FOUNDATION FOR SARCOIDOSIS RESEARCH

The value of partnering with a patient advocacy organization in clinical trial recruitment in sarcoidosis

Tim Legenzoff[1]*, Elise Hoover MPH*[1]*, Mary McGowan MHRM*[1]*, Tricha Shivas MBe*[1]

[1]**Foundation for Sarcoidosis Research | 320 W Ohio St., Suite 300, Chicago, IL 60654**

Introduction

Patient advocacy organizations build unique relationships with their patient communities by serving as a key resource at the time of diagnosis and beyond. This is especially true in rare diseases like sarcoidosis and the Foundation for Sarcoidosis Research (FSR).

FSR has observed key strategies that not only engage patients, but that increase access to research opportunities and therefore accelerate therapeutic discovery.

Sarcoidosis is an inflammatory disease characterized by the formation of granulomas in one or more organs of the body and is estimated to impact between 150,000 and 200,000 individuals in the U.S.

Black individuals not only experience the highest prevalence, but also the greatest disease burden, and yet are vastly underrepresented in clinical trials. FSR's Ignore No More campaign found that 85% of Black individuals with sarcoidosis surveyed felt clinical trials were worth the risk, yet 61% said they'd never been invited to participate by their doctor. This highlights a need for additional awareness and educational activities, and attention paid to patient-reported barriers to participation.

