

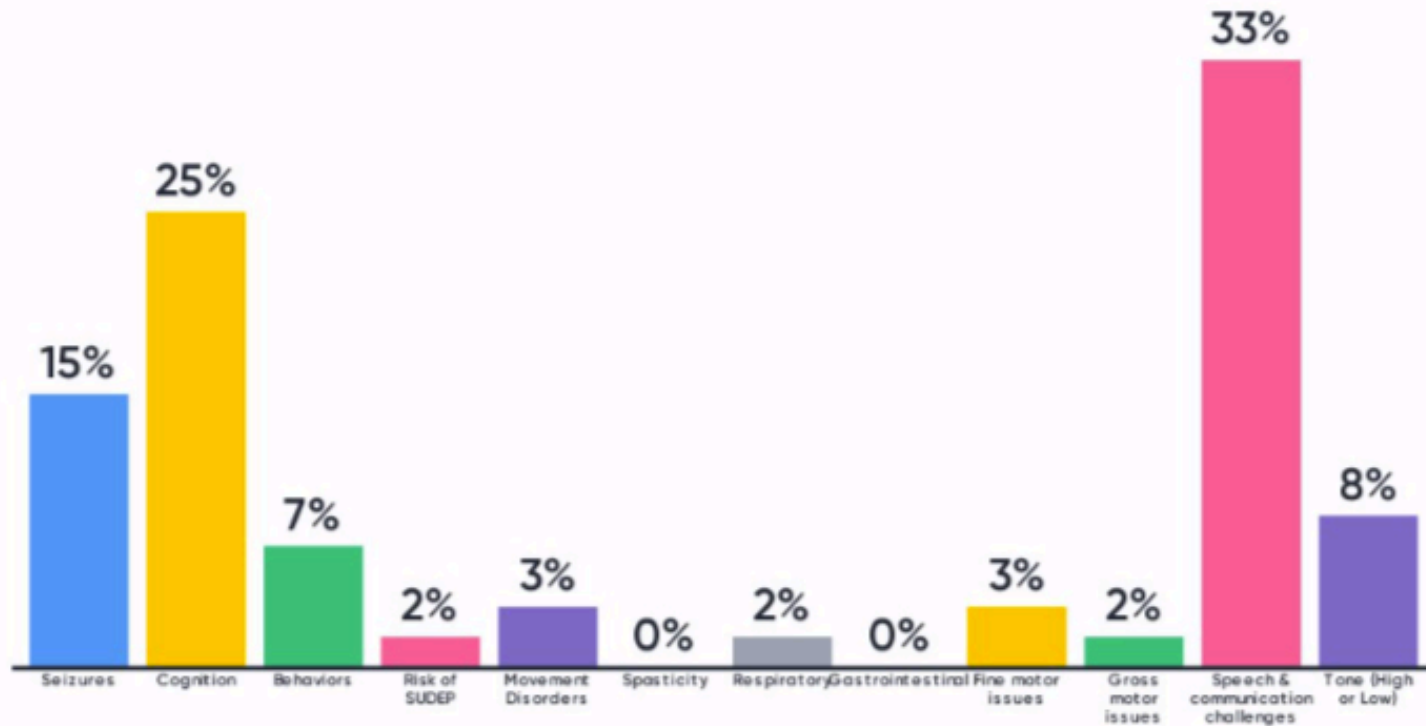


+ Raw Mentimeter Results &
Key Themes

How do you feel coming into this meeting?

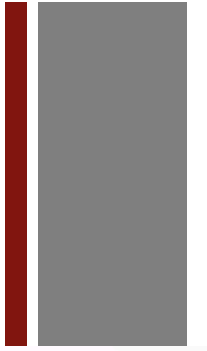


What are the most difficult symptoms of STXBP1?

