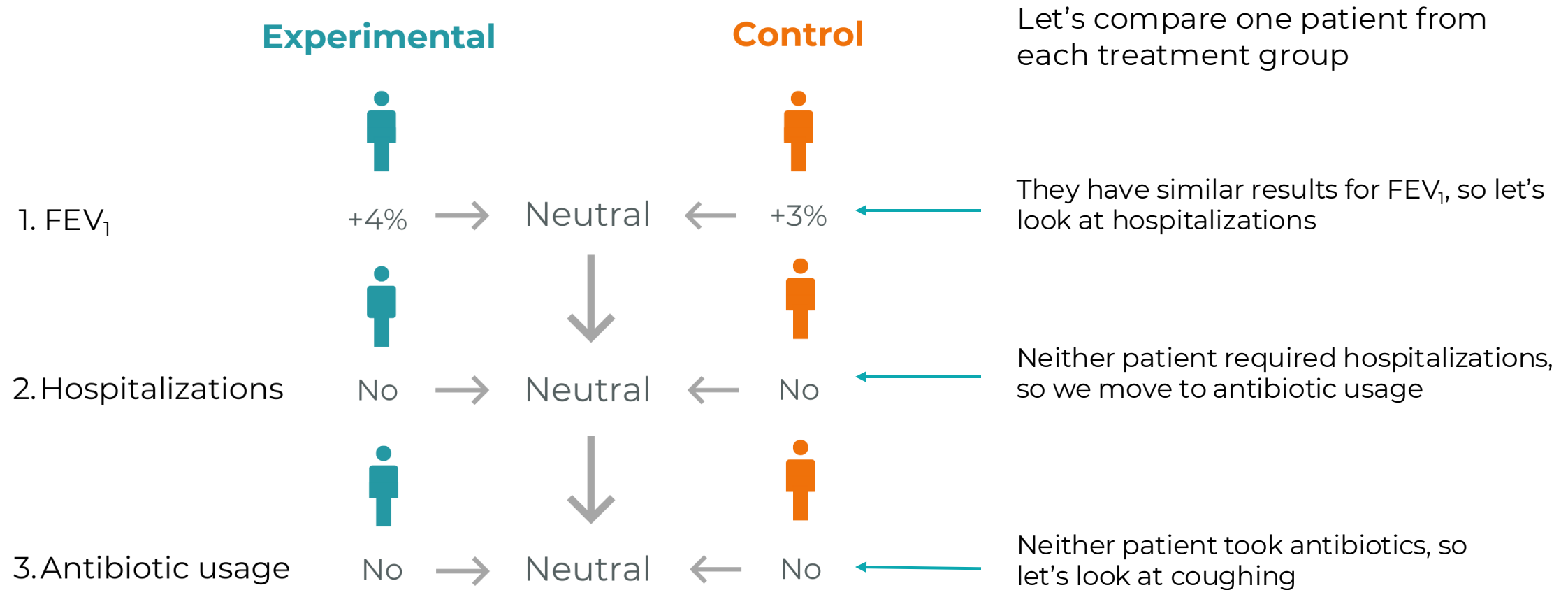
Including multiple prioritized outcomes into a single treatment assessment: a beginner's guide



