

Patient-Focused and Virtual Trial Models to Reduce the Burden of Clinical Trial Participation for Patients and Families Suffering From Rare Neurologic Diseases

Scott Schliebner, Senior Vice President
Center for Rare Diseases, PRA Health Sciences

RARE NEUROLOGIC DISEASES

- There are more than 7,000 different rare diseases
- Neurological disorders make up a large portion of rare diseases that have ineffective diagnoses and treatments¹
- More than 3,000 genetic conditions affect the neurologic system; approximately 75% of these conditions affect children²
- Disease-modifying treatments do not exist for most of these disorders³
- Conducting clinical trials in rare neurologic patient populations poses many unique and complex challenges
- A patient-focused and virtual trial paradigm can reduce the burden of clinical trial participation and accelerate the development of new therapies for rare neurologic conditions

METHODOLOGICAL QUESTIONS ADDRESSED

- Within rare neurologic diseases, what are the challenges faced by industry, patients, families, caregivers, and other stakeholders?
- How can the clinical research paradigm evolve to reduce the burden of clinical trial participation?
- What methodological approaches can be utilized to ensure that clinical trials are more patient-focused?
- Can patient-focused and virtual trials fit more seamlessly into patients' lives and increase clinical trial participation?

METHODS

- Rare disease industry-sponsored clinical trials (N=224) over the last 5 years were reviewed to assess perceived barriers to participation
- PRA Health Sciences Rare Disease Patient Survey (2018) examined patient and caregiver experiences related to clinical trial participation⁴
- Responses and data were assembled to better understand the challenges and burdens faced by patients and caregivers

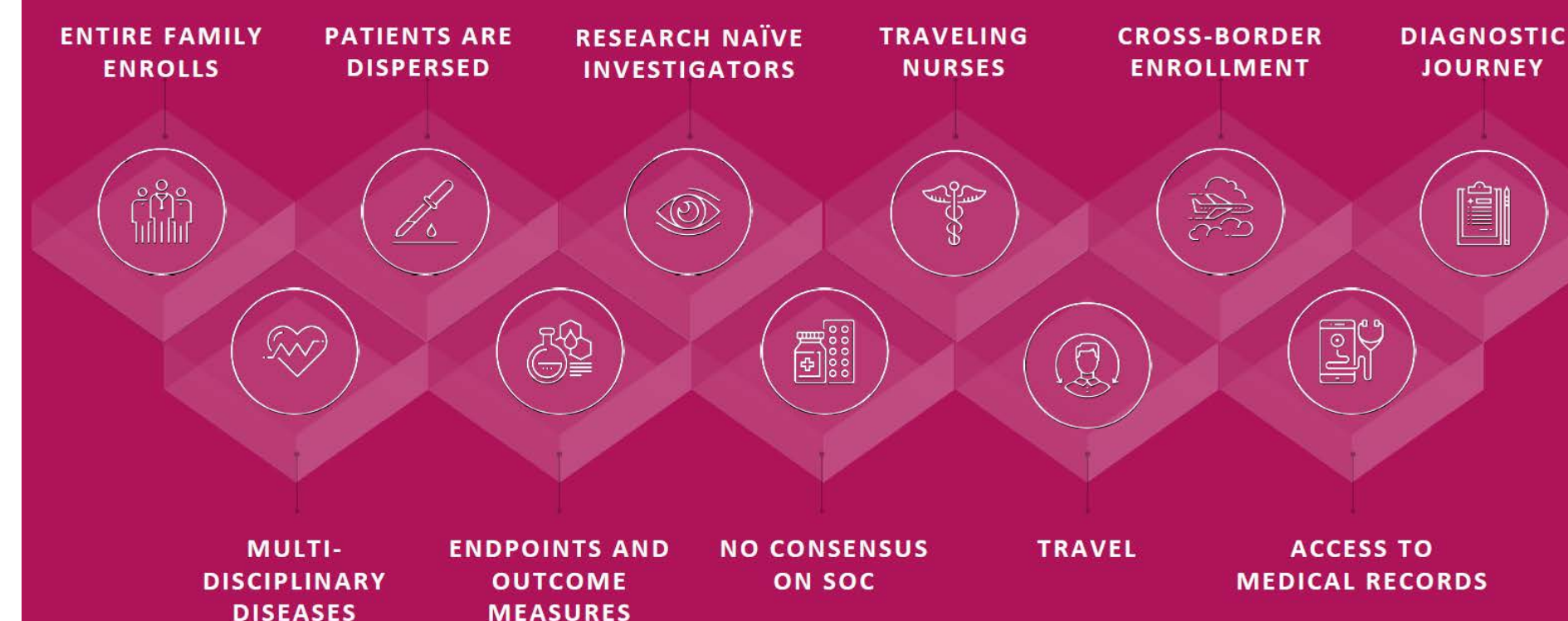
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WHY ARE CLINICAL TRIALS SO CHALLENGING?