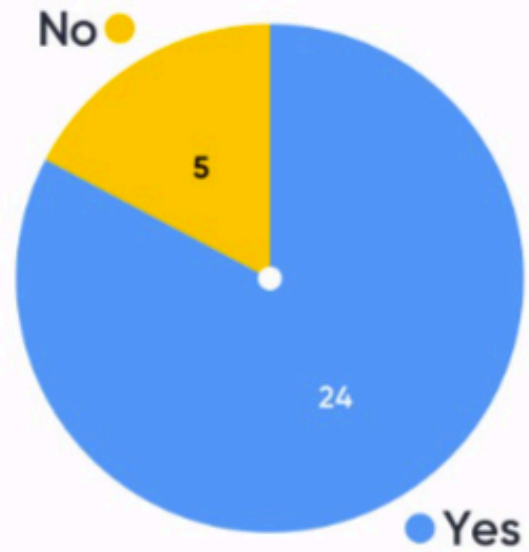


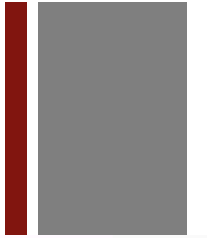
What else?



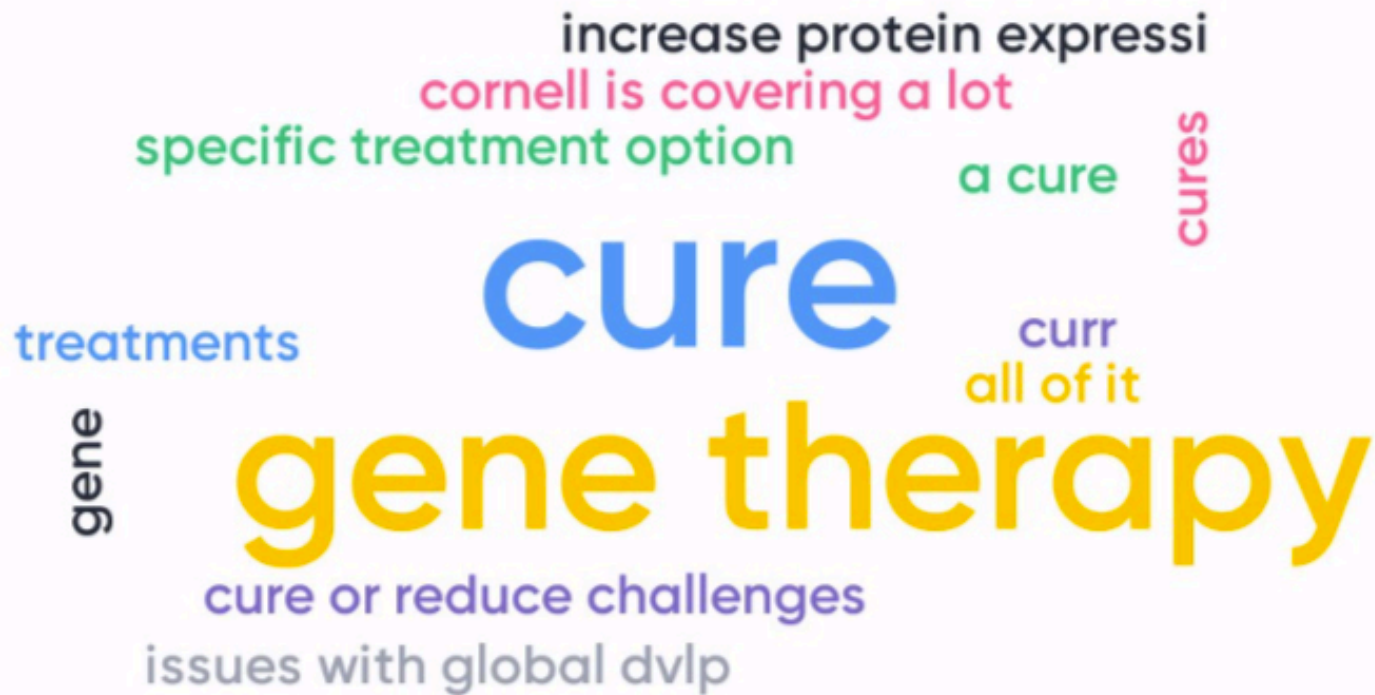


Would you invest in research even if it may not benefit your child?





