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Boosting the Appeal of Clinical Trials—Making Participation of Greater Interest to Patients

Richie Kahn, Canary Advisors

Wes Michael, Rare Patient Voice



Richie Kahn is a health policy professional by training, clinical researcher by trade, and patient advocate by necessity. A rare disease patient and clinical trial participant himself, Richie is intensely passionate about better incorporating the patient perspective into the clinical development process. Through his work at Canary Advisors, he assists organizations set on patient-focused drug and device development by serving as an early stage patient advocacy function; supporting regulatory patient advocacy; crafting patient-facing market access and reimbursement strategies; and providing clinical trial support services.



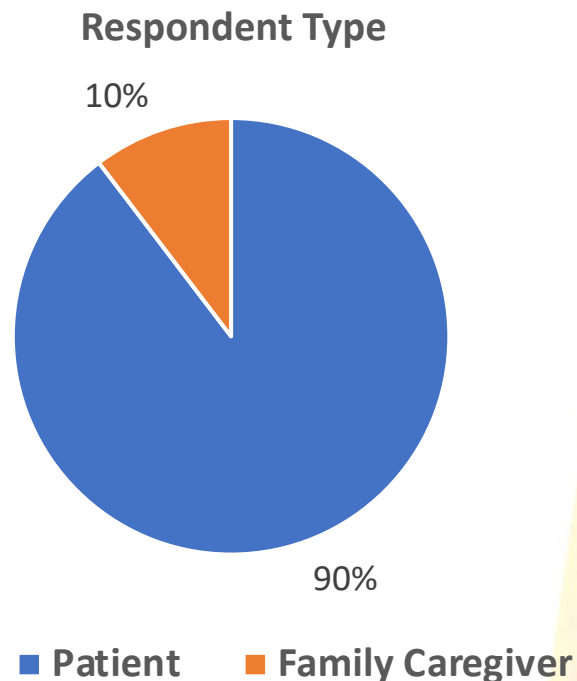
Wes Michael founded Rare Patient Voice LLC in 2013 to give patients and family caregivers with rare diseases the opportunity to voice their opinions in research studies. Over the past decade, the company has conducted thousands of studies and rewarded patients and caregivers with over \$10 million for their participation. Many have been recruited in person by Wes and his team at patient events and through a referral program with patient advocacy and support groups. RPV now covers non-rare as well as rare diseases and conditions and has expanded from the United States to Canada, the United Kingdom, France, Germany, Italy, Spain, Australia, and New Zealand.

Clinical trials – the necessary but challenging step in drug development

- ❖ A critical part of the drug development process, but only 15% of trials meet projected timelines
- ❖ Patient recruitment can be difficult, particularly in the rare disease space, where populations are small.
- ❖ Drug companies stand to lose between \$600,000 and \$8 million each day a product is delayed from coming to market.
- ❖ How can the clinical trial process be made more appealing for patients and family caregivers?

Rare Patient Voice 2022 patient survey

- In September 2022, Rare Patient Voice conducted a survey of 1,989 patients and family caregivers (loved ones helping with patient care) in the United States
- Questions pertained to patient preferences regarding clinical trials

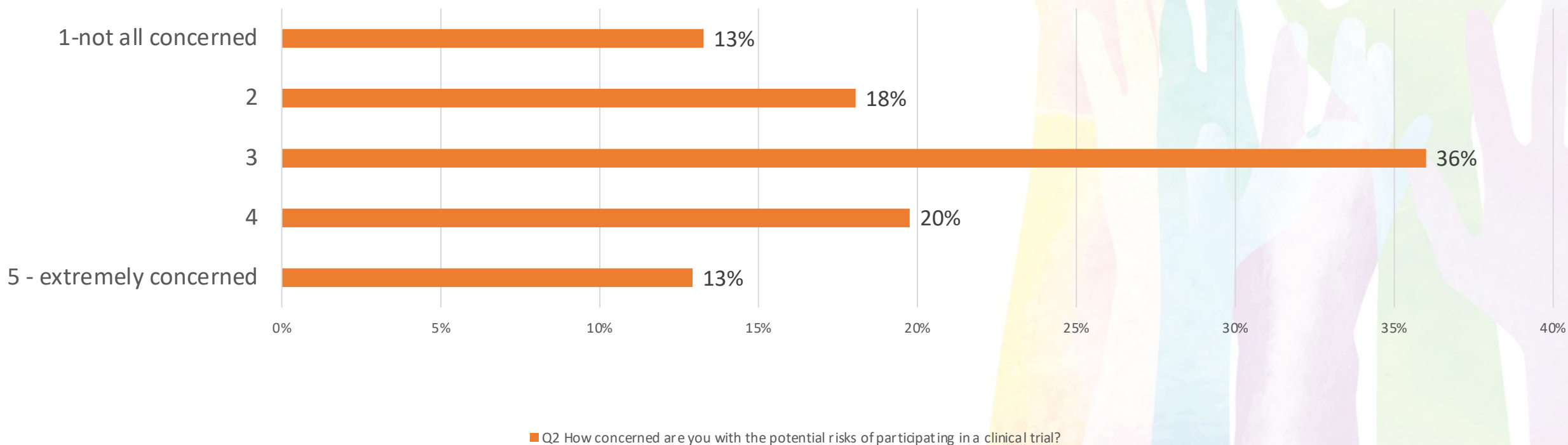


Several areas emerged of greatest interest to patients and family caregivers.