PATIENT SURVEY DEMOGRAPHICS

	Total	Female	Male
Total Survey Respondents	482	463 (96%)	18 (4%)
Age Breakdown		Age Range	# (%)
		<18	6 (1.2%)
		18-24	2 (0.4%)
		25-40	27 (5.2%)
		41-60	240 (50%)
		61+	207 (43%)
Is There an FDA-Approved Therapy Available for Your Disease?	Yes	No	Unsure
	397 (82%)	36 (8%)	47 (10%)
Are You Currently Receiving This Therapy?	Yes	No	Unsure
	358 (75%)	36 (7%)	89 (18%)
Does Your Disease Have a Dedicated Patient Advocacy Group?	Yes	No	Unsure
	330 (68%)	18 (4%)	132 (28%)
Have You Participated in a Clinical Trial?		Yes	No
		47 (10%)	432 (90%)
Are You Currently Participating in a Clinical Trial?		Yes	No
		12 (3%)	467 (97%)
Did the Trial Address the Symptoms of Most Concern to You?	Yes	No	Unsure
	32 (70%)	11 (21%)	4 (9%)
Did You Feel Adequately Prepared for What to Expect in the Trial?	Yes	No	Unsure
	33 (72%)	10 (20%)	3 (8%)

WHY DO PATIENTS PARTICIPATE IN CLINICAL TRIALS?

- ADVANCEMENT OF RESEARCH INTO MY DISEASE
- NO OTHER TREATMENT OPTIONS AVAILABLE
- TO RECEIVE ACCESS TO HIGH QUALITY MEDICATION
- CURRENT APPROVED TREATMENT IS NOT WORKING

WHAT WERE THE MOST CHALLENGING BARRIERS YOU FACED WHILE PARTICIPATING?

- TRAVEL TO/FROM SITE VISITS
- TIME COMMITMENT
- FINANCIAL STRAIN
- STRAIN ON FAMILY AND/OR CAREGIVERS
- INVASIVE PROCEDURES; CULTURAL BARRIERS; BARRIERS RELATED TO DISABILITY; SIDE EFFECTS

WHY HAVEN'T YOU PARTICIPATED IN A CLINICAL TRIAL?

- DID NOT MEET ELIGIBILITY CRITERIA
- CLINICAL TRIAL WAS NO LONGER ENROLLING
- THE TRIAL SITE WAS TOO FAR AWAY
- IT WAS NEVER OFFERED TO ME
- SOCIAL, EMOTIONAL, FINANCIAL; UNFAMILIAR WITH THE PROCESS; SCARED

PATIENT-FOCUSED AND VIRTUAL TRIALS TO OVERCOME BARRIERS TO CLINICAL TRIAL PARTICIPATION

- Clinical trials must fit more seamlessly into the lives of patients and families
- Financial, social, emotional, and familial strains are prohibitive for many patients
- To ensure clinical trials are feasible and reasonable, patients and families should be consulted and included in the clinical trial design process
- Patients should be considered partners and the ultimate customers



- Patient feasibility studies and patient advisory panels can support a patient-focused approach
- Mobile technology, telemedicine, e-consent, and home nursing visits reduce time and travel commitments
- Wearables, online surveys, and remote data collection devices can be employed to bring clinical trials directly to patients
- Adopting less site-centric and more virtual clinical trial models will eliminate barriers to participation, accelerate clinical trials, and bring new therapies to patients faster
- Rare neurologic diseases provide an ideal environment to embrace patient-focused and virtual trial models