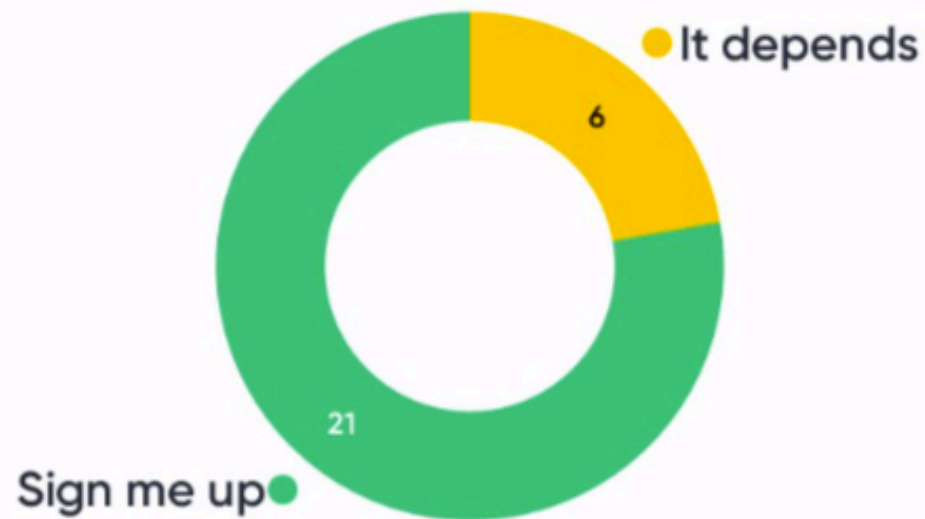
What research do you want to see long-term?



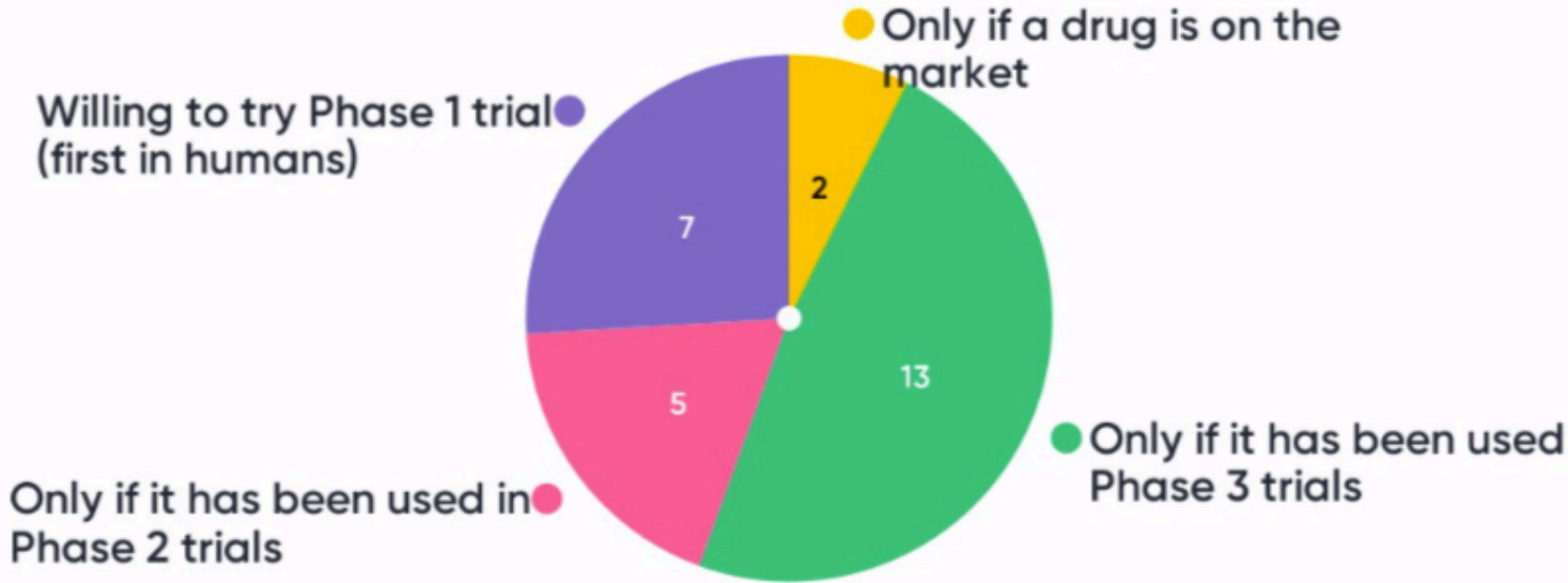
How will you know if a therapy is working?