- Risks vs. benefits of clinical trials
- Need for communication throughout the trial
- Location of trial
- Preference for compensation/reimbursement

Patients were split on potential risks of taking part in clinical trials.

Q2 How concerned are you with the potential risks of participating in a clinical trial?



Thoughts on potential clinical trial risks included:

- **Fear of the unknown**
 - “By nature, clinical trials are testing untested treatments, so the potential risks are unknown.”
 - “I’m putting my child at risk for a plethora of different wretched things that could happen to him.”
 - “It’s a gamble with your life.”
- **Wary of potential side effects**
 - “I can’t risk those side effects. I’m a single mother.”

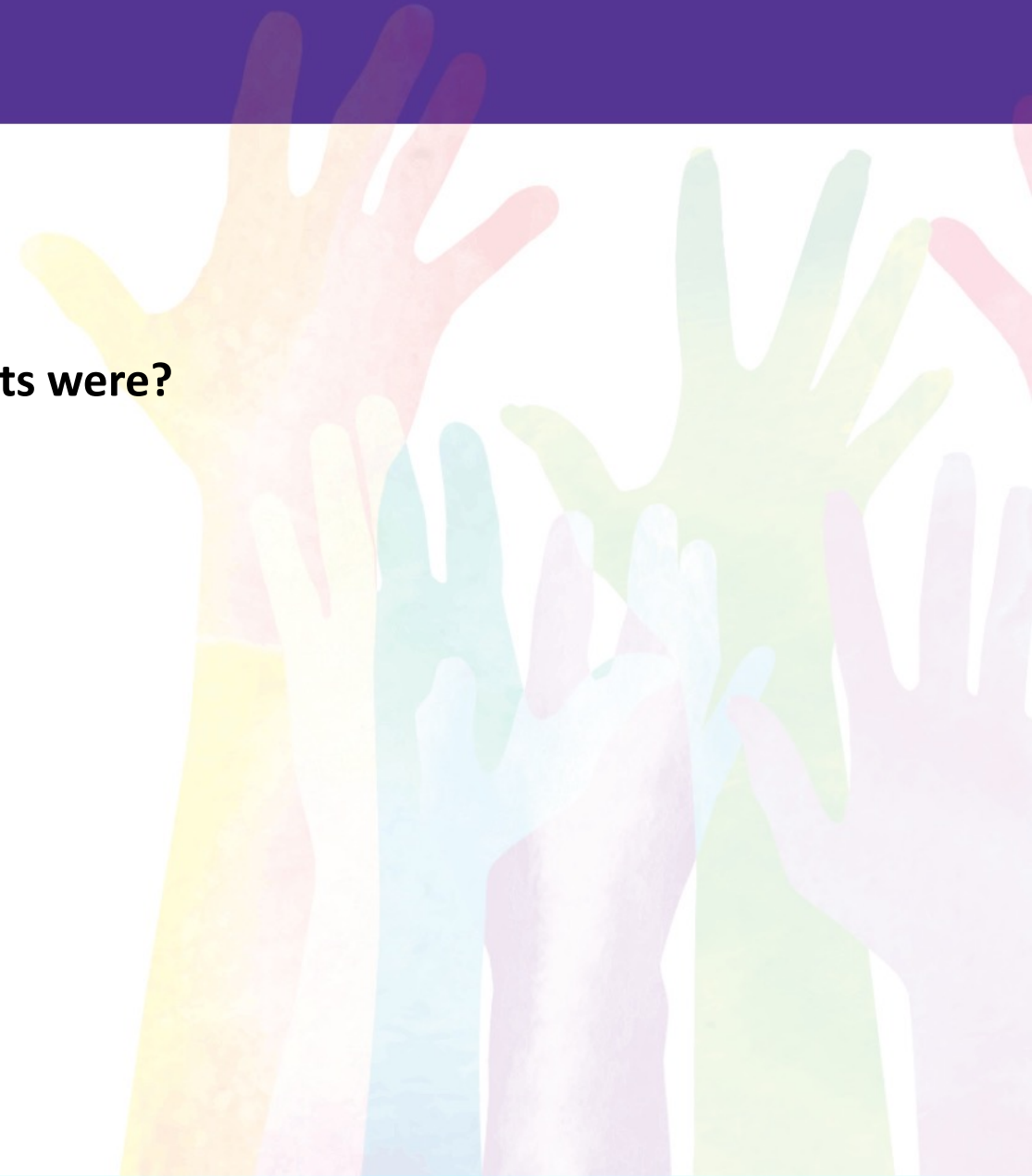
Thoughts on potential clinical trial risks cont'd:

- **They already suffer from a condition, they don't want to make it worse.**
 - ❑ "I already have serious health problems from my diseases and have them relatively managed and don't want to risk that."
- **It might not help.**
 - ❑ "I'm the Guinea pig. And if it's double blind I might get a placebo."
- **It might help but . . .**
 - ❑ "I am told that if I get in a trial and the trial ends, the medication can be terminated even if there are positive results in helping me survive."

