

Your Voice Matters: How Your Opinions Shape the Medicines You Get

For teens living with rare conditions

Think about medicines for things like bad acne, allergies, or even something more serious down the road.

Did you know your thoughts and feelings can actually influence which medicines become available?

Let's find out how!



What's Benefit-Risk Assessment? The Big Decision!

Benefits

- What good things the medicine does. For example, it might clear up a rash or stop a headache.

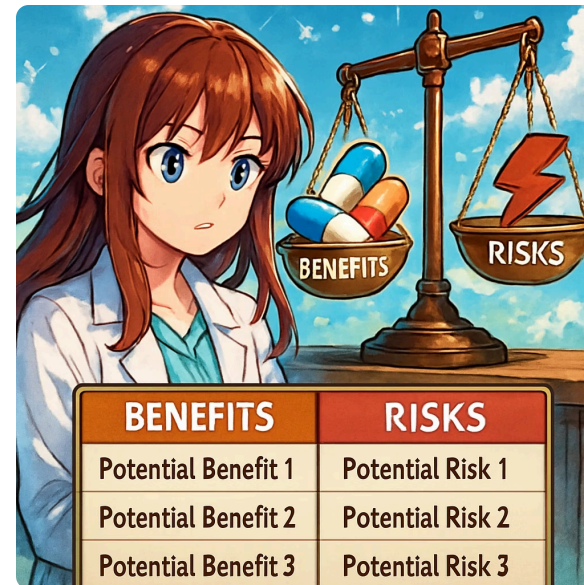
Risks

- What bad things the medicine might cause, like adverse events (nausea/vomiting, stomach ache).

Imagine a new medicine is being developed.

Before it can be used, health authorities (like people who make sure medicines are safe) have a big decision to make. They look at two main things:

They compare the **benefits** to the **risks** to decide if the medicine should be allowed. This is called benefit-risk assessment.



Your Opinion Counts!

What is Patient Preference?

Medicine A

Works fast but might make your skin itchy for a while.

Medicine B

Works slower but is less likely to make you itchy.

What seems like a good balance of benefits and risks can be different for different people. This is where **you** come in!

Think about it: Two medicines for a skin rash:

- Which would **you** prefer? Your choice is your **patient preference!** It's what you think is an acceptable trade-off between the good and bad things a medicine might do.





Why Does Your Preference Matter to the People Deciding About Medicines?

Different People, Different Priorities

What bothers one person a lot might not bother someone else.



Real-Life Choices

You're the one who will be taking the medicine!

Knowing what you prefer helps **make sure** the available **medicines are ones people are actually willing to use** and fit into their lives.



More Than Safe & Effective

Health authorities are starting to realise that just checking if a medicine works and is safe isn't the whole story.

They **need to know what patients actually think!**

Patient Preference Questionnaire for Adolescents with Rare Diseases



<https://forms.office.com/e/zGhkrsfjgS>

How Do They Find Out What You Prefer?

The Patient Preference Studies



You Compare Options

Choose the option you would actually want—or select "none" if none appeal.



Your Choices Reveal Trade-offs

How much benefit feels worth a certain side-effect?

Your selections show what matters most to you personally.



Scientists Analyse Patterns

Researchers identify which features matter most to teens like you.

They find patterns across hundreds of choices from many participants.