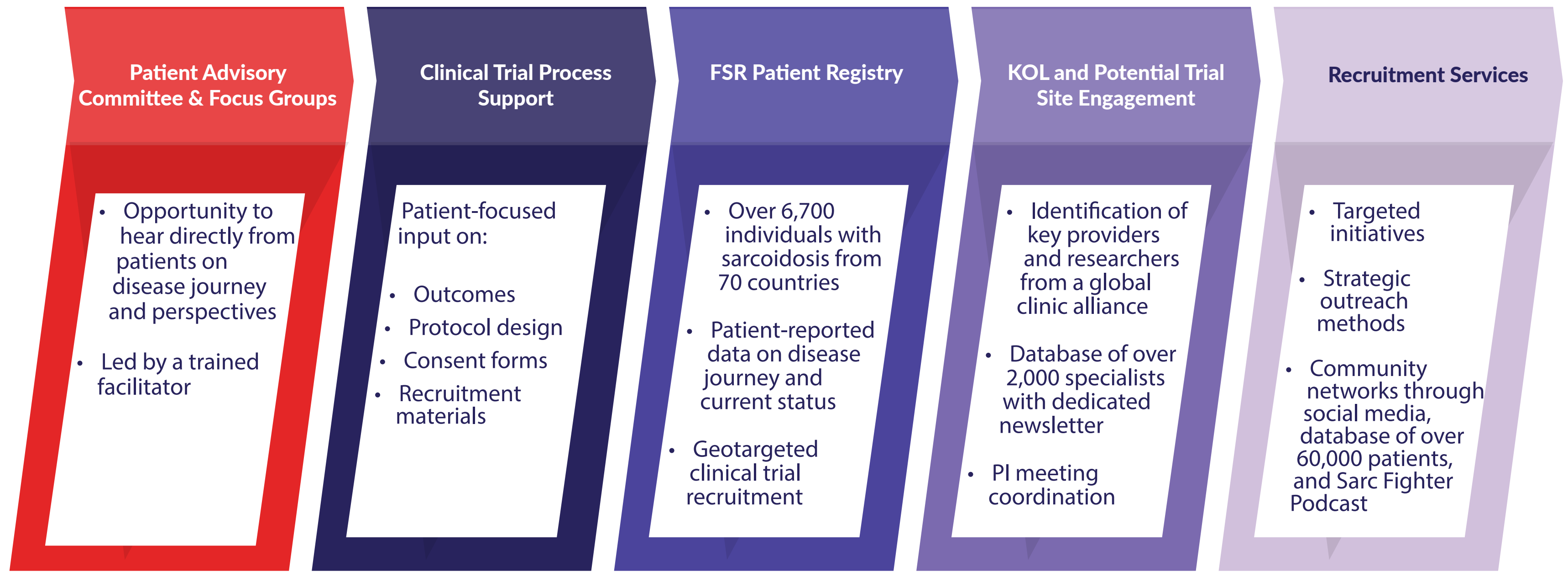
Methods

FSR partners with industry in pulmonary sarcoidosis and cardiac sarcoidosis clinical trials. With the support of FSR's Patient Advisory Committee, FSR's Clinical Studies Network (CSN), Sarcoidosis Clinics, and FSR's Patient Registry, FSR provides support services throughout the clinical trial processes and recruitment phase.

Clinical trial enrollment statistics were collected through partner reporting and feedback. Content engagement for FSR social media, newsletters, our clinical trial indicator list, and registry communications were provided through platform metrics.

Outcomes

Figure 1: FSR SarConnect™ - Clinical Trial Support and Recruitment Services



Patient Advisory Committee & Focus Groups
- Opportunity to hear directly from patients on disease journey and perspectives
- Led by a trained facilitator

Clinical Trial Process Support

Patient-focused input on:
- Outcomes
- Protocol design
- Consent forms
- Recruitment materials

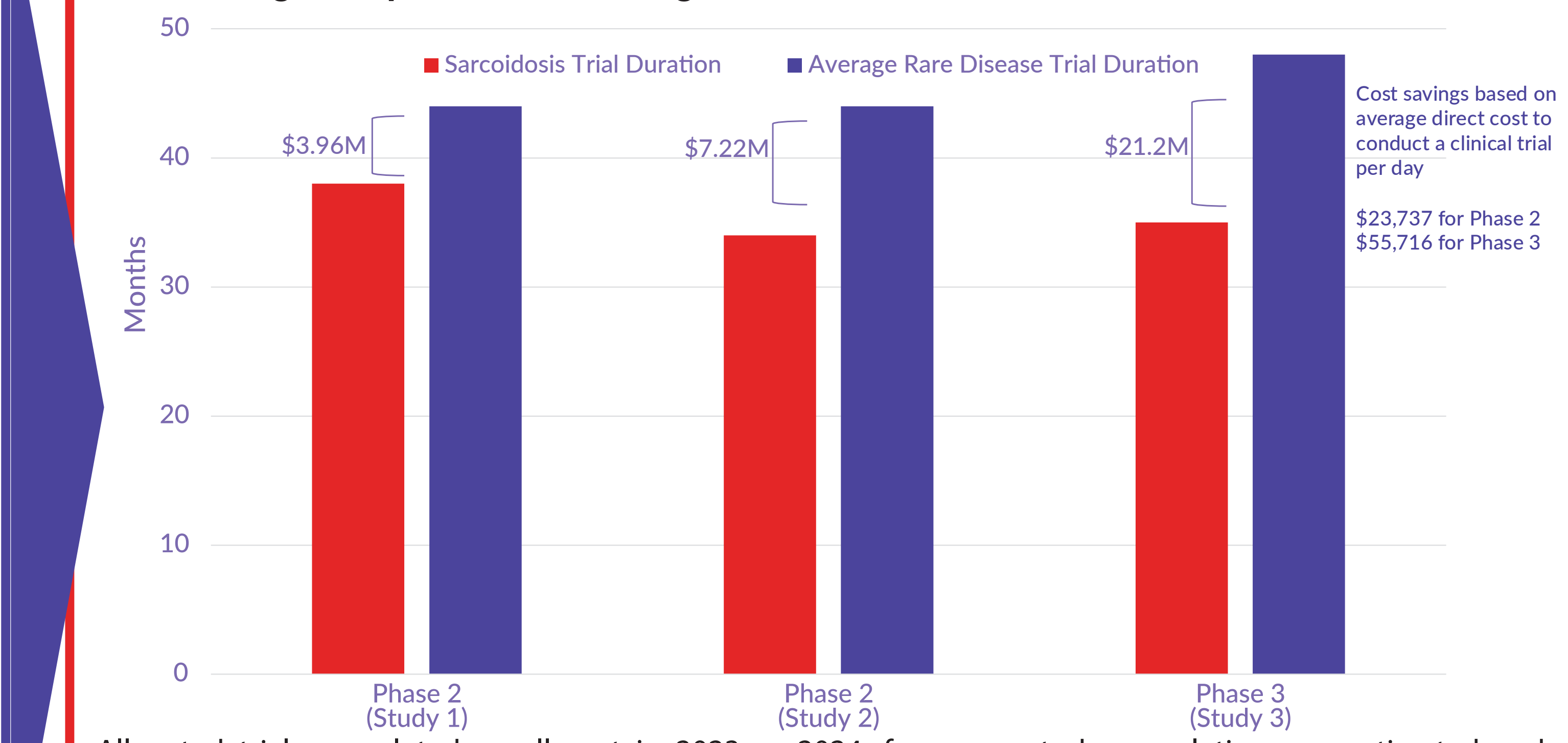
FSR Patient Registry
- Over 6,700 individuals with sarcoidosis from 70 countries
- Patient-reported data on disease journey and current status
- Geotargeted clinical trial recruitment