POLL QUESTION

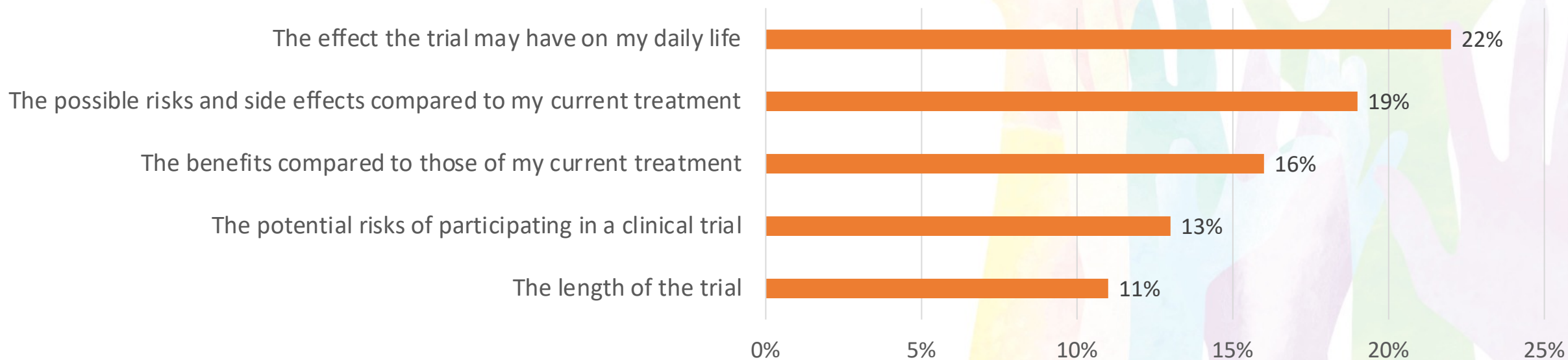
What do you think the key areas of most concern for patients were?

- Benefits, compared to my current treatment
- Effects on my daily life
- Length of the trial
- Possible side effects
- Potential risks of taking part in a trial



What are patients most concerned about?