Would you participate in a natural history study to understand disease progression?



Would you participate in a drug study?



What was the most interesting thing we learned...

Phenylbutyrate and Curcuman Oil

Drug therapy options

The amazing research that is being done already

Where we are for research

Science works in network

Gene therapy

Very hard to narrow down . . . Detailed mechanism of the syntax binding disorder

The increased amount of research and awareness that surrounds STXBP1

Both meeting other families for the first time and possible treatments

Progress with curcurma essential oils

Pharma / compound studies

Model systems and drug information

What was the most interesting thing we learned...

Research underway

Botanical and plant drugs

Even though there is a broad spectrum of disorders, there are still groupings of types of the STX disorder.

About phenylbutyral increasing brain activity

All of the different ways in which people are studying STXBP1

Promising Research

Let the research is paying off and successful in the labs

Botanical and natural drugs

That the scientific community is actively researching STXBP1!

Progression in animal studies being done

Drug therapy options

What was the most interesting thing we learned...

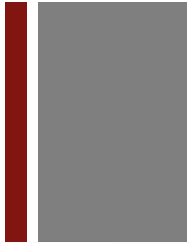
Worm study

How the protein works and what is being done to understand what is normal and what is not...and hopefully that will lead to a list of direct symptoms proven by lack of this protein.

The various forms of research around the world

The attention that our rare disease is receiving in the research field - especially the Cornell study that seems very promising.