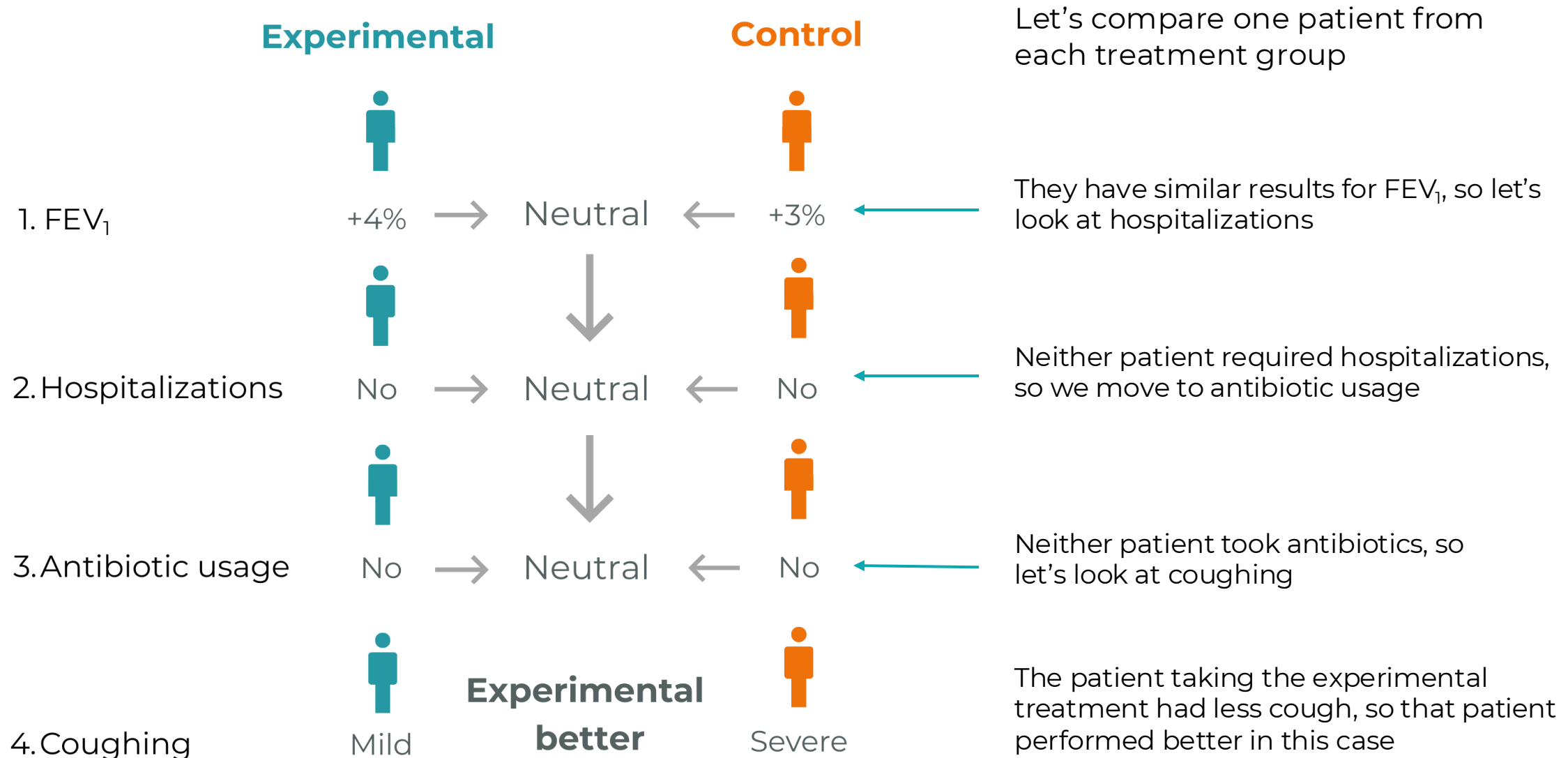
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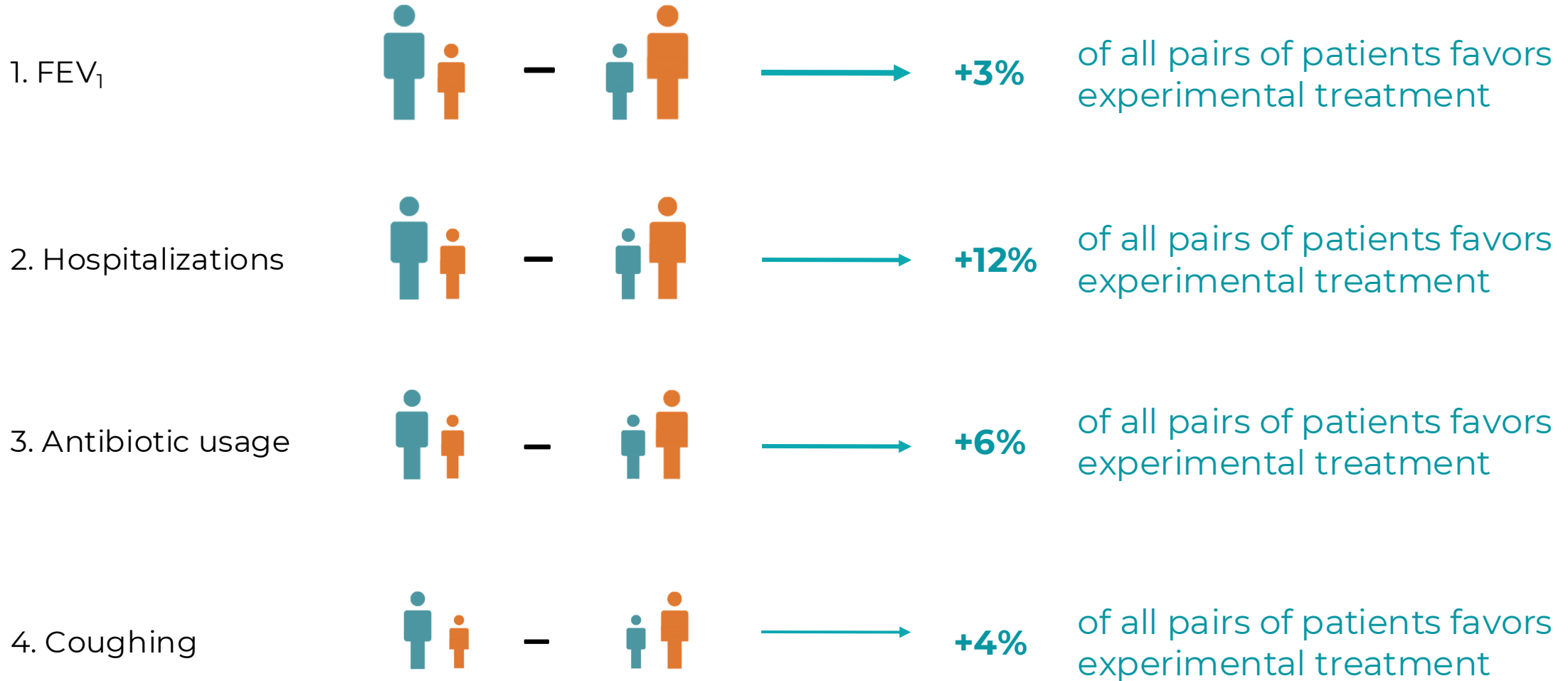