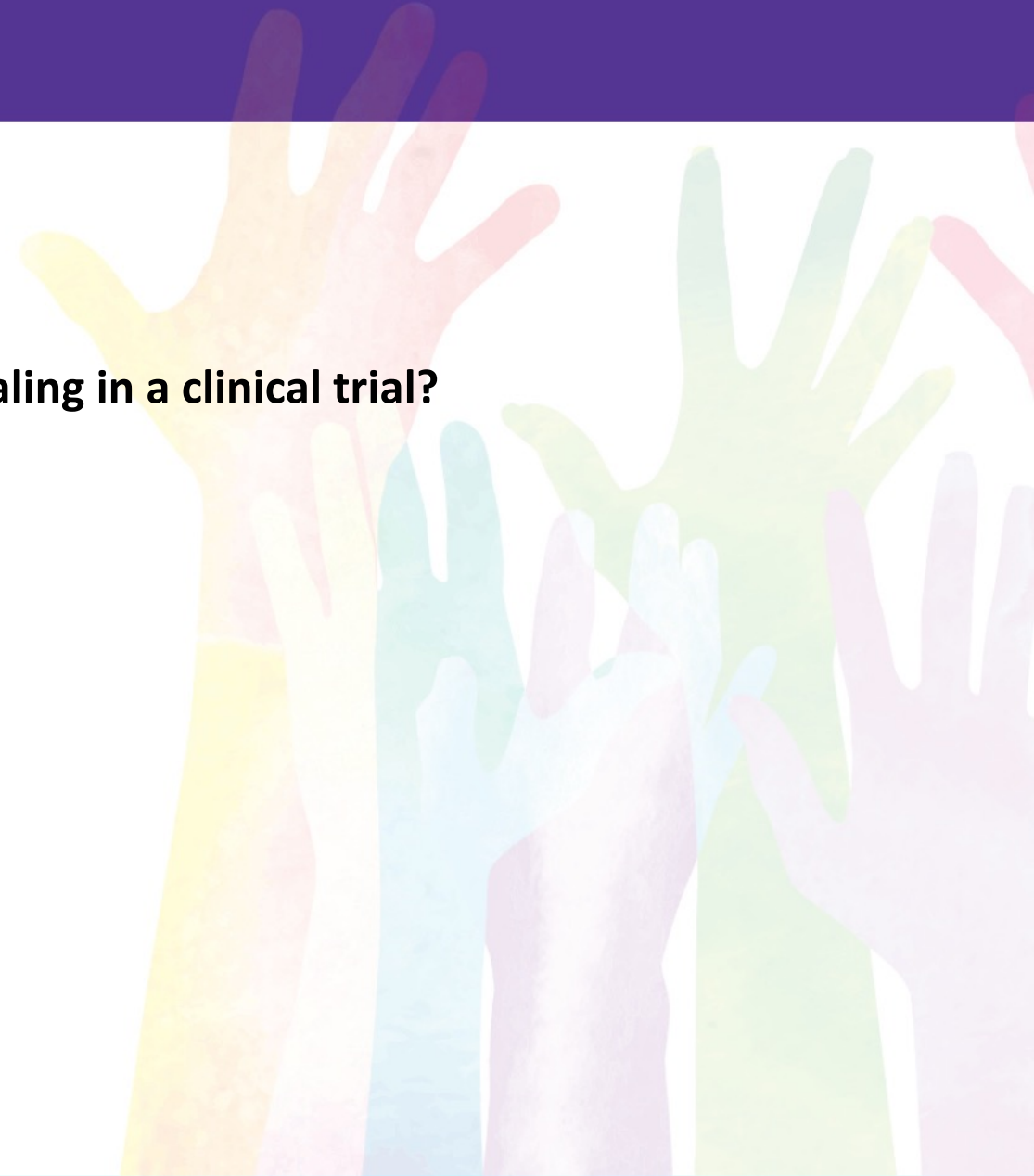
Extremely Concerned



POLL QUESTION

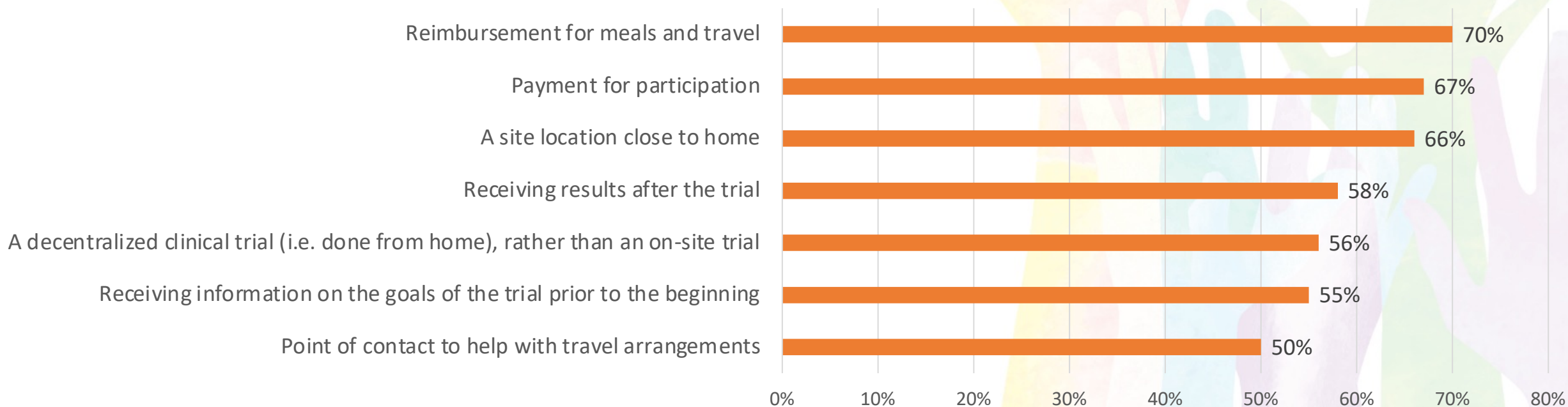
What would you say patients reported finding most appealing in a clinical trial?

- Being informed about the goals of the trial
- Convenient site location
- Decentralized trial
- Help making travel arrangements and other details
- Payment for participating
- Receiving results after the trial was completed
- Reimbursement for travel and meal expenses



What do patients find to be most appealing in a clinical trial?

Extremely Appealing



Aspects of communication that patients want:

- Getting an understanding of the treatment under development
- Being fully informed about the trial before the start of screening
- Having a full understanding of what the trial hopes to discover
- A 24/7 contact for emergencies or concerns relating to the trial
- A communication app or platform to use during the trial
- The ability to be in contact with other people in the trial
- Improved awareness of what clinical research is and what's involved

How can we boost patient interest in clinical trial participation?

- Reduce the burden of trial participation on the daily life of patients and families
- Provide compensation for their time and reimbursement for their expenses
- Offer a convenient site location or decentralized trials
- Communication prior to, during, and after the trial

Thank you for joining us today! We'd love to say in touch with you.



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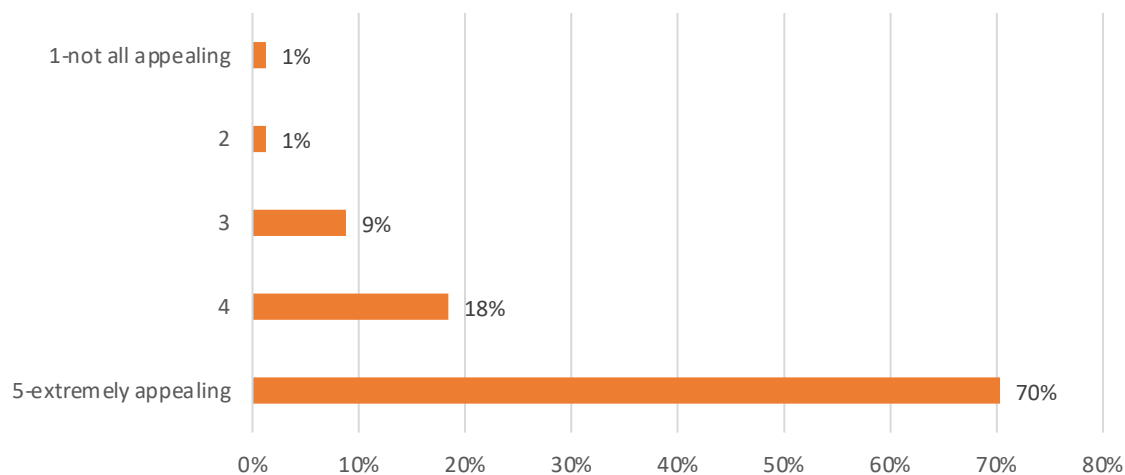
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Appendix