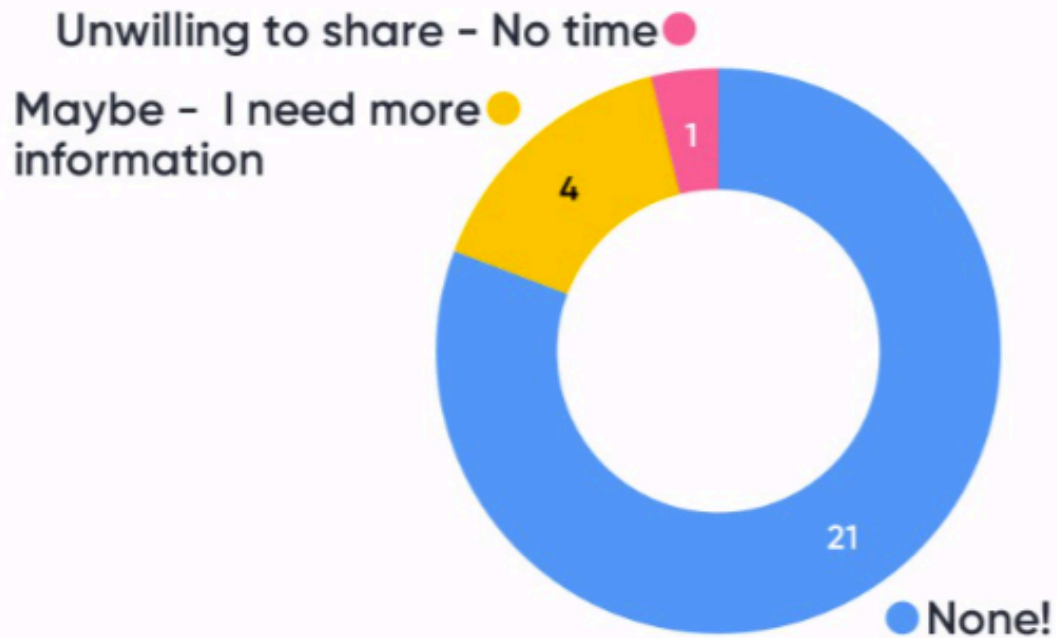
Mortality rate is low for STXBP1



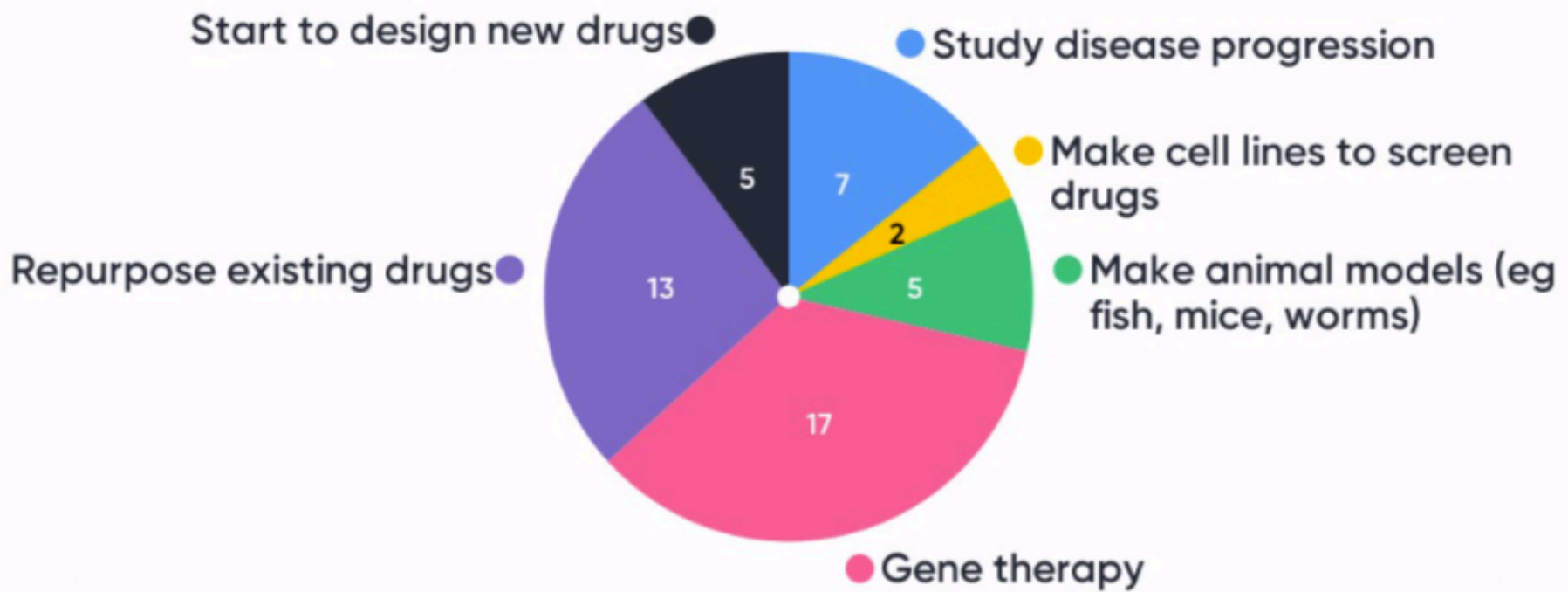
What are your impressions of today?

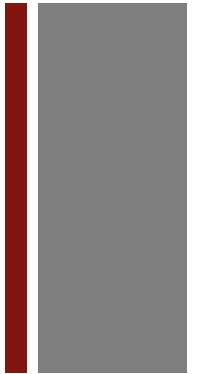


What are hurdles to joining the Simons Registry??