Let's count every possible pair of patients to estimate the "Net Treatment Benefit"



25% Net Treatment Benefit

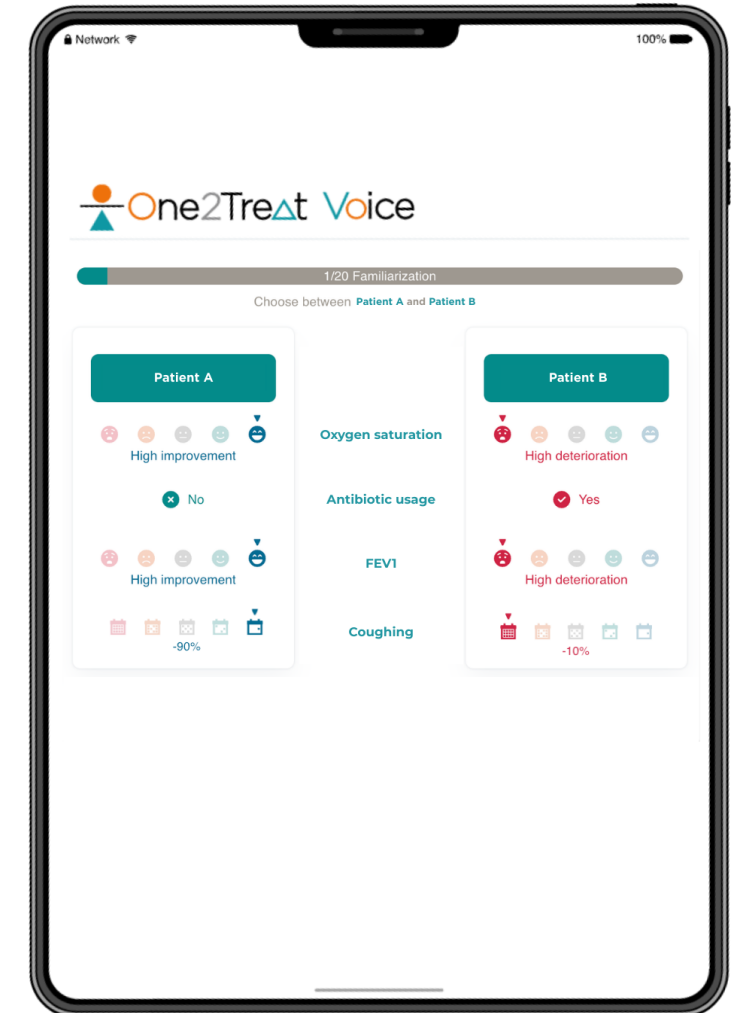
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25% Net Treatment Benefit