Results Guide Real Decisions

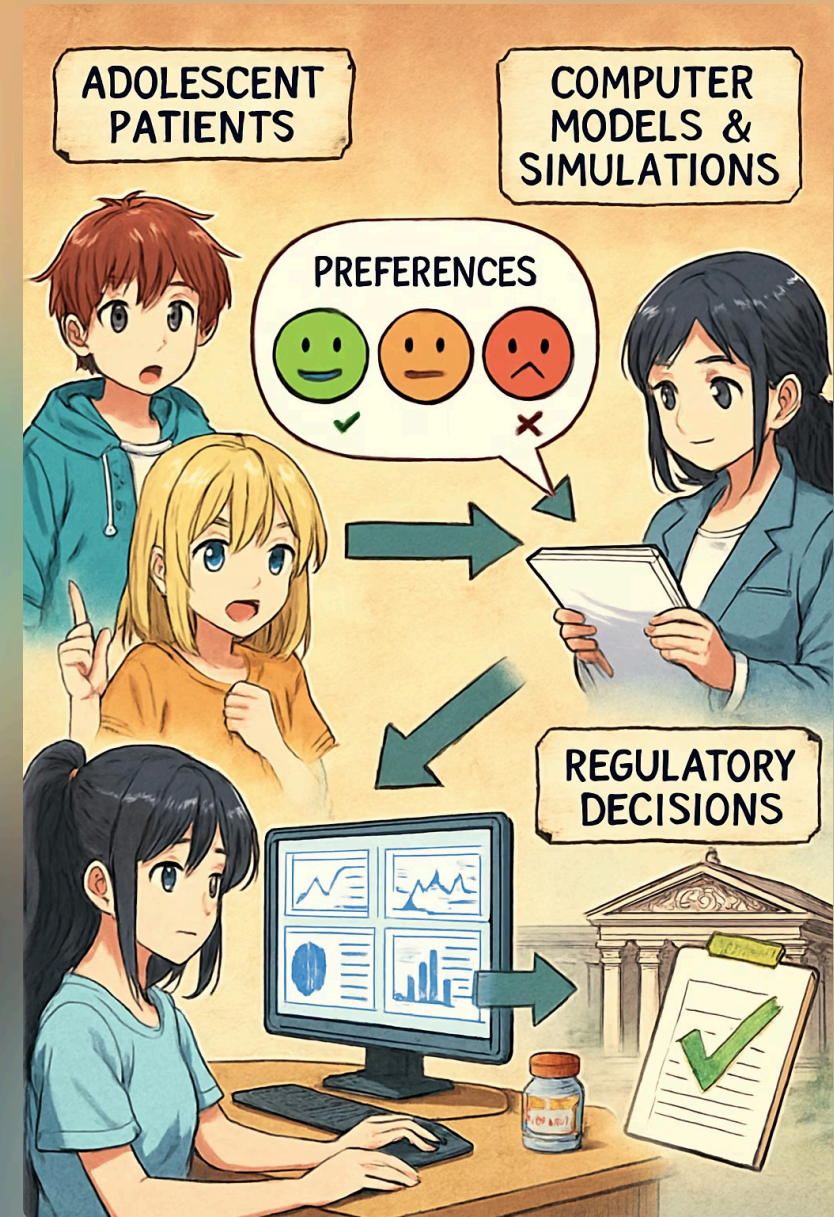
Regulators, companies, and doctors use these findings.

They create medicines that better fit into patients' actual lives.

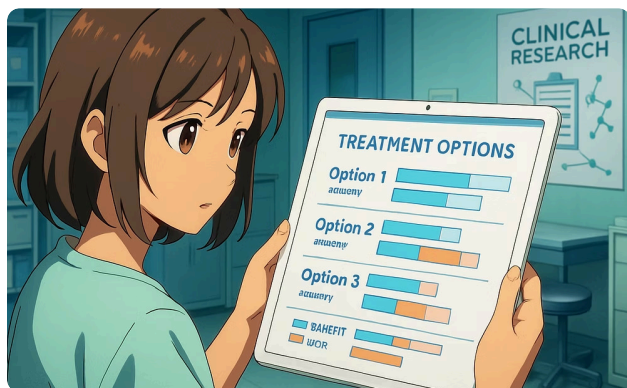
Combining Your Voice with Powerful Predictions

Patient Preferences in Action

Understanding patient preferences helps create better medicines that align with what patients actually want and need.