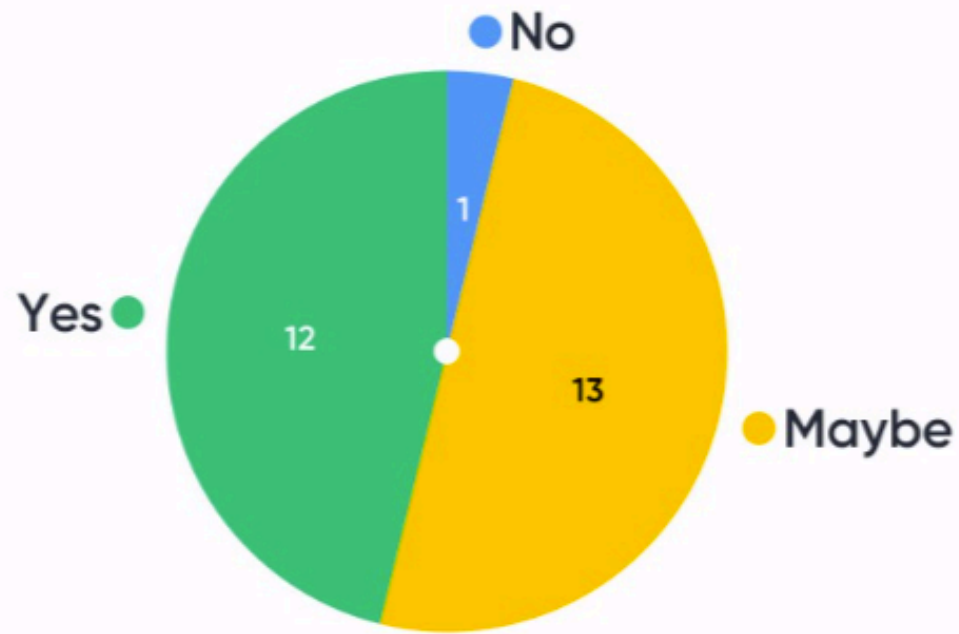


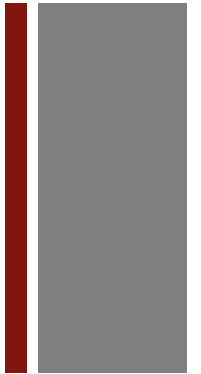
What research do you want in the next 1-5 yrs



