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Rare Disease Studies: Patient-Focused Approaches

The National Organization for Rare Disorders (NORD) indicates that there are approximately 7,000 medical conditions that qualify for a rare disease designation. In the United States, this is defined as an affected population with a prevalence of fewer than 200,000 patients.

Because there is a great unmet need for novel therapies to address these rare conditions, various incentives, such as tax credits (U.S.), additional marketing exclusivity (U.S., EU), user fee waivers (U.S.), and other measures have been instituted to stimulate development of new therapies. The FDA has reported a steady increase in the number of approvals for orphan drugs each year since 1983 and the cumulative number of approvals is now totaling close to 500. However, this number illustrates that there is still a critical need for further advancements to address a large number of these disorders that do not have effective treatments options, many of which affect young people and are debilitating and life-threatening.

The proliferation of products under investigation for these disorders has raised unique challenges within the clinical research environment. Patients may have disabilities due to their disease and may struggle to cope with activities of daily living, let alone visit clinics where clinical research is being conducted and subsequently adhere to traditional clinic-driven research schedules. For many conditions, there are a limited number of centers of excellence and patients may have to travel significant distances for expert medical attention. In order to support patients and their caregivers, one must consider innovative approaches for collecting required study data while minimizing the burden to patients.

A few of the approaches that UBC has developed and successfully implemented in this regard are:

1. Home nursing support
2. Hand-held data capture tools
3. Concierge services

Home Nursing Support

Most clinical studies require that patients are examined by the physician investigator in a clinical setting at particular time points. However, there are often a number of measures or activities that may not necessarily require the patient to present physically to the clinic/office and that can be performed by a nurse trained on clinical trial requirements and protocol procedures. Examples of these may include:

- Specimen collection, including blood draws for laboratory assessment
- Administering study drug, either to avoid a clinic visit or to support patients and caregivers who are unable to self-administer or are concerned about self-administration
- Measurement of vital signs and physical assessments
- Assessment for adverse events
- Initial or ongoing patient and caregiver training
- Assessment of compliance with procedures, such as diary completion or completion of PROs

Using this approach may also allay concerns about continuity of study compliance in patient populations that are known to migrate seasonally. Having a centralized study nursing management staff connected directly to the project management team for the project helps keep control of study progress.

UBC has successfully implemented this field nursing approach in 40 countries in North America, Europe, and Australia. This has included more than 3.8 million patient touches and, in a one-year period, support in 27 rare disease indications.

Hand-Held Devices

Many rare disease protocols involve maintenance of symptom diaries, documentation of medical resource utilization and completion of patient reported outcomes (PROs), and clinical outcome assessments (COAs) to assess the impact of treatment on disease-related quality of life and disease status. With today's widespread use of Android and Apple devices by patients and caregivers, it is possible to deploy a "Bring Your Own Device" strategy, which allows easy download of applications at the time of an initial study visit.

About 95% of patients have such a device or access to one, and a loaner device can be provided to the remainder. For multi-national programs, required assessment tools can be used in local language(s). In a current program, this technology solution is being activated in more than 20 countries. Similarly, patients and caregivers may additionally access tools on their PC, which gives them the flexibility to complete entries using the most convenient method at any given moment.

Training videos and guidance documents can be distributed on these devices as well. Using this method, an audit trail of access to and completion of training by these methods is maintained as part of the system. A practice environment is provided so that patients and caregivers can become comfortable with completion of the required assessments. These remain accessible to the patient and caregiver for future reference.

There is always a concern about compliance with completion of these assessment tools once patients leave the clinic. However, text and email-based reminders to complete data entry at required time points and built-in alerts to investigators for noncompliance trends for a patient can mitigate these concerns. In a recent program involving completion of clinical outcome assessments (COAs) and PROs, there was an overall remote completion rate of greater than 95% out of more than 29,000 expected forms, which in our experience exceeds success rates with other methods of collection for these data.

Concierge Services

For patients who rely heavily on caregivers

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for their transport to the clinic, laboratory, or other points of medical contact, studies with frequent required visits can be a barrier to study participation. Providing a mechanism to allow patients to meet appointment schedules can be impactful in terms of their ability to participate in clinical research. This can be especially important when patients need to travel at a distance to a study center.

This support can be established using a centralized study concierge service. Sites can request taxi, air, and rail transport services for a patient and caregiver via a secure online portal. The concierge support team makes all travel arrangements. Arrangements can also include hotel and other accommodation, as well as a provision of stipends for meals and other necessities. This service has been provided on a global basis in compliance with local law. Often a customized approach is required based on geographic region as optimal methods for transport can vary by region. This approach requires no out-of-pocket expense for the patient or additional effort by the study center.

Conclusions

The changing nature of today's social environment presents challenges requiring clinical research methodologies to adapt to engage the patients needed for participation in rare disease studies. While the strategies outlined above can apply to many clinical study situations, they are particularly relevant in certain rare conditions when the limited number of affected patients drives the need for accommodation.

UBC is uniquely positioned to provide these and other leading-edge approaches to ensure that patients in need can access potential new therapies, clinical investigators are unburdened to focus on priority activities in studies, and pharmaceutical and biotechnology sponsors achieve their goals to bring innovative, life-saving therapies to the patients. PV

UBC *is a leading provider of, comprehensive clinical, safety, and patient support services. Our experts are committed to working in unison with pharmaceutical and biotech organizations to effectively navigate the product lifecycle and make medicine and medical products safer and more accessible.*

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