KOL and Potential Trial Site Engagement
- Identification of key providers and researchers from a global clinic alliance
- Database of over 2,000 specialists with dedicated newsletter
- PI meeting coordination

Recruitment Services
- Targeted initiatives
- Strategic outreach methods
- Community networks through social media, database of over 60,000 patients, and Sarc Fighter Podcast

The purpose of this project is to assist industry in pulmonary sarcoidosis and cardiac sarcoidosis clinical trials. With the support of FSR's Patient Advisory Committee, FSR's Clinical Studies Network (CSN), Sarcoidosis Clinics, and FSR's Patient Registry, FSR provides support services throughout the clinical trial processes and recruitment phase.

Figure 2: Average FSR-Partnered Sarcoidosis Trial Duration (Phase 2 and 3) and Associated Cost-Savings Compared to the Average for Rare Disease(1,2)



All noted trials completed enrollment in 2023 or 2024; for some, study completion was estimated and confirmed on clinicaltrials.gov. FSR also partners with Phase 1, observational, and biorepository studies; they were not featured due to limitations in average clinical trial duration and cost of clinical trial source material. FSR has also collaborated with partners who did not meet enrollment in the past before maturity of the SARConnect program.

Figure 3: Challenges for Sarcoidosis Clinical Trials and FSR's Strategic Solutions

BARRIERS TO RANDOMIZED CLINICAL TRIALS	FSR STRATEGIC SOLUTIONS
Clinical perception that sarcoidosis mostly remits	Engage Patient Community
Disproportionate impact to under-represented, under-resourced patients facing known medical bias	Spread Awareness
Belief that current therapies are "good enough"	Collaborate with FSR Volunteers
Limited understanding of sarcoidosis disease mechanisms	Education for Patients & Clinicians
Unclear regulatory approval pathway	Synthesize Industry Perspectives
Sarcoidosis is vastly complex and heterogenic	Provide Clinical Trial Support
Clinical trial enrollment proves challenging	Foster Institutional Collaboration
	Advocate for Regulatory Changes

Randomized clinical trials for sarcoidosis continue to trail behind sister diseases with similar prevalence due to several key problematic barriers. In November 2023, FSR hosted a focused workshop with leading experts in sarcoidosis with the goal of identifying innovative, directed solutions for overcoming these barriers. This session culminated in a white paper on Advancing Clinical Trials and Research.