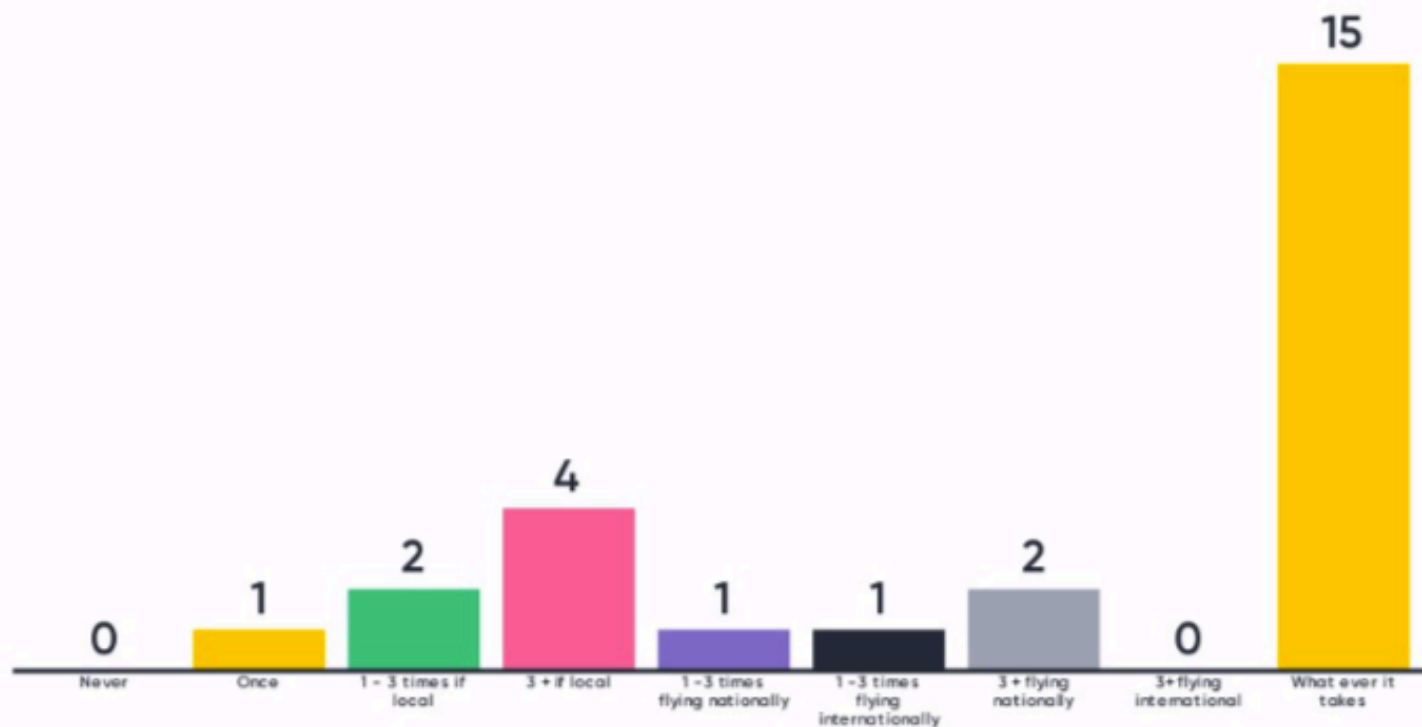


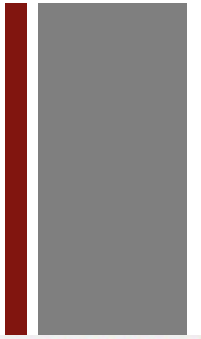
Would you consider gene therapy?





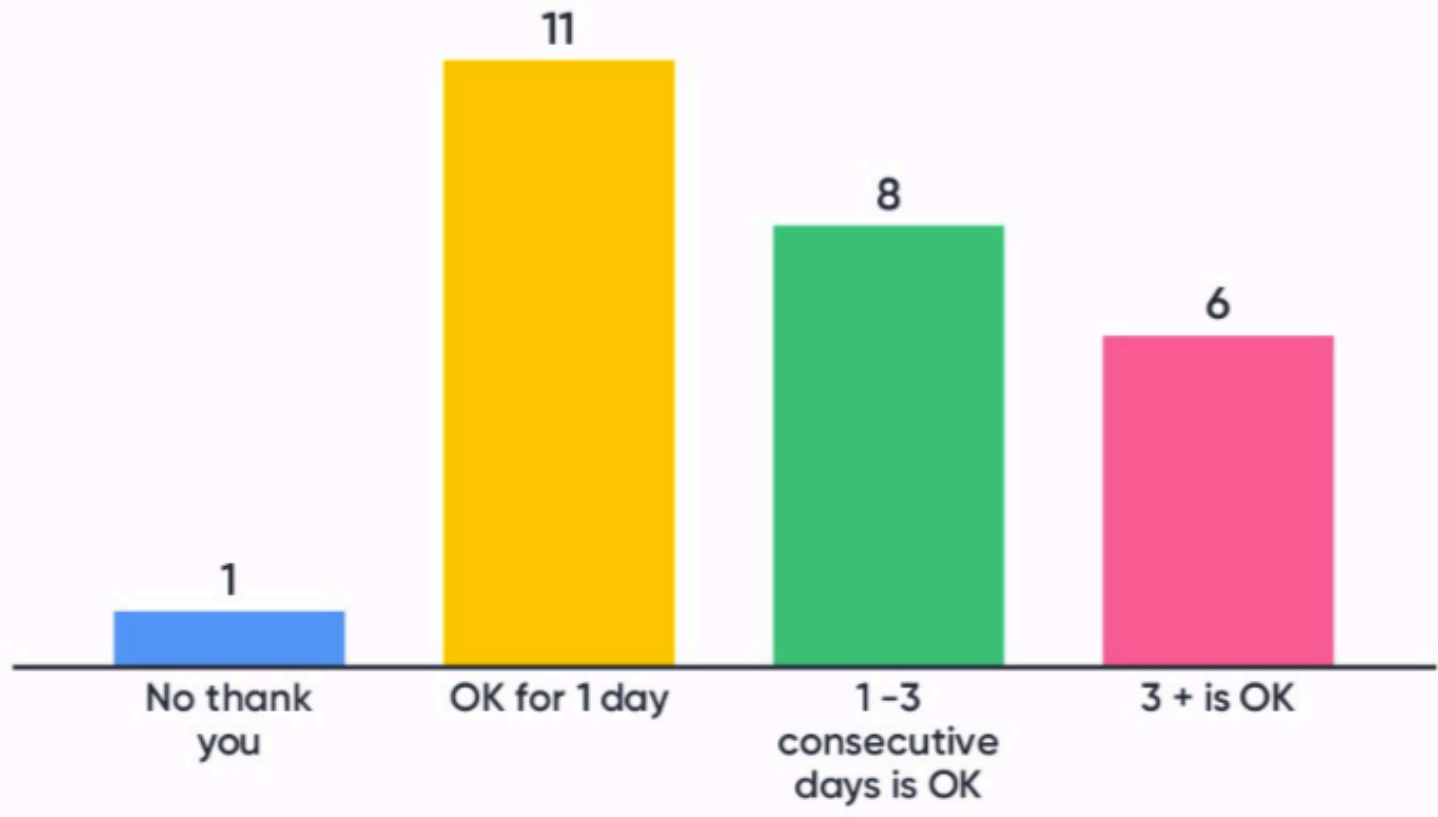
How often would you be willing to travel?