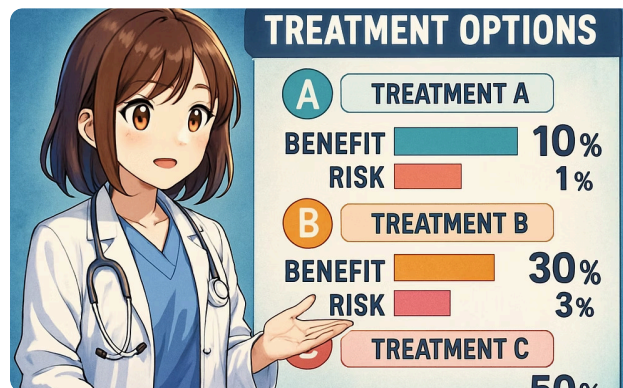
Understanding What Patients Value: example with Discrete Choice Experiments (DCEs)



Treatment Choices

Imagine being asked to choose between different potential treatments.

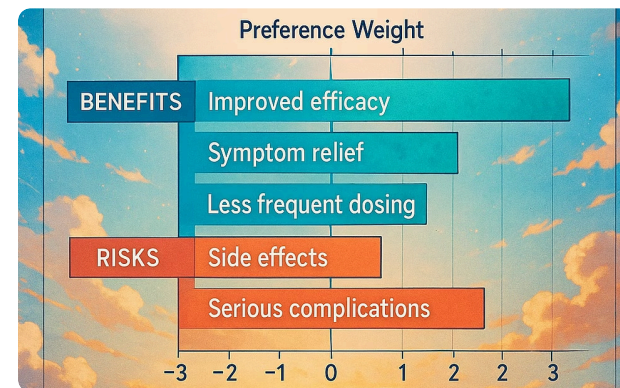
Each treatment option has different features (benefits and risks) at different levels.



Example Features

Benefit Example: Chance of hair regrowth (e.g., 10%, 30%, 50%).

Risk Example: Chance of serious infection (e.g., 1%, 3%, 6%).



Preference Weights

By analysing the choices people make, researchers can understand how much importance (or **preference weight**) patients place on each benefit and risk.

This is a way of **modeling** patient preferences quantitatively.

Essentially, we are modeling **how patients make trade-offs between the good and bad aspects of a treatment.**

Applying Preferences to Clinical Trial Data: A Form of Simulation

Preference Weights

Once we have these preference weights, **we can use them with the results from clinical trials.**

How much risk is okay?

We can **estimate the highest level of risk patients would be willing to accept for a certain level of benefit.**

This is like simulating how much risk is "worth it" for a given result.

Preference Shares

We can **calculate the probability that a typical patient would choose one treatment over another based on their benefit and risk profiles observed in the trial.**

This simulates treatment choices in a population with the identified preferences.

How confident are we?

We can even assess how robust these probabilities are when **we consider the uncertainty in both the clinical trial data and the patient preference data.**

This adds another layer to the simulation by accounting for variability.

Real Example: Alopecia Areata (Hair Loss)



Scientists developed a new medicine for alopecia areata

They knew it could help hair grow back (a big benefit!), but it also had some risks.