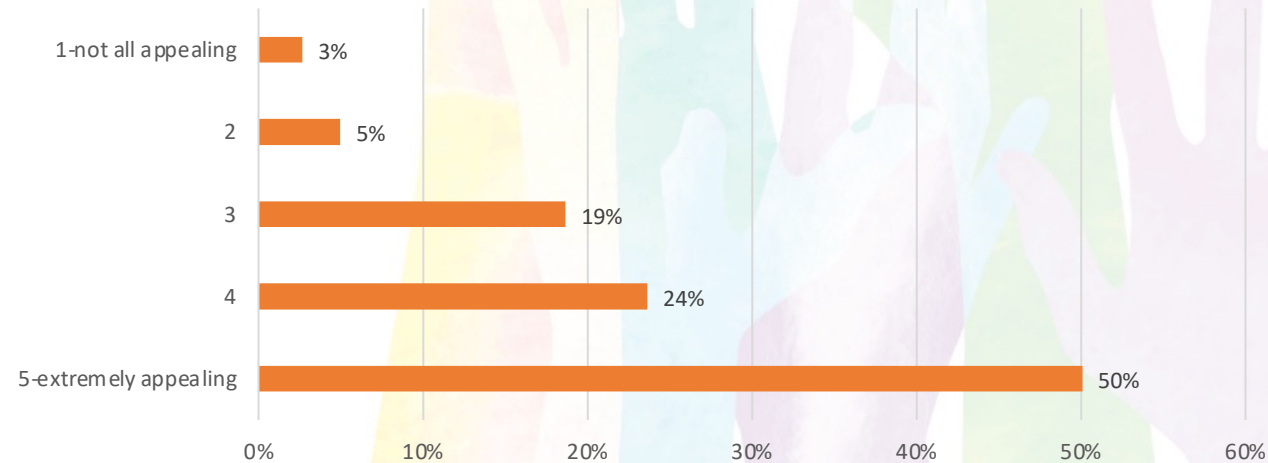
If they need to travel, patients want reimbursement and a point of contact to help with travel and reimbursement.

Q13. Reimbursement for your meals and travel



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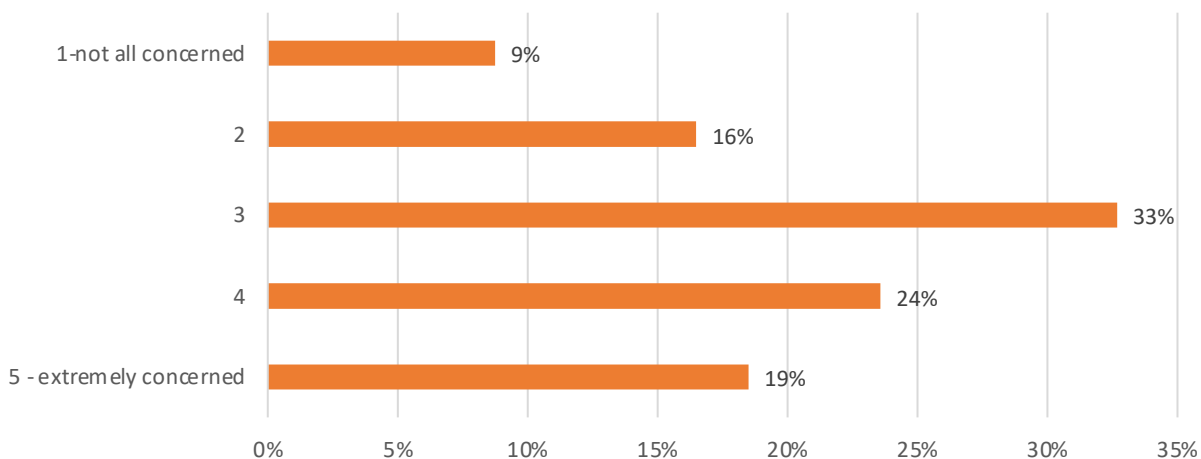
Q12. A point of contact to help with travel arrangements



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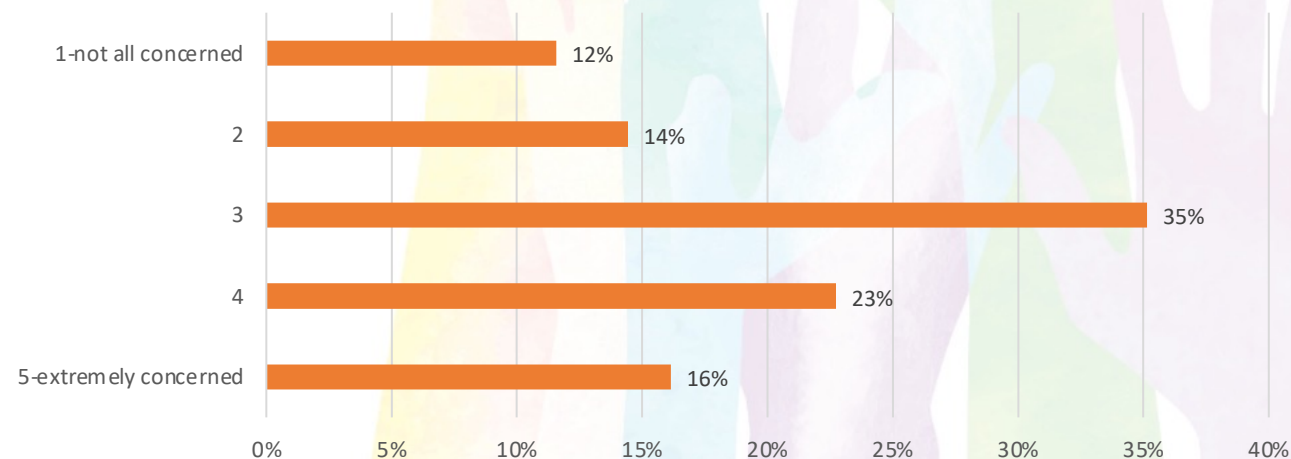
Three-quarters had moderate - high concern about possible risks and side effects, as well as benefits compared with their current treatment

