



# Winning Approaches for Rare Disease Patient Recruitment

24<sup>th</sup> April 2024

# Presenters

## Winning Approaches for Rare Disease Patient Recruitment

Discover effective tactics and methods for rare disease patient recruitment campaigns, drawing from real-world case studies, examples, and best practices to ensure enrollment success in studies.



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# Winning Approaches



- Challenges
- Best Practices
- Case Study Discussion

# Rare Disease Stats

**7,000+**

Rare Diseases

**350 Million**

People Globally Have Rare Disorders

**30 Million**

Americans Have Rare Diseases

**1 out 10**

People are Affected by Rare Diseases

**1 out 5**

Cancers are Rare

**95%**

Of Rare Diseases do Not Have  
Treatment

*Source List in Appendix*



# Challenges Facing Study Teams, Sites and Patients



- **Study Teams:** limited patient populations, complex trial designs, enrollment timelines and objectives
- **Sites:** limited patient populations, complex trial designs, high workloads & staffing challenges, and competing study priorities
- **Patients:** trial participation burden, attractiveness of the study and fit within their current treatment journey and trusting a new provider (site)

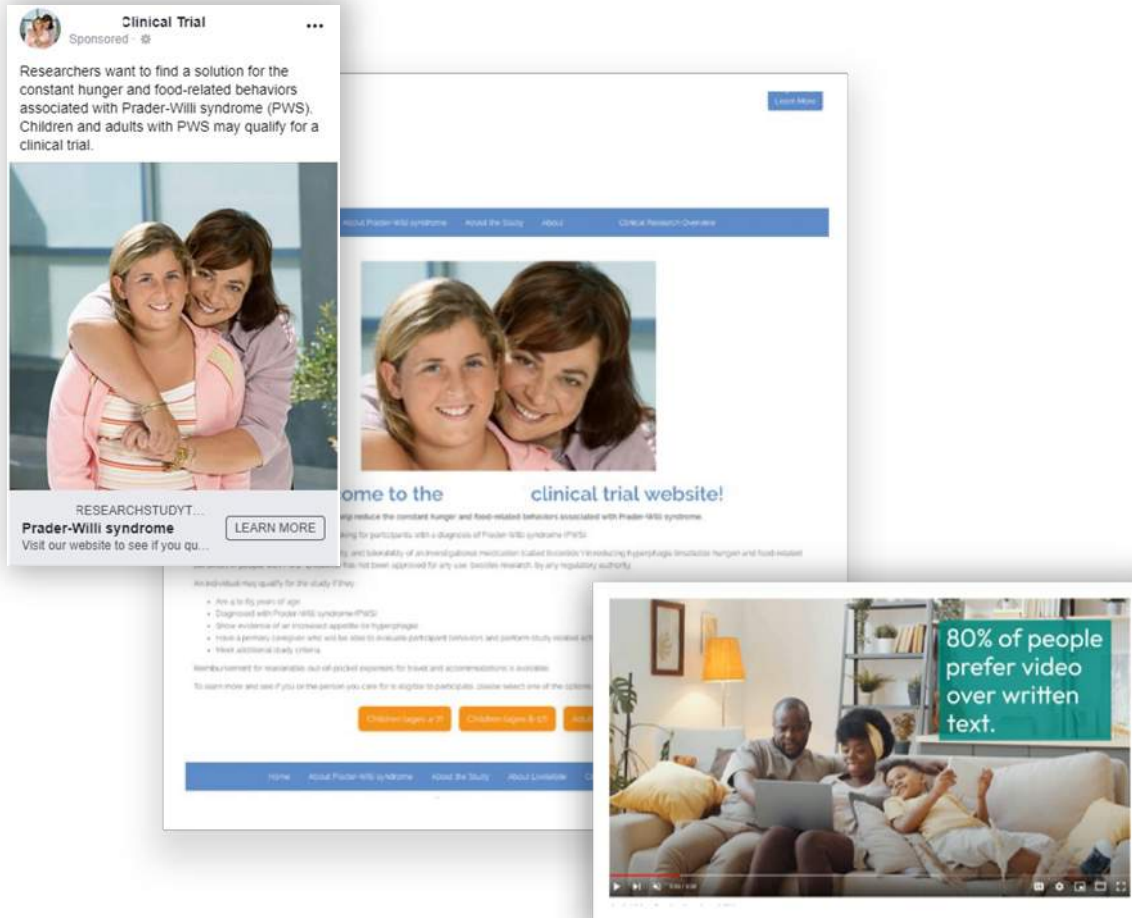
# Special Considerations with Rare Disease Patients



- Patients are often quite knowledgeable about their disease, the trial landscape, and research innovations
- They are often well aligned with their disease specific advocacy group
- Disease burden can dramatically affect their interest in the trial
  - Site location/travel
  - Study design & patient burden
  - Phase of study

# Strategies & Best Practices for Enrolling Rare Disease Trials

# Create Robust Trial Awareness



- Make it easy for patients to learn about the study

- Study Specific Websites
- Online Prescreeners and Site Selectors
- Patient Advocacy Groups
- Social Media Posts
- Videos: About the Study and ICF Videos
- Printed Materials to Support Sites
- HCP Facing Content About the Trial



# Advocacy Group Targeting

## Patient Advocacy Group (PAG)

This is often the very best and first tactic to be used when creating awareness.



# The Patient Enrollment Journey

- Have a plan for engaging with prospective patients and onboarding them into the study:
  - Do they meet the eligibility criteria?
  - Are they interested?
  - Are they ready and prepared to participate?
  - What's the best site for them?
  - Have mitigations to prevent patients from slipping through the enrollment funnel



# New Technologies to Improve Patient Experiences



## Virtual Waiting Rooms

These are websites where patients can register and wait until they and/or the site are ready to screen, keeping them engaged and warm. They are useful in rare disease programs, registry studies, acute and flare-up studies, and studies with narrow screening windows.

## Direct to Patient Engagement Centers

Partner companies can provide instant secondary phone screenings for patients, reducing the burden of new patient follow-up on sites and providing patients with immediate attention.

## Simple Tech Solutions

Offer web-based apps for scheduling phone screenings that fit patients' schedules, along with personalized email reminders and communication touchpoints to close gaps in the recruitment funnel.



# Supporting Study Sites – Your Partners in Success

- Collaborate and treat study sites as your partners in success
  - Continual engagement with sites translates into study success
- Invest in training, resources, tools and support to help your sites be successful
- Share your recruitment plans - - get feedback and buy-in



# Splash's Top Tips for Enrollment Success



1. Have digital & print versions of content and study information to support & educate patients, families, caregivers, sites, & HCPs.
2. Leverage the power of patient advocacy groups - - Advertise on their social pages, websites, newsletters, & grass-roots events.
3. Take advantage of the global reach of social media to spread awareness.
4. Use “patient concierge” services to ensure consistent patient follow up & onboarding into the trial.
5. Train study sites on the protocol & process of enrolling patients & conduct regular site engagement so they are motivated to enroll patients into your study.
6. Track & measure the impact of the tactics & site performance -- optimize what is working best for your trial!

# Global Patient Recruitment Case Study Discussion

# Questions?

# THANK YOU



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# Source List

## FDA

<https://www.fda.gov/patients/rare-diseases-fda>

## NIH

<https://www.nih.gov/about-nih/what-we-do/nih-turning-discovery-into-health/promise-precision-medicine/rare-diseases>

## NIH (National Human Genome Research Institute)

<https://www.genome.gov/dna-day/15-ways/rare-genetic-diseases>

## Global Genes

<https://globalgenes.org/rare-disease-facts/>

