Follow-up Answers to Questions in Q&A Session from Feb. 16 Webinar

What is the best way for small pharma companies to get the study results to the sites and patients?

Mike Wenger, VP of Patient Engagement, Informa Pharma Intelligence: For smaller pharma companies, it makes sense to make results available on a publicly accessible portal, like TrialSummaries.com, where individuals can sign up to be alerted when trial results are posted. Instead of contacting individual patients and/or trial sites, smaller sponsors can post their results in one place where both participants and the public can view them. By listing them on a non-branded website, sponsors meet regulatory requirements that results be presented in a non-promotional manner. In addition, inclusion on a website that lists results for multiple sponsors and disease states increases brand reach and recognition.

How do I qualify for clinical trials? How can I get selected for more clinical trials?

Wes Michael, President, Rare Patient Voice: Each clinical trial has specific inclusion and exclusion criteria. To qualify, you need to meet those. More important is to find clinical trials that may be relevant for you. Work with your physician(s), patient advocacy groups, and online searches, including ClinicalTrials.gov.

What are industry and advocacy groups doing to help train physicians how to talk about clinical trials with patients?

Steve Jones, president of the European Idiopathic Pulmonary Fibrosis and Related Disorders Federation: The only work on this in Europe, which I know about, is Action for Pulmonary Fibrosis (APF) in the UK, which has trained a cadre of "research champions" who are working closely with doctors in their hospitals to recruit and retain patients for clinical trials. This said, as yet, APF has not started a formal training program for doctors, but this would be the next step.

Is transportation to clinical trials a barrier to participation?

Wes: Yes, we have heard from many patients that transportation is a barrier to participation. Some trials are now to some degree "decentralized," meaning some or all of the visits can be conducted remotely. Some sponsors and advocacy groups help patients obtain the transportation. Not only are cost and time factors, but some diseases/conditions make travel very difficult.

Have you seen a correlation between low-income families/high-income families and the capability to participate in clinical trials?

Mike: Yes, financial constraints represent a significant barrier to clinical trial participation. Low-income individuals typically have jobs that are difficult to take time off of during working hours. What's more, these individuals are likely paid on an hourly basis and would forfeit crucial earnings by clocking out to participate in a trial. Low-income people also are more reliant on public transportation, which may pose a logistical challenge in accessing trial sites.

We have noticed disparities in clinical trial participation for patients who are Black/Brown as well as females (specific to cancer trials). Do we know specific ways to reach out to these communities to encourage recruitment? What barriers do you see specific to race/gender, and how can we overcome them?

Mike: As we mentioned in the webinar, there is a definite mistrust in medical research among many people of color. This is due in great part to historical medical injustices such as the infamous Tuskegee Study and the Henrietta Lacks case. That said, grassroots efforts to reach BIPOC individuals are most successful in reaching members of these communities. Working with churches and local community organizations is a good starting point. It should be noted that people of color are more comfortable when the study team members look like them; that's why it's important to have a diverse professional staff as well. Language can be a major barrier for those who do not speak English or for whom English is not their first language. Bilingual staff and having pamphlets/websites in Spanish and other languages helps bridge communication gaps.

How much concern do patients have regarding how the clinical trial will affect their insurance coverage?

Wes: Patients have great concern about insurance coverage. If the trial will or will not have an effect on their coverage, the sponsor should make that very clear.

What's the biggest difference in Rare Patient Voice studies and clinical trials?

Wes: The biggest difference, from RPV's point of view, is how many patients qualify. For many of our market research studies, which are only collecting patient opinions, 70% may qualify. For a clinical trial, with very specific inclusion and exclusion criteria, which might include current medication usage and other comorbid conditions the patient may have, a much smaller percentage will qualify.

Online information about trials (especially via ClinicalTrials.gov) is almost always too technical for ordinary (even educated) patients to understand. Are there any efforts to "translate" this information into lay language that's tailored to patients' information needs/concerns?

Mike: ClinicalTrials.gov is not known for being patient friendly in terms of either usability or content. The government is making an effort to address the issue. At the top of the ClinicalTrials.gov website you'll find: "Try the modernized ClinicalTrials.gov beta website. Learn more about modernization effort." Many sponsors, however, are taking it upon themselves to provide information about clinical trials in general, and their own studies in particular, by hosting dedicated clinical trial websites.

Why do trials disqualify individuals that are most affected by the disorder by stating it is for healthy individuals? For example, a seizure trial disqualifies those with active seizures.

Wes: This is very frustrating to many patients. We also see trials disqualifying patients who have other conditions, when the real-world situation is that most patients have more than one condition. I think the reason for this in many cases is that clinical trials are scientific experiments, and to get a clear result and see if a drug is effective, they need to have as few other variables in play as possible. So they often want patients who are otherwise healthy, which may not be the typical patient.