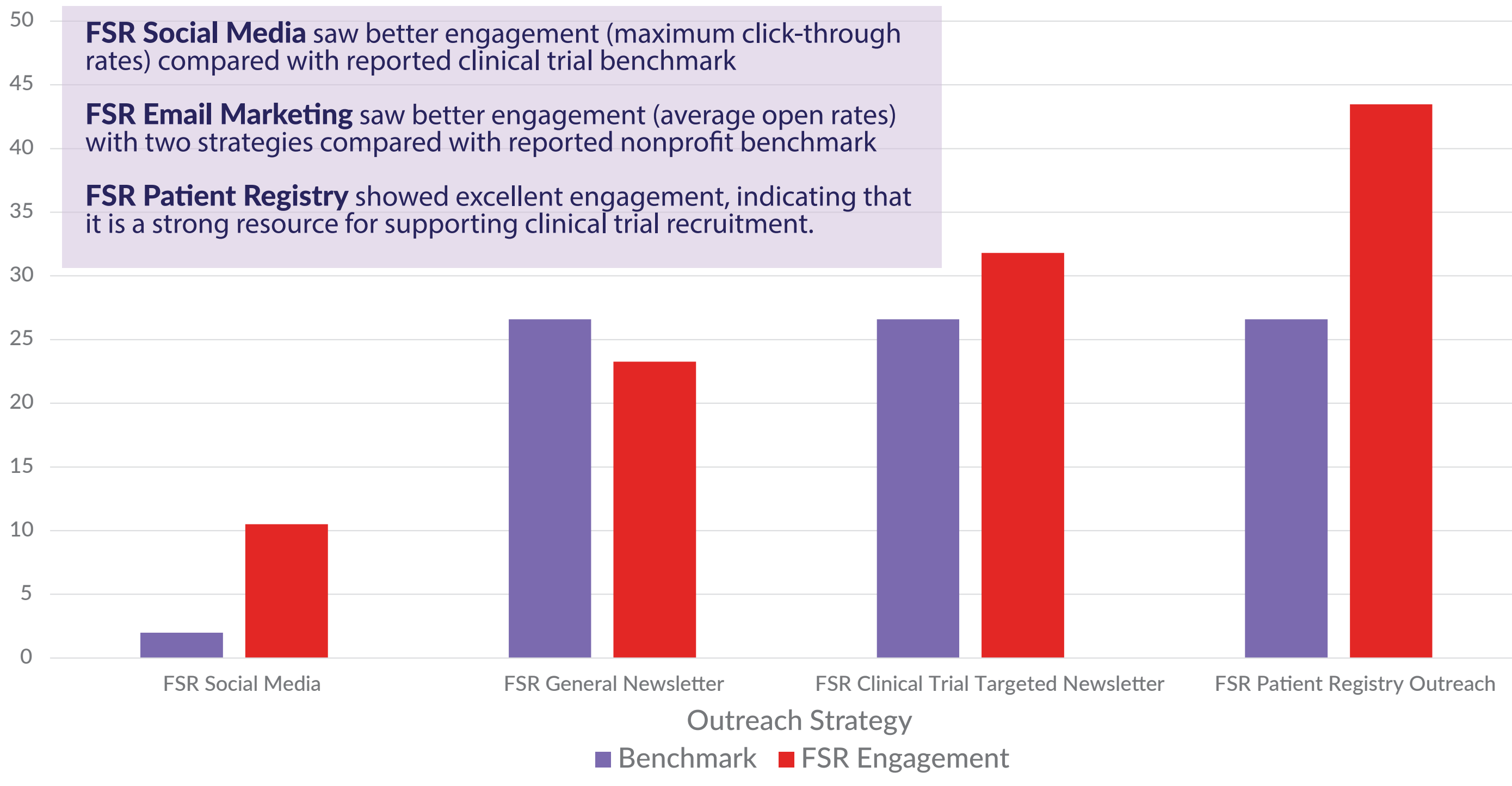
Figure 4: FSR SarConnect™ Engagement Rates Compared to Reported Benchmarks(3,4)

Benchmark References:

Social media benchmark: clinical trial maximum click-through rate – 2%

Email benchmark: Nonprofit average open rates - 26.6%

Data is from 4 separate FSR partnered clinical trial campaigns



FSR Social Media saw better engagement (maximum click-through rates) compared with reported clinical trial benchmark

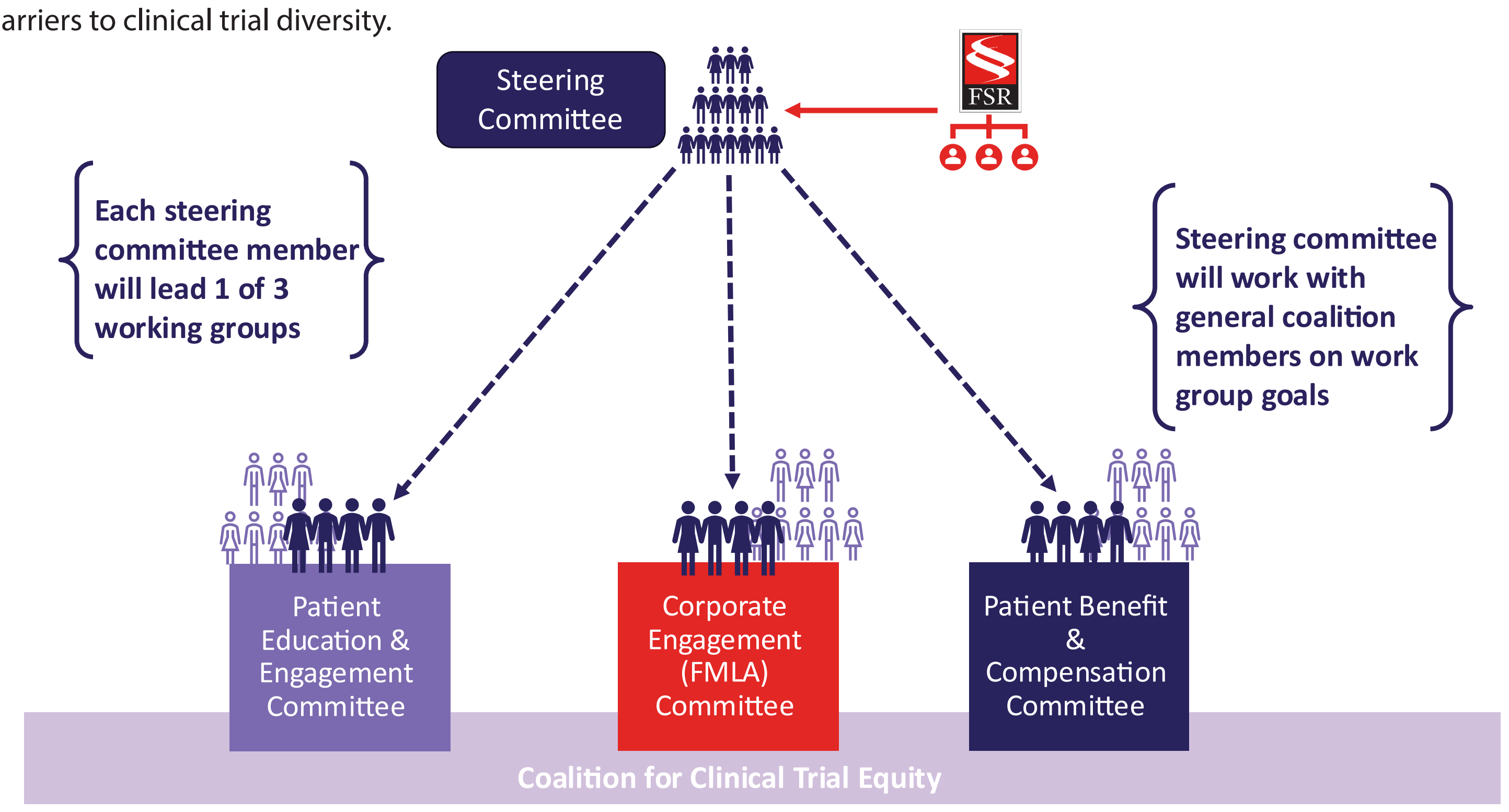
FSR Email Marketing saw better engagement (average open rates) with two strategies compared with reported nonprofit benchmark

FSR Patient Registry showed excellent engagement, indicating that it is a strong resource for supporting clinical trial recruitment.