Q3. The possible risks and side effects compared to my current treatment



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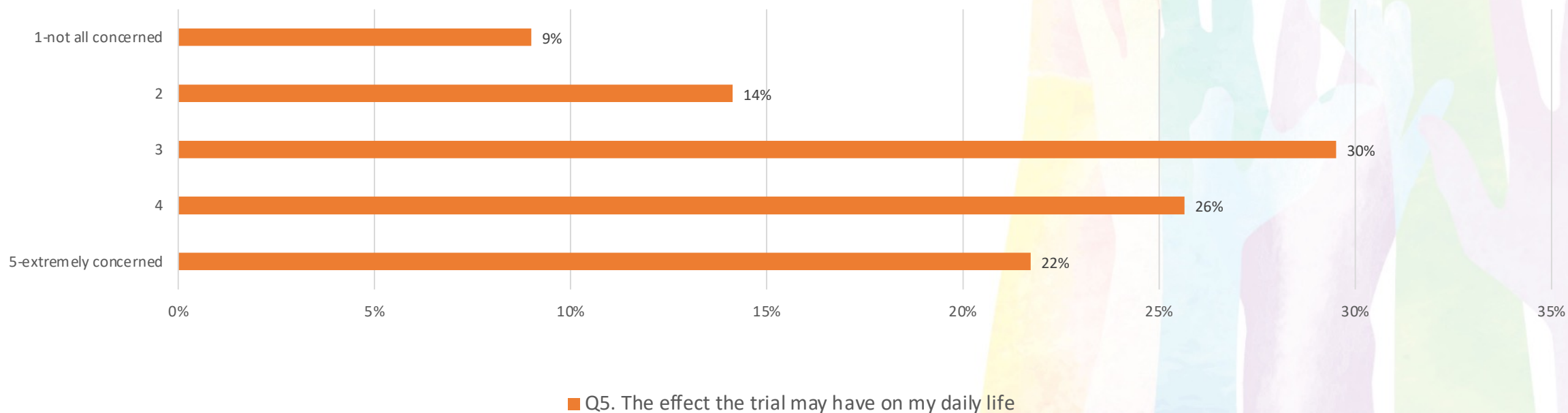
Q4. The benefits compared to those of my current treatment



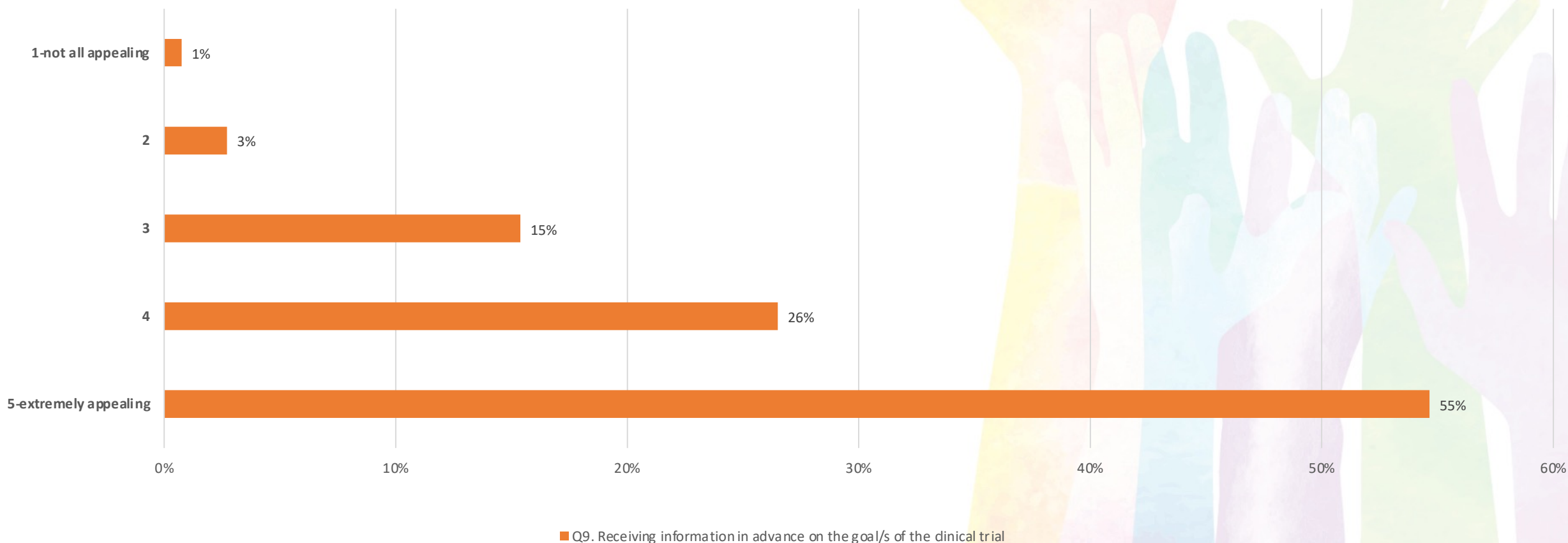
Q4. The benefits compared to those of my current treatment