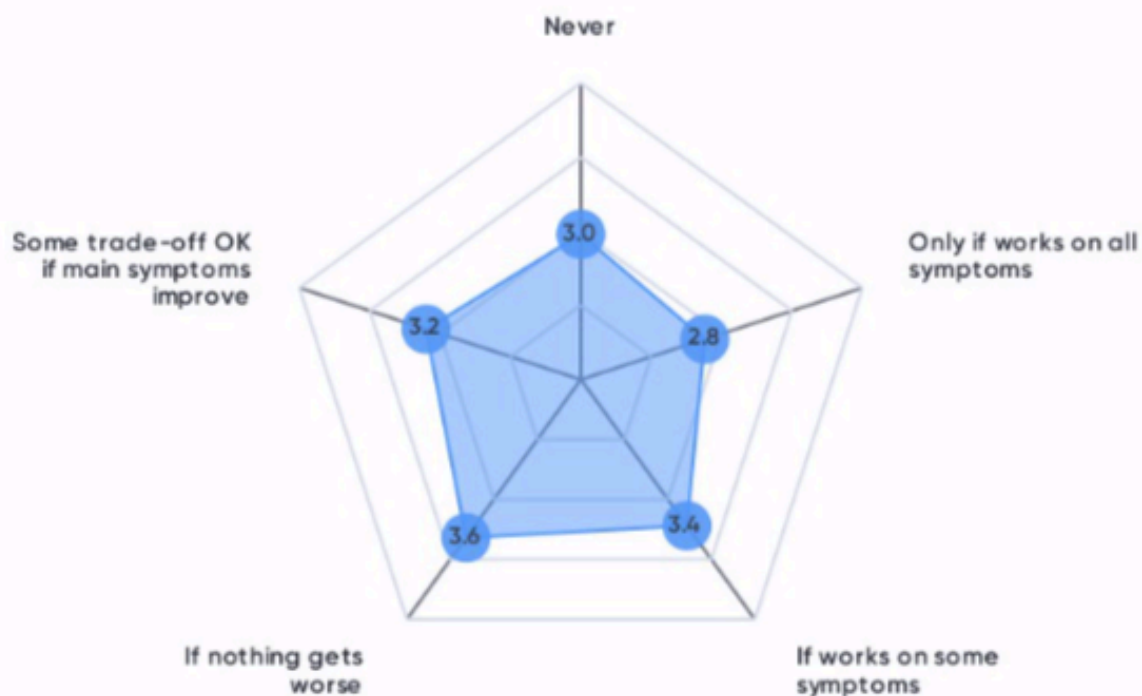


Mentimeter

Is hospitalization OK during a study?



Would you give a drug that is being tested?



What risks are acceptable in a drug study?