FSR's SarConnect™ patient outreach strategies used in four separate clinical trials, including social media and email outreach, were compared against reported benchmarks to assess the difference in engagement rates. Social media engagement is defined as maximum click-through rate and is compared against reported clinical trial benchmarks, while email engagement is defined as average open-rate and is compared against reported nonprofit benchmarks. This graph illustrates the engagement that patient advocacy groups can achieve using these strategies to directly support clinical trial recruitment in rare disease. All four clinical trials represented in this graph completed enrollment.

Discussion

Strategies to address barriers to diversity in clinical trials are ongoing, including comprehensive efforts around the challenges reported by Black sarcoidosis patients that also extending systemically to other diseases. Building on learnings from the FSR Ignore No More Campaign and the learnings from an IRB-approved nationwide survey of Black sarcoidosis patients, the results of which are captured in a 60-page white paper, FSR invited like-minded organizations and individuals committed to implementing innovative and practical solutions to improve access to trials and address barriers to clinical trial diversity.



For more information about the coalition, how to join and sponsorship opportunities, please visit ignorenomore.org.

Limited sponsor feedback revealed that FSR referred over 1,400 potential participants to these partnered clinical trials, all of which achieved complete enrollment. Note: "Referrals" were sometimes noted as patients who passed the pre-screener and sometimes noted as those who completed the pre-screener survey.

Conclusions

- Challenges in measuring effective recruitment include:
  - Limited sponsor reporting
  - Limited understanding of referral conversion to enrollment through study completion
  - Ability to adjust recruitment strategies reliant on external data sources
  - FSR partnership is one of multiple strategies used by drug developers in recruitment efforts
- Collaborations with patient advocacy organizations by clinical trial sponsors maximizes patient engagement in both protocol design and trial participation.
  - Advocacy organizations seeking to replicate this model are recommended to properly value their role as thought partners and cost of dedicated staff time in order to create partnerships that create a sustainable program.
- FSR's insights into patient preferences on therapeutic interventions and clinical trial participation will expand when we host a Patient-Focused Drug Development meeting on October 28, 2024.

Corresponding Author Information:

Tim Legenzoff
tim@stopsarcoidosis.org

Clinical Trials White Paper

IgnoreNoMore White Paper

SARConnect

FSR Homepage StopSarcoidosis.org

FSR Externally-Led Patient Focused Drug Development Meeting

IgnoreNoMore.org

FSR Patient Registry

References: Figure 2: [1]Jayasundara, K., Hollis, A., Krahn, M. et al. Estimating the clinical cost of drug development for orphan versus non-orphan drugs. [2]Orphanet J Rare Dis 14, 12 (2019). https://doi.org/10.1186/s13023-018-0990-4, Getz, K. (2024). How Much Does a Day of Delay in a Clinical Trial Really Cost? Applied Clinical Trials. Retrieved from https://www.appliedclinicaltrialsonline.com/view/how-much-does-a-day-of-delay-in-a-clinical-trial-really-cost- Figure 4: [3]Eustace, Stephen. January 4, 2024. Stark Raving Health. Clinical Trial Marketing Success Using Digital Media Benchmarks. http://starkravinghealth.com/clinical-trial-marketing-success-using-digital-media-benchmarks/ Campaign Monitor. [4]Ultimate Email Marketing Benchmarks for 2022: By Industry and Day. https://campaignmonitor.com/resources/guides/email-marketing-benchmarks/