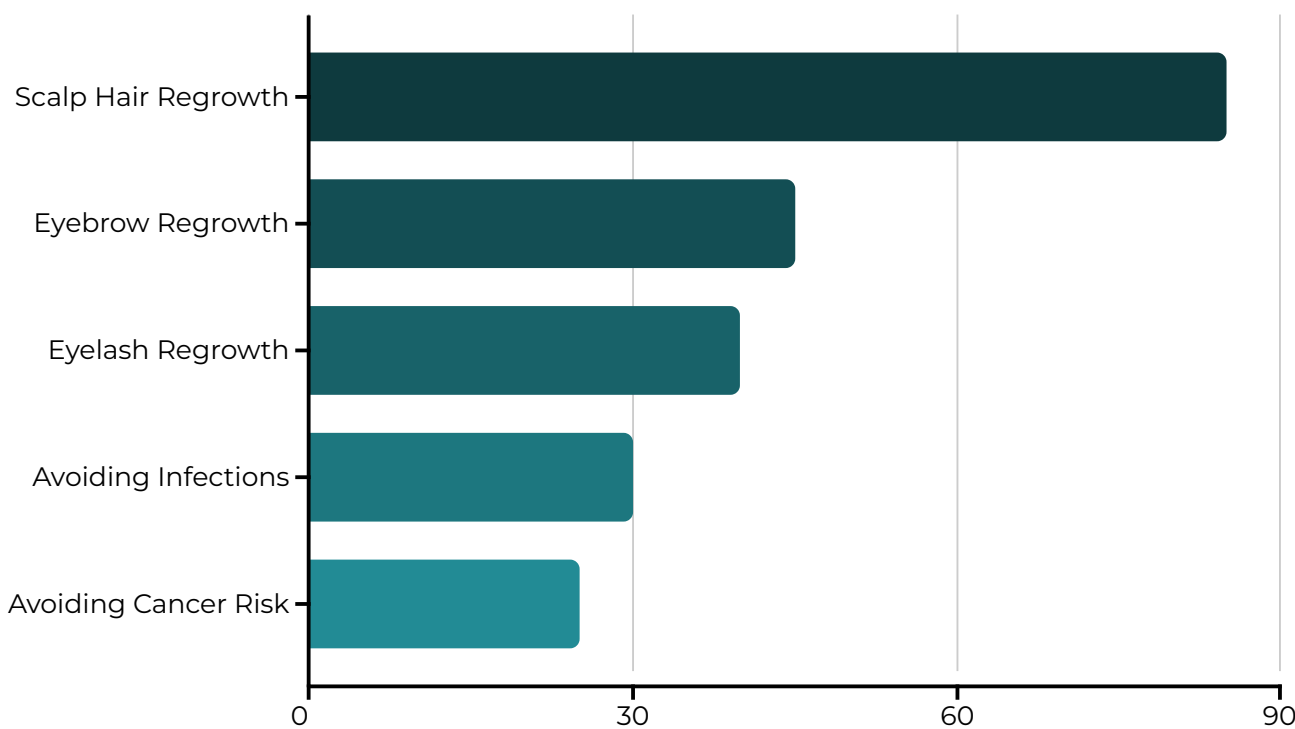
Patient Preference Study

They did a patient preference study, asking people with alopecia areata about their feelings regarding hair regrowth and associated risks.



Discrete Choice Experiments

They used Discrete Choice Experiments to understand patient preferences for hair regrowth on different areas versus the risk of serious infections, thromboembolic events, and malignancies (cancer).



What they found?

Regrowing hair on their scalp was very, very important to these patients, and they were willing to accept some risks to achieve that.

1 Maximum Acceptable Risk Estimation

Researchers **used preference weights with clinical trial data** to estimate the maximum acceptable risk for side effects.

2 Patient Perspective Importance

Health authorities learned that patients prioritize the benefits of the medicine over the risks.

3 Approval Decision Factor

Understanding patient perspectives was **crucial for health authorities when deciding on medicine approval.**

The Goal: Patient-Centered Decisions

Preference Modeling

Understanding what matters most to patients through structured research

Better Decisions

Approving medicines that align with patient values



Clinical Evidence

Gathering scientific data on how treatments work

Benefit-Risk Assessment

Evaluating treatments from the patient's perspective

Benefit-Risk Trade-offs

By combining patient preferences modeling with clinical trial results, we understand the trade-offs from the patient's perspective.

Regulatory Decisions

Informing regulatory decisions on whether a medicine's benefits are acceptable to patients given its risks.

Product Information Development

Developing product information that highlights what matters most to patients.

Alignment with Patient Values

Goal: Medicines aligned with what patients value and are willing to accept.

Conclusion: Your Voice is Important!



Patient-Centered Medicine

Patient preference is all about making sure the medicines available are not just scientifically good, but also good for you as a person.

Valued Opinions

Your opinions and feelings about the benefits and risks of treatments are valuable and are increasingly being considered when deciding which medicines you might have access to in the future.