If we would like to get involved in recruiting for interventional clinical trials, are there any regulations/rules we would need to follow? Any suggestions on how we could help or start getting involved? Would we need to contract with specific sites/hospitals?

Mike: In the US, the Food & Drug Administration (FDA) governs how sponsors can recruit for clinical trial participants. In any participant outreach campaign in the EU, an ethics committee must review all recruitment materials for potential trial. The promotion of clinical trial recruitment is permissible in Belgium, France, Germany, Italy, and the Netherlands. Sponsors must also report the start and end of recruitment efforts in the EU.

Instead of contacting specific sites/hospitals, an efficient way to get involved is to join a patient referral network like Citeline Connect. Citeline Connect offers both a platform and a network of 75+ recruitment partners that simultaneously find, refer and screen highly qualified trial participants.

What kind of information would be useful to make a decision on ClinicalTrials.gov? Assuming it's shared in a patient friendly way.

Mike: Here are key pieces of information that help patients determine if a clinical trial is the right fit for them:

- Purpose of the study
- Length of study
- Whether it is actively enrolling
- Location of trial sites
- Frequency of site visits
- Prescreening inclusion/exclusion criteria (gender, age, disease state, general health, previous treatments, etc.)
- Direct contact information

In addition, the following information also can be determining factors:

- Whether a placebo is part of the study
- Possible side effects
- Compensation, if applicable
- Any follow-up care
- Will treatment be available after the study?

How can we access patients if we do not have access to their personal contact health information due to privacy laws?

Wes: One way is to work with companies like Rare Patient Voice. We have a panel of over 100,000 patients across 700 different diseases, patients who have signed up with us to take part in research. So we can reach out to the relevant patients in our panel and gain their permission to collect more health information.

80% of trials are late and many cite recruitment as the main issue. My research tells me that sites often over-promise at site selection and fall short in implementation. Is this typical? How can it be improved?

Mike: Unfortunately, this is often the case. Falling short of clinical trial enrollment goals is a major culprit, and the reason for this stems from poor prescreening. That's why at Citeline Connect we implement a rigorous prescreening process up front, one that reduces screen fails, minimizes patient frustration, and delivers highly qualified patient referrals to study sponsors.

There are probably timing concerns regarding when results of data analyses can be released. Do pharma companies and/or researchers want to publish their results first, no?

Mike: Your question has perfect timing! It's more than a matter of wanting to publish results; in many areas; it's a regulatory requirement.

In the EU, new regulations went into effect Jan. 31, 2022, with the implementation of the EU Clinical Trial Regulation (EU CTR) and its new Clinical Trial Information System (CTIS). Summary results are required for all studies conducted in the EU within six months of trial completion for pediatric trials or one year of trial completion for most other trials. Interim results are required if specified in the protocol.

In the US, federal law requires that responsible parties, typically trial sponsors, register applicable clinical trials (ACTs) on ClinicalTrials.gov within 21 days after the first human subject is enrolled and submit certain summary results information for those trials, generally no later than one year after the study's completion date unless a deadline extension is obtained. Recently, the FDA has begun issuing notices of noncompliance to study sponsors. The FDA is authorized to seek civil money penalties for violations of the ClinicalTrials.gov disclosure rule, including up to $12,000 for each day that a company fails to submit the required information.

The World Health Organization (WHO) has requirements for publishing clinical trial data: Results must be published open access within 12 months in a peer-reviewed journal or made publicly available within 24 months, and they must be posted on a clinical trial registry within 12 months.

Would you find it useful to receive back medical records that were generated during your participation in the clinical trial (i.e., lab analysis results, imaging from RX, MRI, etc.) so they are available for any other medical checks for the future?

Ashley, patient: I would like to have that information on my own participation in a trial to be able to share with anyone on my medical team. I think that insight would be helpful.

Sarah, patient: Absolutely, I would want a copy of my full medical record accrued during the clinical trial. This would become a very important part of my personal medical history and I would want full access to the information, not just for myself but also to share with my narcolepsy treatment team (and other medical professionals in my life).

How successful are research champions in helping recruit and enroll participants?

Mike: One study focused on the Rare Diseases Clinical Research Network (RDCRN) requirement for each consortium to include patient advocacy groups (PAGs) as research partners. It concluded that PAGs "help ensure the feasibility and success of research protocols by assisting with study design and patient recruitment…."

Another study in London, focusing on staff-based research champions, concluded that "Investing in staff that promote and drive research in clinical services increases opportunities for patients to hear about and engage in clinical research studies."

Where can we reach you?

Wes: Visit Rarepatientvoice.com, our website and a great source of information. I can be reached directly at wes.michael@rarepatientvoice.com.

Mike: Visit CitelineConnect.com for more information. You can reach me at mike.wenger@informa.com.