But how to select and prioritize outcomes across all stakeholders?

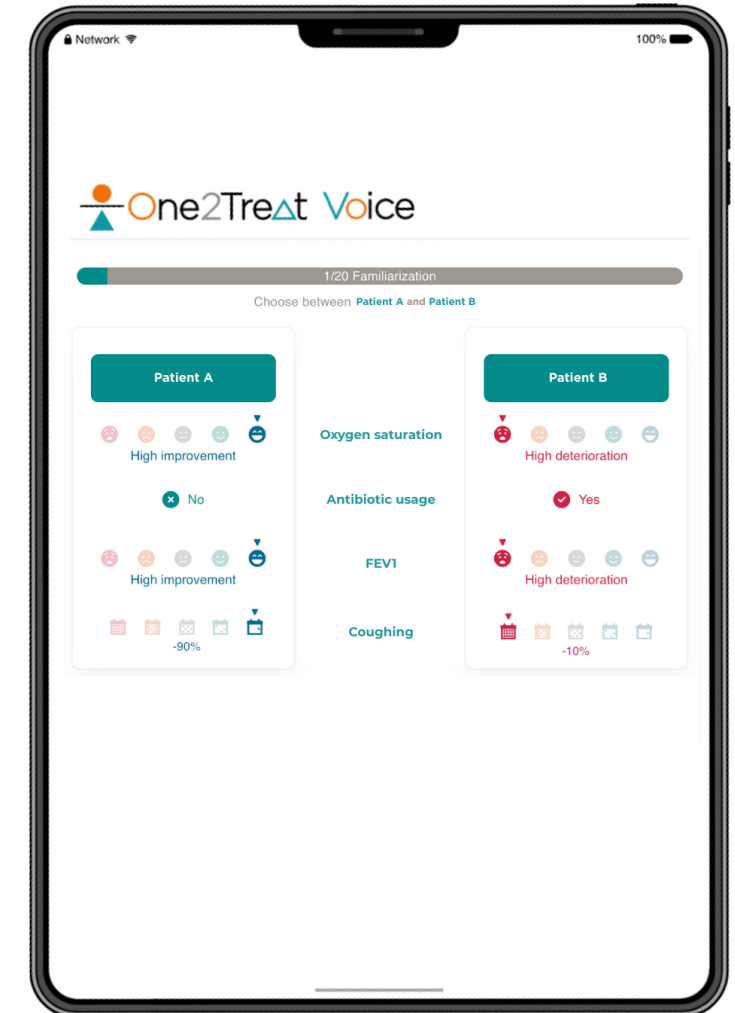
One2Treat Voice[®] software to engage physicians and patients to select outcomes across multiple stakeholder groups



But how to select and prioritize outcomes across all stakeholders?

One2Treat Voice[®] software to engage physicians and patients to select outcomes across multiple stakeholder groups

Quantitative framework to elicit patient preferences and define multidimensional endpoint



Incorporate multiple outcomes into a patient-centric endpoint to:

- Engage stakeholders to **align patients and sponsors**
- Capture multidimensional **patient preferences** instead of relying on a single endpoint
- **De-risk** development by detecting meaningful treatment effects

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- Engage stakeholders to **align patients and sponsors**
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- **De-risk** development by detecting meaningful treatment effects
 - **Better clinical trial endpoints**
 - **More inclusive trials**
 - **Better treatments for patients**

Thank you



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