Over three-quarters had moderate to high concern about the effect a trial would have on their daily life.

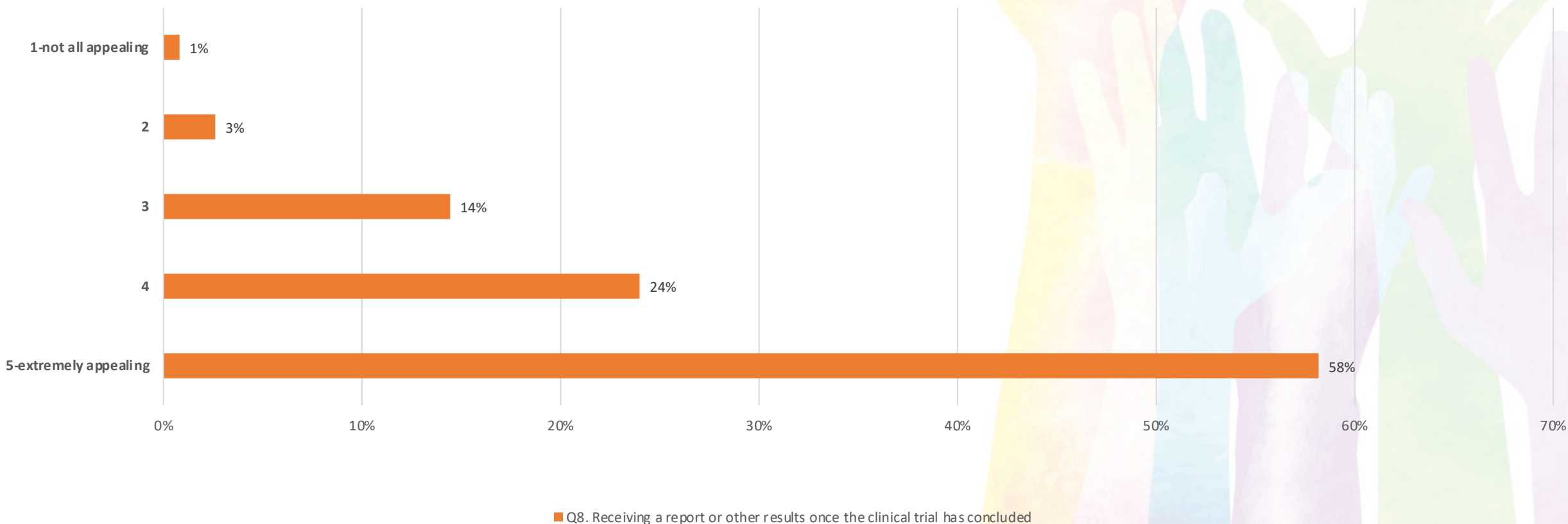
Q5. The effect the trial may have on my daily life



96% said receiving information on the goals of the trial prior to beginning was moderately - extremely appealing.

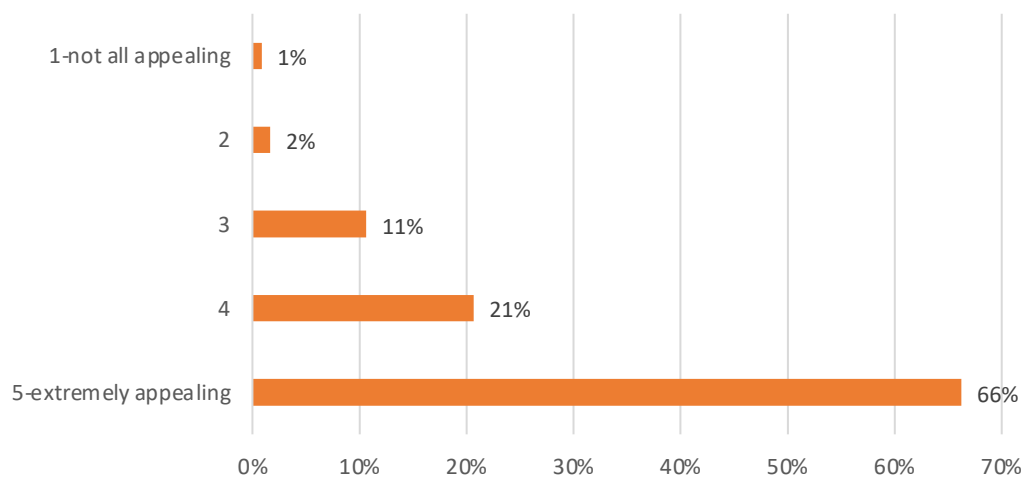


96% said they found receiving results after trial completion would be moderately - extremely appealing.



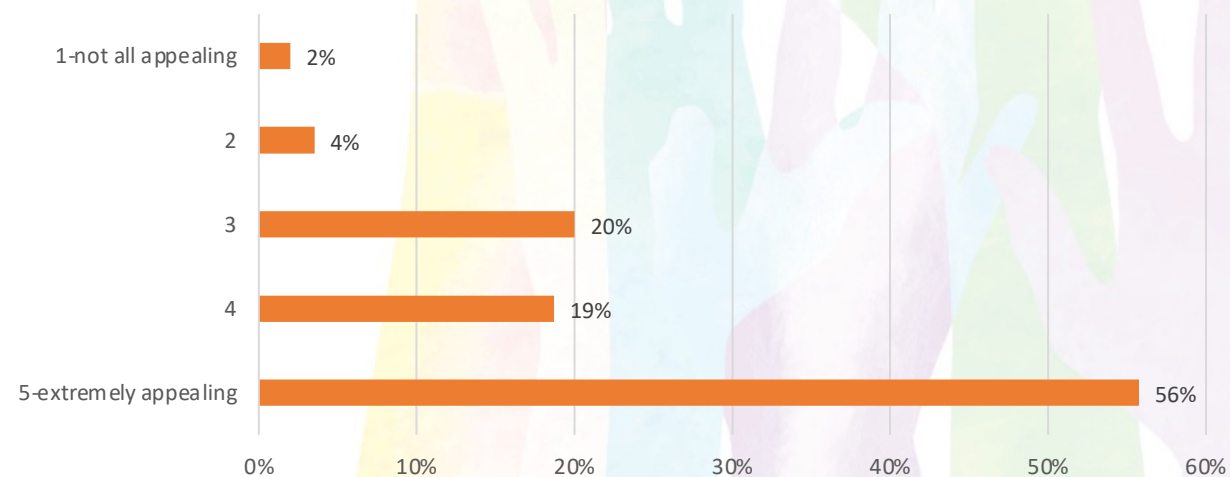
Patients overwhelmingly prefer a site location close to home, as well as decentralized trials that can be conducted at home.

Q7. A site location close to home



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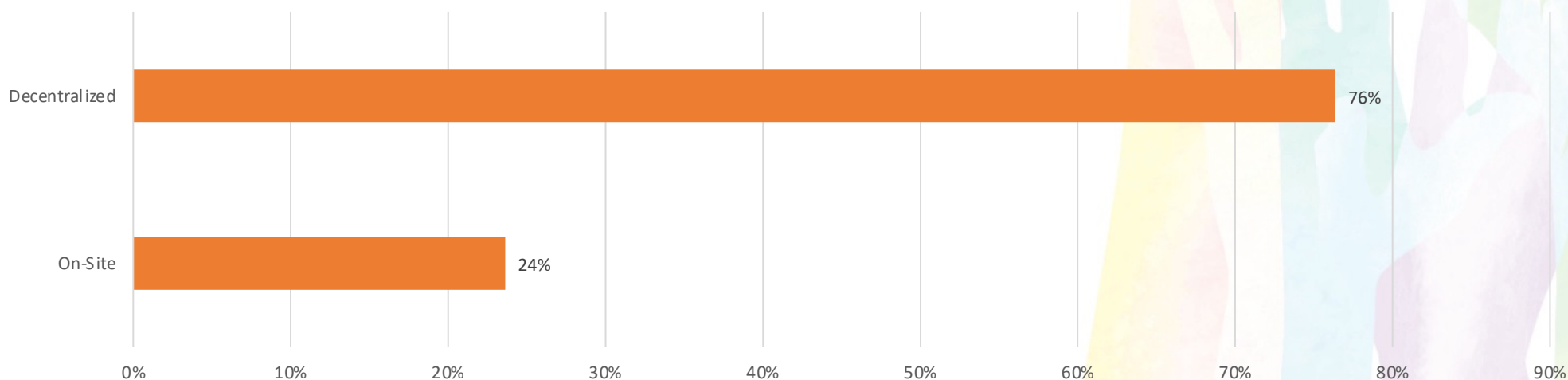
Q10. A decentralized clinical trial (i.e. done from home), rather than an on-site trial



Q10. A decentralized clinical trial (i.e. done from home), rather than an on-site trial

When given the choice of the trial in decentralized or on-site format, decentralized was preferred.

Q15. If the same trial were offered in a decentralized (i.e. from home) format and in an on-site format, which would you prefer participating?



■ Q15. If the same trial were offered in a decentralized (i.e. from home) format and in an on-site format, which would you prefer participating?

Reasons for Preferring Decentralized Trials

- Comfort and convenience of remaining at home
- Patients have children and pets who need care
- Patients have a job and daily lives they don't want to disrupt
- Travel is a hassle
- Less exposure to others
- Travel is especially difficult for some patients (e.g., those with disabilities, Crohn's disease, etc.)



Reasons for Preferring On-Site Trials

- ❖ Professionals and any necessary equipment are immediately available
- ❖ Greater availability to ask questions
- ❖ Patients don't have to worry about doing something wrong
- ❖ Some patients want to get out of the house
- ❖ Some patients don't want others coming into their house